UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

Form 10-Q

(Mark One)

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

For the Quarterly Period Ended September 30, 2026

OR

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

For the transition period from _____ to _____

Commission file number 000-19125

Ionis Pharmaceuticals, Inc.

(Exact name of Registrant as specified in its charter)

Delaware	**33-0336973**
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)
2855 Gazelle Court, Carlsbad, California	**92010**
(Address of Principal Executive Offices)	(Zip Code)

760-931-9200

(Registrant's telephone number, including area code)

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

Title of each class	Trading symbol	Name of each exchange on which registered
Common Stock, $.001 Par Value	"IONS"	The Nasdaq Stock Market LLC

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted and posted 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, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large Accelerated Filer ☒	Accelerated Filer ☐
Non-accelerated Filer ☐	Smaller Reporting Company ☐ Emerging Growth Company ☐

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12(b)-2 of the Securities Exchange Act of 1934). Yes ☐ No ☒

The number of shares of voting common stock outstanding as of November 3, 2026 was 166,545,100.

IONIS PHARMACEUTICALS, INC.
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TRADEMARKS

"Ionis," the Ionis logo, and other trademarks or service marks of Ionis Pharmaceuticals, Inc. appearing in this report are the property of Ionis Pharmaceuticals, Inc. "Akcea," the Akcea logo, and other trademarks or service marks of Akcea Therapeutics, Inc. appearing in this report are the property of Akcea Therapeutics, Inc., Ionis' wholly owned subsidiary. This report contains additional trade names, trademarks and service marks of others, which are the property of their respective owners. Solely for convenience, trademarks and trade names referred to in this report may appear without the ® or TM symbols.

PART I — FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share data)

	September 30, 2026	December 31, 2025
	(unaudited)	
ASSETS		
Current assets:		
Cash and cash equivalents	$ 296,303	$ 372,260
Short-term investments	1,587,604	2,305,176
Contracts receivable	88,340	66,059
Inventories	28,578	10,048
Other current assets	360,676	237,092
Total current assets	2,361,501	2,990,635
Property, plant and equipment, net	162,059	123,048
Right-of-use assets	228,035	238,549
Deposits and other assets	160,995	171,604
Total assets	$ 2,912,590	$ 3,523,836
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 27,113	$ 28,082
Accrued compensation	61,379	116,236
Accrued liabilities	116,177	106,089
Income taxes payable	109	2,713
0 percent convertible senior notes due April 2026, net	-	431,948
Liability related to sale of future royalties, net	138,188	11,689
Current portion of deferred contract revenue	70,203	73,761
Other current liabilities	22,123	11,049
Total current liabilities	435,292	781,567
Long-term deferred contract revenue	48,755	92,001
0 percent convertible senior notes due 2030, net	754,289	751,495
1.75 percent convertible senior notes due 2028, net	569,974	567,830
Liability related to sale of future royalties, net	427,840	551,353
Long-term lease liabilities	262,477	262,383
Long-term obligations	20,323	28,118
Total liabilities	2,518,950	3,034,747
Stockholders' equity:		
Common stock, $0.001 par value; 300,000,000 shares authorized, 166,410,425 and 163,304,875 shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively	166	163
Additional paid-in capital	3,381,632	3,145,402
Accumulated other comprehensive loss	(39,818)	(25,281)
Accumulated deficit	(2,948,340)	(2,631,195)
Total stockholders' equity	393,640	489,089
Total liabilities and stockholders' equity	$ 2,912,590	$ 3,523,836

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Commercial revenue:				
Product sales, net	$ 48,296	$ 32,530	$ 122,390	$ 58,091
Royalty revenue	80,592	75,694	214,874	209,811
Other commercial revenue	7,317	7,374	25,375	26,621
Total commercial revenue	136,205	115,598	362,639	294,523
Research and development revenue:				
Collaborative agreement revenue	102,770	31,436	356,567	413,387
WAINUA joint development revenue	3,746	9,685	37,556	32,471
Total research and development revenue	106,516	41,121	394,123	445,858
Total revenue	242,721	156,719	756,762	740,381
Expenses:				
Cost of sales	3,322	2,338	9,043	7,952
Research, development and patent	215,878	217,754	643,158	635,973
Selling, general and administrative	154,222	96,809	454,977	263,681
Total operating expenses	373,422	316,901	1,107,178	907,606
Loss from operations	(130,701)	(160,182)	(350,416)	(167,225)
Other income (expense):				
Investment income	19,543	23,682	65,257	73,035
Interest expense	(4,628)	(4,212)	(14,405)	(12,434)
Interest expense related to sale of future royalties	(4,129)	(17,952)	(38,972)	(55,422)
Gain on investments, net	9,526	29,908	21,927	9,432
Other income, net	277	363	805	929
Loss before income tax benefit (expense)	(110,112)	(128,393)	(315,804)	(151,685)
Income tax benefit (expense)	141	(213)	(1,341)	(308)
Net loss	$ (109,971)	$ (128,606)	$ (317,145)	$ (151,993)
Basic and diluted net loss per share	$ (0.66)	$ (0.80)	$ (1.92)	$ (0.95)
Shares used in computing basic and diluted net loss per share	166,205	159,765	165,504	159,216

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(in thousands)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Net loss	$ (109,971)	$ (128,606)	$ (317,145)	$ (151,993)
Unrealized gains (losses) on debt securities, net of tax	(5,910)	2,091	(14,338)	4,088
Currency translation adjustment	(11)	(82)	(199)	745
Comprehensive loss	$ (115,892)	$ (126,597)	$ (331,682)	$ (147,160)

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands)
(Unaudited)

Description	Common Stock Shares	Common Stock Amount	Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
Balance at June 30, 2025	159,197	$ 159	$ 2,932,747	$ (27,987)	$ (2,273,195)	$ 631,724
Net loss	-	-	-	-	(128,606)	(128,606)
Change in unrealized gains, net of tax	-	-	-	2,091	-	2,091
Foreign currency translation	-	-	-	(82)	-	(82)
Issuance of common stock in connection with employee stock plans, net	1,941	2	81,257	-	-	81,259
Stock-based compensation expense	-	-	31,581	-	-	31,581
Balance at September 30, 2025	161,138	$ 161	$ 3,045,585	$ (25,978)	$ (2,401,801)	$ 617,967
Balance at June 30, 2026	165,637	$ 166	$ 3,311,837	$ (33,897)	$ (2,838,369)	$ 439,737
Net loss	-	-	-	-	(109,971)	(109,971)
Change in unrealized losses, net of tax	-	-	-	(5,910)	-	(5,910)
Foreign currency translation	-	-	-	(11)	-	(11)
Issuance of common stock in connection with employee stock plans, net	705	-	25,833	-	-	25,833
Stock-based compensation expense	-	-	43,962	-	-	43,962
Issuance of common stock to settle exercised warrants in connection with 0 percent convertible senior notes due 2026	68	-	-	-	-	-
Balance at September 30, 2026	166,410	$ 166	$ 3,381,632	$ (39,818)	$ (2,948,340)	$ 393,640

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands)
(Unaudited)

Description	Common Stock Shares	Common Stock Amount	Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2024	157,909	$ 158	$ 2,868,812	$ (30,811)	$ (2,249,808)	$ 588,351
Net loss	-	-	-	-	(151,993)	(151,993)
Change in unrealized gains, net of tax	-	-	-	4,088	-	4,088
Foreign currency translation	-	-	-	745	-	745
Issuance of common stock in connection with employee stock plans, net	3,229	3	84,784	-	-	84,787
Stock-based compensation expense	-	-	91,989	-	-	91,989
Balance at September 30, 2025	161,138	$ 161	$ 3,045,585	$ (25,978)	$ (2,401,801)	$ 617,967
Balance at December 31, 2025	163,305	$ 163	$ 3,145,402	$ (25,281)	$ (2,631,195)	$ 489,089
Net loss	-	-	-	-	(317,145)	(317,145)
Change in unrealized losses, net of tax	-	-	-	(14,338)	-	(14,338)
Foreign currency translation	-	-	-	(199)	-	(199)
Issuance of common stock in connection with employee stock plans, net	3,870	3	103,390	-	-	103,393
Stock-based compensation expense	-	-	132,850	-	-	132,850
Issuance of common stock to settle conversion obligation of 0 percent convertible senior notes due 2026	1,798	(2)	(13)	-	-	(15)
Retirement of shares received from the exercise of bond hedges related to 0 percent convertible senior notes due 2026	(2,631)	2	3	-	-	5
Issuance of common stock to settle exercised warrants in connection with 0 percent convertible senior notes due 2026	68	-	-	-	-	-
Balance at September 30, 2026	166,410	$ 166	$ 3,381,632	$ (39,818)	$ (2,948,340)	$ 393,640

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2026	**2025**
Operating activities:		
Net loss	$ (317,145)	$ (151,993)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	10,333	6,749
Amortization of right-of-use operating lease assets	10,514	6,803
Amortization of other assets	2,096	1,801
Amortization of discount on investments, net	(12,597)	(18,063)
Amortization of debt issuance costs	6,045	4,940
Non-cash royalty revenue related to sale of royalties	(35,986)	(38,478)
Non-cash interest related to sale of future royalties	38,427	54,964
Stock-based compensation expense	132,294	90,554
Gain on investments, net	(21,929)	(9,437)
Non-cash losses (gains) related to other assets	1,296	(3,643)
Changes in operating assets and liabilities:		
Contracts receivable	(22,281)	67,580
Inventories	(46,622)	(14,600)
Other current and long-term assets	(60,346)	(123,489)
Accounts payable	(2,051)	(29,116)
Income taxes	(2,604)	67
Accrued compensation	(54,857)	(12,595)
Accrued liabilities and other liabilities	15,512	87,683
Deferred contract revenue	(46,804)	(50,601)
Net cash used in operating activities	(406,705)	(130,874)
Investing activities:		
Purchases of short-term investments	(706,278)	(1,126,595)
Proceeds from sale of short-term investments	1,422,166	1,302,751
Purchases of property, plant and equipment	(50,556)	(30,152)
Acquisition of licenses and other assets, net	(5,139)	(4,275)
Net cash provided by investing activities	660,193	141,729
Financing activities:		
Proceeds from issuance of common stock through equity plans, net	103,393	84,787
Repayment of 0 percent convertible senior notes due 2026	(432,511)	-
Principal payments on mortgage debt	(128)	(123)
Net cash provided by (used in) financing activities	(329,246)	84,664
Effects of exchange rates on cash	(199)	745
Net increase (decrease) in cash and cash equivalents	(75,957)	96,264
Cash and cash equivalents at beginning of period	372,260	242,077
Cash and cash equivalents at end of period	$ 296,303	$ 338,341
Supplemental disclosures of cash flow information:		
Interest paid	$ 7,819	$ 5,399
Income taxes paid (refunds received), net	$ 3,794	$ (661)
Supplemental disclosures of non-cash investing and financing activities:		
Right-of-use assets obtained in exchange for lease obligations	$ -	$ 86,939
Amounts accrued for capital and patent expenditures	$ 1,082	$ 478

See accompanying notes.

IONIS PHARMACEUTICALS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
September 30, 2026
(Unaudited)

1. Organization and Basis of Presentation

Organization and Business Activity

We incorporated in California on January 10, 1989. In conjunction with our initial public offering, we reorganized as a Delaware corporation in April 1991. We are a fully integrated commercial-stage biotechnology company and a leader in the discovery and development of RNA-targeted therapeutics.

Basis of Presentation

We prepared the unaudited interim condensed consolidated financial statements for the three and nine months ended September 30, 2026 and 2025 on the same basis as the audited financial statements for the year ended December 31, 2025. We included all normal recurring adjustments in the financial statements, which we considered necessary for a fair presentation of our financial position at such dates and our operating results and cash flows for those periods. Our operating results for the interim periods may not be indicative of what our operating results will be for the entire year. For more complete financial information, these financial statements, and notes thereto, should be read in conjunction with the audited financial statements for the year ended December 31, 2025 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC.

In our condensed consolidated financial statements, we included the accounts of Ionis Pharmaceuticals, Inc. and the consolidated results of its wholly owned subsidiaries (“we”, “us” or “our”).

We operate as a single segment, Ionis operations, because our chief operating decision maker, or CODM, reviews operating results on an aggregate basis and manages our operations as a single operating segment. Refer to Note 13, *Segment Information*, for further details on our segment information.

Use of Estimates

We prepare our condensed consolidated financial statements in conformity with accounting principles generally accepted in the United States, or U.S., that require us to make estimates and assumptions that affect the amounts reported in our condensed consolidated financial statements and accompanying notes. Actual results could differ from our estimates.

2. Significant Accounting Policies

Our significant accounting policies have not changed substantially from those included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Recent Accounting Standards

In July 2025, the FASB issued ASU 2025-05, which amended the guidance in ASC 326 to simplify the estimation of credit losses on accounts receivable and contract assets from revenue transactions. The amended guidance allows companies to elect a practical expedient to assume that conditions as of the balance sheet date will remain unchanged for the remaining life of the asset when estimating the expected credit losses of the asset. This update is effective for annual periods beginning after December 15, 2025 and interim periods within those annual periods. Companies that elect the practical expedient are required to apply the amendments prospectively. We adopted this update in the first quarter of 2026 on a prospective basis and elected the practical expedient. The updated guidance did not have a material impact on our condensed consolidated financial statements.

In September 2025, the FASB issued ASU 2025-06, which amended and simplified the existing guidance for software costs. The amended guidance removes references to software development stages and allows companies to begin capitalizing software costs when management has authorized and committed to funding the software project and it is probable that the project will be completed with the software performing the intended function. This update is effective for annual periods beginning after December 15, 2027 and interim periods within those annual periods. Early adoption of this guidance is permitted at the beginning of an annual reporting period. The guidance may be applied on a prospective or retrospective basis. We early adopted this update in the first quarter of 2026 on a prospective basis. The updated guidance did not have a material impact on our condensed consolidated financial statements.

We do not expect any recently issued accounting standards other than the standards mentioned above and those included in our Annual Report on Form 10-K for the year ended December 31, 2025 to have a material impact to our financial results.

3. Supplemental Financial Data

Inventories

Our inventories consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Raw materials	$ 1,151	$ 1,039
Work in process	66,427	24,051
Finished goods	5,788	1,096
Total	$ 73,366	$ 26,186
Reported as:		
Inventories	$ 28,578	$ 10,048
Deposits and other assets	44,788	16,138
Total	$ 73,366	$ 26,186

Work in process inventory increased as of September 30, 2026 compared to December 31, 2025 primarily due to our purchases of TRYNGOLZA drug substance.

We classify inventories as non-current assets when we expect the inventories to remain on hand beyond one year. We include non-current inventories in deposits and other assets in our condensed consolidated balance sheets. The amounts reported as deposits and other assets as of September 30, 2026 and December 31, 2025 consisted of work in process inventory.

Accrued Liabilities

Our accrued liabilities consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Clinical expenses	$ 53,561	$ 53,659
In-licensing expenses	11,400	8,588
Commercial expenses, including gross-to-net product accruals	33,637	15,556
Other miscellaneous expenses	17,579	28,286
Total accrued liabilities	$ 116,177	$ 106,089

4. Revenues

During the three and nine months ended September 30, 2026 and 2025, our revenues consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Commercial revenue:				
Product sales, net:				
TRYNGOLZA sales, net	$ 9,329	$ 31,792	$ 41,003	$ 57,353
DAWNZERA sales, net	36,773	738	79,193	738
ZANVASTRO sales, net	2,194	-	2,194	-
Total product sales, net	48,296	32,530	122,390	58,091
Royalty revenue:				
SPINRAZA royalties	69,393	55,896	166,589	158,243
WAINUA royalties	6,847	13,320	33,993	33,107
Other royalties	4,352	6,478	14,292	18,461
Total royalty revenue	80,592	75,694	214,874	209,811
Other commercial revenue	7,317	7,374	25,375	26,621
Total commercial revenue	136,205	115,598	362,639	294,523
Research and development revenue:				
Collaborative agreement revenue	102,770	31,436	356,567	413,387
WAINUA joint development revenue	3,746	9,685	37,556	32,471
Total research and development revenue	106,516	41,121	394,123	445,858
Total revenue	$ 242,721	$ 156,719	$ 756,762	$ 740,381

Revenue Sources

The following are sources of revenue and when we typically recognize revenue.

Commercial Revenue

In June 2026, the U.S. Food and Drug Administration, or FDA, approved TRYNGOLZA for the treatment of sHTG. Following the approval, we launched TRYNGOLZA for the treatment of sHTG and began earning revenue from TRYNGOLZA sales for the sHTG indication. TRYNGOLZA is also approved in the U.S. for the treatment of familial chylomicronemia syndrome, or FCS. In April 2026, we lowered the Wholesale Acquisition Cost, or WAC, of TRYNGOLZA to address the sHTG population prior to receiving FDA approval. This allowed us to proactively align with annual payer contracting cycles for 2027 and facilitate access following the approval.

In August 2025, the FDA approved DAWNZERA for prophylaxis to prevent attacks of hereditary angioedema, or HAE, in adult and pediatric patients 12 years of age and older. Following the approval, we launched DAWNZERA and began earning revenue from DAWNZERA sales.

