

**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 June 30, 2023

**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 001-38042

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

**Delaware**  
(State or other jurisdiction of incorporation or organization)

**46-0408024**  
(I.R.S. Employer Identification No.)

**177 E. Colorado Blvd, Suite 700  
Pasadena, California 91105  
(626) 304-3400**  
(Address and telephone number of principal executive offices)

Former name, former address, and former fiscal year, if changed since last report: N/A

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.001 per share	ARWR	The Nasdaq Global Select Market

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

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

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

Large Accelerated Filer	<input checked="" type="checkbox"/>	Accelerated Filer	<input type="checkbox"/>
Non-Accelerated Filer	<input type="checkbox"/>	Smaller Reporting Company	<input type="checkbox"/>
Emerging Growth Company	<input type="checkbox"/>		

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

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

The number of shares of the registrant's common stock outstanding as of July 31, 2023 was 107,192,901.

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## PART I. FINANCIAL INFORMATION

## ITEM 1. FINANCIAL STATEMENTS

**Arrowhead Pharmaceuticals, Inc.**  
**Consolidated Balance Sheets**  
(In thousands, except per share amounts)

	June 30, 2023	September 30, 2022
	(unaudited)	
<b>ASSETS</b>		
Current assets:		
Cash, cash equivalents and restricted cash	\$ 105,334	\$ 108,005
Accounts receivable	1,247	1,410
Short term investments	346,369	268,391
Prepaid expenses	10,053	7,289
Other current assets	7,162	20,204
<b>Total current assets</b>	<b>470,165</b>	<b>405,299</b>
Property and equipment, net	231,369	110,297
Intangible assets, net	10,687	11,962
Long-term investments	42,758	105,872
Right-of-use assets	40,667	58,291
Other assets	210	218
<b>Total Assets</b>	<b>\$ 795,856</b>	<b>\$ 691,939</b>
<b>LIABILITIES, NONCONTROLLING INTEREST AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 7,874	\$ 2,868
Accrued expenses	38,191	46,856
Accrued payroll and benefits	4,434	12,251
Lease liabilities	2,823	2,776
Deferred revenue	16,905	74,099
<b>Total current liabilities</b>	<b>70,227</b>	<b>138,850</b>
Long-term liabilities:		
Lease liabilities, net of current portion	79,911	78,800
Deferred revenue, net of current portion	—	55,950
Liability related to the sale of future royalties	263,064	—
Other liabilities	669	—
<b>Total long-term liabilities</b>	<b>343,644</b>	<b>134,750</b>
Commitments and contingencies (Note 7)		
Noncontrolling interest and stockholders' equity:		
Common stock, \$0.001 par value:		
Authorized 290,000 and 145,000 shares; issued and outstanding 107,102 and 105,960 shares	199	198
Additional paid-in capital	1,281,393	1,219,213
Accumulated other comprehensive loss	(411)	(136)
Accumulated deficit	(916,351)	(820,755)
<b>Total Arrowhead Pharmaceuticals, Inc. stockholders' equity</b>	<b>364,830</b>	<b>398,520</b>
Noncontrolling interest	17,155	19,819
<b>Total noncontrolling interest and stockholders' equity</b>	<b>381,985</b>	<b>418,339</b>
<b>Total Liabilities, Noncontrolling Interest and Stockholders' Equity</b>	<b>\$ 795,856</b>	<b>\$ 691,939</b>

*The accompanying notes are an integral part of these unaudited consolidated financial statements.*

**Arrowhead Pharmaceuticals, Inc.**  
**Consolidated Statements of Operations and Comprehensive Loss**  
(In thousands, except per share amounts)  
(unaudited)

	Three Months Ended June 30,		Nine Months Ended June 30,	
	2023	2022	2023	2022
<b>Revenue</b>	\$ 15,825	\$ 32,412	\$ 224,638	\$ 211,656
<b>Operating expenses:</b>				
Research and development	94,757	72,180	253,333	213,930
General and administrative	23,771	33,141	67,977	92,403
Total operating expenses	<b>118,528</b>	<b>105,321</b>	<b>321,310</b>	<b>306,333</b>
Operating loss	(102,703)	(72,909)	(96,672)	(94,677)
<b>Other income (expense):</b>				
Interest income	4,172	1,240	11,414	3,450
Interest expense	(5,158)	—	(13,064)	—
Other, net	306	(377)	821	675
Total other (loss) income	<b>(680)</b>	<b>863</b>	<b>(829)</b>	<b>4,125</b>
Loss before income tax expense and noncontrolling interest	<b>(103,383)</b>	<b>(72,046)</b>	<b>(97,501)</b>	<b>(90,552)</b>
Income tax expense	742	—	759	—
Net loss including noncontrolling interest	<b>(104,125)</b>	<b>(72,046)</b>	<b>(98,260)</b>	<b>(90,552)</b>
Net loss attributable to noncontrolling interest, net of tax	(1,179)	—	(2,664)	—
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	<b>\$ (102,946)</b>	<b>\$ (72,046)</b>	<b>\$ (95,596)</b>	<b>\$ (90,552)</b>
Net loss per share attributable to Arrowhead Pharmaceuticals, Inc.:				
Basic	\$ (0.96)	\$ (0.68)	\$ (0.90)	\$ (0.86)
Diluted	\$ (0.96)	\$ (0.68)	\$ (0.90)	\$ (0.86)
Weighted-average shares used in calculating				
Basic	107,004	105,753	106,597	105,273
Diluted	107,004	105,753	106,597	105,273
Other comprehensive loss, net of tax:				
Foreign currency translation adjustments	(79)	(33)	(275)	(71)
Comprehensive loss	<b>\$ (104,204)</b>	<b>\$ (72,079)</b>	<b>\$ (98,535)</b>	<b>\$ (90,623)</b>

*The accompanying notes are an integral part of these unaudited consolidated financial statements.*

**Arrowhead Pharmaceuticals, Inc.**  
**Consolidated Statements of Stockholders' Equity**  
(in thousands)  
(unaudited)

	Common Stock	Amount (\$)	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Non- controlling Interest	Totals
<b>Balance at September 30, 2022</b>	<b>105,960</b>	<b>\$ 198</b>	<b>\$ 1,219,213</b>	<b>\$ (136)</b>	<b>\$ (820,755)</b>	<b>\$ 19,819</b>	<b>\$ 418,339</b>
Stock-based compensation	—	—	19,390	—	—	—	19,390
Exercise of stock options	82	—	576	—	—	—	576
Common stock - restricted stock units vesting	98	1	(1)	—	—	—	—
Foreign currency translation adjustments	—	—	—	(122)	—	—	(122)
Interest in joint venture	—	—	—	—	—	(486)	(486)
Net loss for the three months ended December 31, 2022	—	—	—	—	(41,325)	—	(41,325)
<b>Balance at December 31, 2022</b>	<b>106,140</b>	<b>\$ 199</b>	<b>\$ 1,239,178</b>	<b>\$ (258)</b>	<b>\$ (862,080)</b>	<b>\$ 19,333</b>	<b>\$ 396,372</b>
Stock-based compensation	—	—	20,612	—	—	—	20,612
Exercise of stock options	64	—	520	—	—	—	520
Common stock - restricted stock units vesting	665	—	—	—	—	—	—
Foreign currency translation adjustments	—	—	—	(74)	—	—	(74)
Interest in joint venture	—	—	—	—	—	(999)	(999)
Net income for the three months ended March 31, 2023	—	—	—	—	48,675	—	48,675
<b>Balance at March 31, 2023</b>	<b>106,869</b>	<b>\$ 199</b>	<b>\$ 1,260,310</b>	<b>\$ (332)</b>	<b>\$ (813,405)</b>	<b>\$ 18,334</b>	<b>\$ 465,106</b>
Stock-based compensation	—	—	19,947	—	—	—	19,947
Exercise of stock options	198	—	1,136	—	—	—	1,136
Common stock - restricted stock units vesting	35	—	—	—	—	—	—
Foreign currency translation adjustments	—	—	—	(79)	—	—	(79)
Interest in joint venture	—	—	—	—	—	(1,179)	(1,179)
Net loss for the three months ended June 30, 2023	—	—	—	—	(102,946)	—	(102,946)
<b>Balance at June 30, 2023</b>	<b>107,102</b>	<b>\$ 199</b>	<b>\$ 1,281,393</b>	<b>\$ (411)</b>	<b>\$ (916,351)</b>	<b>\$ 17,155</b>	<b>\$ 381,985</b>

  

	Common Stock	Amount (\$)	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Non- controlling Interest	Totals
<b>Balance at September 30, 2021</b>	<b>104,327</b>	<b>\$ 197</b>	<b>\$ 1,053,386</b>	<b>\$ (69)</b>	<b>\$ (644,692)</b>	<b>\$ —</b>	<b>\$ 408,822</b>
Stock-based compensation	—	—	24,504	—	—	—	24,504
Exercise of stock options	208	—	2,145	—	—	—	2,145
Common stock - restricted stock units vesting	263	—	—	—	—	—	—
Foreign currency translation adjustments	—	—	—	(39)	—	—	(39)
Net loss for the three months ended December 31, 2021	—	—	—	—	(62,872)	—	(62,872)
<b>Balance at December 31, 2021</b>	<b>104,798</b>	<b>\$ 197</b>	<b>\$ 1,080,035</b>	<b>\$ (108)</b>	<b>\$ (707,564)</b>	<b>\$ —</b>	<b>\$ 372,560</b>
Stock-based compensation	—	—	33,802	—	—	—	33,802
Exercise of stock options	237	—	1,537	—	—	—	1,537
Common stock - restricted stock units vesting	667	1	(1)	—	—	—	—
Foreign currency translation adjustments	—	—	—	1	—	—	1
Interest in joint venture	—	—	—	—	—	—	—
Net income for the three months ended March 31, 2022	—	—	—	—	44,366	—	44,366
<b>Balance at March 31, 2022</b>	<b>105,702</b>	<b>\$ 198</b>	<b>\$ 1,115,373</b>	<b>\$ (107)</b>	<b>\$ (663,198)</b>	<b>\$ —</b>	<b>\$ 452,266</b>
Stock-based compensation	—	—	33,391	—	—	—	33,391
Exercise of stock options	53	—	599	—	—	—	599
Common stock - restricted stock units vesting	40	—	—	—	—	—	—
Foreign currency translation adjustments	—	—	—	(33)	—	—	(33)
Interest in joint venture	—	—	39,750	—	—	20,250	60,000
Net loss for the three months ended June 30, 2022	—	—	—	—	(72,046)	—	(72,046)
<b>Balance at June 30, 2022</b>	<b>105,795</b>	<b>\$ 198</b>	<b>\$ 1,189,113</b>	<b>\$ (140)</b>	<b>\$ (735,244)</b>	<b>\$ 20,250</b>	<b>\$ 474,177</b>

*The accompanying notes are an integral part of these unaudited consolidated financial statements.*

**Arrowhead Pharmaceuticals, Inc.**  
**Consolidated Statements of Cash Flows**  
(in thousands)  
(unaudited)

	Nine Months Ended June 30,	
	2023	2022
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net loss	\$ (98,260)	\$ (90,552)
Adjustments to reconcile net loss to net cash flow from operating activities		
Stock-based compensation	59,949	91,697
Depreciation and amortization	8,634	7,761
(Accretion) amortization of note premiums/discounts	(1,030)	2,013
Non-cash interest expense on liability related to the sale of future royalties	13,064	—
Unrealized losses on marketable securities	—	5,755
Changes in operating assets and liabilities:		
Accounts receivable	164	10,016
Prepaid expenses and other current assets	27,913	(8,867)
Accounts payable	5,001	(3,563)
Accrued expenses	(32,082)	1,713
Deferred revenue	(113,144)	(87,100)
Operating lease liabilities	1,158	3,733
Net cash used in operating activities	(128,633)	(67,394)
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of property and equipment	(112,830)	(20,066)
Purchases of investments	(233,984)	(223,391)
Proceeds from maturities of investments	220,150	201,595
Net cash used in investing activities	(126,664)	(41,862)
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from the exercises of stock options	2,232	4,331
Proceeds from the sale of future royalties	250,000	—
Proceeds from investment in joint venture	—	60,000
Proceeds from additional tenant improvement allowance	669	—
Net cash provided by financing activities	252,901	64,331
Net decrease in cash, cash equivalents and restricted cash	(2,396)	(44,925)
Effect of exchange rate on cash, cash equivalents and restricted cash	(275)	(70)
<b>CASH, CASH EQUIVALENTS AND RESTRICTED CASH:</b>		
BEGINNING OF PERIOD	108,005	184,434
END OF PERIOD	\$ 105,334	\$ 139,439
Supplementary disclosures:		
Interest paid	\$ —	\$ —
Income taxes (paid) refunded	\$ —	\$ —

*The accompanying notes are an integral part of these unaudited consolidated financial statements.*

**Arrowhead Pharmaceuticals, Inc.**  
**Notes to Consolidated Financial Statements**  
**(unaudited)**

**NOTE 1. ORGANIZATION AND SIGNIFICANT ACCOUNTING POLICIES**

**General and Recent Developments**

Arrowhead Pharmaceuticals, Inc. and its subsidiaries (referred to herein collectively as the “Company”) are primarily engaged in developing medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, the Company’s therapies trigger the RNA interference mechanism to induce rapid, deep and durable knockdown of target genes. RNA interference (“RNAi”) is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. The Company’s RNAi-based therapeutics may leverage this natural pathway of gene silencing to target and shut down specific disease-causing genes.