In September 2026, the FDA approved ZANVASTRO (zilganersen) for the treatment of Alexander disease, or AxD, in pediatric and adult patients. Following the approval, we launched ZANVASTRO and began earning revenue from ZANVASTRO sales.

We earn royalty payments primarily on net sales of SPINRAZA, WAINUA and QALSODY. We include QALSODY royalties within other royalties in the table above.

We earn commercial revenue from TEGSEDI and WAYLIVRA sales under our distribution agreements with Swedish Orphan Biovitrum AB, or Sobi. In addition, we receive royalties from PTC Therapeutics International Limited, or PTC, for TEGSEDI and WAYLIVRA sales. Refer to Part IV, Item 15, Note 4, *Collaborative Arrangements and Licensing Agreements*, of our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2025 for details on our commercialization partnerships with Sobi and PTC.

Under our distribution agreements with Sobi, we concluded that our performance obligation is to provide services to Sobi over the term of the agreement, which includes supplying finished goods inventory to Sobi. We are also responsible for maintaining the marketing authorization for TEGSEDI and WAYLIVRA in major markets and for leading the global commercial strategy for each medicine. We view this performance obligation as a series of distinct activities that are substantially the same. We recognize as revenue the price Sobi pays us for the inventory when we deliver the finished goods inventory to Sobi. We also recognize distribution fee revenue based on Sobi's net sales of TEGSEDI and WAYLIVRA. Under our agreements with Sobi, Sobi does not generally have a right of return.

Research and development revenue under collaboration agreements

We enter into collaboration agreements to license and sell our technology on an exclusive or non-exclusive basis. Our collaboration agreements typically contain multiple elements, or performance obligations, including technology licenses or options to obtain technology licenses, R&D services and manufacturing services.

For R&D services that we recognize over time, we measure our progress using an input method. The input methods we use are based on the effort we expend or costs we incur toward the satisfaction of our performance obligation. We estimate the amount of effort we expend, including the time we estimate it will take us to complete the activities, or costs we incur in a given period, relative to the estimated total effort or costs to satisfy the performance obligation. This results in a percentage that we multiply by the transaction price to determine the amount of revenue we recognize each period. This approach requires us to make numerous estimates and use judgement. If our estimates or judgements change over the course of the collaboration, they may affect the timing and amount of revenue that we recognize in the current and future periods.

Upfront payments: When we enter into a collaboration agreement and receive an upfront payment, we record the entire upfront payment as deferred revenue if our only performance obligation is for R&D services we will provide in the future. We amortize the upfront payment into revenue as we perform the R&D services. If part or all of the upfront payment is a license fee, we recognize as revenue the portion related to the license when we deliver the license to our partner because our partner has full use of the license and we do not have any additional performance obligations related to the license after delivery.

Milestone payments: We include variable consideration in the transaction price when it is probable. We typically include milestone payments for R&D services in the transaction price when they are achieved. We include these milestone payments when they are achieved because there is considerable uncertainty in the research and development processes that trigger these payments. Similarly, we include regulatory milestone payments in the transaction price once the medicine is approved by the applicable regulatory agency. We will recognize sales-based milestone payments in the period in which we achieve the milestone under the sales-based royalty exception allowed under accounting rules.

We recognize milestone payments that relate to an ongoing performance obligation over our period of performance. For example, when we achieve a milestone payment from a partner for advancing a clinical study under a collaboration agreement, we add the milestone payment to the transaction price if the milestone relates to an ongoing R&D services performance obligation and recognize revenue related to the milestone payment over our estimated period of performance. If we have partially completed our performance obligation, we record a cumulative-effect adjustment in the period we add the milestone payment to the transaction price.

Conversely, we recognize in full those milestone payments that we earn based on our partners' activities when our partner achieves the milestone event and we do not have a remaining performance obligation.

License fees: We recognize as revenue the total amount we determine to be the relative stand-alone selling price of a license when we deliver the license to our partner because our partner has full use of the license and we do not have any additional performance obligations related to the license after delivery.

WAINUA (Eplontersen) Collaboration with AstraZeneca

In 2021, we entered into a collaboration agreement with AstraZeneca to develop and commercialize WAINUA for the treatment of transthyretin amyloidosis, or ATTR, with the parties sharing responsibility for development, and AstraZeneca having responsibility for commercializing WAINUA. Under the terms of the agreement, we received a $200 million upfront payment in 2021.

At inception of the agreement, we evaluated our WAINUA collaboration under ASC Topic 808, *Collaborative Arrangements*, or ASC 808, and identified four material components: (i) the license we granted to AstraZeneca in 2021, (ii) the shared development activities that we and AstraZeneca were to perform, (iii) the shared commercialization activities that we and AstraZeneca were to perform and (iv) the shared medical affairs activities that we and AstraZeneca were to perform.

We determined that we had a vendor-customer relationship within the scope of ASC Topic 606, *Revenue from Contracts with Customers*, or ASC 606, for the license we granted to AstraZeneca and as a result we had one performance obligation. For our sole performance obligation, we determined the transaction price was the $200 million upfront payment we received. We recognized the upfront payment in full in 2021 because we did not have any remaining performance obligations after we delivered the license to AstraZeneca.

We also concluded that the shared development, commercialization and medical affairs activities are within the scope of ASC 808 because we and AstraZeneca are active participants exposed to the risks and benefits of the activities under the collaboration and therefore do not have a vendor-customer relationship. From inception through December 31, 2025, AstraZeneca was responsible for 55 percent of the costs associated with the ongoing global Phase 3 development program. Beginning on January 1, 2026, AstraZeneca was responsible for 75 percent of costs for development activities intended solely to support U.S. regulatory approvals and 87.5 percent of costs for development activities intended to support global regulatory approvals. Because we were leading the Phase 3 development program, we made an accounting policy election to recognize as non-customer revenue the cost-share funding from AstraZeneca, net of our share of AstraZeneca's development expenses, in the same period we incurred the related development expenses. As AstraZeneca was responsible for the majority of the commercial and medical affairs costs in the U.S. and all costs associated with bringing WAINUA to market outside the U.S., we made an accounting policy election to recognize cost-share funding we received from AstraZeneca related to commercial and medical affairs activities as reductions of our selling, general and administrative, or SG&A, expense and R&D expense, respectively.

In June 2026, we exercised our right to opt-out of the shared responsibility and costs for development and U.S. commercialization activities under the agreement. The opt-out became effective on August 1, 2026. Effective August 1, 2026, AstraZeneca is responsible for 100 percent of WAINUA costs under this agreement. We concluded that the development activities we perform and related cost reimbursements we receive from AstraZeneca after the opt-out are within the scope of ASC 606 because we determined AstraZeneca is our customer for the remaining R&D services performance obligation under this collaboration. As a result, we are recognizing cost funding from AstraZeneca related to development expenses incurred after the opt-out as collaborative agreement revenue.

In connection with the opt-out, certain additional pre-opt-out expenses are reimbursable from AstraZeneca. As of September 30, 2026, these amounts were constrained and no amounts have been recognized in the financial statements.

5. Collaborative Arrangements and Licensing Agreements

Below, we have included our AstraZeneca, Biogen, GSK, Novartis, Ono, Otsuka, Recordati and Roche collaborations, which were the collaborations that had either substantive changes or were new from those included in Part IV, Item 15, Note 4, *Collaborative Arrangements and Licensing Agreements*, of our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2025.

AstraZeneca

We have two collaborations with AstraZeneca: one focused on the development and commercialization of WAINUA and one focused on the treatment of cardiovascular, renal and metabolic diseases. From inception through September 30, 2026, we have received approximately $1.1 billion from these collaborations.

In July 2026, we and AstraZeneca announced that the CARDIO-TTRansform trial for eplontersen in patients with ATTR-CM missed the primary efficacy endpoint of the composite outcome of cardiovascular, or CV, mortality and recurrent CV clinical events up to Week 140 compared with placebo. In this contemporary patient population treated with standard of care, including a majority on a stabilizer, adding eplontersen did not provide a statistically significant benefit. In June 2026, we exercised our opt-out right under the agreement. Refer to Note 4, *Revenues*, for further details on the opt-out.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with AstraZeneca (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with AstraZeneca	$ 21,507	$ 27,128	$ 82,463	$ 101,705
Percentage of total revenue	9%	17%	11%	14%

We did not have any deferred revenue from our relationship with AstraZeneca at September 30, 2026 and December 31, 2025.

Biogen

We have multiple collaborations with Biogen focused on using antisense technology to advance the treatment of neurological disorders, including our 2017 spinal muscular atrophy, or SMA, collaboration and 2018 neurology collaboration.

In 2017, we entered into a collaboration agreement with Biogen to identify new antisense medicines for the treatment of SMA. In 2021, Biogen exercised its option to license salanersen, a drug we discovered under this collaboration.

In the second quarter of 2026, we earned a $45 million milestone payment when Biogen initiated a Phase 3 trial of salanersen for SMA under this collaboration. We recognized this milestone payment as R&D revenue in full in the second quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. We will achieve the next payment of $55 million if Biogen achieves approval in a major market, which includes the U.S., Japan, the United Kingdom, Germany, France, Italy and Spain. From inception through September 30, 2026, we have received $130 million in payments under this collaboration.

Under our 2018 neurology collaboration, Biogen gained exclusive rights to the use of our antisense technology to develop therapies for certain neurological diseases and the option to license certain medicines resulting from this collaboration. If Biogen exercises its option to license a medicine, it will assume global development, regulatory and commercialization responsibilities and costs for that medicine.

In the second quarter of 2026, we earned a $15 million license fee payment when Biogen exercised its option to license an investigational candidate for the treatment of amyotrophic lateral sclerosis, or ALS, under this collaboration. We recognized this license fee payment as R&D revenue in full in the second quarter of 2026 because we did not have any remaining performance obligations related to this payment after we delivered the license to Biogen.

In the third quarter of 2026, we earned a $7.5 million milestone payment when Biogen advanced a program under this collaboration. We recognized this milestone payment as R&D revenue in full in the third quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. We will achieve the next payment of $15 million if Biogen licenses a program under this collaboration. From inception through September 30, 2026, we have received approximately $1.1 billion in payments under this collaboration, including payments to purchase our stock.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Biogen (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Biogen	$ 95,269	$ 74,237	$ 290,820	$ 211,296
Percentage of total revenue	39%	47%	38%	29%

Our condensed consolidated balance sheets at September 30, 2026 and December 31, 2025 included deferred revenue of $104.5 million and $151.2 million, respectively, from our relationship with Biogen.

GSK

In 2010, we entered into a collaboration with GSK using our antisense drug discovery platform to discover and develop new medicines against targets for serious and rare diseases, including infectious diseases. Under our collaboration, GSK is developing and commercializing HIBSAGO (bepirovirsen) for the treatment of chronic hepatitis B, or CHB, infection. In 2019, following positive Phase 2 results, GSK licensed our CHB program. GSK is responsible for all global development, regulatory and commercialization activities and costs for the CHB program.

In the second quarter of 2026, we amended our CHB collaboration agreement with GSK, resulting in increased milestone payments that we are eligible to receive under this collaboration and amended royalty terms that exclude net sales in China, Hong Kong and Macau. Under the amended agreement, we are eligible to receive more than $510 million, which is comprised of a $25 million license fee, up to $46.5 million in development milestone payments, up to $320 million in regulatory milestone payments and up to $120 million in sales milestone payments if GSK successfully develops and commercializes HIBSAGO. In addition, we are eligible to receive tiered royalties ranging from 10 to 12 percent on net sales of HIBSAGO worldwide, excluding China, Hong Kong and Macau. The amendments to the agreement did not change existing performance obligations or result in new performance obligations. Therefore, we did not record any adjustments to previously recognized revenue.

In the first quarter of 2026, we earned a $15 million milestone payment when the Japanese Ministry of Health, Labour and Welfare, or MHLW, accepted for review a New Drug Application, or NDA, filing for HIBSAGO. In addition, we earned a $15 million milestone payment when the European Medicines Agency, or EMA, accepted for review a Marketing Authorization Application, or MAA, filing for HIBSAGO. We recognized these milestone payments as R&D revenue in full in the first quarter of 2026 because we did not have any remaining performance obligations related to the milestone payments.

In the third quarter of 2026, we earned a $35 million milestone payment when the MHLW approved HIBSAGO for the treatment of CHB infection. We recognized this milestone payment as R&D revenue in full in the third quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. From inception through September 30, 2026, we have received more than $135 million in an upfront payment and other payments related to the CHB program.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with GSK (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with GSK	$ 35,000	$ -	$ 65,000	$ -
Percentage of total revenue	14%	-%	9%	-%

We did not have any deferred revenue from our relationship with GSK at September 30, 2026 and December 31, 2025.

In October 2026, we achieved a $30 million milestone payment when the FDA approved HIBSAGO for the treatment of CHB infection. We will achieve the next payment of up to $35 million if HIBSAGO is approved in China.

Novartis

In 2017, we initiated a collaboration with Novartis to develop and commercialize pelacarsen. Novartis is responsible for conducting and funding development and regulatory activities for pelacarsen, including a global Phase 3 cardiovascular outcomes study that Novartis initiated in 2019. In September 2026, Novartis announced that the pelacarsen Phase 3 Lp(a)HORIZON study did not meet its primary endpoint of reducing the risk of cardiovascular events compared to placebo. From inception through September 30, 2026, we have received more than $275 million in payments under this collaboration.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Novartis (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Novartis	$ -	$ 1,235	$ 655	$ 3,569
Percentage of total revenue	-%	1%	-%	-%

We did not have any deferred revenue from our relationship with Novartis at September 30, 2026. Our consolidated balance sheet at December 31, 2025 included deferred revenue of $0.6 million from our relationship with Novartis.

Ono

In March 2025, we entered into an agreement with Ono Pharmaceutical Co., Ltd., or Ono, to develop and commercialize sapablursen, an investigational RNA-targeted medicine for the potential treatment of polycythemia vera, or PV, a rare and potentially life-threatening hematologic disease. We are responsible for completing the Phase 2 IMPRSSION study of sapablursen, including the ongoing extension period. Ono is solely responsible for subsequent development, regulatory filings and commercialization of sapablursen.

In the second quarter of 2026, we earned a $20 million milestone payment when Ono initiated a pivotal clinical trial for sapablursen under this collaboration. We recognized this milestone payment as R&D revenue in full in the second quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. We will achieve the next payment of $15 million if the FDA accepts a New Drug Application, or NDA, filing for sapablursen in the U.S. From inception through September 30, 2026, we have received more than $305 million in payments from Ono.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Ono (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Ono	$ 719	$ 2,979	$ 24,525	$ 282,979
Percentage of total revenue	-%	2%	3%	38%

We did not have any deferred revenue from our relationship with Ono at September 30, 2026. Our consolidated balance sheet at December 31, 2025 included deferred revenue of $2.9 million from our relationship with Ono.

Otsuka

In 2023, we entered into an agreement with Otsuka Pharmaceutical Co., Ltd., or Otsuka, to commercialize DAWNZERA in Europe. In the second quarter of 2024, we expanded the agreement to include commercialization rights for DAWNZERA in the Asia-Pacific region. We are responsible for the ongoing development of DAWNZERA. We retained the rights to commercialize DAWNZERA in the U.S. and in the rest of the world, assuming regulatory approval. In November 2024, we entered into an agreement with Otsuka to commercialize ulefnersen, an investigational medicine for the treatment of ALS, caused by mutations in the *FUS* gene, worldwide. We are responsible for the ongoing development of ulefnersen.

In the first quarter of 2026, we achieved a $15 million milestone payment when the European Commission approved DAWNZERA in the EU. We recognized this milestone payment as R&D revenue in full in the first quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. We will achieve the next payment of $20 million if Otsuka receives reimbursement approval in three of the five major European countries, which include the United Kingdom, France, Germany, Italy and Spain. From inception through September 30, 2026, we have received more than $140 million in payments from Otsuka.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Otsuka (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Otsuka	$ 6,665	$ 3,980	$ 35,984	$ 15,433
Percentage of total revenue	3%	3%	5%	2%

Our condensed consolidated balance sheets at September 30, 2026 and December 31, 2025 included deferred revenue of $1.7 million and $4.3 million, respectively, from our relationship with Otsuka.

Recordati

In June 2026, we entered into an agreement with Recordati AG., or Recordati, to commercialize zilganersen for the treatment of Alexander disease, or AxD, in countries outside of the U.S. We are responsible for the ongoing development of zilganersen globally. We retained the rights to commercialize zilganersen in the U.S., assuming regulatory approval. Recordati is responsible for regulatory filings and commercialization outside the U.S., including country-specific support for early access pathways based on local regulations and access dynamics.

Over the term of this collaboration, we are eligible to receive up to $75 million, which is comprised of a $30 million upfront payment, up to $15 million in regulatory milestone payments and up to $30 million in sales milestone payments. In addition, we are eligible to receive tiered royalties of up to the mid-20 percent range on net sales. We are also eligible to receive reimbursements for clinical development costs that we incur outside the U.S. after March 2028. We will achieve the next payment of up to $10 million if zilganersen is approved in a country outside of the U.S. From inception through September 30, 2026, we have received the $30 million upfront payment from Recordati.

At inception, we identified two performance obligations under this agreement, comprised of our license of zilganersen to Recordati and R&D services for zilganersen. We determined the transaction price to be the $30 million upfront payment. We allocated the transaction price based on the estimated stand-alone selling price of each performance obligation as follows:

- $21.0 million for the license of zilganersen; and
- $9.0 million for the R&D services for zilganersen.