The following table presents the Company’s current pipeline:

<b>Therapeutic Area</b>	<b>Name</b>	<b>Stage</b>	<b>Product Rights</b>
<b>Cardiometabolic</b>	ARO-APOC3	Phase 2b and Phase 3	Arrowhead
	ARO-ANG3	Phase 2b	Arrowhead
	Olpasiran	Phase 3	Amgen
<b>Pulmonary</b>	ARO-ENAC2	Pre-Clinical	Arrowhead
	ARO-RAGE	Phase 1/2	Arrowhead
	ARO-MUC5AC	Phase 1/2a	Arrowhead
	ARO-MMP7	Phase 1/2a	Arrowhead
<b>Liver</b>	GSK-4532990 (formerly ARO-HSD)	Phase 2	GSK
	Fazirsiran	Phase 3	Takeda and Arrowhead
	JNJ-3989	Phase 2	Janssen
	HZN-457 (formerly ARO-XDH)	Phase 1	Horizon
	ARO-C3	Phase 1/2	Arrowhead
	ARO-PNPLA3 (formerly JNJ-75220795)	Phase 1	Arrowhead
<b>Muscle</b>	ARO-DUX4	Pre-Clinical	Arrowhead
<b>Central Nervous System (CNS)</b>	ARO-SOD1	Pre-Clinical	Arrowhead

The Company operates lab facilities in San Diego, California and Madison, Wisconsin, where its research and development activities, including the development of RNAi therapeutics, take place. The Company’s principal executive offices are located in Pasadena, California.

During the first three quarters of fiscal 2023, the Company continued to develop and advance its pipeline and partnered candidates. Several key recent developments include:

- hosted a Research & Development (R&D) Day on June 1, 2023 to discuss progress of the Company’s pipeline of RNAi Therapeutics, at which the following updates were discussed:
  - ARO-RAGE showed continued dose response with single inhaled dose of 184 mg achieving mean knockdown of 90% and max of 95%;
  - adipose delivery platform achieved single dose target gene silencing of greater than 90% with six months of duration in non-human primates;
  - improved hepatic dimer platform achieved equivalent or better knockdown of two target genes with longer duration than monomer mixture in non-human primates;
  - TRiM™ platform now has potential to address multiple cell types including liver, solid tumors, lung, central nervous system, skeletal muscle, and adipose;
  - announced progress towards the Company’s “20 in 25” goal to grow its pipeline of RNAi therapeutics that leverage the proprietary Targeted RNAi Molecule (TRiM™) platform to a total of

20 clinical stage or marketed products in the year 2025;

- presented updated data from the Phase 2 SEQUOIA study of investigational RNAi therapy Fazirsiran in patients with alpha-1 antitrypsin deficiency liver disease which included:
  - Fazirsiran reduced serum Z-AAT concentration in a dose-dependent manner;
  - Fazirsiran significantly reduced liver Z-AAT;
  - Fazirsiran consistently reduced hepatic globule burden;
  - Fazirsiran treatment reduced histological signs of hepatic inflammation;
  - 50% of the pooled Fazirsiran treated patients showed at least a one-point improvement in METAVIR liver fibrosis versus 38% in the placebo group;
  - Fazirsiran has been well tolerated to date;
  - pulmonary function test results (FEV1 and DLCO) for both Fazirsiran and placebo were stable over time with no apparent dose-dependent effects;
  - updated Phase 2 clinical data were presented at the European Association for the Study of the Liver (EASL) Congress 2023 in an oral presentation titled, “Fazirsiran reduces liver Z-alpha-1 antitrypsin synthesis, decreases globule burden and improves histological measures of liver disease in adults with alpha-1 antitrypsin deficiency: a randomized placebo-controlled phase 2 study”;
- presented interim data from the ongoing Phase 2 GATEWAY clinical study of ARO-ANG3 which included:
  - mean reduction in LDL-C of 48.1% (200mg) and 44.0% (300mg);
  - ANPTL3 inhibition with ARO-ANG3 also reduced HDL-C, non-HDL-C, and triglycerides, consistent with published human genetic data;
  - safety and tolerability;
- completed enrollment of the Phase 3 PALISADE clinical trial evaluating ARO-APOC3 for treatment of familial chylomicronemia syndrome;
- announced interim results from ARO-RAGE administration in Part 1 of the ongoing Phase 1/2 study in normal healthy volunteers which included:
  - reductions in soluble RAGE (sRAGE) as measured in serum after two doses on Day 1 and Day 29;
  - duration of pharmacologic effect persisted for at least 6 weeks after the second administration of the 92 mg does with further follow up ongoing;
  - reduction in sRAGE as measured in bronchoalveolar lavage fluid (BALF) at Day 31 after a single dose;
  - reduction in in serum sRAGE was observed after a single dose;
  - the pooled placebo groups experienced a mean sRAGE increase of 8% in BALF and a mean decrease of 1% serum;
  - safety and tolerability;
- expanded TRiM™ platform to include an optimized intrathecal administration for CNS delivery with distribution throughout the brain and in all relevant brain cell types. The first development candidate to utilize this new delivery platform is ARO-SOD1. In June 2023, the Company filed a clinical trial application (CTA) for approval to initiate a Phase 1 clinical study. In preclinical studies, ARO-SOD1 achieved 95% spinal cord tissue mRNA knockdown after a single intrathecal dose in human SOD1 transgenic rats and maintained greater than 80% spinal cord tissue mRNA knockdown three months after a single intrathecal dose in non-human primates;
- dosed the first patient in Takeda’s Phase 3 REDWOOD clinical study of Fazirsiran for the treatment of alpha-1 antitrypsin deficiency associated liver diseases, triggering a \$40.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023;
- dosed the first patient in GSK’s Phase 2b trial of GSK4532990, formerly called ARO-HSD, an investigational RNAi therapeutic for the treatment of patients with non-alcoholic steatohepatitis (NASH), triggering a \$30.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023;

- announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to ARO-APOC3 for reducing triglycerides in adult patients with familial chylomicronemia syndrome (FCS). ARO-APOC3 was previously granted Orphan Drug designation by the FDA and the European Union;
- announced interim results from Part 1 of AROC3-1001, an ongoing Phase 1/2 clinical study of ARO-C3, which included:
  - a dose-dependent reduction in serum C3, with 88% mean reduction at highest dose tested;
  - a dose-dependent reduction in AH50, a marker of alternative complement pathway hemolytic activity, with 91% mean reduction at highest dose tested;
  - duration of pharmacologic effect supportive of quarterly or less frequent subcutaneous dose administration;
  - safety and tolerability;
- received notice from Janssen of its decision to voluntarily terminate the Research Collaboration and Option Agreement (the “Janssen Collaboration Agreement”) between the Company and Janssen. The Company regained full rights to ARO-PNPLA3, formerly called JNJ-75220795, upon termination of the Janssen Collaboration Agreement, which took effect on April 7, 2023. ARO-PNPLA3 is in Phase 1 clinical trials that are now being developed by the Company;
- initiated dosing in ARO-MMP7-1001 (NCT05537025), a Phase 1/2a single ascending dose and multiple ascending dose clinical study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-MMP7, an investigational RNAi therapeutic designed to reduce expression of matrix metalloproteinase 7 (MMP7) as a potential treatment for idiopathic pulmonary fibrosis (IPF), in up to 56 healthy volunteers and in up to 21 patients with IPF;
- enrolled the first subject in a Phase 1 randomized, placebo-controlled trial to assess the safety tolerability, pharmacokinetics and pharmacodynamics of a development-stage medicine, HZN-457 (previously known as ARO-XDH), which is out-licensed to Horizon, triggering a \$15.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023;
- enrolled the first subject in Amgen’s Phase 3 trial of Olpasiran, triggering a \$25.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023;
- entered into a Royalty Purchase Agreement (the “Royalty Pharma Agreement”) with Royalty Pharma Investments 2019 ICAV (“Royalty Pharma”) on November 9, 2022, pursuant to which Royalty Pharma paid \$250.0 million upfront (See Note 11 — Liability Related to the Sale of Future Royalties of Notes to Consolidated Financial Statements of Part I, “Item 1. Financial Statements.”);
- announced top line results from the SEQUOIA Phase 2 Study of Fazirsiran in patients with Alpha-1 Antitrypsin Deficiency-Associated Liver Disease in which:
  - fibrosis regression was observed in 50% of patients receiving Fazirsiran;
  - median reductions of 94% of Z-AAT accumulation in the liver and mean reductions of 68% in histologic globule burden were observed;
  - treatment emergent adverse events were generally well balanced between Fazirsiran and placebo groups;
  - results were consistent with AROAAT-2002 open-label study previously published in The New England Journal of Medicine.

### ***Consolidation and Basis of Presentation***

The interim Consolidated Financial Statements include the accounts of Arrowhead Pharmaceuticals, Inc. and its subsidiaries (wholly-owned subsidiaries and a variable interest entity for which the Company is the primary beneficiary). Subsidiaries refer to Arrowhead Madison, Inc., Visirna Therapeutics, Inc. (“Visirna”), and Arrowhead Australia Pty Ltd. For subsidiaries in which the Company owns or is exposed to less than 100% of the economics, the Company records net loss attributable to noncontrolling interests in its consolidated statements of operations equal to the percentage of the economic or ownership interests retained in such entity by the respective noncontrolling party.

The interim Consolidated Financial Statements have been prepared in conformity with U.S. generally accepted accounting principles (“GAAP”). The financial data of the Company included herein are unaudited. In the opinion of management, all material adjustments of a normal recurring nature have been made to present fairly the Company’s

financial position at June 30, 2023 and the results of operations and cash flows for the periods presented. All intercompany transactions and balances have been eliminated. Certain prior period amounts have been reclassified to conform with the current period presentation.

Certain financial information that is normally included in annual financial statements prepared in accordance with GAAP, but that is not required for interim reporting purposes, has been omitted from the accompanying interim consolidated financial statements and related notes. Readers are urged to review the Company's Annual Report on Form 10-K for the year ended September 30, 2022 for more complete descriptions and discussions. Operating results and cash flows for the nine months ended June 30, 2023 are not necessarily indicative of the results that may be expected for the fiscal year ending September 30, 2023.

### **Liquidity**

The Company's primary sources of financing have been through the sale of its securities, revenue from its licensing and collaboration agreements and the sale of certain future royalties. Research and development activities have required significant capital investment since the Company's inception and are expected to continue to require significant cash expenditure in the future, particularly as the Company's pipeline of drug candidates and its headcount have both expanded significantly. Additionally, significant capital investment will be required as the Company's pipeline matures into later stage clinical trials and as the Company plans to increase its internal manufacturing capabilities.

At June 30, 2023, the Company had \$105.3 million in cash and cash equivalents (including \$7.3 million in restricted cash), \$346.4 million in short-term investments and \$42.8 million in long-term investments to fund operations. During the nine months ended June 30, 2023, the Company's cash and cash equivalents and investments balance increased by \$12.2 million which was primarily due to the \$250.0 million upfront payment received from Royalty Pharma (Note 11) and \$110.0 million in milestone payments from the Company's collaboration and license agreements, partially offset by cash used to fund its operations.

In total, the Company is eligible to receive up to \$3.4 billion in developmental, regulatory and sales milestones, and may receive various royalties on net sales from its licensing and collaboration agreements, subject to the terms and conditions of those agreements. The revenue recognition for these collaboration agreements is discussed further in Note 2.

### **Summary of Significant Accounting Policies**

There have been no changes to the significant accounting policies disclosed in the Company's most recent Annual Report on Form 10-K for the fiscal year ended September 30, 2022.

### **Recent Accounting Pronouncements**

There have been no recent accounting pronouncements that have significantly impacted this Quarterly Report on Form 10-Q, beyond those disclosed in the Company's most recent Annual Report on Form 10-K for the fiscal year ended September 30, 2022.