We recognized $21.0 million of R&D revenue for the license of zilganersen in the second quarter of 2026 because we completed the performance obligation when we delivered the license to Recordati. We are recognizing revenue for our R&D services performance obligation relative to our total effort expected to satisfy our performance obligation. We currently estimate we will satisfy our performance obligation in the second quarter of 2029. In addition, we will recognize reimbursements for clinical development costs as R&D revenue in the period that we incur the costs.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Recordati (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Recordati	$ 705	$ -	$ 21,802	$ -
Percentage of total revenue	-%	-%	3%	-%

Our condensed consolidated balance sheet at September 30, 2026 included deferred revenue of $8.2 million from our relationship with Recordati.

Roche

In 2018, we entered into a collaboration agreement with Hoffmann-La Roche Inc and F. Hoffmann-La Roche Ltd, collectively Roche, to develop sefaxersen for the treatment of complement-mediated diseases. In 2022, Roche exercised its right to exclusively develop and commercialize sefaxersen.

In the third quarter of 2026, we earned a $23.5 million milestone payment when Roche advanced the Phase 3 IMAgINATION study of sefaxersen for the treatment of IgAN. We recognized this milestone payment as R&D revenue in full in the third quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. We will achieve the next payment of $15 million if the FDA accepts an NDA filing for sefaxersen.

In 2023, we entered into an agreement with Roche to develop RNA-targeted programs for Alzheimer's disease, or AD, and Huntington's disease, or HD.

In the first quarter of 2026, we earned a $50 million milestone payment when Roche initiated a Phase 1 trial for an investigational medicine for the treatment of AD. We recognized this milestone payment as R&D revenue in full in the first quarter of 2026 because we did not have any remaining performance obligations related to the milestone payment. In the third quarter of 2026, we received a termination notice from Roche for this collaboration that became effective in November 2026.

In 2013, we entered into an agreement with Roche to develop treatments for Huntington's disease, or HD. In the third quarter of 2026, we received a termination notice from Roche for this collaboration that became effective in October 2026.

From inception through September 30, 2026, we have received more than $430 million in payments from our Roche collaborations.

During the three and nine months ended September 30, 2026 and 2025, we earned the following revenue from our relationship with Roche (in thousands, except percentage amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue from our relationship with Roche	$ 23,500	$ 2,618	$ 73,500	$ 6,986
Percentage of total revenue	10%	2%	10%	1%

We did not have any deferred revenue from our relationship with Roche at September 30, 2026 and December 31, 2025.

6. Basic and Diluted Net Loss Per Share

Basic net loss per share

We calculated our basic net loss per share for the three and nine months ended September 30, 2026 and 2025 by dividing our net loss by our weighted-average number of common shares outstanding during the period.

Diluted net loss per share

For the three and nine months ended September 30, 2026 and 2025, we incurred a net loss; therefore, we did not include dilutive common equivalent shares in the computation of diluted net loss per share because the effect would have been anti-dilutive. Common stock from the following would have had an anti-dilutive effect on net loss per share:

- 1.75 percent convertible senior notes due 2028, or 1.75% Notes due 2028;
- Warrants related to the 0% Notes due 2026;
- Dilutive stock options;
- Unvested restricted stock units, or RSUs;
- Unvested performance restricted stock units, or PRSUs; and
- Employee Stock Purchase Plan, or ESPP.

For the three and nine months ended September 30, 2026, common stock underlying the 0 percent convertible senior notes due 2030, or 0% Notes due 2030, would also have had an anti-dilutive effect on net loss per share. For the three and nine months ended September 30, 2025, common stock underlying the 0 percent convertible senior notes due 2026, or 0% Notes due 2026, and the related note hedges would also have had an anti-dilutive effect on net loss per share.

7. Investments

The following table summarizes the contract maturity of the available-for-sale securities we held as of September 30, 2026:

One year or less	66%
After one year but within two years	26%
After two years but within three and a half years	8%
Total	100%

As illustrated above, at September 30, 2026, 92 percent of our available-for-sale securities had a maturity of less than two years.

All of our available-for-sale debt securities are available to us for use in our current operations. As a result, we categorize all of these securities as current assets even though the stated maturity of some individual securities may be one year or more beyond the balance sheet date.

We invest in debt securities with strong credit ratings and an investment grade rating at or above A-1, P-1 or F-1 by Standard & Poor's, Moody's or Fitch, respectively.

At September 30, 2026, we had an equity ownership interest of less than 20 percent in four private companies and four public companies with which we conduct business.

The following is a summary of our investments (in thousands):

September 30, 2026	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Available-for-sale debt securities:				
Corporate debt securities (1)	$ 598,983	$ 84	$ (1,275)	$ 597,792
Debt securities issued by U.S. government agencies	55,151	3	(252)	54,902
Debt securities issued by the U.S. Treasury (1)	415,527	8	(1,027)	414,508
Debt securities issued by states of the U.S. and political subdivisions of the states	4,165	-	(16)	4,149
Total debt securities with a maturity of one year or less	1,073,826	95	(2,570)	1,071,351
Corporate debt securities	320,994	30	(4,120)	316,904
Debt securities issued by U.S. government agencies	94,803	-	(1,857)	92,946
Debt securities issued by the U.S. Treasury	153,024	-	(1,946)	151,078
Total debt securities with a maturity of more than one year	568,821	30	(7,923)	560,928
Total available-for-sale debt securities	$ 1,642,647	$ 125	$ (10,493)	$ 1,632,279
Equity securities:				
Publicly traded equity securities included in other current assets (2)	$ 11,897	$ 55,437	$ (10,055)	$ 57,279
Privately held equity securities included in deposits and other assets (3)	10,000	9,816	-	19,816
Total equity securities	21,897	65,253	(10,055)	77,095
Total available-for-sale debt and equity securities	$ 1,664,544	$ 65,378	$ (20,548)	$ 1,709,374

December 31, 2025	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Available-for-sale debt securities:				
Corporate debt securities (1)	$ 746,814	$ 991	$ (27)	$ 747,778
Debt securities issued by U.S. government agencies	55,768	92	(5)	55,855
Debt securities issued by the U.S. Treasury (1)	769,034	843	(13)	769,864
Debt securities issued by states of the U.S. and political subdivisions of the states	5,709	11	-	5,720
Total debt securities with a maturity of one year or less	1,577,325	1,937	(45)	1,579,217
Corporate debt securities	435,350	1,586	(106)	436,830
Debt securities issued by U.S. government agencies	92,770	100	(94)	92,776
Debt securities issued by the U.S. Treasury	242,288	595	(1)	242,882
Other municipal debt securities	1,219	4	-	1,223
Total debt securities with a maturity of more than one year	771,627	2,285	(201)	773,711
Total available-for-sale debt securities	$ 2,348,952	$ 4,222	$ (246)	$ 2,352,928
Equity securities:				
Publicly traded equity securities included in other current assets (2)	$ 11,897	$ 35	$ (8,920)	$ 3,012
Privately held equity securities included in deposits and other assets (3) (2)	4,905	54,395	(7,091)	52,209
Total equity securities	16,802	54,430	(16,011)	55,221
Total available-for-sale debt and equity securities	$ 2,365,754	$ 58,652	$ (16,257)	$ 2,408,149

(1) Includes investments classified as cash equivalents in our condensed consolidated balance sheets.

(2) Our publicly traded equity securities are included in other current assets. We recognize publicly traded equity securities at fair value. In the first quarter of 2026, one of our privately held investees became a publicly traded entity after completing an initial public offering. As a result, we recorded a $23.4 million unrealized gain in our condensed consolidated statements of operations in the first quarter of 2026, which reflected the difference between the carrying value of this investment as of December 31, 2025 and its fair value of $55.8 million as of March 31, 2026. In the third quarter of 2026, we recorded an unrealized gain of $9.8 million, which reflected the difference between the carrying value of this investment as of June 30, 2026 and its fair value of $55.4 million as of September 30, 2026. This investment is subject to a 12-month contractual sale restriction that ends in January 2027. In the nine months ended September 30, 2026, we recorded a $1.1 million net unrealized loss in our condensed consolidated statements of operations related to changes in the fair value of our other investments in publicly traded companies.

(3) Our privately held equity securities are included in deposits and other assets. We recognize our privately held equity securities at cost minus impairments, plus or minus changes resulting from observable price changes in orderly transactions for the identical or similar investment of the same issuer, which are Level 3 inputs. In the nine months ended September 30, 2026, there were no changes to the carrying value of our privately held equity securities.

The following is a summary of our investments we consider to be temporarily impaired at September 30, 2026 (in thousands, except for number of investments):

		Less than 12 Months of Temporary Impairment		More than 12 Months of Temporary Impairment		Total Temporary Impairment	
	Number of Investments	Estimated Fair Value	Unrealized Losses	Estimated Fair Value	Unrealized Losses	Estimated Fair Value	Unrealized Losses
Corporate debt securities	409	$ 814,052	$ (5,343)	$ 5,924	$ (52)	$ 819,976	$ (5,395)
Debt securities issued by U.S. government agencies	64	127,901	(2,077)	2,826	(32)	130,727	(2,109)
Debt securities issued by the U.S. Treasury	86	531,707	(2,973)	-	-	531,707	(2,973)
Debt securities issued by states of the U.S. and political subdivisions of the states	4	3,359	(16)	-	-	3,359	(16)
Total temporarily impaired debt securities	563	$ 1,477,019	$ (10,409)	$ 8,750	$ (84)	$ 1,485,769	$ (10,493)

We believe that the decline in value of these securities is temporary and is primarily related to the change in market interest rates since purchase rather than underlying credit deterioration for any of the issuers. We believe it is more likely than not that we will be able to hold our debt securities with declines in value to maturity. Therefore, we intend to hold these securities to maturity and anticipate full recovery of our debt securities' amortized cost basis at maturity.

8. Fair Value Measurements

The following tables present the major security types we held at September 30, 2026 and December 31, 2025 that we regularly measure and carry at fair value. The following tables segregate each security type by the level within the fair value hierarchy of the valuation techniques we utilized to determine the respective security's fair value (in thousands):

	At September 30, 2026	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)
Cash equivalents (1)	$ 146,829	$ 146,829	$ -
Corporate debt securities (2)	914,696	-	914,696
Debt securities issued by U.S. government agencies (3)	147,848	-	147,848
Debt securities issued by the U.S. Treasury (4)	565,586	565,586	-
Debt securities issued by states of the U.S. and political subdivisions of the states (3)	4,149	-	4,149
Publicly traded equity securities included in other current assets (5)	57,279	57,279	-
Total	$ 1,836,387	$ 769,694	$ 1,066,693

	At December 31, 2025	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)
Cash equivalents (1)	$ 213,579	$ 213,579	$ -
Corporate debt securities (6)	1,184,608	-	1,184,608
Debt securities issued by U.S. government agencies (3)	148,631	-	148,631
Debt securities issued by the U.S. Treasury (3)	1,012,746	1,012,746	-
Debt securities issued by states of the U.S. and political subdivisions of the states (3)	6,943	-	6,943
Publicly traded equity securities included in other current assets (5)	3,012	3,012	-
Total	$ 2,569,519	$ 1,229,337	$ 1,340,182

The following footnotes reference lines in our condensed consolidated balance sheets:

(1) Included in cash and cash equivalents.

(2) $33.8 million was included in cash and cash equivalents, with the difference included in short-term investments.

(3) Included in short-term investments.

(4) $10.9 million was included in cash and cash equivalents, with the difference included in short-term investments.

(5) Included in other current assets.

(6) $47.8 million was included in cash and cash equivalents, with the difference included in short-term investments.

Convertible Notes

Our 0% Notes due 2030 and 1.75% Notes due 2028 had a fair value of $635.7 million and $633.1 million at September 30, 2026, respectively. Our 0% Notes due 2030, 1.75% Notes due 2028 and 0% Notes due 2026 had a fair value of $830.3 million, $918.1 million and $594.6 million at December 31, 2025, respectively. We determine the fair value of our notes based on quoted market prices for these notes, which are Level 2 measurements because the notes do not trade regularly.

9. Stock-based Compensation Expense

The following table summarizes stock-based compensation expense for the three and nine months ended September 30, 2026 and 2025 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Cost of sales	$ 193	$ 145	$ 516	$ 855
Research, development and patent expense	24,399	20,757	74,203	60,597
Selling, general and administrative expense	18,832	10,244	57,575	29,102
Stock-based compensation expense, net of amounts capitalized	43,424	31,146	132,294	90,554
Capitalized stock-based compensation expense	538	435	556	1,435
Total stock-based compensation expense	$ 43,962	$ 31,581	$ 132,850	$ 91,989

As of September 30, 2026, total unrecognized estimated stock-based compensation expense related to non-vested stock options, RSUs and PRSUs was $47.6 million, $138.7 million and $20.6 million, respectively. Our actual expenses will likely differ from these estimates because we will adjust our unrecognized stock-based compensation expense for future forfeitures, including any PRSUs that do not vest. We expect to recognize the cost of stock-based compensation expense related to our non-vested stock options, RSUs and PRSUs over a weighted average amortization period of 1.1 years, 1.4 years and 1.4 years, respectively.

Stock Options:

The weighted-average grant date fair value of stock options granted to employees for the nine months ended September 30, 2026 and 2025 was $37.74 and $17.21 per share, respectively. The weighted-average grant date fair value of stock options granted to non-employee directors for the nine months ended September 30, 2026 and 2025 was $39.97 and $20.86 per share, respectively.

In the second quarter of 2026, two new members were appointed to the Board of Directors following the retirement of two Board members. In June 2026, we issued stock options to the two new Board members.

RSUs:

The weighted-average grant date fair value of RSUs granted to employees for the nine months ended September 30, 2026 and 2025 was $76.30 and $33.78 per share, respectively. The weighted-average grant date fair value of RSUs granted to non-employee directors for the nine months ended September 30, 2026 and 2025 was $71.93 and $33.78 per share, respectively.

PRSUs:

Under the terms of the PRSUs we granted in 2026 and 2025, the PRSUs may vest at the end of the three-year performance period based on our relative total shareholder return, or TSR, as compared to a peer group of companies and as measured at the end of the performance period. Under the terms of the grants, no number of PRSUs is guaranteed to vest and the actual number of PRSUs that will vest at the end of each performance period may be anywhere from zero to 200 percent of the target number depending on our relative TSR.

The weighted-average grant date fair value of PRSUs we granted to our executive officers for the nine months ended September 30, 2026 and 2025 was $115.05 and $48.81 per share, respectively.

Black-Scholes Assumptions:

For the nine months ended September 30, 2026 and 2025, we used the following weighted-average assumptions in our Black-Scholes calculations:

Employee Stock Options:

	Nine Months Ended September 30,	
	2026	**2025**
Risk-free interest rate	4.0%	4.4%
Dividend yield	0.0%	0.0%
Volatility	43.1%	42.1%
Expected life	6.3 years	6.3 years

Board of Directors Stock Options:

	Nine Months Ended September 30,	
	2026	2025
Risk-free interest rate	4.4%	4.0%
Dividend yield	0.0%	0.0%
Volatility	42.0%	43.0%
Expected life	7.2 years	7.4 years

ESPP:

	Nine Months Ended September 30,	
	2026	2025
Risk-free interest rate	3.7%	4.2%
Dividend yield	0.0%	0.0%
Volatility	51.5%	48.2%
Expected life	6 months	6 months

10. Liability Related to Sale of Future Royalties

In 2023, we entered into a royalty purchase agreement with Royalty Pharma Investments, or Royalty Pharma, to monetize a portion of our future SPINRAZA and pelacarsen royalties we are entitled to under our arrangements with Biogen and Novartis, respectively. As a result, we received an upfront payment of $500 million and we are eligible to receive up to $625 million in additional milestone payments. Under the terms of the agreement, Royalty Pharma will receive 25 percent of our SPINRAZA royalty payments from 2023 through 2027, increasing to 45 percent of royalty payments in 2028, on up to $1.5 billion in annual sales. In addition, Royalty Pharma is eligible to receive 25 percent of any future royalty payments on pelacarsen, our medicine in development to treat patients with elevated lipoprotein(a)-driven cardiovascular disease. Royalty Pharma's royalty interest in SPINRAZA will revert to us after total SPINRAZA royalty payments to Royalty Pharma reach either $475 million or $550 million, depending on the timing and occurrence of FDA approval of pelacarsen.

We recorded the upfront payment of $500 million as a liability related to the sale of future royalties, net of transaction costs of $10.4 million, which we are amortizing over the estimated life of the arrangement using the effective interest rate method. We recognize royalty revenue in the period in which the counterparty sells the related product and recognizes the related revenue. We record royalty payments made to Royalty Pharma as a reduction of the liability. From inception through September 30, 2026, we have paid Royalty Pharma approximately $178.0 million under this arrangement.

We determine the effective interest rate used to record interest expense under this agreement based on an estimate of future royalty payments to Royalty Pharma. As of September 30, 2026 and 2025, the estimated effective interest rate under the agreement was 10.0 percent and 12.4 percent, respectively.

In September 2026, Novartis announced that the pelacarsen Phase 3 Lp(a)HORIZON study did not meet its primary endpoint of reducing the risk of cardiovascular events compared to placebo. As a result, we no longer expect the FDA approval of pelacarsen and changed our estimate of future pelacarsen royalty payments to zero and total SPINRAZA royalty payments payable to Royalty Pharma from $475 million to $550 million. We accounted for this change in estimate using the prospective interest method by recalculating the effective interest rate based on the carrying amount and the revised estimated future payments to Royalty Pharma as of September 2026. The revised estimated future payments was less than the net carrying amount of our liability. As a result, we began recognizing contra-interest expense over the remaining term of the Royalty Pharma arrangement. This reflects the reversal of previously recognized interest expense that is greater than the updated estimate of total interest expense that we will recognize over the term of the arrangement.

The following table sets forth information on our liability related to sale of future royalties (in thousands):

Liability related to sale of future royalties, net as of December 31, 2025	$	563,042
Royalty payments to Royalty Pharma		(35,986)
Interest expense related to sale of future royalties		38,427
Amortization of issuance costs related to sale of future royalties		545
Liability related to sale of future royalties, net as of September 30, 2026	$	566,028
Less: Current portion, net (1)		(138,188)
Liability related to sale of future royalties, net as of September 30, 2026 – Non-current	$	427,840

(1) The current portion represents the estimated reduction in the liability balance over the next twelve months after September 30, 2026, which includes estimated royalty payments to Royalty Pharma and estimated contra-interest expense.

There are numerous factors, most of which are not within our control, that could materially impact the amount and timing of royalty payments from Biogen, and result in changes to our estimate of future royalty payments to Royalty Pharma. Such factors include, but are not limited to, the commercial sales of SPINRAZA, competing products or other significant events.

11. Convertible Debt

0 Percent Convertible Senior Notes due 2030

In November 2025, we completed a $770.0 million offering of our 0% Notes due 2030.

At September 30, 2026, we had the following 0% Notes due 2030 outstanding (in millions except interest rate and price per share data):

		0% Notes due 2030
Outstanding principal balance	$	770.0
Unamortized debt issuance costs	$	15.7
Maturity date		December 2030
Interest rate		0%
Effective interest rate		0.5%
Conversion price per share	$	98.10
Total shares of common stock subject to conversion		7.8

1.75 Percent Convertible Senior Notes due 2028

In 2023, we completed a $575.0 million offering of our 1.75% Notes due 2028.

At September 30, 2026, we had the following 1.75% Notes due 2028 outstanding (in millions except interest rate and price per share data):

		1.75% Notes due 2028
Outstanding principal balance	$	575.0
Unamortized debt issuance costs	$	5.0
Maturity date		June 2028
Interest rate		1.75%
Effective interest rate		2.3%
Conversion price per share	$	53.73
Total shares of common stock subject to conversion		10.7

0 Percent Convertible Senior Notes due 2026 and Call Spread

In April 2026, we paid the remaining principal balance of our 0% Notes due 2026 with $432.5 million of cash at maturity. In addition, we settled the conversion obligation in excess of principal by issuing 1.8 million shares of common stock to the holders of the 0% Notes due 2026 who exercised their conversion option.

In conjunction with the 2021 offering, we entered into a call spread transaction, which was comprised of purchasing note hedges and selling warrants, to minimize the impact of potential economic dilution upon conversion of our 0% Notes due 2026 by increasing the effective conversion price on these notes. We increased our effective conversion price to $76.39 with the same number of underlying shares as our 0% Notes due 2026. Our note hedges were exercisable upon conversion of the 0% Notes due 2026. In April 2026, we exercised the note hedges upon maturity of these notes. As a result, we received 2.6 million shares of common stock, which we retired upon receipt. In the three and nine months ended September 30, 2026, we issued 0.1 million shares in relation to warrant exercises. The warrants fully expired in September 2026.

Upon the execution of the call spread transaction in 2021, we recorded the amount we paid for the note hedges and the amount we received for the warrants in additional paid-in capital in our condensed consolidated balance sheets. Refer to Part IV, Item 15, Note 1, *Organization and Significant Accounting Policies*, of our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2025 for our Call Spread accounting policy. We reassess our ability to continue to classify the warrants in shareholders' equity at each reporting period.

Other Terms of Convertible Senior Notes

The 0% Notes due 2030 and 1.75% Notes due 2028 are convertible under certain conditions, at the option of the note holders. We can settle conversions of the notes, at our election, in cash, shares of our common stock or a combination of both. We may not redeem the notes prior to maturity, and we do not have to provide a sinking fund for them. Holders of the notes may require us to purchase some or all of their notes upon the occurrence of certain fundamental changes, as set forth in the indentures governing the notes, at a purchase price equal to 100 percent of the principal amount of the notes to be purchased, plus any accrued and unpaid interest. The 0% Notes due 2026 were subject to similar terms.

12. Legal Proceedings

From time to time, we are involved in legal proceedings arising in the ordinary course of our business. Periodically, we evaluate the status of each legal matter and assess our potential financial exposure. If we consider the potential loss from any legal proceeding to be probable and we can reasonably estimate the amount, we accrue a liability for the estimated loss. The outcome of any proceeding is not determinable in advance. Therefore, we are required to use significant judgment to determine the probability of a loss and whether the amount of the loss is reasonably estimable. Our assessment of a potential liability and the amount of accruals we recorded are based only on the information available to us at the time. As additional information becomes available, we reassess the potential liability related to the legal proceeding and may revise our estimates.

On September 11, 2025, we sued Arrowhead Pharmaceuticals, Inc., in the Central District of California asserting that Arrowhead's announced intention to commercialize plozasiran in November 2025 would infringe our patent US9,593,333. Arrowhead is now commercializing plozasiran and the lawsuit is ongoing. Arrowhead has filed its Answer to our Complaint and Counterclaims asserting that our patent US9,593,333 is invalid or not infringed by use of plozasiran.

On June 1, 2026, Biogen received notice that Somerset Therapeutics, LLC had filed an Abbreviated New Drug Application, or ANDA, seeking approval to commercialize a generic version of SPINRAZA intrathecal injection, 12 mg/5 ml. The ANDA alleged that certain patents that cover SPINRAZA and its use are invalid or would not be infringed by Somerset. On June 22, 2026, Biogen, Cold Spring Harbor Laboratory and Ionis jointly filed a lawsuit in the District Court of Delaware asserting patent infringement. That proceeding has since been consolidated with two other ANDA proceedings directed to SPINRAZA: one initiated by Cipla LTD., and another initiated by Somerset listing different patents. The parties have filed their complaints, counterclaims and answers, and the consolidated lawsuit is ongoing.

13. Segment Information

We operate as a single operating segment, Ionis operations, focused on the research, development and commercialization of our RNA-targeted medicines to bring better futures to people with serious diseases. The CODM, our Chief Executive Officer, manages our company, reviews operating results, assesses performance and allocates resources on an aggregate basis using consolidated net income or loss as the key measure of segment profit or loss. As such, results of our operations are reported on a consolidated basis for purposes of management and segment reporting.

Ionis operations derives its revenues from commercial and R&D revenue sources. Refer to Note 4, *Revenues*, for further details on our sources of revenue.

The following table sets forth information on segment profit or loss, including significant segment expenses (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue	$ 242,721	$ 156,719	$ 756,762	$ 740,381
Less:				
Cost of sales	3,129	2,193	8,527	7,097
Drug discovery	28,904	28,412	86,581	85,656
Drug development	101,333	120,828	309,473	351,463
Medical affairs	11,453	8,874	32,760	22,342
Manufacturing and development chemistry	23,266	15,790	64,213	51,616
R&D support	26,523	23,092	75,928	64,252
Selling, general and administrative	135,389	86,565	397,401	234,626
Other segment items (1)	22,695	(429)	99,024	75,322
Consolidated net loss	$ (109,971)	$ (128,606)	$ (317,145)	$ (151,993)

(1) Other segment items include stock-based compensation expense, investment income, interest expense, gain or loss on investments, other income or expense and income tax expense or benefit.

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

In this Report on Form 10-Q, unless the context requires otherwise, "Ionis," the "Company," "we," "our," and "us," means Ionis Pharmaceuticals, Inc. and its subsidiaries.

Forward-Looking Statements

In addition to historical information contained in this Report on Form 10-Q, the Report includes forward-looking statements regarding our business and the therapeutic and commercial potential of our commercial medicines, additional medicines in development, technologies and our expectations regarding development and regulatory milestones. Any statement describing our goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Our forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause our results to differ materially from those expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in this report and described in additional detail in our annual report on Form 10-K for the year ended December 31, 2025, which is on file with the U.S. Securities and Exchange Commission and is available from us, and those identified within Part II Item 1A, *Risk Factors*, of this Report. Although our forward-looking statements reflect the good faith judgment of our management, these statements are based only on facts and factors currently known by us. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. As a result, you are cautioned not to rely on these forward-looking statements.

Overview

For three decades, we have invented medicines that bring better futures to people with serious diseases. As a pioneer in RNA-targeted medicines with a deep understanding of disease biology and an industry-leading drug discovery technology, we are driven to deliver innovative, life-changing advances for patients.

With multiple independent commercial launches now underway, we have transitioned into a fully integrated commercial-stage biotechnology company. We currently have nine marketed medicines to treat serious diseases, which include three Ionis-owned medicines: TRYNGOLZA (olezarsen), DAWNZERA (donidalorsen) and ZANVASTRO (zilganersen), and six partnered medicines: HIBSAGO (bepirovirsen), WAINUA (eplontersen), SPINRAZA (nusinersen), QALSODY (tofersen), TEGSEDI (inotersen) and WAYLIVRA (volanesorsen). Following approval by the U.S. Food and Drug Administration, or FDA, in June 2026, we independently launched TRYNGOLZA for the treatment of severe hypertriglyceridemia, or sHTG. In addition, we independently launched ZANVASTRO for the treatment of Alexander disease, or AxD, after receiving FDA approval in September 2026. Our partner, GSK, launched HIBSAGO for the treatment of chronic hepatitis B, or CHB, in Japan after receiving approval from the Ministry of Health, Labour and Welfare, or MHLW, in August 2026. HIBSAGO also received FDA approval in October 2026. We also have a rich innovative pipeline across our focus areas of neurology, cardiometabolic diseases and select areas of high patient needs. We currently have multiple medicines advancing in Phase 3 development, including obudanersen for Angelman syndrome, or AS, which is wholly owned and recently completed enrollment. In addition, our partnered medicines, ulefnersen and sefaxersen, recently delivered positive Phase 3 results. We also have additional medicines in early and mid-stage development.

Our multiple sources of revenue and solid financial foundation enable our continued investments to support ongoing and planned launches and to advance our wholly owned medicines in development. Our key recent achievements, combined with our independent and partnered product launches anticipated by the end of 2027, position us well to help millions of patients with serious diseases and deliver increasing product and royalty revenue.

Our Marketed Medicines

TRYNGOLZA is a once monthly, self-administered RNA-targeted medicine approved in the United States, or U.S., as an adjunct to diet to reduce triglycerides and the risk of acute pancreatitis in adults with sHTG and as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome, or FCS. TRYNGOLZA is the first and only treatment approved by the FDA that significantly and substantially reduces triglyceride levels in adults with sHTG and provides a clinically meaningful reduction in acute pancreatitis, or AP, events. TRYNGOLZA is also approved in the European Union, or EU, Canada and the United Kingdom, or UK, as an adjunct to diet in adult patients for the treatment of genetically confirmed FCS. TRYNGOLZA is the first medicine we began commercializing independently in the U.S. Sobi has exclusive rights to commercialize TRYNGOLZA in countries outside of the U.S., Canada and China.

DAWNZERA is an RNA-targeted medicine approved in the U.S. for prophylaxis to prevent attacks of hereditary angioedema, or HAE, in adult and pediatric patients 12 years of age and older. DAWNZERA is the first and only FDA-approved RNA-targeted prophylactic therapy for HAE. DAWNZERA is also approved in the EU, UK and Canada for the routine prevention of recurrent attacks of HAE in the same age group. DAWNZERA is self-administered via subcutaneous autoinjector once every four or eight weeks. DAWNZERA has the potential to offer durable efficacy, a favorable safety and tolerability profile, and the longest available dosing interval. DAWNZERA is the second medicine we are commercializing independently in the U.S. We licensed commercialization rights for DAWNZERA in Europe and the Asia-Pacific region to Otsuka Pharmaceutical Co., Ltd., or Otsuka.

ZANVASTRO is an RNA-targeted medicine approved in the U.S. for the treatment of AxD in pediatric and adult patients. It is administered intrathecally every three months. ZANVASTRO is the first and only FDA-approved disease modifying treatment for AxD. ZANVASTRO is the third medicine we are commercializing independently in the U.S. We licensed commercialization rights for ZANVASTRO in countries outside of the U.S. to Recordati. ZANVASTRO was granted Orphan Drug designation from the European Medicines Agency, or EMA.

SPINRAZA is an antisense medicine for the treatment of patients with spinal muscular atrophy, or SMA, a progressive, debilitating and often fatal genetic disease. Higher dose SPINRAZA was approved and launched in the U.S., EU and Japan for the treatment of SMA. Higher dose SPINRAZA is also approved in the UK and Switzerland. Our partner, Biogen, is responsible for commercializing SPINRAZA worldwide.

HIBSAGO (bepirovirsen) is an RNA-targeted medicine approved in the U.S. and Japan for CHB virus infection in adult patients without cirrhosis receiving nucleos(t)ide analogue therapy and with hepatitis B virus, or HBV, surface antigen, or HBsAg, levels ≤3000 IU/ml as determined by an FDA-authorized test. It is a six-month finite treatment that can be administered at-home after the first dose. HIBSAGO offers unprecedented functional cure rates compared to current standard of care. Our partner, GSK, is responsible for commercializing HIBSAGO worldwide.

WAINUA (WAINZUA in Europe) is a once monthly, self-administered subcutaneous RNA-targeted medicine that is approved in numerous countries, including the U.S., EU, UK, Canada and China, for the treatment of adults with polyneuropathy of hereditary transthyretin-mediated amyloidosis, or ATTRv-PN, a debilitating, progressive, and fatal disease. In January 2024, we and AstraZeneca launched WAINUA in the U.S. for the treatment of adults with ATTRv-PN. The launch of WAINUA is underway in numerous countries, including the countries in the EU, following the approval by the European Commission, or EC, in March 2025. AstraZeneca is our commercialization partner for WAINUA.

QALSODY is an antisense medicine that received accelerated approval from the FDA in April 2023 and marketing authorization under exceptional circumstances from the EMA in May 2024 for the treatment of adult patients with superoxide dismutase 1 amyotrophic lateral sclerosis, or SOD1-ALS, a rare, neurodegenerative disorder that causes progressive loss of motor neurons leading to death. QALSODY was the first treatment approved to target a genetic cause of ALS. Our partner, Biogen, is responsible for commercializing QALSODY worldwide. Biogen is also evaluating QALSODY as a potential treatment for presymptomatic SOD1-ALS patients in the ongoing ATLAS study. QALSODY was granted Orphan Drug designation by the FDA and EMA.

TEGSEDI is a once weekly, self-administered subcutaneous medicine approved in Europe and Brazil for the treatment of patients with ATTRv-PN. We currently sell TEGSEDI in Europe through our distribution agreement with Swedish Orphan Biovitrum AB, or Sobi. In Latin America, PTC Therapeutics International Limited, or PTC, is commercializing TEGSEDI in Brazil and is pursuing access in additional Latin American countries through its exclusive license agreement with us.

WAYLIVRA is a once weekly, self-administered, subcutaneous medicine approved in Europe and Brazil as an adjunct to diet in adult patients with genetically confirmed FCS and at high risk for pancreatitis. We sell WAYLIVRA in Europe through our distribution agreement with Sobi. In Latin America, PTC is commercializing WAYLIVRA in Brazil for two indications, FCS and familial partial lipodystrophy, or FPL, and is pursuing access in additional Latin American countries through its exclusive license agreement with us.

Our Innovative Late-Stage Pipeline of Investigational Medicines

Obudanersen is our wholly owned medicine in development for AS. In July 2026, we completed enrollment of the Phase 3 study, REVEAL, which we designed to evaluate the efficacy and safety of obudanersen. In September 2026, we initiated the Phase 3 CHAMPION study in patients with AS due to paternal uniparental disomy or imprinting center defects. In addition, we are continuing to conduct the open label Phase 1/2 study, HALOS, of obudanersen in patients with AS designed to assess the safety, tolerability and activity of multiple ascending doses of obudanersen administered intrathecally. In 2025, we presented positive 12- and 18-month long-term extension data from the HALOS study which supports continued development. The FDA and EMA granted Orphan Drug designation to obudanersen. Additionally, the FDA granted Breakthrough Therapy, Fast Track and Rare Pediatric designations to obudanersen.

Eplontersen is our medicine in development to treat patients with transthyretin amyloidosis cardiomyopathy, or ATTR-CM. In July 2026, we and AstraZeneca announced that the CARDIO-TTRansform trial for eplontersen in patients with ATTR-CM missed the primary efficacy endpoint of the composite outcome of cardiovascular, or CV, mortality and recurrent CV clinical events up to Week 140 compared with placebo. In this contemporary patient population treated with standard of care, including a majority on a stabilizer, adding eplontersen did not provide a statistically significant benefit. AstraZeneca shared the results with the scientific community at the European Society of Cardiology, or ESC, Congress in August 2026 and is continuing to analyze the full data set.