## **NOTE 2. COLLABORATION AND LICENSE AGREEMENTS**

The following table provides a summary of revenue recognized:

	<b>Three Months Ended June 30,</b>		<b>Nine Months Ended June 30,</b>	
	<b>2023</b>	<b>2022</b>	<b>2023</b>	<b>2022</b>
	<b>(in thousands)</b>			
GSK	\$ 277	\$ —	\$ 29,600	\$ 120,000
Horizon	1,539	6,666	23,206	21,251
Takeda	14,009	25,507	146,477	67,100
Janssen	—	239	355	3,305
Amgen	—	—	25,000	—
<b>Total</b>	<b>\$ 15,825</b>	<b>\$ 32,412</b>	<b>\$ 224,638</b>	<b>\$ 211,656</b>

The following table summarizes the balance of receivables and contract liabilities related to the Company's

collaboration and license agreements:

	June 30, 2023	September 30, 2022	
	(in thousands)		
Receivables included in accounts receivable	\$	1,539	\$ 6,174
Contract liabilities included in deferred revenue	\$	16,905	\$ 130,049

### ***Glaxosmithkline Intellectual Property (No. 3) Limited (“GSK”)***

On November 22, 2021, GSK and the Company entered into an Exclusive License Agreement (the “GSK License Agreement”). Under the GSK License Agreement, GSK has received an exclusive license for GSK-4532990 (formerly ARO-HSD). The exclusive license is worldwide with the exception of greater China. The Company completed its Phase 1/2 study of GSK-4532990, and GSK is wholly responsible for all clinical development and commercialization of GSK-4532990 in its territory. Under the terms of the agreement, the Company has received an upfront payment of \$120.0 million and recognized an additional \$30.0 million at the start of a Phase 2 trial. The Company is also eligible for an additional payment of \$100.0 million upon achieving a successful Phase 2 trial readout and the first patient dosed in a Phase 3 trial. Furthermore, should the Phase 3 trial read out positively, and the potential new medicine receives regulatory approval in major markets, the deal provides for commercial milestone payments to the Company of up to \$190.0 million at first commercial sale, and up to \$590.0 million in sales-related milestone payments. The Company is further eligible to receive tiered royalties on net product sales in a range of mid-teens to twenty percent.

At the inception of the GSK License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services, including the Company’s responsibility to complete the Phase 1/2 study (the “GSK R&D Services”). Due to the specialized and unique nature of the GSK R&D Services and their direct relationship with the license, the Company determined that these deliverables represented one distinct bundle and, thus, one performance obligation. Beyond the GSK R&D Services, which are the responsibility of the Company, GSK will be responsible for managing future clinical development and commercialization in its territory.

The Company determined the initial transaction price totaled \$120.0 million, including the upfront payment, which was collected in January 2022. The Company has excluded any future estimated milestones or royalties from this transaction price to date. The Company has allocated the total \$120.0 million initial transaction price to its one distinct performance obligation for the GSK-4532990 license and the associated GSK R&D Services. As the Company has completed its performance obligation related to this agreement, the upfront payment of \$120.0 million was fully recognized during the six months ended March 31, 2022. Further, GSK dosed the first patient in a Phase 2 trial in March 2023, triggering a \$30.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023. There were no contract assets and liabilities recorded as of June 30, 2023.

### ***Horizon Therapeutics Ireland DAC (“Horizon”)***

On June 18, 2021, Horizon and the Company entered into a collaboration and license agreement (the “Horizon License Agreement”). Under the terms of the Horizon License Agreement, Horizon received a worldwide exclusive license for HZN-457, a clinical-stage medicine being developed by Horizon as a potential treatment for people with uncontrolled gout. The Company conducted all activities through the preclinical stages of development of, and Horizon is now wholly responsible for clinical development and commercialization of, HZN-457. The Company received \$40.0 million as an upfront payment in July 2021 and an additional \$15.0 million upon Horizon’s initiation of a Phase 1 clinical trial in January 2023, and is eligible to receive up to \$645.0 million in additional potential development, regulatory and sales milestones. The Company is also eligible to receive royalties in the low- to mid-teens range on net product sales.

At the inception of the Horizon License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services, including the Company’s responsibilities to conduct all activities through the preclinical stages of development of HZN-457 (the “Horizon R&D Services”). Due to the specialized and unique nature of these Horizon R&D Services and their direct relationship with the license, the Company determined that these deliverables represented one distinct bundle and, thus, one performance obligation. Beyond the Horizon R&D Services, which are the responsibility of the Company, Horizon is responsible for managing future clinical development and commercialization of HZN-457.

The Company determined the initial transaction price totaled \$40.0 million, including the upfront payment. The Company has excluded any future estimated milestones or royalties from this transaction price to date. The Company allocated the total \$40.0 million initial transaction price to its one distinct performance obligation for the HZN-457 license and the associated Horizon R&D Services. Revenue was recognized on a straight-line basis over the timeframe for

completing the Horizon R&D Services. The Company determined that the straight-line basis was appropriate as its efforts were expended evenly over the course of completing its performance obligation. Further, Horizon enrolled the first subject in December 2022 in a Phase 1 randomized, placebo-controlled trial to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of HZN-457, triggering a \$15.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023. There was \$1.5 million in contract assets recorded as accounts receivable and \$0 contract liabilities as of June 30, 2023.

#### ***Takeda Pharmaceutical Company Limited (“Takeda”)***

On October 7, 2020, Takeda and the Company entered into an Exclusive License and Co-Funding Agreement (the “Takeda License Agreement”). Under the Takeda License Agreement, Takeda and the Company will co-develop its Fazirsiran program, the Company’s second-generation subcutaneously administered RNAi therapeutic candidate being developed as a treatment for liver disease associated with alpha-1 antitrypsin deficiency. Within the United States, Fazirsiran, if approved, will be co-commercialized under a 50/50 profit sharing structure. Outside the United States, Takeda will lead the global commercialization strategy and received an exclusive license to commercialize Fazirsiran, while the Company will be eligible to receive tiered royalties of 20% to 25% on net sales. The Company received \$300.0 million as an upfront payment in January 2021, recognized an additional \$40.0 million upon Takeda’s initiation of a Phase 3 clinical study in March 2023, and is eligible to receive potential development, regulatory and commercial milestones of up to \$527.5 million.

At the inception of the Takeda License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services including the Company’s responsibilities to complete the initial portion of the SEQUOIA study, to complete the ongoing Phase 2 AROAAT2002 study and to ensure certain manufacturing of Fazirsiran drug product is completed and delivered to Takeda (the “Takeda R&D Services”). Due to the specialized and unique nature of these Takeda R&D Services and their direct relationship with the license, the Company determined that these deliverables represent one distinct bundle and, thus, one performance obligation. Beyond the Takeda R&D Services, which are the responsibility of the Company, Takeda will be responsible for managing future clinical development and commercialization outside the United States. Within the United States, the Company will also participate in co-development and co-commercialization efforts and will co-fund these efforts with Takeda as part of the 50/50 profit sharing structure within the United States. The Company considers the collaborative activities, including the co-development and co-commercialization, to be a separate unit of account within Topic 808, and as such, these co-funding amounts are recorded as research and development expenses or general and administrative expenses, as appropriate.

The Company has allocated the total \$300.0 million initial transaction price to its one distinct performance obligation for the Fazirsiran license and the associated Takeda R&D Services. Revenue is recognized using a proportional performance method (based on actual patient visits completed versus total estimated visits completed for the ongoing SEQUOIA and AROAAT2002 clinical studies). The Company previously expected these clinical trials to extend to September 2025 in order to demonstrate long term safety and efficacy in the open label extension (OLE) part of the studies; however, Takeda now intends to initiate a new OLE study available to patients participating in these Phase 2 studies that will initiate as early as the fourth quarter of fiscal 2023. Based on this new information, patients enrolled in the SEQUOIA and AROAAT2002 studies are expected to complete their Phase 2 study visits between June 2023 and June 2024, shortening the Company’s performance obligation. As a result, effective the second quarter of fiscal 2023, the Company changed its estimates of the revenue recognition to better reflect this newly estimated performance period. The effect of these changes in estimates resulted in accelerated revenue by \$61.4 million, or \$0.58 per share (diluted) for each of the three and nine months ended June 30, 2023. There were \$16.9 million of contract liabilities recorded as deferred revenue, of which \$16.9 million was classified as current as of June 30, 2023.

In March 2023, Takeda dosed the first patient in the Phase 3 REDWOOD clinical study of Fazirsiran, triggering a \$40.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023. The Company also recorded \$1.4 million as accrued expenses as of June 30, 2023 that was primarily driven by co-development and co-commercialization activities.

#### ***Janssen Pharmaceuticals, Inc. (“Janssen”)***

On October 3, 2018, Janssen, part of the Janssen Pharmaceutical Companies of Johnson & Johnson, and the Company entered into a License Agreement (the “Janssen License Agreement”) and the Janssen Collaboration Agreement. The Company also entered into a stock purchase agreement with JJDC, Inc. (“JJDC”), Johnson & Johnson’s venture capital arm (the “JJDC Stock Purchase Agreement”).

Under the Janssen License Agreement, Janssen received a worldwide, exclusive license to the Company’s JNJ-3989 (ARO-HBV) program, the Company’s third-generation subcutaneously administered RNAi therapeutic candidate being

developed as a potential therapy for patients with chronic hepatitis B virus infection. Beyond the Company's Phase 1/2 study of JNJ-3989 (ARO-HBV), which the Company was responsible for completing, Janssen is wholly responsible for clinical development and commercialization of JNJ-3989 (ARO-HBV). Under the terms of the Janssen License Agreement, the Company has received \$175.0 million as an upfront payment, \$75.0 million in the form of an equity investment by JJDC in the Company's common stock under the JJDC Stock Purchase Agreement, and milestone and option payments totaling \$73.0 million, and the Company may receive up to \$0.8 billion in development and sales milestone payments for the Janssen License Agreement. The Company is further eligible to receive tiered royalties on product sales up to mid-teens under the Janssen License Agreement.

In May 2021, Janssen exercised its option right for JNJ-75220795 (ARO-JNJ1) which resulted in a \$10.0 million milestone payment to the Company. This \$10.0 million milestone payment was recognized entirely as of September 30, 2021. The Company conducted its discovery, optimization and preclinical research and development of JNJ-75220795 (ARO-JNJ1), ARO-JNJ2, and ARO-JNJ3 under the Janssen Collaboration Agreement. All costs and labor hours spent by the Company have been entirely funded by Janssen. On April 7, 2023, Janssen voluntarily terminated the Janssen Collaboration Agreement. Upon termination, the Company regained full rights to ARO-PNPLA3, formerly called JNJ-75220795, the only candidate for which Janssen had exercised its option.

At the inception of the Janssen License Agreement, the Company determined that the key deliverables included the license and certain R&D services including the Company's responsibility to complete the Phase 1/2 study of JNJ-3989 (ARO-HBV) and the Company's responsibility to ensure certain manufacturing of JNJ-3989 (ARO-HBV) drug product is completed and delivered to Janssen (the "Janssen R&D Services"). Due to the specialized and unique nature of these Janssen R&D Services and their direct relationship with the license, the Company determined that these deliverables represent one distinct bundle and, thus, one performance obligation.

The Company determined the transaction price totaled approximately \$252.7 million, which includes the upfront payment, the premium paid by JJDC for its equity investment in the Company, two \$25.0 million milestone payments related to JNJ-3989 (ARO-HBV), and estimated payments for reimbursable Janssen R&D Services to be performed. The Company has allocated the total \$252.7 million initial transaction price to its one distinct performance obligation for the JNJ-3989 (ARO-HBV) license and the associated Janssen R&D Services. The Company recognized this transaction price in its entirety as of September 30, 2021, as its performance obligations were substantially completed. Future milestones and royalties achieved will be recognized in their entirety when earned. There were no contract assets and liabilities recorded as of June 30, 2023.

#### ***Amgen Inc. ("Amgen")***

On September 28, 2016, Amgen and the Company entered into two collaboration and license agreements and a common stock purchase agreement. Under the Second Collaboration and License Agreement (the "Olpasiran Agreement"), Amgen received a worldwide, exclusive license to the Company's novel RNAi Olpasiran program. These RNAi molecules are designed to reduce elevated lipoprotein(a), which is a genetically validated, independent risk factor for atherosclerotic cardiovascular disease. Under the first collaboration and license agreement (the "First Collaboration and License Agreement" or the "ARO-AMG1 Agreement"), Amgen received an option to a worldwide, exclusive license to ARO-AMG1, an RNAi therapy for an undisclosed genetically validated cardiovascular target. Under both agreements, Amgen is wholly responsible for clinical development and commercialization.

Under the Olpasiran Agreement and the ARO-AMG1 Agreement, the Company has received \$35.0 million in upfront payments and \$21.5 million in the form of an equity investment by Amgen in the Company's common stock. Further, the Company received additional an \$55.0 million in milestone payments; \$10.0 million upon Amgen's initiation of Phase 1 study in September 2018, \$20.0 million upon its initiation of a Phase 2 clinical study in July 2020, and \$25.0 million upon its first subject enrollment in a Phase 3 trial in December 2022. The Company has substantially completed its performance obligations under the Olpasiran Agreement and the ARO-AMG1 Agreement. There were no contract assets and liabilities recorded as of June 30, 2023.

In November 2022, Royalty Pharma and the Company entered into the Royalty Pharma Agreement. In consideration for the payments under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive up to an additional \$535.0 million in remaining development, regulatory and sales milestone payments payable from Amgen and Royalty Pharma. See Note 11.

#### ***Joint Venture and License Agreement with Visirna Therapeutics, Inc. ("Visirna")***

On April 25, 2022, Visirna and the Company entered into a License Agreement (the "Visirna License Agreement"), pursuant to which Visirna received an exclusive license to develop, manufacture and commercialize four of the Company's

RNAi-based investigational cardiometabolic medicines in Greater China (including the People’s Republic of China, Hong Kong, Macau and Taiwan). Pursuant to a Share Purchase Agreement (the “Visirna SPA”) entered into simultaneously with the Visirna License Agreement, the Company acquired a majority stake in Visirna as partial consideration for the Visirna License Agreement. Under the Visirna SPA, entities affiliated with Vivo Capital also acquired a minority stake in Visirna in exchange for \$60.0 million in upfront capital to support the operations of Visirna. As further consideration under the Visirna License Agreement, the Company is also eligible to receive potential royalties on commercial sales.