Pelacarsen is our medicine in development to treat patients with elevated lipoprotein(a)-driven cardiovascular disease, or Lp(a)-driven CVD. In September 2026, Novartis announced that the pelacarsen Phase 3 Lp(a)HORIZON study did not meet its primary endpoint of reducing the risk of cardiovascular events compared to placebo. The full Lp(a)HORIZON data will be presented at the American Heart Association, or AHA, conference in November 2026.

Salanersen is our medicine in development for SMA. In the second quarter of 2026, Biogen advanced salanersen into Phase 3 development in patients with SMA and the FDA granted Breakthrough Therapy designation to salanersen based on positive interim Phase 1 results. Salanersen has also received Orphan Drug designation for the treatment of SMA.

Sapablursen is our medicine in development for polycythemia vera, or PV. In the second quarter of 2026, Ono advanced sapablursen into Phase 3 development in patients with PV. Sapablursen has received FDA Fast Track, Orphan Drug and Breakthrough Therapy designations for the treatment of PV.

Sefaxersen is our medicine in Phase 3 development for immunoglobulin A, or IgA, nephropathy, or IgAN. In September 2026, we and Roche announced positive interim data from the ongoing Phase 3 IMAgINATION study evaluating investigational sefaxersen in adults with primary IgAN, which were presented in October 2026 at the American Society of Nephrology, or ASN. Roche plans to share these data with health authorities.

Tofersen is our medicine in development for presymptomatic SOD1-ALS. Biogen is evaluating tofersen for treatment of presymptomatic individuals who have a *SOD1* genetic mutation.

Ulefnersen is our medicine in development for amyotrophic lateral sclerosis, or ALS, with mutations in the fused in sarcoma gene, or FUS. In September 2026, we and Otsuka announced positive topline results from the Phase 3 FUSION study of ulefnersen in juvenile and adult patients with FUS-ALS. We licensed global commercialization rights for ulefnersen to Otsuka. The FDA and EMA granted Orphan Drug designation to ulefnersen. The FDA also granted Fast Track designation to ulefnersen.

Critical Accounting Estimates

We prepare our condensed consolidated financial statements in conformity with accounting principles generally accepted in the U.S. As such, we make certain estimates, judgments and assumptions that we believe are reasonable, based upon the information available to us. These judgments involve making estimates about the effect of matters that are inherently uncertain and may significantly impact our quarterly or annual results of operations and financial condition. Each quarter, our senior management reviews the development, selection and disclosure of such estimates with the audit committee of our board of directors. The following are our significant accounting estimates, which we believe are the most critical to aid in fully understanding and evaluating our reported financial results:

- Assessing the propriety of revenue recognition and associated deferred revenue;
- Determining the appropriate cost estimates for unbilled preclinical studies and clinical development activities; and
- Assessing the appropriate estimate of anticipated future royalty payments under our royalty purchase agreement

There have been no material changes to our critical accounting estimates from the information provided in Item 7, *Management's Discussion and Analysis of Financial Condition and Results of Operations*, included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Results of Operations

The following is a summary of our financial results (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Total revenue	$ 242.7	$ 156.7	$ 756.8	$ 740.4
Total operating expenses	$ 373.4	$ 316.9	$ 1,107.2	$ 907.6
Loss from operations	$ (130.7)	$ (160.2)	$ (350.4)	$ (167.2)
Net loss	$ (110.0)	$ (128.6)	$ (317.1)	$ (152.0)

Revenue

Total revenue for the three and nine months ended September 30, 2026 was $242.7 million and $756.8 million, respectively, compared to $156.7 million and $740.4 million for the same periods in 2025 respectively, and was comprised of the following (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue:				
Commercial revenue:				
Product sales, net:				
TRYNGOLZA sales, net	$ 9.3	$ 31.8	$ 41.0	$ 57.4
DAWNZERA sales, net	36.8	0.7	79.2	0.7
ZANVASTRO sales, net	2.2	-	2.2	-
Total product sales, net	48.3	32.5	122.4	58.1
Royalty revenue:				
SPINRAZA royalties	69.4	55.9	166.6	158.2
WAINUA royalties	6.8	13.3	34.0	33.1
Other royalties	4.4	6.5	14.3	18.5
Total royalty revenue	80.6	75.7	214.9	209.8
Other commercial revenue	7.3	7.4	25.4	26.6
Total commercial revenue	136.2	115.6	362.7	294.5
Research and development revenue:				
Collaborative agreement revenue	102.8	31.4	356.5	413.4
WAINUA joint development revenue	3.7	9.7	37.6	32.5
Total research and development revenue	106.5	41.1	394.1	445.9
Total revenue	$ 242.7	$ 156.7	$ 756.8	$ 740.4

Commercial revenue for the three months and nine months ended September 30, 2026 increased 18% and 23%, respectively, compared to the same periods in 2025. This increase was primarily driven by DAWNZERA product sales.

Research and development, or R&D, revenue for the three and nine months ended September 30, 2026 increased compared to the same periods in 2025 due to multiple payments we earned for programs advancing under our R&D collaborations and excluding the $280 million upfront payment we earned for the global license of sapablursen to Ono Pharmaceutical Co., Ltd. in the second quarter of 2025.

WAINUA (Eplontersen) Collaboration with AstraZeneca

Our financial results for the three and nine months ended September 30, 2026 and 2025 reflected the cost-sharing provisions related to our collaboration with AstraZeneca to develop and commercialize WAINUA for the treatment of ATTR. From inception through December 31, 2025, AstraZeneca was responsible for 55 percent of the costs associated with the ongoing global Phase 3 development program. Beginning on January 1, 2026, AstraZeneca was responsible for 75 percent of costs for development activities intended solely to support U.S. regulatory approvals and 87.5 percent of costs for development activities intended to support global regulatory approvals. Because we were leading and conducting the Phase 3 development program, we recognized as R&D revenue the percentage of cost-share funding AstraZeneca was responsible for, net of our share of AstraZeneca's development expenses, in the same period we incurred the related development expenses.

In June 2026, we exercised our right to opt-out of the shared responsibility and costs for development and U.S. commercialization activities under the agreement. The opt-out became effective on August 1, 2026. Effective August 1, 2026, AstraZeneca is responsible for 100 percent of WAINUA costs under this agreement.

Prior to August 2026, AstraZeneca was responsible for the majority of the medical affairs and commercial costs in the U.S. and all costs associated with bringing WAINUA to market outside the U.S. As a result, we recognized cost-share funding we received from AstraZeneca related to these activities as a reduction of our medical affairs and commercialization expenses, which we classified as R&D and selling, general and administrative, or SG&A expenses, respectively.

The following table sets forth information on revenue and expenses under this collaboration (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
WAINUA joint development revenue	$ 3.7	$ 9.7	$ 37.6	$ 32.5
Research and development expenses related to Phase 3 development of WAINUA	15.5	19.5	55.1	65.8
Medical affairs expenses for WAINUA	1.0	1.7	5.6	5.7
Commercialization expenses for WAINUA	2.6	7.5	18.9	22.0

Operating Expenses

The following table sets forth information on operating expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses, excluding non-cash compensation expense related to equity awards	$ 330.0	$ 285.8	$ 974.9	$ 817.0
Non-cash compensation expense related to equity awards	43.4	31.1	132.3	90.6
Total operating expenses	$ 373.4	$ 316.9	$ 1,107.2	$ 907.6

Operating expenses, excluding non-cash compensation expense related to equity awards, for the three months and nine months ended September 30, 2026 increased compared to the same periods in 2025. SG&A expenses increased as anticipated year over year primarily due to investments related to the commercialization efforts for TRYNGOLZA, DAWNZERA and ZANVASTRO. We expect our operating expenses, excluding non-cash compensation expense related to equity awards, to continue to increase during the remainder of 2026 as we advance our commercialization activities.

Non-cash compensation expense related to equity awards for the three and nine months ended September 30, 2026 increased compared to the same periods in 2025 due to a higher stock price on the grant date of annual equity awards in 2026 compared to 2025 and increased headcount. We employed 1,485 people as of September 30, 2026 compared to 1,225 people as of September 30, 2025. We believe non-cash compensation expense related to equity awards is not indicative of our operating results or cash flows from our operations.

Cost of Sales

Our cost of sales is comprised of costs related to our commercial revenue, which consisted of manufacturing costs, transportation and freight, indirect overhead costs associated with the manufacturing and distribution of TRYNGOLZA, DAWNZERA, TEGSEDI and WAYLIVRA and associated period costs.

Cost of sales for newly launched products, such as TRYNGOLZA, DAWNZERA and ZANVASTRO, does not include the full cost of manufacturing until we manufacture and sell additional inventory after exhausting pre-launch inventory, which we previously recorded as R&D expense.

The following table sets forth information on cost of sales (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Cost of sales, excluding non-cash compensation expense related to equity awards	$ 3.1	$ 2.2	$ 8.5	$ 7.1
Non-cash compensation expense related to equity awards	0.2	0.1	0.5	0.9
Total cost of sales	$ 3.3	$ 2.3	$ 9.0	$ 8.0

Research, Development and Patent Expenses

Our research, development and patent expenses consist of expenses for drug discovery, drug development, medical affairs, manufacturing and development chemistry and R&D support expenses.

The following table sets forth information on research, development and patent expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research, development and patent expenses, excluding non-cash compensation expense related to equity awards	$ 191.5	$ 197.0	$ 569.0	$ 575.4
Non-cash compensation expense related to equity awards	24.4	20.8	74.2	60.6
Total research, development and patent expenses	$ 215.9	$ 217.8	$ 643.2	$ 636.0

Drug Discovery

We use our proprietary technologies to generate information about the function of genes and to determine the value of genes as drug discovery targets. We use this information to direct our own drug discovery research, and that of our partners. Drug discovery is also the function that is responsible for advancing our core technology. This function is also responsible for making investments in complementary technologies to expand the reach of our technologies.

The following table sets forth information on drug discovery expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Drug discovery expenses, excluding non-cash compensation expense related to equity awards	$ 28.9	$ 28.4	$ 86.6	$ 85.7
Non-cash compensation expense related to equity awards	5.4	4.0	15.4	11.7
Total drug discovery expenses	$ 34.3	$ 32.4	$ 102.0	$ 97.4

Drug discovery expenses, excluding non-cash compensation expense related to equity awards, were essentially flat in the three and nine months ended September 30, 2026 compared to the same periods in 2025.

Drug Development

The following table sets forth drug development expenses, including expenses for our marketed medicines and those in Phase 3 development for which we have incurred significant costs (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Eplontersen	$ 13.3	$ 19.1	$ 47.2	$ 64.5
Donidalorsen	3.8	3.7	9.2	11.8
Olezarsen	10.6	25.6	37.7	68.7
Zilganersen	1.9	2.2	6.6	9.7
Obudanersen	10.6	6.8	30.2	24.3
Ulefnersen	3.4	2.8	8.9	8.0
Other development projects	23.7	23.6	67.7	59.3
Development overhead expenses	34.0	37.0	102.1	105.2
Total drug development expenses, excluding non-cash compensation expense related to equity awards	101.3	120.8	309.6	351.5
Non-cash compensation expense related to equity awards	11.0	9.2	34.3	26.2
Total drug development expenses	$ 112.3	$ 130.0	$ 343.9	$ 377.7

Our development expenses, excluding non-cash compensation expense related to equity awards, decreased for the three and nine months ended September 30, 2026 compared to the same periods in 2025 as several late-stage studies ended.

We may conduct multiple clinical trials on a drug candidate, including multiple clinical trials for the various indications we may be studying. Furthermore, as we obtain results from trials, we may elect to discontinue clinical trials for certain drug candidates in certain indications in order to focus our resources on more promising drug candidates or indications. Our Phase 1 and Phase 2 programs are clinical research programs that fuel our Phase 3 pipeline. When our medicines are in Phase 1 or Phase 2 clinical trials, they are in a dynamic state in which we may adjust the development strategy for each medicine. Although we may characterize a medicine as “in Phase 1” or “in Phase 2,” it does not mean that we are conducting a single, well-defined study with dedicated resources. Instead, we allocate our internal resources on a shared basis across numerous medicines based on each medicine’s particular needs at that time. This means we are constantly shifting resources among medicines. Therefore, what we spend on each medicine during a particular period is usually a function of what is required to keep the medicines progressing in clinical development, not what medicines we think are most important. For example, the number of people required to start a new study is large, the number of people required to keep a study going is modest and the number of people required to finish a study is large. However, such fluctuations are not indicative of a shift in our emphasis from one medicine to another and cannot be used to accurately predict future costs for each medicine. Because we always have numerous medicines in preclinical and varying stages of clinical research, the fluctuations in expenses from medicine to medicine, in large part, offset one another. If we partner a medicine, it may affect the size of a trial, its timing, its total cost and the timing of the related costs.

Medical Affairs

Our medical affairs function is responsible for funding and coordinating investigator-sponsored trials, communicating scientific and clinical information to healthcare providers, medical professionals and patients, and managing publications.

The following table sets forth information on medical affairs expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Medical affairs expenses, excluding non-cash compensation expense related to equity awards	$ 11.5	$ 8.9	$ 32.8	$ 22.3
Non-cash compensation expense related to equity awards	2.5	1.0	7.1	3.8
Total medical affairs expenses	$ 14.0	$ 9.9	$ 39.9	$ 26.1

Medical affairs expenses, excluding non-cash compensation expense related to equity awards, increased in the three and nine months ended September 30, 2026 compared to the same periods in 2025 as we continued advancing our late-stage pipeline.

Manufacturing and Development Chemistry

Expenditures in our manufacturing and development chemistry function consist primarily of personnel costs, specialized chemicals for oligonucleotide manufacturing, validation batches to support regulatory approvals, laboratory supplies and outside services. Our manufacturing and development chemistry function is responsible for providing drug supplies to drug development and our collaboration partners. Our manufacturing procedures include testing to satisfy good laboratory and good manufacturing practice requirements.

The following table sets forth information on manufacturing and development chemistry expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Manufacturing and development chemistry expenses, excluding non-cash compensation expense related to equity awards	$ 23.3	$ 15.8	$ 64.2	$ 51.6
Non-cash compensation expense related to equity awards	2.5	2.1	8.5	5.8
Total manufacturing and development chemistry expenses	$ 25.8	$ 17.9	$ 72.7	$ 57.4

Manufacturing and development chemistry expenses, excluding non-cash compensation expense related to equity awards, increased in the three and nine months ended September 30, 2026 due to the timing of manufacturing performed by our contract manufacturing organizations for drug product and active pharmaceutical ingredients related to several late-stage programs.

R&D Support

In our research, development and patent expenses, we include support costs such as rent, repair and maintenance for buildings and equipment, utilities, depreciation of laboratory equipment and facilities, amortization of our intellectual property, information technology costs, procurement costs and waste disposal costs. We call these costs R&D support expenses.

The following table sets forth information on R&D support expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Occupancy	$ 10.5	$ 7.8	$ 30.3	$ 21.6
Personnel costs	9.4	7.6	25.8	21.8
Patent expenses	0.1	2.6	3.0	4.1
Computer software and licenses	0.4	3.4	1.3	9.8
Insurance	1.0	0.9	1.7	2.6
Taxes	1.2	0.7	3.7	3.9
Other	3.9	0.1	10.0	0.5
Total R&D support expenses, excluding non-cash compensation expense related to equity awards	26.5	23.1	75.8	64.3
Non-cash compensation expense related to equity awards	3.0	4.5	8.9	13.1
Total R&D support expenses	$ 29.5	$ 27.6	$ 84.7	$ 77.4

R&D support expenses, excluding non-cash compensation expense related to equity awards, increased in the three and nine months ended September 30, 2026 compared to the same periods in 2025 primarily due to increased occupancy costs attributed to our lease of the laboratory and office space facility that commenced in September 2025. In addition, personnel costs increased period over period due to increased headcount.

Selling, General and Administrative Expenses

SG&A expenses include personnel, information technology systems and outside costs associated with the commercialization and pre-commercialization activities for our medicines and costs to support our company, our employees and our stockholders including, legal, human resources, investor relations and finance. Additionally, we include in SG&A expenses such costs as rent, repair and maintenance of buildings and equipment, depreciation and utilities costs that we need to support the corporate functions listed above. We also include fees we owe under our in-licensing agreements related to SPINRAZA and QALSODY and cost sharing payments associated with shared commercialization activities under our WAINUA collaboration with AstraZeneca.

The following table sets forth information on SG&A expenses (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Selling, general and administrative expenses, excluding non-cash compensation expense related to equity awards	$ 135.4	$ 86.6	$ 397.4	$ 234.6
Non-cash compensation expense related to equity awards	18.8	10.2	57.6	29.1
Total selling, general and administrative expenses	$ 154.2	$ 96.8	$ 455.0	$ 263.7

SG&A expenses, excluding non-cash compensation expense related to equity awards, increased in the three and nine months ended September 30, 2026 compared to the same periods in 2025 primarily due to investments related to commercialization efforts for TRYNGOLZA, DAWNZERA and ZANVASTRO. We expect SG&A expenses to increase as we continue to invest in our independent commercial launches.

Investment Income

The following table sets forth information on investment income (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Investment income	$ 19.5	$ 23.7	$ 65.3	$ 73.0

Investment income for the three and nine months ended September 30, 2026 decreased compared to the same periods in 2025 due to a decrease in our cash balance during the nine months ended September 30, 2026 compared to the same period in 2025. Our cash balance decreased due to the repayment upon maturity of our 0% Notes due 2026 in April 2026.

Interest Expense

The following table sets forth information on interest expense (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Convertible notes:				
Non-cash amortization of debt issuance costs	$ 1.7	$ 1.6	$ 5.5	$ 4.6
Interest expense payable in cash	2.5	2.5	7.5	7.6
Interest on mortgage for manufacturing facility	0.1	0.1	0.3	0.2
Other	0.3	-	1.1	-
Total interest expense	$ 4.6	$ 4.2	$ 14.4	$ 12.4

Interest Expense Related to Sale of Future Royalties

We recorded $4.1 million and $39.0 million of interest expense related to the sale of future royalties in the three and nine months ended September 30, 2026, respectively, compared to $18.0 million and $55.4 million in the same periods in 2025. These amounts are related to the Royalty Pharma Investments, or Royalty Pharma, transaction, in which we sold a minority interest in our future SPINRAZA and pelacarsen royalties to Royalty Pharma for a $500 million upfront payment and $625 million of potential future payments. In September 2026, we began recognizing contra-interest expense related to the Royalty Pharma arrangement due to a change in estimate. Refer to Part I, Item 1, Note 10, *Liability Related to Sale of Future Royalties*, in the Notes to Condensed Consolidated Financial Statements for further details related to the Royalty Pharma arrangement.

Gain on Investments

The following table sets forth information on gain on investments, net (in millions):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Gain on investments	$ 9.5	$ 29.9	$ 21.9	$ 9.4

The period over period fluctuations in gain on investments were primarily due to changes in the fair value of investments in publicly traded biotechnology companies.

Net Loss and Net Loss per Share

The following table sets forth information on net loss and net loss per share (in millions, except per share amounts):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Net loss	$ (110.0)	$ (128.6)	$ (317.1)	$ (152.0)
Basic and diluted net loss per share	$ (0.66)	$ (0.80)	$ (1.92)	$ (0.95)

The period-over-period fluctuations in our net loss were driven by factors discussed in the sections above.

Liquidity and Capital Resources

We have financed our operations primarily from research and development collaborative agreements. We also financed our operations from commercial revenue from SPINRAZA, WAINUA and QALSODY royalties and TEGSEDI and WAYLIVRA commercial revenue. In addition, we began earning commercial revenue from TRYNGOLZA product sales in late December 2024, DAWNZERA product sales in late August 2025 and ZANVASTRO product sales in September 2026. From our inception through September 30, 2026, we have earned approximately $9.6 billion in revenue. We have also financed our operations through the sale of our equity securities, the issuance of long-term debt and the sale of future royalties. From the time we were founded through September 30, 2026, we have raised net proceeds of approximately $2.9 billion from the sale of our equity securities. Additionally, from our inception through September 30, 2026, we have borrowed approximately $3.5 billion under long-term debt arrangements and received proceeds of $0.5 billion from the sale of future royalties to finance a portion of our operations.

From December 31, 2025 to September 30, 2026, our working capital decreased primarily due to a decrease in cash, cash equivalents and short-term investments, and our long-term obligations did not change significantly. In April 2026, we paid the remaining principal balance of our 0% Notes due 2026 upon maturity. Refer to Part I, Item 1, Note 11, *Convertible Debt*, in the Notes to Condensed Consolidated Financial Statements for further details.

The following table summarizes our contractual obligations, excluding our liability related to the sale of future royalties, as of September 30, 2026. The table provides a breakdown of when obligations become due.

Contractual Obligations	**Payments Due by Period (in millions)**		
(selected balances described below)	**Total**	**Less than 1 year**	**More than 1 year**
0% Notes due 2030 (principal payable)	$ 770.0	$ -	$ 770.0
1.75% Notes due 2028 (principal and interest payable)	595.2	10.1	585.1
Operating leases	458.3	36.4	421.9
Building mortgage payments (principal and interest payable)	8.8	8.8	-
Other obligations (principal and interest payable)	0.6	0.1	0.5
Total	$ 1,832.9	$ 55.4	$ 1,777.5

Our contractual obligations consist primarily of our convertible debt. In addition, we also have a facility mortgage, facility leases, equipment financing arrangements and other obligations. We believe our cash, cash equivalents and short-term investments, as well as plans for cash in the future, will be sufficient to fund our planned operations and these obligations. We have not entered into, nor do we currently have, any off-balance sheet arrangements (as defined under SEC rules).

Convertible Debt and Call Spread

Refer to Part I, Item 1, Note 11, *Convertible Debt*, in the Notes to Condensed Consolidated Financial Statements for the significant terms of each convertible debt instrument.

Operating Facilities

Refer to Part IV, Item 15, Note 7 of our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2025 for further details on our operating facilities.

Operating Leases

Refer to Part IV, Item 15, Note 7 of our audited financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2025 for further details on our operating leases.

Liability Related to Sale of Future Royalties

Refer to Part I, Item 1, Note 10, *Liability Related to Sale of Future Royalties,* in the Notes to Condensed Consolidated Financial Statements for further details on our royalty purchase agreement with Royalty Pharma.

Other Obligations

In addition to contractual obligations, we had outstanding purchase orders as of September 30, 2026 for the purchase of services, capital equipment and materials as part of our normal course of business.

We may enter into additional collaborations with partners which could provide for additional revenue to us and we may incur additional cash expenditures related to our obligations under any of the new agreements we may enter into. We currently intend to use our cash, cash equivalents and short-term investments to finance our activities. However, we may also pursue other financing alternatives, like issuing additional shares of our common stock, issuing debt instruments, refinancing our existing debt, securing lines of credit or executing royalty monetization agreements. Whether we use our existing capital resources or choose to obtain financing will depend on various factors, including the future success of our business, the prevailing interest rate environment and the condition of financial markets generally.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to changes in interest rates primarily from our investments in certain short-term investments. We primarily invest our excess cash in highly liquid short-term investments of the U.S. Treasury and reputable financial institutions, corporations, and U.S. government agencies with strong credit ratings. We typically hold our investments for the duration of the term of the respective instrument. We do not utilize derivative financial instruments, derivative commodity instruments or other market risk sensitive instruments, positions or transactions to manage exposure to interest rate changes. Accordingly, we believe that, while the securities we hold are subject to changes in the financial standing of the issuer of such securities, we are not subject to any material risks arising from changes in interest rates, foreign currency exchange rates, commodity prices, equity prices or other market changes that affect market risk sensitive instruments.

We are also exposed to changes in foreign currency exchange rates as we have foreign subsidiaries with functional currencies other than the U.S. dollar. We translate our subsidiaries' functional currencies into our reporting currency, the U.S. dollar. As a result, our financial position, results of operations and cash flows can be affected by market fluctuations in the foreign currencies to U.S. dollar exchange rate, which are difficult to predict. A hypothetical 10 percent change in foreign exchange rates during any of the periods presented would not have had a material impact on our condensed consolidated financial statements.

ITEM 4. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures that are designed to ensure that information we are required to disclose in our Exchange Act reports is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. We design and evaluate our disclosure controls and procedures recognizing that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance and not absolute assurance of achieving the desired control objectives.

In January 2026, we deployed a new global enterprise resource planning, or ERP, system for the company. We designed the ERP system to accurately maintain our financial records, support operational functionality and provide information to our management teams related to business operations. We have updated our internal control over financial reporting, where applicable, to accommodate related changes in our financial management processes resulting from this implementation. Except for changes in controls related to the new ERP system, there were no other changes to our internal control over financial reporting during nine months ended September 30, 2026 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

As of our most recently completed fiscal year and as of the end of the period covered by this Quarterly Report on Form 10-Q, we carried out an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer. Based on our evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective as of September 30, 2026. There have been no significant changes in our internal controls or in other factors that could significantly affect internal controls subsequent to September 30, 2026, except as mentioned above.

We also performed an evaluation of any changes in our internal controls over financial reporting that occurred during our last fiscal quarter and that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting. We conducted this evaluation under the supervision of and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer. That evaluation did not identify any changes in our internal controls over financial reporting that occurred during our latest fiscal quarter and that have materially affected, or are reasonably likely to materially affect, our internal controls over financial reporting.

PART II — OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

For details of legal proceedings, refer to Part I, Item 1, Note 12, *Legal Proceedings*, in the Notes to Condensed Consolidated Financial Statements.

ITEM 1A. RISK FACTORS

Investing in our securities involves a high degree of risk. You should carefully consider the following information about the risks described below, together with the other information contained in this report and in our other public filings in evaluating our business. If any of the following risks actually occur, our business could be materially harmed, and our financial condition and results of operations could be materially and adversely affected. As a result, the trading price of our securities could decline, and you might lose all or part of your investment. We have marked with an asterisk those risk factors that reflect substantive changes from the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2025.

Summary of Risk Factors

There are a number of risks related to our business and our securities. Some of the principal risks related to our business include the following:

- Our ability to generate substantial revenue from the sale of our medicines;
- The availability of adequate coverage and payment rates for our medicines;
- Our and our partners' ability to compete effectively;
- Our ability to successfully manufacture our medicines;
- Our ability to successfully develop and obtain marketing approvals for our medicines;
- Our ability to secure and maintain effective corporate partnerships;
- Our ability to sustain cash flows and achieve consistent profitability;
- Our ability to protect our intellectual property;
- Our ability to maintain the effectiveness of our personnel;
- The impacts of health epidemics, climate change, war and other events;
- Our dependence upon our own and third-party data, information technology systems and infrastructure; and
- The other factors set forth below.

Risks Related to the Commercialization of our Medicines

We have limited experience as a company in commercializing medicines and we will have to continue to invest significant resources to develop our capabilities. If we are unable to establish or maintain an effective commercialization infrastructure, or enter into agreements with third parties to commercialize our medicines, we may not be able to successfully commercialize our medicines.*

We have historically relied on third parties to commercialize our marketed medicines and have limited experience as a company in commercializing medicines. We currently have three independently launched medicines, TRYNGOLZA, DAWNZERA, and ZANVASTRO, and we expect to independently launch additional medicines in the future. Any failure to effectively commercialize our medicines, including our failure to allocate resources to our commercial launches efficiently or timely, could adversely impact the revenue we generate from our medicines. If the commercialization of our independently launched medicines and future sales of such are less successful than anticipated by us or our investors or securities analysts, our stock price could decline and our business may be harmed.

We will have to continue to invest significant financial and management resources to build and maintain the infrastructure required to successfully commercialize our medicines. We will need to establish and maintain effective sales teams for each of our independently launched medicines and there are significant risks involved in managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. We must also continue to scale-up existing internal support functions to aid our commercialization efforts, which will need to work effectively in coordination with commercial functional areas. Any failure to establish or maintain an effective commercialization infrastructure, including our sales, marketing, market access, distribution, and related capabilities, scale-up our existing support functions, or effectively integrate new functional areas, could adversely affect our ability to successfully commercialize our medicines.

If we choose to rely on third parties to assist us in commercializing our medicines, we may not be able to enter into collaborations or hire consultants or external service providers on acceptable financial terms, or at all. In addition, if we continue to engage third parties to assist us in the commercialization of our medicines, our product revenues and profitability may be lower than if we commercialized such medicines ourselves.

The proximity of our current and planned independent launches could increase the likelihood that the risks set forth above will occur.

If the market does not adopt our medicines, including our commercial medicines and our medicines in development, we are not likely to generate substantial revenues or become consistently profitable.

Even if our medicines are authorized for marketing, our success will depend upon the medical community, patients and third-party payers considering our medicines medically useful, cost-effective, safe and convenient. Even when the FDA or foreign regulatory authorities authorize our or our partners' medicines for commercialization, doctors may not prescribe our medicines to treat patients. Furthermore, we and our partners may not successfully commercialize additional medicines.

Additionally, in many of the markets where we or our partners may sell our medicines in the future, if we or our partners cannot agree with the government or other third-party payers regarding the price we can charge for our medicines, we or our partners may not be able to sell our medicines in that market. Similarly, cost control initiatives by governments or third-party payers could decrease the price received for our medicines or increase patient coinsurance to a level that makes our medicines, including our commercial medicines and our medicines in development, economically unviable. If the pricing of any of our medicines decreases for any reason, it will reduce our revenue for such medicine. For example, Biogen has in the past disclosed that SPINRAZA revenue decreased in part due to lower pricing in the U.S. and certain rest-of-world markets.

The degree of market adoption of our medicines, including our commercial medicines and our medicines in development, depends upon several factors, including the:

- receipt and scope of marketing authorizations;
- establishment and demonstration in the medical and patient community of the efficacy and safety of our medicines, public perception regarding our medicines and their potential advantages over competing products;
- cost and effectiveness of our medicines compared to other available therapies;
- patient convenience of the dosing regimen for our medicines; and
- reimbursement policies of government and third-party payers.

With respect to TRYNGOLA, it is the first and only treatment approved to reduce triglycerides and the risk of acute pancreatitis in people living with sHTG. As such, we are building a new market that requires health care practitioners to adopt a new approach to patient care, which takes time. Prescribers want to see how a patient responds to treatment with TRYNGOLZA before prescribing more broadly. This dynamic has and may continue to result in a more gradual launch ramp than we originally expected.

Based on the profile of our medicines, physicians, patients, patient advocates, payers or the medical community in general may not adopt any of the medicines that we or our partners may develop. For example, the product label for WAYLIVRA in the EU requires regular blood monitoring, which has negatively affected our ability to attract and retain patients for this medicine.

If government or other third-party payers fail to provide adequate coverage and payment rates for our medicines, including our commercial medicines and our medicines in development, or if healthcare reform measures increase our costs, decrease our sales, or negatively impact reimbursement for our products, our revenue will be limited.*

In both domestic and foreign markets, sales of our current and future products will depend in part upon the availability of coverage and reimbursement from third-party payers. The majority of patients in the U.S. who would fit within our target patient populations for our medicines have their healthcare supported by a combination of Medicare coverage, other government health programs such as Medicaid, managed care providers, private health insurers and other organizations. Coverage decisions may depend upon clinical and economic standards that disfavor new medicines when more established or lower cost therapeutic alternatives are already available or subsequently become available. Assuming coverage is approved, the resulting reimbursement payment rates might not be enough to make our medicines affordable. Even if favorable coverage status and adequate reimbursement rates are attained, less favorable coverage policies and reimbursement rates may be implemented in the future. Accordingly, our commercial medicines and our medicines in development will face competition from other therapies and medicines for limited financial resources. Furthermore, we or our partners may need to conduct post-marketing studies to demonstrate the cost-effectiveness of any future products to satisfy third-party payers. These studies might require us to commit a significant amount of management time and financial and other resources. In addition, third-party payers may never consider our future products as cost-effective and adequate third-party coverage and reimbursement might not be available to enable us to maintain price levels sufficient to realize an appropriate return on investment in product development.

Third-party payers, whether foreign or domestic, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In addition, in the U.S., no uniform policy of coverage and reimbursement for medicines exists among third-party payers. Therefore, coverage and reimbursement for medicines can differ significantly from payer to payer.

For example, the TRYNGOLZA launch for sHTG is at an early stage where a majority of TRYNGOLZA prescriptions require a medical exemption. Some health care practitioners are hesitant to prescribe a newly approved medicine, like TRYNGOLZA, before formal coverage with payors is in place. We are working with payors to establish coverage for TRYNGOLZA but our success may be limited. In addition, regarding DAWNZERA, we are experiencing increased price pressure from payors in the competitive HAE market.

Further, we believe that future coverage, reimbursement and pricing will likely be subject to increased restrictions both in the U.S. and in international markets. In the U.S., recent health reform measures have resulted in reductions in Medicare and other healthcare funding, and there have been several recent U.S. Congressional inquiries, legislation and executive orders designed to, among other things, reduce drug prices, increase competition (including by enhancing support for generic and biosimilar drugs), lower out-of-pocket drug costs for patients, curtail spread pricing practices by pharmacy benefit managers, and foster scientific innovation to promote better health care and improved health. For example, the Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers and continues to significantly impact the U.S. pharmaceutical industry. Since its enactment, there have been amendments and judicial, Congressional and executive branch challenges to certain aspects of the Affordable Care Act, as well as efforts to repeal or replace certain aspects of the Affordable Care Act. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand or revenue for our commercial medicines and our medicines in development.

In addition, the Inflation Reduction Act of 2022, or the IRA, includes key actions aimed at reducing the costs of prescription drugs and allows the U.S. Department of Health and Human Services, or HHS, to negotiate the price of certain single-source drugs covered under Medicare and establish a price cap on such drugs. The IRA, among other things, (1) directed HHS to negotiate the price of certain single-source drugs and biologics that have been on the market for at least seven years covered under Medicare, or the Medicare Drug Price Negotiation Program, and (2) imposed rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. Each year, up to 20 products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis.