During the nine months ended June 30, 2023, the Company performed manufacturing and development work pursuant to a Clinical Supply Agreement between the parties contemplated by the Visirna License Agreement. The Company received \$0.9 million as consideration for this manufacturing and development work, and there were no contract assets and liabilities recorded as of June 30, 2023.

### NOTE 3. PROPERTY AND EQUIPMENT

The following table summarizes the Company's major classes of property and equipment:

	June 30, 2023	September 30, 2022
	(in thousands)	
Computers, software, office equipment and furniture	\$ 2,198	\$ 2,182
Land	2,996	2,996
Research equipment	50,472	38,283
Leasehold improvements	96,344	42,017
Construction in progress	118,279	56,373
	270,289	141,851
Less: Accumulated depreciation and amortization	(38,920)	(31,554)
Property and equipment, net	\$ 231,369	\$ 110,297

Depreciation and amortization expense for property and equipment for the three months ended June 30, 2023 and 2022 was \$2.9 million and \$2.2 million, respectively. Depreciation and amortization expense for property and equipment for the nine months ended June 30, 2023 and 2022 was \$7.4 million and \$6.5 million, respectively.

The increase in the construction in progress during the nine months ended June 30, 2023 was mainly due to the continuing developments of manufacturing, laboratory and office facilities in Verona, Wisconsin as well as a new laboratory and office facility in San Diego, California. In May 2023, the Company completed the development of the San Diego facility, which resulted in the reclassification of construction in progress as leasehold improvements as of June 30, 2023. See Note 7.

**NOTE 4. INVESTMENTS**

The Company's investments consisted of the following:

	As of June 30, 2023			
	(in thousands)			
	Adjusted Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
<b>Short-term investments (due within one year)</b>				
Held to maturity debt securities	\$ 346,369	\$ —	\$ (4,204)	\$ 342,165
Held to maturity certificate of deposit	—	—	—	—
<b>Total short-term investments</b>	<u>\$ 346,369</u>	<u>\$ —</u>	<u>\$ (4,204)</u>	<u>\$ 342,165</u>
<b>Long-term investments (due within one through three years)</b>				
Held to maturity debt securities	\$ 42,758	\$ —	\$ (294)	\$ 42,464
<b>Total long-term investments</b>	<u>\$ 42,758</u>	<u>\$ —</u>	<u>\$ (294)</u>	<u>\$ 42,464</u>

	As of September 30, 2022			
	(in thousands)			
	Adjusted Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
<b>Short-term investments (due within one year)</b>				
Held to maturity debt securities	\$ 218,391	\$ —	\$ (3,661)	\$ 214,730
Held to maturity certificate of deposit	50,000	—	—	50,000
<b>Total short-term investments</b>	<u>\$ 268,391</u>	<u>\$ —</u>	<u>\$ (3,661)</u>	<u>\$ 264,730</u>
<b>Long-term investments (due within one through three years)</b>				
Held to maturity debt securities	\$ 105,872	\$ —	\$ (5,569)	\$ 100,303
<b>Total long-term investments</b>	<u>\$ 105,872</u>	<u>\$ —</u>	<u>\$ (5,569)</u>	<u>\$ 100,303</u>

## NOTE 5. INTANGIBLE ASSETS

Intangible assets subject to amortization include patents and a license agreement capitalized as part of the Novartis RNAi asset acquisition in March 2015. The following table presents the components of intangible assets:

	Gross Carrying Amount	Accumulated Amortization	Impairment	Net Carrying Amount	Useful Lives
	(in thousands)				(in years)
<b>As of June 30, 2023</b>					
Patents	\$ 21,728	\$ 12,933	\$ —	\$ 8,795	14
License	3,129	1,237	—	1,892	21
<b>Total intangible assets, net</b>	<b>\$ 24,857</b>	<b>\$ 14,170</b>	<b>\$ —</b>	<b>\$ 10,687</b>	
<b>As of September 30, 2022</b>					
Patents	\$ 21,728	\$ 11,770	\$ —	\$ 9,958	14
License	3,129	1,125	—	2,004	21
<b>Total intangible assets, net</b>	<b>\$ 24,857</b>	<b>\$ 12,895</b>	<b>\$ —</b>	<b>\$ 11,962</b>	

Intangible assets are reviewed annually for impairment and more frequently if potential impairment indicators exist. No impairment indicators were identified during the nine months ended June 30, 2023 and 2022.

Intangible assets with definite useful lives are amortized on a straight-line basis over their useful lives. Intangible assets amortization expense was \$0.4 million for each of the three months ended June 30, 2023 and 2022, and \$1.3 million and for each of the nine months ended June 30, 2023 and 2022. None of the intangible assets with definite useful lives are anticipated to have a residual value.

The following table presents the estimated future amortization expense related to intangible assets as of June 30, 2023:

Year Ending September 30,	Amortization Expense
	(in thousands)
2023 (remainder)	\$ 425
2024	1,700
2025	1,700
2026	1,700
2027	1,700
Thereafter	3,462
<b>Total</b>	<b>\$ 10,687</b>

## NOTE 6. STOCKHOLDERS' EQUITY

The following table summarizes the Company's shares of common stock and preferred stock:

	Par Value	Shares		
		Authorized	Issued	Outstanding
(in thousands)				
<b>As of June 30, 2023</b>				
Common stock	\$ 0.001	290,000	107,102	107,102
Preferred stock	\$ 0.001	5,000	—	—
<b>As of September 30, 2022</b>				
Common stock	\$ 0.001	145,000	105,960	105,960
Preferred stock	\$ 0.001	5,000	—	—

On March 16, 2023, the Company's stockholders approved an increase in authorized common shares, par value 0.001 per share, from 145,000,000 to 290,000,000. The amendment to the Amended and Restated Certificate of Incorporation was filed on April 27, 2023.

As of June 30, 2023 and September 30, 2022, respectively, 12,914,571 and 14,000,392 shares of common stock were reserved for issuance upon exercise of options and vesting of restricted stock units granted or available for grant under the Company's 2004 Equity Incentive Plan, 2013 Incentive Plan, and 2021 Incentive Plan, as well as for inducement grants made to new employees under Rule 5635(c)(4) of the Nasdaq Listing Rules.

On December 2, 2022, the Company entered into an open market sale agreement (the "Open Market Sale Agreement"), pursuant to which the Company may, from time to time, sell up to \$250,000,000 in shares of the Company's common stock through Jefferies LLC, acting as the sales agent and/or principal, in an at-the-market offering ("ATM Offering"). The Company is not required to sell shares under the Open Market Sale Agreement. The Company will pay Jefferies LLC a commission of up to 3.0% of the aggregate gross proceeds received from all sales of the common stock under the Open Market Sale Agreement. Unless otherwise terminated, the ATM Offering shall terminate upon the earlier of (i) the sale of all shares of common stock subject to the Sales Agreement and (ii) the termination of the Sales Agreement as permitted therein. The Company and Jefferies may each terminate the Open Market Sale Agreement at any time upon prior notice. As of June 30, 2023, no shares have been issued under the Open Market Sale Agreement.

## NOTE 7. COMMITMENTS AND CONTINGENCIES

### *Litigation*

From time to time, the Company may be subject to various claims and legal proceedings in the ordinary course of business. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount is reasonably estimable, the Company will accrue a liability for the estimated loss. There were no contingent liabilities recorded as of June 30, 2023.

### *Commitments*

On December 20, 2021, the Company completed a purchase of 13 acres of land in the Verona Technology Park in Verona, Wisconsin, which is being developed into an approximately 160,000 square foot drug manufacturing facility and an approximately 140,000 square foot laboratory and office facility which will support the Company's process development and analytical activities. As of June 30, 2023, the Company has incurred \$102.7 million and intends to spend an additional \$160.0 million to \$180.0 million to complete the build out of the facilities. As part of this acquisition, the Company entered into a development agreement with the City of Verona to construct certain infrastructure improvements within the tax incremental district and will be reimbursed up to \$16.0 million by the City of Verona by future tax increment revenue generated from the developed property. The total amount of funding that the City of Verona will pay under the Tax Incremental Financing program is not guaranteed and will depend on future tax revenues generated from the developed property. The Company will also receive up to \$2.5 million of refundable Wisconsin state income tax credits from the Wisconsin Economic Development Corporation (WEDC) as incentives to invest in the local community and create new jobs.

### *Technology License Commitments*

The Company has licensed from third parties the rights to use certain technologies for its research and development activities, as well as in any products it may develop using these licensed technologies. These agreements and other similar

agreements often require the Company to make milestone and royalty payments. Milestone payments, for example, may be required as the research and development process progresses through various stages of development, such as when clinical candidates enter or progress through clinical trials, upon NDA and/or certain sales level milestones. During the three and nine months ended June 30, 2023 and 2022, the Company did not reach any milestones.

## NOTE 8. LEASES

On November 19, 2021, the Company entered into a 15-year lease for approximately 144,000 square feet of office and research and development laboratory space in San Diego, California. This new facility accommodates increased personnel for its expanding pipeline of current and future drug candidates. The lease payments, which began on April 19, 2023, the rent commencement date, will be approximately \$119.0 million over the initial 15-year term. The Company also estimates annual operating expenses to be approximately \$3.0 million for the first year of the lease, and these payments will continue throughout the initial 15-year term. The Company expects to pay approximately \$32.0 million for leasehold improvements, net of tenant improvement allowances. Pursuant to the lease, within twelve months of the expiration of the initial 15-year term, the Company has the option to extend the lease for up to one additional ten-year term, with certain annual increases in base rent.

Further, the lease agreement grants the Company the right to receive an Additional Tenant Improvement Allowance (“ATIA”) funded by the lessor. The maximum amount of ATIA is \$7.2 million, and as of June 30, 2023, the Company has received approximately \$0.7 million, which has been recorded as other liabilities on its consolidated balance sheets. The Company will repay the ATIA through equal monthly payments, including 7% interest per annum over the base term, starting from the rent commencement date. Interest begins accruing on the date the lessor first disburses the ATIA.

### Other Significant Leases

**Pasadena, California:** The Company leases 49,000 square feet of office space located at 177 Colorado Blvd. for its corporate headquarters from 177 Colorado Owner, LLC, which lease expires on April 30, 2027. The lease contains an option to renew for one term of five years.

**San Diego, California:** The Company subleased space from Halozyme, Inc. for additional research and development space in San Diego, California. The term of this sublease commenced on April 1, 2020 and ended on January 14, 2023. On December 23, 2022, the Company entered into a new six-month lease agreement with 11404 & 11408 Sorrento Valley Owner (DE) LLC, effective January 15, 2023. The lease ended on July 15, 2023.

**Madison, Wisconsin:** The Company leases space for office and laboratory facilities, which expires on September 30, 2031. The lease contains options to renew for two terms of five years. After accounting for additional rental square feet added pursuant to amendments to the lease agreement in 2019 and 2020, the Company currently leases a total of 111,000 square feet.

The components of lease assets and liabilities along with their classification on the Company’s consolidated balance sheets were as follows:

Lease Assets and Liabilities		Classification	June 30, 2023		September 30, 2022	
(in thousands)						
Operating lease assets	Right-of-use assets		\$	40,667	\$	58,291
Current operating lease liabilities	Lease liabilities			2,823		2,776
Non-current operating lease liabilities	Lease liabilities, net of current portion			79,911		78,800

  

Lease Cost	Classification	Three Months Ended June 30,		Nine Months Ended June 30,					
		2023	2022	2023	2022				
(in thousands)									
Operating lease cost	Research and development	\$	3,323	\$	2,974	\$	7,735	\$	4,757
	General and administrative expense		509		448		1,542		1,288
Variable lease cost <sup>(1)</sup>	Research and development		257		179		627		519
	General and administrative expense		—		—		—		—
<b>Total</b>		<b>\$</b>	<b>4,089</b>	<b>\$</b>	<b>3,601</b>	<b>\$</b>	<b>9,904</b>	<b>\$</b>	<b>6,564</b>

(1) Variable lease cost is primarily related to operating expenses associated with the Company's operating leases.

There was \$0.6 million and \$0.2 million short-term lease cost during the three months ended June 30, 2023, and 2022, respectively. There was \$1.2 million and \$0.7 million short-term lease cost during the nine months ended June 30, 2023, and 2022, respectively.

The following table presents payments of operating lease liabilities on an undiscounted basis as of June 30, 2023:

Year	Amounts (in thousands)
2023 (remainder of fiscal year)	\$ 1,143
2024	8,094
2025	11,800
2026	12,148
2027	11,320
2028 and thereafter	102,812
<b>Total</b>	<b>\$ 147,317</b>
Less imputed interest	\$ (64,583)
<b>Total operating lease liabilities (includes current portion)</b>	<b>\$ 82,734</b>

Supplemental cash flow and other information related to leases was as follows:

	Nine Months Ended June 30,	
	2023	2022
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases (in thousands)	4,430	3,398
	June 30,	
	2023	2022
Weighted-average remaining lease term (in years)	13.4	7.3
Weighted-average discount rate	8.0 %	8.5 %

## NOTE 9. STOCK-BASED COMPENSATION

The Company has three plans that provide for equity-based compensation. Under the 2004 Equity Incentive Plan (the “2004 Plan”) and 2013 Incentive Plan (the “2013 Plan”), 68,555 and 3,440,076 shares, respectively, of the Company’s common stock are reserved for grants of stock options and restricted stock awards to employees and directors as of June 30, 2023.