The current U.S. Presidential administration is pursuing policies to reduce regulations and expenditures across government agencies, including at the FDA, HHS, Centers for Medicare & Medicaid Services, or CMS, and other related agencies, with a particular focus on most favored nation pricing equal to or lower than those paid in other developed nations. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose additional policy changes that create uncertainty for our business. For example, in furtherance of the administration's drug pricing initiatives, CMS has finalized or proposed several new mandatory and voluntary payment models to implement a most favored nation rebate model. These models are referred to as the GENEROUS Model, the GLOBE Model, and the GUARD Model. CMS is currently operationalizing the voluntary GENEROUS Model, and the GLOBE Model was finalized on September 30, 2026. We do not expect the implementation of the GLOBE Model to have a significant impact on our business. The GUARD Model has not yet been finalized, and it remains unclear whether any substantive changes will be made to such model prior to its implementation. These and other recent actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks.

Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. Our future product sales may be subject to additional discounts from list price in the form of rebates and discounts provided to covered entities under the Public Health Service Act 340B drug pricing program. Changes to the 340B program or to Medicare or Medicaid programs at the federal or state level, including outcomes of ongoing litigation in our industry, may impact our product prices and rebate liability.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Third-party coverage and reimbursement for medicines may not be available or adequate in either the U.S. or international markets, which would negatively affect the potential commercial success of our products, our revenue and our profits.

If we or our partners fail to compete effectively, our medicines, including our commercial medicines and our medicines in development, will not generate significant revenues.*

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. Our competitors engage in drug discovery throughout the world, are numerous, and include, among others, major pharmaceutical companies and specialized biopharmaceutical firms. In addition, other companies are engaged in developing RNA-targeted technology. Our competitors may, among other things, relative to our medicines or medicines in development:

- develop safer or more effective products;
- develop less costly products;
- receive more favorable reimbursement coverage;
- implement more effective approaches to sales and marketing;
- develop products that are more convenient to use than our medicines;
- have access to increased manufacturing capacity;
- obtain regulatory approval for products more quickly; or
- establish superior intellectual property positions.

These competitive advantages could make our medicines, including our commercial medicines and our medicines in development, obsolete or non-competitive.

Many of our competitors have substantially greater financial, technical and human resources than we do. In addition, many of these competitors have significantly greater experience than we do in conducting preclinical testing and human clinical studies of new pharmaceutical products, in obtaining FDA and other regulatory authorizations of such products and in commercializing such products.

There are several pharmaceutical and biotechnology companies engaged in the development or commercialization in certain geographic markets of products against targets that are also targets of products in our development pipeline or of medicines we are commercializing. For example:

- TRYNGOLZA faces competition in FCS from a commercial competitor and, in sHTG, could face competition from commercial competitors in the future;
- DAWNZERA faces competition from several commercial competitors, including an oral product, and could face competition from additional commercial competitors in the future;
- WAINUA faces competition from numerous competitors, including an oral product in markets outside the U.S.;
- HIBSAGO faces competition from several commercial competitors, including oral products, and could face competition from additional commercial competitors in the future;
- SPINRAZA faces competition from both a gene therapy product and an oral product for the treatment of SMA. Biogen has in the past disclosed that SPINRAZA revenue decreased due to a reduction in demand as a result of increased competition and that future sales of SPINRAZA may be adversely affected by competing products;
- QALSODY could face competition from a commercial competitor in the future; and
- Obudanersen, if approved, could face competition from commercial competitors in the future, including oral products.

For details regarding medicines that compete or may compete directly with our marketed medicines and late-stage medicines, refer to the section titled, *Competition*, in Part I, Item 1, *Business*, in our Annual Report on Form 10-K for the year ended December 31, 2025.

Certain of our partners are pursuing other technologies or developing other medicines either on their own or in collaboration with others, including our competitors, to treat some of the same diseases that our own programs target. Competition may negatively impact a partner's focus on and commitment to our medicines and, as a result, could delay or otherwise negatively affect the commercialization of our partnered medicines. Additionally, companies that are developing medicines that target the same patient populations as our medicines in development may compete with us to enroll participants in the clinical trials for such medicines, which could make it more difficult for us to complete enrollment for these clinical trials.

Our medicines could be subject to regulatory limitations following approval.

Following approval of a medicine, we and our partners must comply with comprehensive government regulations regarding the manufacture, marketing and distribution of medicines. The FDA and foreign regulatory bodies have the authority to impose significant restrictions on an approved medicine through the product label. We or our partners may not obtain the labeling claims necessary or desirable to successfully commercialize our medicines, including our commercial medicines and our medicines in development.

Promotional communications regarding prescription medicines must be consistent with the information in the product's approved labeling. Additionally, prescription medicines may be promoted only for the approved indication(s) in accordance with the approved label. The FDA and other regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, when approved, the FDA or a foreign regulatory authority may condition approval on the performance of post-approval clinical studies or patient monitoring, which could be time-consuming and expensive. For example, in connection with the conditional marketing approval for WAYLIVRA in the EU, we are required to conduct a post-authorization safety study to evaluate the safety of WAYLIVRA on thrombocytopenia and bleeding in FCS patients taking WAYLIVRA. If the results of such post-marketing studies are not satisfactory, the FDA, EC or foreign regulatory authorities may withdraw the marketing authorization or may condition continued marketing on commitments from us or our partners that may be expensive and time-consuming to fulfill.

If we or others identify side effects after any of our medicines are on the market, or if manufacturing problems occur subsequent to regulatory approval, or if we, our contract manufacturing organizations, or our partners fail to comply with regulatory requirements, we or our partners may, among other things, lose regulatory approval and be forced to withdraw products from the market, need to conduct additional clinical studies, incur restrictions on the marketing, distribution or manufacturing of the product, and/or change the labeling of our medicines.

We depend on our collaborations for the development and commercialization of certain of our medicines, including our collaborations with Biogen and GSK.*

We have entered into significant collaborative arrangements with biopharmaceutical companies, including separate collaborations with Biogen to develop and commercialize SPINRAZA and QALSODY, and a collaboration with GSK to develop and commercialize HIBSAGO. We entered into these collaborations primarily to:

- fund our development activities;
- seek and obtain regulatory approvals;
- manufacture; and
- successfully commercialize such medicines globally.

We are relying on these collaborators to obtain additional regulatory approvals for these medicines, generate additional clinical data for these medicines, manufacture these medicines, and successfully commercialize these medicines. In general, we cannot control the amount and timing of resources that our collaborators devote to our collaborations. If either of these collaborators fails to further develop, obtain regulatory approvals or additional regulatory approvals for, manufacture, or successfully commercialize medicine under the collaboration, revenues for such medicine would be negatively affected.

In addition, our collaborations may not continue for various reasons. Biogen and GSK can terminate the respective collaborations at any time. If either collaborator stops developing or commercializing a medicine under the applicable collaboration, we would have to seek or spend additional funding, and the commercialization for such medicine may be harmed.

We depend on our collaboration with AstraZeneca for the development and commercialization of WAINUA.*

In December 2021, we entered into a collaborative agreement with AstraZeneca under which we granted AstraZeneca exclusive worldwide rights to develop and commercialize WAINUA, with the parties sharing responsibility and costs for development and U.S. commercialization activities, and AstraZeneca assuming sole responsibility for commercialization outside the U.S. We exercised our opt-out right under the agreement and the opt-out process set forth in the agreement is underway. In general, we cannot control the amount and timing of resources that AstraZeneca devotes to our collaboration. In addition, we do not have control over whether AstraZeneca elects to terminate the collaborative arrangement. If AstraZeneca fails to further develop or successfully commercialize WAINUA for any reason, our revenues for WAINUA would be negatively affected.

If we are not successful in expanding our manufacturing capabilities or cannot manufacture our medicines or contract with a third party to manufacture our medicines at costs that allow us to charge competitive prices to buyers, we cannot market our products profitably.*

To successfully commercialize any of our medicines, we need to optimize and manage large-scale commercial manufacturing capabilities either on a standalone basis or through a third-party manufacturer. As our drug development and commercial pipeline increases and matures, we will have a greater need for clinical trial and commercial manufacturing capacity. We also must ensure that we have the manufacturing capabilities in place to support advances in our drug development activities, such as new chemistries. While we believe our current capabilities and those we obtain through third-party manufacturers support our manufacturing needs now, it will be important to expand our manufacturing infrastructure in the future, which will likely require substantial expenditures. If we are not successful in executing this expansion, or if the demand for any of our commercial medicines exceeds our expectations, it could limit our ability to meet our manufacturing requirements and commercial objectives in the future.

In addition, we have limited experience manufacturing pharmaceutical products of the chemical class represented by our medicines, called oligonucleotides, on a commercial scale for the systemic administration of a medicine. There are a small number of suppliers for certain capital equipment and raw materials that we use to manufacture our medicines, and some of these suppliers will need to increase their scale of production to meet our projected needs for commercial manufacturing. If a supplier chooses to devote more resources to other products, especially products with higher manufacturing capacity needs, that could impact such supplier’s capability to deliver our requirements timely. Further, we must continue to improve our manufacturing processes to allow us to reduce our drug costs. We or our partners may not be able to manufacture our medicines at a cost or in quantities necessary to make commercially successful products.

Manufacturers, including us, must adhere to the FDA's cGMP regulations and similar regulations in foreign countries, which the applicable regulatory authorities enforce through facilities inspection programs. We, our partners and our contract manufacturers may not comply or maintain compliance with cGMP, or similar foreign regulations. Non-compliance could significantly delay or prevent receipt of marketing authorizations for our medicines, including authorizations for our commercial medicines and our medicines in development, or could result in enforcement action after authorization that might limit the commercial success of our medicines.

We rely on third-party manufacturers to supply the drug substance and drug product for TRYNGOLZA, DAWNZERA and WAINUA and drug product for WAYLIVRA and ZANVASTRO. The operations of our suppliers, many of which are located outside of the United States, are subject to additional risks that are beyond our control. For example, tariffs on the raw materials, components, or equipment used to manufacture our products, or on our drug substance or finished products, will increase our manufacturing costs. On April 2, 2026, President Trump issued a proclamation that imposes tariffs of up to 100% on imported patented pharmaceutical products and active pharmaceutical ingredients, with specified exceptions. We believe the impacts of such tariffs on our business will be limited and manageable. In addition, merger and acquisition activity within the commercial manufacturing space could reduce the availability of resources from our third-party manufacturers. Delays or disruption to our own or third-party commercial manufacturing capabilities for any reason could limit the commercial success of our medicines.

Risks Related to the Development and Regulatory Approval of our Medicines

If we or our partners fail to obtain regulatory approval for our medicines and additional approvals for our commercial medicines, we or our partners cannot sell them in the applicable markets.

We cannot guarantee that any of our medicines will be considered safe and effective or will be approved for commercialization. In addition, it is possible that our commercial medicines may not be approved in additional markets or for additional indications. We and our partners must conduct time-consuming, extensive and costly clinical studies to demonstrate the safety and efficacy of each of our medicines before they can be approved or receive additional approvals for sale. We and our partners must conduct these studies in compliance with FDA regulations and with comparable regulations in other countries.

We and our partners may not obtain necessary regulatory approvals on a timely basis, if at all, for our medicines. It is possible that regulatory authorities will not approve our medicines for marketing or our commercial medicines in additional markets or for additional indications. If the FDA or another regulatory authority believes that we or our partners have not sufficiently demonstrated the safety or efficacy of any of our medicines, including our commercial medicines or our medicines in development, the authority will not approve such medicine or will require additional studies, which could be time-consuming and expensive and delay or harm commercialization of the medicine. For example, in August 2018 we received a complete response letter from the FDA regarding the new drug application for WAYLIVRA in which the FDA determined that the safety concerns identified with WAYLIVRA in our clinical development program outweighed the expected benefits of triglyceride lowering in patients with FCS. We also received a Notice of Non-Compliance Withdrawal Letter, or Non-W, from Health Canada for WAYLIVRA in November 2018.

The FDA or other comparable foreign regulatory authorities can delay, limit or deny approval of a medicine for many reasons, including:

- such authorities may disagree with the design or implementation of our clinical studies;
- we or our partners may be unable to satisfactorily demonstrate that a medicine is safe and effective for any indication;
- such authorities may not accept clinical data from studies conducted at clinical facilities that have deficient clinical practices or that are in countries where the standard of care is potentially different from that in the U.S.;
- we or our partners may be unable to demonstrate that our medicine's clinical and other benefits outweigh its safety risks to support approval;
- such authorities may disagree with the interpretation of data from preclinical or clinical studies;
- such authorities may find deficiencies in the manufacturing processes or facilities of third-party manufacturers who manufacture clinical and commercial supplies for our medicines; and
- the approval policies or regulations of such authorities or their prior guidance to us or our partners during clinical development may significantly change in a manner rendering our clinical data insufficient for approval.

Failure to receive marketing authorization for our medicines in development, or failure to receive additional marketing authorizations for our commercial medicines, or delays in these authorizations, could prevent or delay commercial introduction of the medicine, and, as a result, could negatively impact our ability to generate revenue from product sales.

If the results of clinical testing indicate that any of our medicines are not suitable for commercial use, we may need to abandon one or more of our drug development programs.

Drug discovery and drug development have inherent risks and the historical failure rate for drugs is high. Antisense medicines are a relatively new approach to therapeutics. If we cannot demonstrate that our medicines are safe and effective for human use in the intended indication(s), we may need to abandon one or more of our drug development programs.

Even if our medicines are successful in preclinical and human clinical studies, the medicines may not be successful in late-stage clinical studies. Similarly, topline, preliminary or interim data we release for any of our clinical studies may not be indicative of full or final results from such study.*

Successful results in preclinical or initial human clinical studies, including the Phase 2 results for some of our medicines in development, may not predict the results of subsequent clinical studies. If any of our medicines in Phase 3 clinical studies do not show sufficient safety and efficacy in patients with the targeted indication, or if such studies are discontinued for any other reason, it could negatively impact our development and commercialization goals for these medicines and our stock price could decline. In addition, we may release topline, preliminary or interim data for any of our clinical studies. The interim, topline or preliminary results we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. As a result, such data should be viewed with caution until the final data are available.

In the past, we have invested in clinical studies of medicines that have not met the primary clinical endpoints in their Phase 3 studies or have been discontinued for other reasons. For example, in September 2026, we and Novartis announced that the pelacarsen Phase 3 Lp(a) HORIZON study did not meet its primary endpoint of reducing the risk of cardiovascular events, a composite of cardiovascular death, non-fatal myocardial infarction, non-fatal stroke, and urgent coronary revascularization requiring hospitalization, compared to placebo. In addition, in July 2026, we and AstraZeneca announced that the CARDIO-TTRansform Phase 3 trial for eplontersen in patients with ATTR-CM did not meet the primary efficacy endpoint of the composite outcome of cardiovascular, or CV, mortality and recurrent CV clinical events up to Week 140 compared with placebo.

There are a number of factors that could cause a clinical study to fail or be delayed, including:

- the clinical study may produce negative or inconclusive results;
- regulators may require that we hold, suspend or terminate clinical research for noncompliance with regulatory requirements;
- we, our partners, the FDA or foreign regulatory authorities could suspend or terminate a clinical study due to adverse side effects of a medicine on subjects or lack of efficacy in the trial;
- we or our partners may decide, or regulators may require us, to conduct additional preclinical testing or clinical studies;
- enrollment in our clinical studies may be slower than we anticipate;
- we or our partners, including our independent clinical investigators, contract research organizations and other third-party service providers on which we rely, may not identify, recruit or train suitable clinical investigators at a sufficient number of study sites or timely enroll a sufficient number of study subjects in the clinical study;
- the institutional review board for a prospective site might withhold or delay its approval for the study;
- people who enroll in the clinical study may later drop out due to adverse events, a perception they are not benefiting from participating in the study, fatigue with the clinical study process or personal issues;
- a clinical study site may deviate from the protocol for the study;
- the cost of our clinical studies may be greater than we anticipate;
- our partners may decide not to exercise any existing options to license and conduct additional clinical studies for our medicines; and
- the supply or quality of our medicines or other materials necessary to conduct our clinical studies may be insufficient, inadequate or delayed.

Further, the FDA or other regulatory authorities could request, among other things, additional information or commitments before we can start or continue a clinical study, protocol amendments, increased safety monitoring, additional product labeling information, and post-approval commitments. This happened in connection with the conditional marketing approval for WAYLIVRA in the EU, as the European Commission is requiring us to conduct a post-authorization safety study to evaluate the safety of WAYLIVRA on thrombocytopenia and bleeding in FCS patients taking WAYLIVRA. In addition, under accelerated approval, the FDA is requiring completion of the ongoing Phase 3 trial for QALSODY to confirm the clinical benefit of QALSODY.

Moreover, our commercial medicines are chemically similar to each other. As a result, a safety observation we encounter with one of our medicines could have, or be perceived by a regulatory authority to have, an impact on a different medicine we are developing. This could cause the FDA or other regulators to ask questions or take actions that could harm or delay our ability to develop and commercialize our medicines or increase our costs. Any failure or delay in our clinical studies could reduce the commercial potential or viability of our medicines.