On March 18, 2021, the Company’s Board of Directors approved the Arrowhead Pharmaceuticals, Inc. 2021 Incentive Plan (the “2021 Plan”), which authorized 8,000,000 shares (subject to certain adjustments) available for grants of stock options, stock appreciation rights, restricted and unrestricted stock, performance awards, cash awards and other awards convertible into or otherwise based on shares of the Company’s common stock. The maximum number of shares authorized under the 2021 Plan will be (i) reduced by any shares subject to awards made under the 2013 Plan after January 1, 2021, and (ii) increased by any shares subject to outstanding awards under the 2013 Plan as of January 1, 2021 that, after January 1, 2021, are canceled, expired, forfeited or otherwise not issued under such awards (other than as a result of being tendered or withheld to pay the exercise price or withholding taxes in connection with any such awards) or settled in cash. As of June 30, 2023, the total number of shares reserved for issuance was 6,186,644 shares, which included 197,596 shares that were forfeited under the 2013 Plan, and 1,977,114 shares have been granted under the 2021 Plan.

In addition, there were 712,454 shares reserved for options and 746,175 shares reserved for restricted stock units issued as inducement grants to new employees granted outside of the Company’s equity-based compensation plans under Rule 5635(c)(4) of the Nasdaq Listing Rules.

The following table presents a summary of awards outstanding:

	As of June 30, 2023				
	2004 Plan	2013 Plan	2021 Plan	Inducement Awards	Total
<b>Granted and outstanding awards:</b>					
Options	68,555	1,550,951	33,838	712,454	2,365
Restricted stock units	—	1,889,125	1,686,766	746,175	4,322
<b>Total</b>	<b>68,555</b>	<b>3,440,076</b>	<b>1,720,604</b>	<b>1,458,629</b>	<b>6,687</b>

The following table summarizes stock-based compensation expenses included in operating expenses:

	Three Months Ended June 30,		Nine Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Research and development		8,982	8,098	26,129
General and administrative		10,965	25,292	33,820
<b>Total</b>	<b>\$</b>	<b>19,947</b>	<b>\$</b>	<b>33,390</b>
			<b>\$</b>	<b>59,949</b>
			<b>\$</b>	<b>9</b>

### Stock Option Awards

The following table presents a summary of the stock option activity for the nine months ended June 30, 2023:

	Shares	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding at September 30, 2022	2,721,384	\$ 20.73		
Granted	32,151	33.03		
Cancelled or expired	(43,936)	62.31		
Exercised	(343,801)	6.48		
Outstanding at June 30, 2023	2,365,798	\$ 22.20	4.4 years	\$ 43,813,599
Exercisable at June 30, 2023	2,188,690	\$ 20.28	4.2 years	\$ 43,480,339

The aggregate intrinsic values represents the amount by which the market price of the underlying stock exceeds the exercise price of the option. The total intrinsic value of the options exercised during the three months ended June 30, 2023 and

2022 was \$6.5 million and \$1.6 million, respectively. The total intrinsic value of the options exercised during the nine months ended June 30, 2023 and 2022 was \$10.1 million and \$24.9 million, respectively.

Stock-based compensation expense related to stock options outstanding for the three months ended June 30, 2023 and 2022, was \$2.1 million and \$2.6 million, respectively. Stock-based compensation expense related to stock options for the nine months ended June 30, 2023 and 2022 was \$6.7 million and \$8.3 million, respectively.

As of June 30, 2023, the pre-tax compensation expense for all outstanding unvested stock options in the amount of \$5.3 million will be recognized in the Company's results of operations over a weighted average period of 12 months.

The fair value of each stock option award is estimated on the date of grant using the Black-Scholes option pricing model. The Black-Scholes option valuation model was developed for use in estimating the fair value of traded options, which do not have vesting restrictions and are fully transferable. The determination of the fair value of each stock option is affected by the Company's stock price on the date of grant, as well as assumptions regarding a number of highly complex and subjective variables. Because the Company's employee stock options have characteristics significantly different from those of traded options, and because changes in the subjective input assumptions can materially affect the fair value estimate, the existing models do not necessarily provide a reliable single measure of the fair value of its employee stock options.

The following table provides the assumptions used in the calculation of grant-date fair values of these stock options based on the Black-Scholes option pricing model:

	Nine Months Ended June 30,	
	2023	2022 <sup>(5)</sup>
Expected dividend yield <sup>(1)</sup>	—	N/A
Risk-free interest rate <sup>(2)</sup>	3.69 %	N/A
Expected volatility <sup>(3)</sup>	86.4 %	N/A
Expected term (in years) <sup>(4)</sup>	6.25	N/A
Weighted average grant date fair value per share of options granted	\$ 24.80	N/A

(1) The dividend yield is zero as the Company currently does not pay a dividend.

(2) The risk-free interest rate is based on that of the U.S. Treasury yields with equivalent terms in effect at the time of the grant.

(3) Volatility is estimated based on volatility average of the Company's common stock price.

(4) The expected term represents the period of time that stock options granted are expected to be outstanding, by using historical exercise patterns and post-vesting termination behavior.

(5) No options were granted during the nine months ended June 30, 2022.

#### Restricted Stock Units

Restricted stock units ("RSUs"), including market-based, time-based and performance-based awards, have been granted under the Company's 2013 and 2021 Plans and as inducements grants granted outside of the Company's equity-based compensation plans. At vesting, each outstanding RSU will be exchanged for one share of the Company's common stock. RSU awards generally vest subject to the satisfaction of service requirements or the satisfaction of both service requirements and achievement of certain performance targets.

The following table summarizes the activity of the Company's RSUs:

	Number of RSUs	Weighted- Average Grant Date Fair Value Per Share
Outstanding at September 30, 2022	4,069,431	\$ 62.96
Granted	1,144,594	34.27
Vested	(798,271)	53.72
Forfeited	(93,688)	54.58
Outstanding at June 30, 2023	4,322,066	\$ 57.45

The fair value of RSUs was determined based on the closing price of the Company's common stock on the grant date, with consideration given to the probability of achieving service and/or performance conditions for awards.

For the three months ended June 30, 2023 and 2022, the Company recorded \$17.8 million and \$33.7 million of expense

related to RSUs, respectively. For the nine months ended June 30, 2023 and 2022, the Company recorded \$53.2 million and \$83.4 million of expense related to RSUs, respectively. As of June 30, 2023, there was \$131.6 million of total unrecognized compensation cost related to RSUs that is expected to be recognized over a weighted-average period of 2.3 years.

## NOTE 10. FAIR VALUE MEASUREMENTS

The Company employs a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The fair value of a financial instrument is the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date using the exit price. Accordingly, when market observable data are not readily available, the Company's own assumptions are used to reflect those that market participants would be presumed to use in pricing the asset or liability at the measurement date.

Assets and liabilities recorded at fair value on the consolidated balance sheets are categorized based on the level of judgment associated with inputs used to measure their fair values and the level of market price observability, as follows:

Level 1 Unadjusted quoted prices are available in active markets for identical assets or liabilities as of the reporting date.

Level 2 Pricing inputs are other than quoted prices in active markets, which are based on the following:

- Quoted prices for similar assets or liabilities in active markets;
- Quoted prices for identical or similar assets or liabilities in non-active markets; or
- Either directly or indirectly observable inputs as of the reporting date.

Level 3 Pricing inputs are unobservable and significant to the overall fair value measurement, and the determination of fair value requires significant management judgment or estimation.

In certain cases, inputs used to measure fair value may fall into different levels of the fair value hierarchy. In such cases, the level in the fair value hierarchy within which the fair value measurement in its entirety falls has been determined based on the lowest level input that is significant to the fair value measurement in its entirety. Thus, a Level 3 fair value measurement may include inputs that are observable (Level 1 or Level 2) and unobservable (Level 3). The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and consideration of factors specific to the asset or liability.

The Company uses prices and inputs that are current as of the measurement date, including during periods of market disruption. In periods of market disruption, the ability to observe prices and inputs may be reduced for many instruments. This condition could cause an instrument to be reclassified from Level 1 to Level 2, or from Level 2 to Level 3. The Company recognizes transfers between levels at either the actual date of the event or a change in circumstances that caused the transfer. At June 30, 2023 and September 30, 2022, the Company did not have any financial assets or financial liabilities based on Level 3 measurements.

The following table presents information about the Company's assets and liabilities measured at fair value on a recurring basis, and indicates the fair value hierarchy of the valuation techniques utilized by the Company:

	June 30, 2023			
	Level 1	Level 2	Level 3	Total
	(in thousands)			
<b>Financial assets:</b>				
U.S. government bonds	\$ 27,092	\$ —	\$ —	\$ 27,092
Municipal securities	—	7,033	—	7,033
Commercial notes	—	62,755	—	62,755
Corporate debt securities	—	287,749	—	287,749
Certificate of deposits	—	—	—	—
Money market instruments	49,199	—	—	49,199

September 30, 2022

	Level 1	Level 2	Level 3	Total
	(in thousands)			
U.S. government bonds	\$ 1,973	\$ —	\$ —	\$ 1,973
Commercial notes	—	41,727	—	41,727
Corporate debt securities	—	271,333	—	271,333
Certificate of deposits	50,000	—	—	50,000
Money market instruments	39,262	—	—	39,262

#### NOTE 11. LIABILITY RELATED TO THE SALE OF FUTURE ROYALTIES

On November 9, 2022, the Company and Royalty Pharma entered into the Royalty Pharma Agreement, pursuant to which Royalty Pharma agreed to pay up to \$410.0 million in cash to the Company in consideration for the Company's future royalty interest in Olpasiran, a small interfering RNA (siRNA) originally developed by the Company and licensed to Amgen in 2016 under the Olpasiran Agreement.

Pursuant to the Royalty Pharma Agreement, Royalty Pharma paid \$250.0 million upfront and agreed to pay up to an additional \$160.0 million in aggregate one-time milestone payments due if and when the following milestone events occur: (i) \$50.0 million on completion of enrollment in the OCEAN Phase 3 clinical trial for Olpasiran, (ii) \$50.0 million upon receipt of FDA approval of Olpasiran for an approved indication (reduction in the risk of myocardial infarction, urgent coronary revascularization, or coronary heart disease death in adults with established cardiovascular disease and elevated Lp(a)), and (iii) \$60.0 million upon Royalty Pharma's receipt of at least \$70.0 million of royalty payments under the Royalty Pharma Agreement in any single calendar year.

In consideration for the payment of the foregoing amounts under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive any milestone payments potentially payable by Amgen under the Olpasiran Agreement.

The Company has evaluated the terms of the Royalty Pharma Agreement and concluded in accordance with the relevant accounting guidance that the Company accounted for the transaction as debt and the funding of \$250.0 million from Royalty Pharma was recorded as a liability related to the sale of future royalties on its consolidated balance sheets. The Company is not obligated to repay this upfront funding received under the Royalty Pharma Agreement. This liability is amortized over the expected repayment term using an effective interest rate method. The effective interest rate is calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement. The interest rate may vary during the term of the agreement depending on a number of factors, including the amount and timing of forecasted net revenues which affects the repayment timing and ultimate amount of repayment. The Company will evaluate the effective interest rate periodically based on its current revenue forecasts utilizing the prospective method. For the three and nine months ended June 30, 2023, the Company recognized non-cash interest expense of \$5.2 million and \$13.1 million, respectively, on the consolidated statements of operations and comprehensive loss.

#### NOTE 12. EARNINGS PER SHARE

The following table presents the computation of basic and diluted earnings per share for the nine months ended

June 30, 2023 and 2022.

	Three Months Ended June 30,		Nine Months Ended June 30,	
	2023	2022	2023	2022
(in thousands, except per share amounts)				
<b>Numerator:</b>				
Net loss	\$ (102,946)	\$ (72,046)	\$ (95,596)	\$ (90,552)
<b>Denominator:</b>				
Weighted-average basic shares outstanding	107,004	105,753	106,597	105,273
Effect of dilutive securities	—	—	—	—
Weighted-average diluted shares outstanding	107,004	105,753	106,597	105,273
Basic earnings per share	\$ (0.96)	\$ (0.68)	\$ (0.90)	\$ (0.86)
Diluted earnings per share	\$ (0.96)	\$ (0.68)	\$ (0.90)	\$ (0.86)

Potentially dilutive securities representing approximately 3,467,000 and 4,024,000 shares of common stock were excluded from the computation of diluted earnings per share for the three and nine months ended June 30, 2023, respectively, because their effect would have been anti-dilutive.

## ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

### FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and we intend that such forward-looking statements be subject to the safe harbors created thereby. For this purpose, any statements contained in this Quarterly Report on Form 10-Q except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as “may,” “will,” “expect,” “believe,” “anticipate,” “intend,” “plan,” “project,” “could,” “estimate,” “target,” “forecast” or “continue” or the negative of these words or other variations thereof or comparable terminology are intended to identify forward-looking statements. In addition, any statements that refer to projections of the Company's future financial performance, trends in its business, or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of the Company's preclinical studies and clinical trials, and its research and development programs; its expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions the Company has entered into or may enter into in the future; its beliefs and expectations regarding the amount and timing of future milestone, royalty or other payments that could be due to or from third parties under existing agreements; and its estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties.

The forward-looking statements included herein are based on current expectations of the Company's management based on available information and involve a number of risks and uncertainties, all of which are difficult or impossible to predict accurately, and many of which are beyond the Company's control. As such, the Company's actual results and timing of certain events may differ materially from the results discussed, projected, anticipated or indicated in any forward-looking statements. Forward-looking statements are not guarantees of future performance and the Company's actual results of operations, financial condition and cash flows may differ materially. Factors that may cause or contribute to such differences include, but are not limited to, those discussed in more detail in “Item 1. Business” and “Item 1A. Risk Factors” of Part I and “Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations” of Part II of the Company's most recent Annual Report on Form 10-K. Readers should carefully review these risks, as well as the additional risks described in other documents the Company files from time to time with the Securities and Exchange Commission (the “SEC”). In light of the significant risks and uncertainties inherent in the forward-looking information included herein, the inclusion of such information should not be regarded as a representation by the Company or any other person that such results will be achieved, and readers are cautioned not to place undue reliance on such forward-looking information. Statements made herein are as of the date of the filing of this Quarterly Report on Form 10-Q with the SEC and should not be relied upon as of any subsequent date. Except as may be required by law, the Company disclaims any intent to revise the forward-looking statements contained herein to reflect events or circumstances after the date hereof or to reflect the occurrence of unanticipated events.

### OVERVIEW

The Company develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, the Company's therapies trigger the RNAi mechanism to induce rapid, deep and durable knockdown of target genes. RNAi is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. RNAi-based therapeutics may leverage this natural pathway of gene silencing to target and shut down specific disease-causing genes.

The Company has focused its resources on therapeutics that exclusively utilize its high levels of pharmacologic activity in multiple animal models spanning several therapeutic areas. The Company believes that TRiM™ enabled therapeutics offer several potential advantages over prior generation and competing technologies, including: simplified manufacturing and reduced costs; multiple routes of administration including subcutaneous injection and inhaled administration; the ability to target multiple tissue types including liver, lung, muscle, CNS and others; and the potential for improved safety and reduced risk of intracellular buildup, because there are fewer metabolites from smaller, simpler molecules.

The Company's pipeline includes:

- Hypertriglyceridemia - ARO-APOC3
- Dyslipidemia - ARO-ANG3
- Cardiovascular disease - Olpasiran (formerly AMG 890 or ARO-LPA, out-licensed to Amgen)

- Cystic fibrosis - ARO-ENAC2
- Mucob obstructive or inflammatory pulmonary conditions - ARO-MUC5AC and ARO-RAGE
- Idiopathic pulmonary fibrosis - ARO-MMP7
- Non-alcoholic steatohepatitis (NASH) - GSK-4532990 (formerly ARO-HSD, out-licensed to GSK)
- Alpha-1 antitrypsin deficiency (AATD) - Fazirsiran (formerly ARO-AAT, a collaboration with Takeda)
- Chronic hepatitis B virus - JNJ-3989 (formerly ARO-HBV, out-licensed to Janssen)
- Uncontrolled gout - HZN-457 (formerly ARO-XDH, out-licensed to Horizon)
- Complement mediated diseases - ARO-C3
- Non-alcoholic steatohepatitis (NASH) - ARO-PNPLA3 (formerly JNJ-75220795 or ARO-JNJ1)
- Facioscapulohumeral muscular dystrophy - ARO-DUX4
- Amyotrophic lateral sclerosis "ALS" (CNS) - ARO-SOD1

The Company operates lab facilities in San Diego, California and Madison, Wisconsin, where its research and development activities, including the development of RNAi therapeutics, take place. The Company's principal executive offices are located in Pasadena, California.

The Company continues to develop other clinical candidates for future clinical trials. Clinical candidates are tested internally and through GLP toxicology studies at outside laboratories. Drug materials for such studies and clinical trials are either manufactured internally or contracted to third-party manufacturers. The Company engages third-party contract research organizations (CROs) to manage clinical trials and works cooperatively with such organizations on all aspects of clinical trial management, including plan design, patient recruiting, and follow up. These outside costs, relating to the preparation for and administration of clinical trials, are referred to as "candidate costs." As clinical candidates progress through clinical development, candidate costs will increase.

### ***The First Three Quarters of Fiscal 2023 Business Highlights***

Key recent developments during the first three quarters of fiscal 2023 included the following:

- hosted a Research & Development (R&D) Day on June 1, 2023 to discuss progress of the Company's pipeline of RNAi Therapeutics, at which the following updates were discussed:
  - ARO-RAGE showed continued dose response with single inhaled dose of 184 mg achieving mean knockdown of 90% and max of 95%;
  - adipose delivery platform achieved single dose target gene silencing of greater than 90% with six months of duration in non-human primates;
  - improved hepatic dimer platform achieved equivalent or better knockdown of two target genes with longer duration than monomer mixture in non-human primates;
  - TRiM™ platform now has potential to address multiple cell types including liver, solid tumors, lung, central nervous system, skeletal muscle, and adipose;
  - announced progress towards the Company's "20 in 25" goal to grow its pipeline of RNAi therapeutics that leverage the proprietary Targeted RNAi Molecule (TRiM™) platform to a total of 20 clinical stage or marketed products in the year 2025;
- presented updated data from the Phase 2 SEQUOIA study of investigational RNAi therapy Fazirsiran in patients with alpha-1 antitrypsin deficiency liver disease which included:
  - Fazirsiran reduced serum Z-AAT concentration in a dose-dependent manner;
  - Fazirsiran significantly reduced liver Z-AAT;
  - Fazirsiran consistently reduced hepatic globule burden;
  - Fazirsiran treatment reduced histological signs of hepatic inflammation;
  - 50% of the pooled Fazirsiran treated patients showed at least a one-point improvement in METAVIR liver fibrosis versus 38% in the placebo group;
  - Fazirsiran has been well tolerated to date;
  - pulmonary function test results (FEV1 and DLCO) for both Fazirsiran and placebo were stable over time with no apparent dose-dependent effects;
  - updated Phase 2 clinical data were presented at the European Association for the Study of the Liver

(EASL) Congress 2023 in an oral presentation titled, “Fazirsiran reduces liver Z-alpha-1 antitrypsin synthesis, decreases globule burden and improves histological measures of liver disease in adults with alpha-1 antitrypsin deficiency: a randomized placebo-controlled phase 2 study”;

- presented interim data from the ongoing Phase 2 GATEWAY clinical study of ARO-ANG3 which included:
  - mean reduction in LDL-C of 48.1% (200mg) and 44.0% (300mg);
  - ANPTL3 inhibition with ARO-ANG3 also reduced HDL-C, non-HDL-C, and triglycerides, consistent with published human genetic data;
  - safety and tolerability;
- completed enrollment of the Phase 3 PALISADE clinical trial evaluating ARO-APOC3 for treatment of familial chylomicronemia syndrome;
- secured stockholder approval to increase authorized common shares to 290,000,000 from 145,000,000 to provide the Company with additional flexibility to issue common stock for a variety of general corporate purposes;
- announced interim results from ARO-RAGE administration in Part 1 of the ongoing Phase 1/2 study in normal healthy volunteers which included:
  - reductions in soluble RAGE (sRAGE) as measured in serum after two doses on Day 1 and Day 29;
  - duration of pharmacologic effect persisted for at least 6 weeks after the second administration of the 92 mg does with further follow up ongoing;
  - reduction in sRAGE as measured in bronchoalveolar lavage fluid (BALF) at Day 31 after a single dose;
  - reduction in in serum sRAGE was observed after a single dose;
  - the pooled placebo groups experienced a mean sRAGE increase of 8% in BALF and a mean decrease of 1% serum;
  - safety and tolerability;
- expanded TRiM™ platform to include an optimized intrathecal administration for CNS delivery with distribution throughout the brain and in all relevant brain cell types. The first development candidate to utilize this new delivery platform is ARO-SOD1. In June 2023, the Company filed a clinical trial application (CTA) for approval to initiate a Phase 1 clinical study. In preclinical studies, ARO-SOD1 achieved 95% spinal cord tissue mRNA knockdown after a single intrathecal dose in human SOD1 transgenic rats and maintained greater than 80% spinal cord tissue mRNA knockdown three months after a single intrathecal dose in non-human primates;
- dosed the first patient in Takeda’s Phase 3 REDWOOD clinical study of Fazirsiran for the treatment of alpha-1 antitrypsin deficiency associated liver diseases, triggering a \$40.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023;
- dosed the first patient in GSK’s Phase 2b trial of GSK4532990, formerly called ARO-HSD, an investigational RNAi therapeutic for the treatment of patients with non-alcoholic steatohepatitis (NASH), triggering a \$30.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023;
- announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to ARO-APOC3 for reducing triglycerides in adult patients with familial chylomicronemia syndrome (FCS). ARO-APOC3 was previously granted Orphan Drug designation by the FDA and the European Union;
- announced interim results from Part 1 of AROC3-1001, an ongoing Phase 1/2 clinical study of ARO-C3, which included:
  - a dose-dependent reduction in serum C3, with 88% mean reduction at highest dose tested;
  - a dose-dependent reduction in AH50, a marker of alternative complement pathway hemolytic activity, with 91% mean reduction at highest dose tested;
  - duration of pharmacologic effect supportive of quarterly or less frequent subcutaneous dose administration;
  - safety and tolerability;
- received notice from Janssen of its decision to voluntarily terminate the Janssen Collaboration Agreement

between the Company and Janssen. The Company regained full rights to ARO-PNPLA3, formerly called JNJ-75220795, upon termination of the Janssen Collaboration Agreement which took effect on April 7, 2023. ARO-PNPLA3 is in Phase 1 clinical trials that are now being developed by the Company;

- initiated dosing in ARO-MMP7-1001 (NCT05537025), a Phase 1/2a single ascending dose and multiple ascending dose clinical study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-MMP7, an investigational RNAi therapeutic designed to reduce expression of matrix metalloproteinase 7 (MMP7) as a potential treatment for idiopathic pulmonary fibrosis (IPF), in up to 56 healthy volunteers and in up to 21 patients with IPF;
- enrolled the first subject in a Phase 1 randomized, placebo-controlled trial to assess the safety tolerability, pharmacokinetics and pharmacodynamics of a development-stage medicine, HZN-457 (previously known as ARO-XDH), which is out-licensed to Horizon, triggering a \$15.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023;
- enrolled the first subject in Amgen's Phase 3 trial of Olpasiran, which triggered a \$25.0 million milestone payment to the Company, which was paid in the second quarter of fiscal 2023;
- entered into the Royalty Pharma Agreement on November 9, 2022, pursuant to which Royalty Pharma paid \$250.0 million upfront (See Note 11 — Liability Related to the Sale of Future Royalties of Notes to Consolidated Financial Statements of Part I, "Item 1. Financial Statements.");
- announced top line results from the SEQUOIA Phase 2 Study of Fazirsiran in patients with Alpha-1 Antitrypsin Deficiency-Associated Liver Disease in which;
  - fibrosis regression was observed in 50% of patients receiving Fazirsiran;
  - median reductions of 94% of Z-AAT accumulation in the liver and mean reductions of 68% in histologic globule burden were observed;
  - treatment emergent adverse events were generally well balanced between Fazirsiran and placebo groups;
  - results were consistent with AROAAT-2002 open-label study previously published in The New England Journal of Medicine.

Net loss was \$102.9 million for the three months ended June 30, 2023 as compared to \$72.0 million for the three months ended June 30, 2022. Net loss was \$95.6 million for the nine months ended June 30, 2023 as compared to \$90.6 million for the nine months ended June 30, 2022. Net loss per share – diluted was \$0.96 for the three months ended June 30, 2023 as compared to \$0.68 for the three months ended June 30, 2022. Net loss per share – diluted was \$0.90 for the nine months ended June 30, 2023 as compared to \$0.86 for the nine months ended June 30, 2022.

The changes in net loss for the three and nine months ended June 30, 2023 reflect an increase in research and development expenses, which have continued to increase as the Company's pipeline of candidates has expanded and progressed through clinical trial phases.

The Company had \$105.3 million of cash, cash equivalents and restricted cash, \$346.4 million in short-term investments, \$42.8 million of long-term investments and \$795.9 million of total assets as of June 30, 2023, as compared to \$108.0 million of cash, cash equivalents and restricted cash, \$268.4 million in short-term investments, \$105.9 million of long-term investments and \$691.9 million of total assets as of September 30, 2022. Based upon the Company's current cash and investment resources and operating plan, the Company expects to have sufficient liquidity to fund operations for at least the next twelve months.

### ***Critical Accounting Estimates***

There have been no significant changes to the Company's critical accounting estimates disclosed in the most recent Annual Report on Form 10-K for the fiscal year ended September 30, 2022, except the Takeda revenue recognition described in Note 2 — Collaboration and License Agreements of Notes to Consolidated Financial Statements of Part I, "Item 1. Financial Statements."

## RESULTS OF OPERATIONS

The following data summarizes the Company's results of operations for the following periods indicated:

	Three Months Ended June 30,		Nine Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands, except per share amounts)			
Revenues	\$ 15,825	\$ 32,412	\$ 224,638	\$ 211,656
Operating loss	\$ (102,703)	\$ (72,909)	\$ (96,672)	\$ (94,677)
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	\$ (102,946)	\$ (72,046)	\$ (95,596)	\$ (90,552)
Net loss per share-diluted	\$ (0.96)	\$ (0.68)	\$ (0.90)	\$ (0.86)

### **Revenue**

Total revenue for the three months ended June 30, 2023 decreased to \$15.8 million, or 51.2% from the same period of 2022. Total revenue for the nine months ended June 30, 2023 increased to \$224.6 million, or 6.1% from the same period of 2022. The changes were primarily driven by the revenue recognition associated with GSK, Horizon, Takeda and Amgen license agreements, as discussed below. The Company has evaluated each agreement in accordance with FASB Topic 808—*Collaborative Arrangements* and Topic 606—*Revenue from Contracts from Customers*. See Note 2 — *Collaboration and License Agreements* to Consolidated Financial Statements of Part I, "Item 1. Financial Statements" for more information on revenue recognized under the collaboration and license agreements.

### **GSK**

At the inception of the GSK License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services, including the Company's responsibility to complete the Phase 1/2 study (the "GSK R&D Services"). Due to the specialized and unique nature of the GSK R&D Services and their direct relationship with the license, the Company determined that these deliverables represented one distinct bundle and, thus, one performance obligation. Beyond the GSK R&D Services, which are the responsibility of the Company, GSK will be responsible for managing future clinical development and commercialization in its territory.

The Company determined the initial transaction price totaled \$120.0 million, including the upfront payment, which was collected in January 2022 (see Note 2 — *Collaboration and License Agreements* to Consolidated Financial Statements of Part I, "Item 1. Financial Statements" for more information on revenue recognized under the GSK License Agreement). The Company has excluded any future estimated milestones or royalties from this transaction price to date. The Company has allocated the total \$120.0 million initial transaction price to its one distinct performance obligation for the GSK-4532990 license and the associated GSK R&D Services. As the Company has completed its performance obligation related to this agreement, the upfront payment of \$120.0 million was fully recognized during the six months ended March 31, 2022. Further, GSK dosed the first patient in a Phase 2b trial in March 2023, triggering a \$30.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023.

### **Horizon**

On June 18, 2021, Horizon and the Company entered into the Horizon License Agreement. At the inception of the Horizon License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services, including the Company's responsibilities to conduct all activities through the preclinical stages of development of HZN-457 (the "Horizon R&D Services"). Due to the specialized and unique nature of these Horizon R&D Services and their direct relationship with the license, the Company determined that these deliverables represented one distinct bundle and, thus, one performance obligation. Beyond the Horizon R&D Services, which are the responsibility of the Company, Horizon is responsible for managing future clinical development and commercialization of HZN-457.

The Company determined the initial transaction price totaled \$40.0 million, including the upfront payment (see Note 2 — *Collaboration and License Agreements* to Consolidated Financial Statements of Part I, "Item 1. Financial Statements" for more information on revenue recognized under the Horizon License Agreement). The Company has excluded any future estimated milestones or royalties from this transaction price to date. The Company allocated the total \$40.0 million initial transaction price to its one distinct performance obligation for the HZN-457 license and the associated Horizon R&D Services. Revenue was recognized on a straight-line basis over the timeframe for completing the Horizon R&D Services. The Company determined that the straight-line basis was appropriate as its efforts were expended evenly over the course of completing its performance obligation. Further, Horizon enrolled the first subject in December 2022 in a Phase 1

randomized, placebo-controlled trial to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of HZN-457, triggering a \$15.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023.

#### **Takeda**

On October 7, 2020, Takeda and the Company entered into the Takeda License Agreement. At the inception of the Takeda License Agreement, the Company identified one distinct performance obligation. The Company determined that the key deliverables included the license and certain R&D services including the Company's responsibilities to complete the initial portion of the SEQUOIA study, to complete the ongoing Phase 2 AROAAT2002 study and to ensure certain manufacturing of Fazirsiran drug product is completed and delivered to Takeda (the "Takeda R&D Services"). Due to the specialized and unique nature of these Takeda R&D Services and their direct relationship with the license, the Company determined that these deliverables represent one distinct bundle and, thus, one performance obligation. Beyond the Takeda R&D Services, which are the responsibility of the Company, Takeda will be responsible for managing future clinical development and commercialization outside the United States. Within the United States, the Company will also participate in co-development and co-commercialization efforts and will co-fund these efforts with Takeda as part of the 50/50 profit sharing structure within the United States. The Company considers the collaborative activities, including the co-development and co-commercialization, to be a separate unit of account within Topic 808, and as such, these co-funding amounts are recorded as research and development expenses or general and administrative expenses, as appropriate.

The Company has allocated the total \$300.0 million initial transaction price to its one distinct performance obligation for the Fazirsiran license and the associated Takeda R&D Services. Revenue is recognized using a proportional performance method (based on actual patient visits completed versus total estimated visits for the ongoing SEQUOIA and AROAAT2002 clinical studies). See Note 2 — Collaboration and License Agreements to Consolidated Financial Statements of Part I, "Item 1. Financial Statements" for more information on revenue recognized under the Takeda License Agreement. The Company previously expected these clinical trials to extend to September 2025 in order to demonstrate long term safety and efficacy in the open label extension (OLE) part of the studies; however, Takeda now intends to initiate a new OLE study available to patients participating in these Phase 2 studies that initiated in July 2023. Based on this new information, patients enrolled in the SEQUOIA and AROAAT2002 studies are expected to complete their Phase 2 study visits between June 2023 and June 2024, shortening the Company's performance obligation. As a result, effective the second quarter of fiscal 2023, the Company changed its estimates of the revenue recognition to better reflect these newly estimated proportional performance periods. The effect of these changes in estimates resulted in accelerated revenue by \$61.4 million, or \$0.58 per share (diluted) for each of the three and nine months ended June 30, 2023. There were \$16.9 million of contract liabilities recorded as deferred revenue, which was classified as current as of June 30, 2023.

In March 2023, Takeda dosed the first patient in the Phase 3 REDWOOD clinical study of Fazirsiran, triggering a \$40.0 million milestone payment to the Company which was paid in the third quarter of fiscal 2023.

#### **Amgen Inc. ("Amgen")**

On September 28, 2016, Amgen and the Company entered into two collaboration and license agreements and a common stock purchase agreement. Under the Olpasiran Agreement, Amgen received a worldwide, exclusive license to the Company's novel RNAi Olpasiran program. Olpasiran is designed to reduce elevated lipoprotein(a), which is a genetically validated, independent risk factor for atherosclerotic cardiovascular disease. Amgen is wholly responsible for clinical development and commercialization. The Company has substantially completed its performance obligations under the Olpasiran Agreement.

Further, in November 2022, Royalty Pharma and the Company entered into the Royalty Pharma Agreement. In consideration for the payments under the Royalty Pharma Agreement, Royalty Pharma is entitled to receive all royalties otherwise payable by Amgen to the Company under the Olpasiran Agreement. The Company remains eligible to receive any milestone payments potentially payable by Amgen under the Olpasiran Agreement.

In December 2022, Amgen enrolled the first subject in its Phase 3 trial of Olpasiran, which triggered a \$25.0 million milestone payment to the Company which was paid in the second quarter of fiscal 2023. The Company is further eligible to receive up to an additional \$535.0 million in aggregate development, regulatory, and sales milestone payments from Amgen and Royalty Pharma.

## Operating Expenses

The analysis below details the operating expenses and discusses the expenditures of the Company within the major expense categories. For purposes of comparison, the amounts for the three and nine months ended June 30, 2023 and 2022 are shown in the tables below.

### Research and Development (R&D) Expenses

R&D expenses are related to the Company's research and development discovery efforts and related candidate costs, which are comprised primarily of outsourced costs related to the manufacturing of clinical supplies, toxicity/efficacy studies and clinical trial expenses. Internal costs primarily relate to discovery operations at the Company's research facilities in San Diego, California and Madison, Wisconsin, including facility costs and laboratory-related expenses. The Company does not separately track R&D expenses by individual research and development projects, or by individual drug candidates. The Company operates in a cross-functional manner across projects and does not separately allocate facilities-related costs, candidate costs, discovery costs, compensation expenses, depreciation and amortization expenses, and other expenses related to research and development activities.

The following table provides details of research and development expenses for the periods indicated:

(in thousands)	Three Months Ended	% of	Three Months Ended	% of	Increase (Decrease)	
	June 30, 2023	Expense Category	June 30, 2022	Expense Category	\$	%
Candidate costs	\$ 41,209	44 %	\$ 31,732	44 %	\$ 9,477	30 %
R&D discovery costs	20,253	21 %	15,081	21 %	5,172	34 %
Salaries	16,632	18 %	11,243	16 %	5,389	48 %
Facilities related	4,810	5 %	3,827	5 %	983	26 %
Total research and development expense, excluding non-cash expense	\$ 82,904	88 %	\$ 61,883	86 %	\$ 21,021	34 %
Stock compensation	8,982	9 %	8,098	11 %	884	11 %
Depreciation and amortization	2,871	3 %	2,200	3 %	671	31 %
Total research and development expense	\$ 94,757	100 %	\$ 72,181	100 %	\$ 22,576	31 %

(in thousands)	Nine Months Ended	% of	Nine Months Ended	% of	Increase (Decrease)	
	June 30, 2023	Expense Category	June 30, 2022	Expense Category	\$	%
Candidate costs	\$ 110,079	43 %	\$ 101,789	47 %	\$ 8,290	8 %
R&D discovery costs	50,377	20 %	40,347	19 %	10,030	25 %
Salaries	47,725	19 %	33,641	16 %	14,084	42 %
Facilities related	11,601	5 %	7,644	4 %	3,957	52 %
Total research and development expense, excluding non-cash expense	\$ 219,782	87 %	\$ 183,421	86 %	\$ 36,361	20 %
Stock compensation	26,129	10 %	23,958	11 %	2,171	9 %
Depreciation and amortization	7,422	3 %	6,551	3 %	871	13 %
Total research and development expense	\$ 253,333	100 %	\$ 213,930	100 %	\$ 39,403	18 %

Candidate costs increased \$9.5 million, or 30%, for the three months ended June 30, 2023 and \$8.3 million, or 8%, for the nine months ended June 30, 2023 compared to the same period of 2022. This increase was primarily due to the additional progression of the Company's pipeline of candidates into and through clinical trials, which resulted in higher outsourced clinical trial, toxicity study and manufacturing costs.

R&D discovery costs increased \$5.2 million, or 34%, for the three months ended June 30, 2023 and \$10.0 million, or 25%, for the nine months ended June 30, 2023 compared to the same period of 2022. This increase was due to the growth of the Company's discovery efforts and continued advancement into novel therapeutic areas and tissue types.

Salaries and stock compensation expense consist of salary, bonuses, payroll taxes, related benefits and stock compensation for the Company's R&D personnel. The increases in salaries and stock comp expenses for the nine months ended June 30, 2023 were primarily due to an increase in R&D headcount that has occurred as the Company has expanded its pipeline of candidates, in addition to annual salary increases. Stock compensation expense was based upon the valuation of

stock options and restricted stock units granted to employees and directors.

Facilities-related expense included lease costs for the Company's research and development facilities in San Diego, California and Madison, Wisconsin. Facilities-related costs increased \$1.0 million, or 26%, for the three months ended June 30, 2023 and \$4.0 million, or 52%, for the nine months ended June 30, 2023 compared to the same period of 2022. This increase was mainly due to the additional lease expense as the Company expands discovery efforts to identify new drug candidates.

Depreciation and amortization expense, a non-cash expense, relates to depreciation on lab equipment and leasehold improvements at the facilities.

The Company anticipates these R&D expenses to continue to increase as its pipeline of candidates grows and progresses to later phase clinical trials, in addition to inflationary pressure on goods and services and the labor market.

### General & Administrative Expenses

The following table provides details of the Company's general and administrative expenses for the periods indicated:

(in thousands)	Three Months Ended June 30, 2023	% of Expense Category	Three Months Ended June 30, 2022	% of Expense Category	Increase (Decrease)	
					\$	%
Salaries	\$ 5,063	21 %	\$ 3,175	10 %	\$ 1,888	59 %
Professional, outside services, and other	5,987	25 %	3,568	11 %	2,419	68 %
Facilities related	1,352	6 %	703	2 %	649	92 %
Total general & administrative expense, excluding non-cash expense	\$ 12,402	52 %	\$ 7,446	23 %	\$ 4,956	67 %
Stock compensation	10,965	46 %	25,292	76 %	(14,327)	(57)%
Depreciation and amortization	404	2 %	403	1 %	1	— %
Total general & administrative expense	\$ 23,771	100 %	\$ 33,141	100 %	\$ (9,370)	(28)%

(in thousands)	Nine Months Ended June 30, 2023	% of Expense Category	Nine Months Ended June 30, 2022	% of Expense Category	Increase (Decrease)	
					\$	%
Salaries	\$ 14,275	21 %	\$ 10,365	11 %	\$ 3,910	38 %
Professional, outside services, and other	15,293	22 %	11,004	12 %	4,289	39 %
Facilities related	3,377	5 %	2,085	2 %	1,292	62 %
Total general & administrative expense, excluding non-cash expense	\$ 32,945	48 %	\$ 23,454	25 %	\$ 9,491	40 %
Stock compensation	33,820	50 %	67,739	73 %	(33,919)	(50)%
Depreciation and amortization	1,212	2 %	1,210	2 %	2	— %
Total general & administrative expense	\$ 67,977	100 %	\$ 92,403	100 %	\$ (24,426)	(26)%

Salaries expense increased \$1.9 million, or 59%, for the three months ended June 30, 2023 and \$3.9 million, or 38%, for the nine months ended June 30, 2023 compared to the same period of 2022. The increase was driven by the combination of annual salary increases and increased headcount required to support the Company's growth.

Professional, outside services, and other expense includes legal, consulting, patent expenses, business insurance expenses, other outside services, travel, communication and technology expenses. This expense increased \$2.4 million, or 68%, for the three months ended June 30, 2023 and \$4.3 million, or 39%, for the nine months ended June 30, 2023 compared to the same period of 2022. The increase was mainly due to consulting expenses related to software implementation and administrative expenses in support of additional headcount.

Facilities related expense primarily includes rental costs and other facilities-related costs for the Company's corporate headquarters in Pasadena, California. Depreciation and amortization expense, a noncash expense, was primarily related to amortization of leasehold improvements for the Company's corporate headquarters.

Stock compensation expense, a non-cash expense, decreased by \$14.3 million, or 57%, for the three months ended June 30, 2023 and \$33.9 million, or 50%, for the nine months ended June 30, 2023 compared to the same periods of 2022. The decrease was mainly due to the lower amount of recognized compensation costs and the reversal of recognized compensation costs related to a performance award where the minimum performance goal was not met. The fair value of

market condition-based awards was expensed ratably over the service period and was not adjusted for actual achievement.

Other than with respect to the stock compensation costs described above, the Company anticipates these general and administrative expenses to continue to increase as its pipeline of candidates grows and progresses to later phase clinical trials, in addition to inflationary pressure on goods and services and the labor market.

**Other Income (Loss)**

Other income (loss) is primarily related to interest income and expense. Other income decreased \$1.5 million and \$5.0 million for the three and nine months ended June 30, 2023, respectively, compared to the same periods of 2022. The decrease was primarily due to the interest expense on the liability related to the sale of future royalties, offset by higher yields on investments due to increased interest rates as well as various credits the Company received during the first three quarters of fiscal 2023.

## LIQUIDITY AND CAPITAL RESOURCES

The Company has historically financed its operations through the sale of its equity securities, revenue from its licensing and collaboration agreements, and the sale of certain future royalties. Research and development activities have required significant capital investment since the Company's inception and are expected to continue to require significant cash expenditure as the Company's pipeline continues to expand and matures into later stage clinical trials. Additionally, the Company expanded its facilities in Verona, Wisconsin and commenced the lease agreement for additional facilities in San Diego, California. Each of these expansions is designed to increase the Company's internal manufacturing and discovery capabilities, and each will require significant capital investment.

The Company's cash, cash equivalents and restricted cash decreased to \$105.3 million at June 30, 2023 compared to \$108.0 million at September 30, 2022. Cash invested in short-term fixed income securities was \$346.4 million at June 30, 2023 compared to \$268.4 million at September 30, 2022. Cash invested in long-term fixed income securities was \$42.8 million at June 30, 2023, compared to \$105.9 million at September 30, 2022. On December 2, 2022, the Company entered into the Open Market Sale Agreement, pursuant to which the Company may, from time to time, sell up to \$250.0 million in shares of the Company's common stock through Jefferies LLC, acting as the sales agent and/or principal, in an at-the-market offering. As of June 30, 2023, no shares have been issued under the Open Market Sale Agreement. The Company believes its current financial resources are sufficient to fund its operations through at least the next twelve months.

The following table presents a summary of cash flows:

	Nine Months Ended June 30,	
	2023	2022
	(in thousands)	
<b>Cash Flow from:</b>		
Operating activities	\$ (128,633)	\$ (67,394)
Investing activities	(126,664)	(41,862)
Financing activities	252,901	64,331
Net decrease in cash, cash equivalents and restricted cash	\$ (2,396)	\$ (44,925)
Cash, cash equivalents and restricted cash at end of period	\$ 105,334	\$ 139,439

During the nine months ended June 30, 2023, cash flows used by operating activities was \$128.6 million, which was primarily due to the ongoing expenses related to the Company's research and development programs and general and administrative expenses, partially offset by the receipt of \$110.0 million from collaboration and license agreements (see Note 2 — Collaboration and License Agreements to Consolidated Financial Statements of Part I, "Item 1. Financial Statements"). Cash used in investing activities was \$126.7 million, which was primarily related to capital expenditures, \$112.8 million of construction in progress, and \$234.0 million purchases of investments, offset by maturities of investments of \$220.2 million. Cash provided by financing activities of \$252.9 million was primarily related to the \$250.0 million payment from Royalty Pharma as well as cash received from stock option exercises. See Note 11 — Liability Related to the Sale of Future Royalties of Notes to Consolidated Financial Statements of Part I, "Item 1. Financial Statements."

During the nine months ended June 30, 2022, cash flows used by operating activities was \$67.4 million, which was primarily due to the receipt of the \$120.0 million upfront payment from GSK, offset by the ongoing expenses related to the Company's research and development programs and general and administrative expenses. Cash used in investing activities was \$41.9 million, which was primarily related to the purchase of property and equipment of \$20.1 million and net purchases and maturities of investments of \$21.8 million. Cash provided by financing activities of \$64.3 million was related to the formation of the Company's joint venture, Visirna, as well as cash received from stock option exercises.

On December 20, 2021, the Company completed a purchase of 13 acres of land in the Verona Technology Park in Verona, Wisconsin, which is being developed into an approximately 160,000 square foot drug manufacturing facility and an approximately 140,000 square foot laboratory and office facility which will support the Company's process development and analytical activities. The Company has incurred \$102.7 million and intends to spend an additional \$160.0 million to \$180.0 million to complete the build out of the facilities with cash on hand. As part of this land purchase, the Company entered into a development agreement with the City of Verona to construct certain infrastructure improvements within the tax incremental district and expects to be reimbursed up to \$16.0 million by the City of Verona by future tax increment revenue generated from the developed property. The total amount of funding that City of Verona is expected to pay under the Tax Incremental Financing program is not guaranteed and will depend on future tax revenues generated from the developed property. The Company also expects receive up to \$2.5 million of refundable Wisconsin state income tax

credits from the Wisconsin Economic Development Corporation (WEDC) as incentives to invest in the local community and create new jobs.

### **ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

There has been no material change in the Company's exposure to market risk from that described in Item 7A of its Annual Report on Form 10-K for the year ended September 30, 2022.

### **ITEM 4. CONTROLS AND PROCEDURES**

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

The Company maintains disclosure controls and procedures designed to ensure that information required to be disclosed in its reports filed under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC rules and forms, and that such information is accumulated and communicated to its management, including its Chief Executive Officer and Chief Financial Officer, as appropriate, to allow for timely decisions regarding required disclosure. In designing and evaluating the 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.

As required by Rule 13a-15(b) of the Exchange Act, the Company carried out an evaluation, under the supervision and with the participation of its management, including its Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of the Company's disclosure controls and procedures as of the end of the quarter covered by this Quarterly Report on Form 10-Q. Based on the foregoing, the Company's Chief Executive Officer and Chief Financial Officer concluded that the Company's disclosure controls and procedures were effective at the reasonable assurance level.

#### **Changes in Internal Control Over Financial Reporting**

There has been no change in the Company's internal control over financial reporting during the Company's most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting. The Company regularly evaluates its controls and procedures and makes improvements in the design and effectiveness of established controls and procedures and the remediation of any deficiencies which may be identified during this process.

## PART II—OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

From time to time, the Company may be involved in routine legal proceedings, as well as demands, claims and threatened litigation, which arise in the normal course of its business. Litigation can be expensive and disruptive to normal business operations. Moreover, the results of legal proceedings, particularly complex legal proceedings, cannot be predicted with any certainty. There have been no material developments in the legal proceedings that the Company disclosed in Part I, Item 3 of its Annual Report on Form 10-K for the year ended September 30, 2022.

### ITEM 1A. RISK FACTORS

The Company's business, results of operations and financial conditions are subject to various risks. These risks are described elsewhere in this Quarterly Report on Form 10-Q and in the Company's other filings with the SEC, including the Company's Annual Report on Form 10-K for the year ended September 30, 2022. There have been no material changes from the risk factors identified in the Company's Annual Report on Form 10-K for the year ended September 30, 2022.

### ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

### ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

### ITEM 4. MINE SAFETY DISCLOSURES

Not Applicable.

### ITEM 5. OTHER INFORMATION

#### *(c) Trading Plans*

During the quarter ended June 30, 2023, no director or officer adopted or terminated any Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement.

## ITEM 6. EXHIBITS

Exhibit Number	Document Description
3.1	<a href="#">Amended and Restated Certificate of Incorporation (incorporated by reference from Exhibit 3.3 of the Company's Form 8-K filed on April 6, 2016)</a>
3.2	<a href="#">Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Arrowhead Pharmaceuticals, Inc. (incorporated by reference from Exhibit 3.2 of the Company's Form 10-Q filed on May 2, 2023)</a>
3.3	<a href="#">Second Amended and Restated Bylaws of Arrowhead Pharmaceuticals, Inc., as amended January 24, 2023</a> (incorporated by reference from Exhibit 3.3 of the Company's Form 10-Q filed on May 2, 2023)
31.1*	<a href="#">Certification of Chief Executive Officer pursuant to Rule 13a-14(a) of 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 Chief Financial Officer pursuant to Rule 13a-14(a) of 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 Chief 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 Chief 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
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
104*	The cover page from this Quarterly Report on Form 10-Q, formatted in Inline XBRL (included as Exhibit 101)

\* Filed herewith.

\*\* Furnished herewith.

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Quarterly Report on Form 10-Q to be signed on its behalf by the undersigned, thereunto duly authorized.

Dated: August 7, 2023

ARROWHEAD PHARMACEUTICALS, INC.

By: /s/ Kenneth A. Myszkowski

Kenneth A. Myszkowski

Chief Financial Officer

(Principal Financial Officer and Duly Authorized Officer)

**CERTIFICATION PURSUANT SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Christopher Anzalone, Chief Executive Officer of Arrowhead Pharmaceuticals, Inc., certify that:

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

Date: August 7, 2023

/s/ CHRISTOPHER ANZALONE

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**Christopher Anzalone**  
**Chief Executive Officer**

**CERTIFICATION PURSUANT SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Kenneth A. Myszkowski, Chief Financial Officer of Arrowhead Pharmaceuticals, Inc., certify that:

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

Date: August 7, 2023

/s/ Kenneth A. Myszkowski

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**Kenneth A. Myszkowski,**  
**Chief Financial Officer**

**CERTIFICATION PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Christopher Anzalone, Chief Executive Officer of Arrowhead Pharmaceuticals, Inc. (the "Company"), certify, pursuant to Rule 13(a)-14(b) or Rule 15(d)-14(b) of the Securities Exchange Act of 1934 and 18 U.S.C. Section 1350, that (i) the Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2023, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and (ii) the information contained in such Quarterly Report on Form 10-Q fairly presents in all material respects the financial condition and results of operations of the Company.

Date: August 7, 2023

/s/ CHRISTOPHER ANZALONE

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**Christopher Anzalone**  
**Chief Executive Officer**

A signed original of these written statements required by 18 U.S.C. Section 1350 has been provided to Arrowhead Pharmaceuticals, Inc. and will be retained by Arrowhead Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

**CERTIFICATION PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Kenneth A. Myszkowski, Chief Financial Officer of Arrowhead Pharmaceuticals, Inc. (the "Company"), certify, pursuant to Rule 13(a)-14(b) or Rule 15(d)-14(b) of the Securities Exchange Act of 1934 and 18 U.S.C. Section 1350, that (i) the Quarterly Report on Form 10-Q of the Company for the quarterly period ended June 30, 2023, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and (ii) the information contained in such Quarterly Report on Form 10-Q fairly presents in all material respects the financial condition and results of operations of the Company.

Date: August 7, 2023

/s/ Kenneth A. Myszkowski

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**Kenneth A. Myszkowski**  
**Chief Financial Officer**

A signed original of these written statements required by 18 U.S.C. Section 1350 has been provided to Arrowhead Pharmaceuticals, Inc. and will be retained by Arrowhead Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.