We depend on third parties to conduct clinical studies for our medicines and any failure of those parties to fulfill their obligations could adversely affect our development and commercialization plans.*

We depend on independent clinical investigators, contract research organizations and other third-party service providers to conduct our clinical studies for our medicines and expect to continue to do so in the future. For example, we use clinical research organizations, such as Icon Clinical Research Limited, Medpace, Inc., Parexel International Corporation, Syneos Health, Inc. and Thermo Fisher Scientific Inc. for the clinical studies for our medicines, including obudanersen and ulefnersen. We rely heavily on these parties for successful execution of our clinical studies, but do not control many aspects of their activities. For example, the investigators are not our employees, but we are responsible for ensuring that such investigators conduct each of our clinical studies in accordance with the general investigational plan and approved protocols for the study. Third parties may not complete activities on schedule or may not conduct our clinical studies in accordance with regulatory requirements or our stated protocols. For example, some of our key vendors have in the past experienced labor shortages, which impacted their ability to perform services for us for certain of our clinical trials. Subsequent failures of these third parties to carry out their obligations, or a termination of our relationship with such third parties, could delay or prevent the development, marketing authorization and commercialization of our medicines.

In addition, while we do not have any clinical trial sites in Russia, Ukraine, Gaza or Iran, we do have a limited number of clinical trial sites in Israel, Turkey and Cyprus that may be materially impacted by the war between Russia and Ukraine and the armed conflict between the United States/Israel and Iran and surrounding areas, or collectively, conflicts in Eastern Europe and the Middle East, and could result in difficulties enrolling or completing our clinical trials in such areas on schedule.

Since corporate partnering is part of our strategy to fund the advancement and commercialization of some of our development programs, if any of our collaborative partners fail to fund our collaborative programs, or if we cannot obtain additional partners, we may have to delay or stop progress on those drug development programs.*

To date, corporate partnering has played a significant role in our strategy to fund our development programs and to add key development resources. While we are now commercializing some of our medicines independently, we still plan to continue to rely on additional collaborative arrangements to develop and commercialize some of our unpartnered medicines. However, we may not be able to negotiate favorable collaborative arrangements for these drug programs. If we cannot continue to secure additional collaborative partners, our revenues could decrease and the development of our medicines could suffer.

Our corporate partners are developing and funding many of the medicines in our development pipeline. For example, we are relying on:

- AstraZeneca for the development and funding of eplontersen;
- GSK for development and funding of HIBSAGO;
- Ono for development and funding of sapablursen;
- Roche for development and funding of sefaxersen; and
- Otsuka for development and funding of ulefnersen.

If any of these pharmaceutical companies stops developing and funding these medicines, our business could suffer and we may not have, or be willing to dedicate, the resources available to develop these medicines on our own. Our collaborators can terminate their relationships with us under certain circumstances, many of which are outside of our control. For example, in 2025, Novartis decided to return the clinical development program for a follow on to pelacarsen, and in 2022, Pfizer and Bayer decided to discontinue the clinical development programs for vupanorsen and fesomersen, respectively.

Even with funding from corporate partners, if our partners do not effectively perform their obligations under our agreements with them, it would delay or stop the progress of our drug development and commercial programs.

In addition to receiving funding, we enter into collaborative arrangements with third parties to:

- conduct clinical studies;
- seek and obtain marketing authorizations; and
- manufacture and commercialize our medicines.

Once we have secured a collaborative arrangement to further develop and commercialize one of our drug development programs, such as our collaborations with AstraZeneca, Biogen, GSK, Ono, Otsuka and Roche, these collaborations may not continue or result in commercialized medicines, or may not progress as quickly as we anticipated.

For example, a collaborator such as AstraZeneca, Biogen, GSK, Ono, Otsuka or Roche, could determine that it is in its financial interest to:

- pursue alternative technologies or develop alternative products that may be competitive with the medicine that is part of the collaboration with us;
- pursue higher-priority programs or change the focus of its own development programs; or
- devote fewer resources to our medicines than it does to its own medicines.

If any of these occur, it could affect our partner's commitment to the collaboration with us and could delay or otherwise negatively affect the commercialization of our medicines, including DAWNZERA, HIBSAGO, QALSODY, SPINRAZA, WAINUA, sapablursen, sefaxersen, and ulefnersen.

We may not be able to benefit from designations for our medicines from regulatory authorities that are intended to confer benefits such as financial incentives or an accelerated regulatory pathway.*

In the U.S., under the Orphan Drug Act, the FDA may designate a medicine as an Orphan Drug if it is intended to treat a rare disease or condition affecting fewer than 200,000 individuals in the U.S. Orphan Drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but it can provide financial incentives, such as tax advantages and user-fee waivers, as well as longer regulatory exclusivity periods. The FDA has granted Orphan Drug designation to WAINUA for the treatment of patients with ATTR, to obudanersen for the treatment of patients with Angelman syndrome, to salanersen for the treatment of patients with SMA, to ulefnersen for the treatment of patients with FUS-ALS, and to some of our earlier stage medicines. The FDA and EMA have granted Orphan Drug designation to DAWNZERA for the treatment of patients with HAE, to QALSODY for the treatment of patients with SOD1-ALS, to TRYNGOLZA for the treatment of patients with FCS, to WAYLIVRA for the treatment of patients with FCS, to ZANVASTRO for the treatment of patients with AxD, to sapablursen for the treatment of patients with PV, and to some of our earlier stage medicines. In addition, the EMA has granted Orphan Drug designation to WAYLIVRA for the treatment of patients with FPL. Even if approval is obtained for a medicine that has been designated as an Orphan Drug, we may lose Orphan Drug exclusivity if the FDA or EMA determines that the request for designation was materially defective or if we cannot assure sufficient quantity of the applicable medicine to meet the needs of patients with the rare disease or condition, or if a competitor is able to gain approval for the same or a substantially similar medicine in a safer or more effective form or that makes a major contribution to patient care. If we lose Orphan Drug exclusivity on any of our medicines, we may face increased competition and lose market share for such medicine.

We may also seek rare pediatric disease designation for some of our medicines. The FDA defines "rare pediatric disease" as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years or is a rare disease or condition within the meaning of the Orphan Drug Act. Designation of a medicine as a medicine for a rare pediatric disease does not guarantee that a marketing application for such medicine will meet the eligibility criteria for a rare pediatric disease priority review voucher, or PRV, at the time the application is approved. Under the Federal Food, Drug and Cosmetic Act, we will need to request a rare pediatric disease PRV in our original marketing application for any potential medicine for which we have received rare pediatric disease designation. The FDA may determine that a marketing application for any such medicine, if approved, does not meet the eligibility criteria for a PRV. Under the current statutory sunset provisions, the PRV program will sunset after September 30, 2029, and the FDA may not award PRVs under this program after such date.

Risks Associated with our Businesses as a Whole

Risks related to our financial condition

If we fail to obtain timely funding, we may need to curtail or abandon some of our programs.

Many of our medicines are undergoing clinical studies or are in the early stages of research and development. Most of our programs will require significant additional research, development, manufacturing, preclinical and clinical testing, marketing authorizations, preclinical activities and commitment of significant additional resources prior to their successful commercialization. In addition, as we commercialize more medicines on our own, we will need to invest significant financial resources to continue developing the infrastructure required to successfully commercialize our medicines, including building and maintaining new support functions, scaling up existing internal support functions and expanding our manufacturing capabilities. All of these activities will require significant cash. As of September 30, 2026, we had cash, cash equivalents and short-term investments equal to $1.9 billion. If we or our partners do not meet our goals to successfully commercialize our medicines, including our commercial medicines, or to license certain medicines and proprietary technologies, we will need additional funding in the future. Our future capital requirements will depend on many factors such as:

- successful commercialization of our commercial medicines;
- the profile and launch timing of our medicines in development;
- changes in existing collaborative relationships and our ability to establish and maintain additional collaborative arrangements;
- continued scientific progress in our research, drug discovery and development programs;
- the size of our programs and progress with preclinical and clinical studies;
- the time and costs involved in obtaining marketing authorizations;
- competing technological and market developments, including the introduction by others of new therapies that address our markets; and
- our manufacturing requirements and capacity to fulfill such requirements.

If we need additional funds, we may need to raise them through public or private financing. Additional financing may not be available on acceptable terms or at all. If we raise additional funds by issuing equity securities, the shares of existing stockholders will be diluted and the price, as well as the price of our other securities, may decline. For example, in September 2024, we completed an underwritten public offering of 11,500,000 shares of our common stock for total net proceeds, after deducting underwriting discounts and commissions and other offering expenses payable by us, of approximately $489.1 million. If adequate funds are not available or not available on acceptable terms, we may have to cut back on one or more of our research, drug discovery or development programs, or commercial operations. Alternatively, we may obtain funds through arrangements with collaborative partners or others, which could require us to give up rights to certain of our technologies or medicines.

We have incurred losses, and our business will suffer if we fail to consistently achieve profitability in the future.

Because drug discovery and development require substantial lead-time and money prior to commercialization, our expenses have generally exceeded our revenue since we were founded in January 1989. As of September 30, 2026, we had an accumulated deficit of approximately $2.9 billion and stockholders' equity of approximately $0.4 billion. Most of our income has historically come from collaborative arrangements, including commercial revenue from royalties and R&D revenue, with additional income from research grants and the sale or licensing of our patents, as well as interest income. We will need to continue investing significant financial resources to commercialize medicines on our own, and we expect our future revenue to be driven primarily by commercial sales. If we do not earn substantial revenue from commercial sales, we may incur additional operating losses in the future, which could restrict our ability to successfully develop additional medicines or sustain future profitability.

Risks related to our intellectual property

If we cannot protect our patent rights or our other proprietary rights, others may compete more effectively against us.*

Our success depends to a significant degree upon whether we can continue to develop, secure and maintain intellectual property rights to proprietary products and services. However, we may not receive issued patents on any of our pending patent applications in the U.S. or in other countries and we may not be able to obtain, maintain or enforce our patents and other intellectual property rights, any of which could impact our ability to compete effectively. In addition, the scope of any of our issued patents may not be sufficiently broad to provide us with a competitive advantage. Furthermore, other parties may successfully challenge, invalidate or circumvent our issued patents or patents licensed to us so that our patent rights do not create an effective competitive barrier or revenue source. For example, on June 22, 2026, Biogen, Cold Spring Harbor Laboratory, and Ionis filed a patent infringement lawsuit in the District Court of Delaware following notice that Somerset Therapeutics, LLC had filed an Abbreviated New Drug Application, or ANDA, seeking approval to commercialize a generic version of SPINRAZA and alleging that certain patents covering SPINRAZA and its use are invalid or would not be infringed. If Somerset Therapeutics obtains a favorable ruling in such lawsuit, our revenues from SPINRAZA could be materially adversely affected.

We cannot be certain that the U.S. Patent and Trademark Office, or U.S. PTO, and courts in the U.S. or the patent offices and courts in foreign countries will consider the claims in our patents and applications covering our commercial medicines, or any of our medicines in development, as patentable. Method-of-use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products off-label. Although off-label prescriptions may infringe or contribute to the infringement of method-of-use patents, the practice is common and such infringement is difficult to prevent, even through legal action.

If we or any licensor partner loses or cannot obtain patent protection for our commercial medicines or any of our medicines in development, it could have a material adverse impact on our business.

Intellectual property litigation could be expensive and prevent us from pursuing our programs.*

From time to time, we have to defend our intellectual property rights. If we are involved in an intellectual property dispute, we may need to litigate to defend our rights or assert them against others. Disputes can involve arbitration, litigation or proceedings declared by the U.S. PTO or the International Trade Commission or foreign patent authorities. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. For example, in September 2025, we filed a claim against Arrowhead Pharmaceuticals, Inc., or Arrowhead, for patent infringement over Arrowhead's commercialization of plozasiran, and Arrowhead filed a counterclaim against us seeking to invalidate that same patent. We are also a party to ongoing litigation involving a third party seeking regulatory approval to commercialize a generic version of SPINRAZA in the U.S. as described above. For details regarding these proceedings, refer to Part II, Item 1, *Legal Proceedings*.

If a third party claims that our medicines or technology infringe its patents or other intellectual property rights, we may have to discontinue an important product or product line, alter our products and processes, pay license fees or cease certain activities. We may not be able to obtain a license to needed intellectual property on favorable terms, if at all. There are many patents issued or applied for in the biotechnology industry, and we may not be aware of patents or patent applications held by others that relate to our business. This is especially true since patent applications in the U.S. are filed confidentially for the first 18 months. Moreover, the validity and breadth of biotechnology patents involve complex legal and factual questions for which important legal issues remain.

Risks related to product liability

We are exposed to potential product liability claims, and insurance against these claims may not be available to us at a reasonable rate in the future or at all.

Our business exposes us to potential product liability risks that are inherent in the testing, manufacturing, marketing and sale of therapeutic products, including potential product liability claims related to our commercial medicines and our medicines in development. We have clinical study insurance coverage and commercial product liability insurance coverage. However, this insurance coverage may not be adequate to cover claims against us, or be available to us at an acceptable cost, if at all. Regardless of their merit or eventual outcome, product liability claims may result in decreased demand for our medicines, injury to our reputation, withdrawal of clinical study volunteers and loss of revenues. Thus, whether or not we are insured, a product liability claim or product recall may result in losses that could be material.

Risks related to our personnel

The loss of key personnel, or the inability to attract and retain highly skilled personnel, could make it more difficult to run our business and reduce our likelihood of success.

We are dependent on the principal members of our management, scientific and commercial staff. We do not have employment agreements with any of our employees that would prevent them from leaving us. The loss of our key management, scientific or commercial employees might slow the achievement of important research and development or commercial goals. It is also critical to our success that we recruit and retain qualified scientific personnel to perform research and development work and that we recruit and retain qualified marketing, sales, market access, distribution, and related personnel to commercialize our medicines. We may not be able to attract and retain skilled and experienced personnel on acceptable terms because of intense competition for experienced personnel among many pharmaceutical and health care companies, universities and non-profit research institutions. In addition, failure to succeed in clinical studies or in commercializing our medicines may make it more challenging to recruit and retain qualified personnel.

Risks related to health epidemics, climate change and other events

Our business may be adversely affected by health epidemics, climate change, extreme weather events, fires, earthquakes, war, civil or political unrest, terrorism or disruptions of the U.S. government.*

Our business could be adversely affected by health epidemics in regions where we or our partners are commercializing our medicines, have concentrations of clinical trial sites or other business operations, and could cause disruption in the operations of third-party manufacturers and contract research organizations upon whom we rely. For example, enrollment in some of our clinical trials was delayed due to the COVID-19 pandemic.

In recent years, extreme weather events and changing weather patterns have become more common. As a result, we are potentially exposed to varying natural disaster or extreme weather risks such as fires, hurricanes, tornadoes, droughts, floods, or other events that may result from the impact of climate change on the environment, any of which could impact our business and manufacturing operations. The potential impacts of climate change may also include increased operating costs associated with additional regulatory requirements and investments in reducing energy, water use and greenhouse gas emissions. In addition, we currently manufacture most of our research and clinical supplies in a manufacturing facility located in Carlsbad, California, and various regions within California have experienced numerous catastrophic wildfires. We manufacture the finished drug product for TRYNGOLZA, DAWNZERA, WAYLIVRA, and ZANVASTRO at third-party contract manufacturers. Biogen manufactures the finished drug product for SPINRAZA and QALSODY, AstraZeneca is responsible for WAINUA’s commercial drug supply, and GSK is responsible for HIBSAGO's commercial drug supply. The facilities and the equipment we, our partners and our contract manufacturers use to research, develop and manufacture our medicines would be costly to replace and could require substantial lead time to repair or replace.

Our facilities or those of our partners or contract manufacturers may be harmed by natural disasters or other events outside our control, such as earthquakes, war, civil or political unrest, deliberate acts of sabotage, terrorism or industrial accidents such as fire and explosion, whether due to human or equipment error, and if such facilities are affected by a disaster or other event, our development and commercialization efforts would be delayed. Although we possess property damage and business interruption insurance coverage, this insurance may not be sufficient to cover all of our potential losses and may not continue to be available to us on acceptable terms, or at all.

In addition, our development and commercialization activities could be harmed or delayed by staffing shortages or a shutdown or other significant disruption of the U.S. government, including the FDA or the U.S. PTO.

Risks related to personal information, cybersecurity, social media and artificial intelligence

We are dependent on data as well as information technology systems and infrastructure, which exposes us to data protection risks.*

We are dependent upon our own and third-party data as well as information technology systems and infrastructure, including mobile technologies, to operate our business. The personal information we process subjects us to stringent and evolving U.S. and foreign laws, rules and regulations, contractual obligations, industry standards and other obligations related to data privacy and security. Our obligations (including in relation to personal information) require us to maintain certain practices, including those in relation to cross-border transfers of data. The multitude and complexity of our information technology systems and infrastructure (including those of third parties with whom we work) make them vulnerable to a variety of evolving threats that may result in systems or data interruption, corruption, destruction, disruption of data integrity, malicious intrusion, or random attacks or other compromise (such as due to malfunctions, software vulnerabilities, natural disasters, telecommunications failures, malicious actors and personnel error). Data privacy or security incidents or breaches pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, customers or other business partners may be exposed to unauthorized persons. Like other companies in our industry, we have, from time to time, experienced threats to and security incidents related to our data and systems, including phishing/vishing attacks and attacks to the security of the systems of our third-party vendors and service providers.

Cyber-attacks are increasing in their frequency, sophistication and intensity, particularly as companies (including us) continue to leverage remote work structures. Advances in artificial intelligence, and our and our third parties' increased use of artificial intelligence, may heighten certain risks, including adversarial attacks, data leaks and unauthorized access, and may enhance bad actors' ability to conduct cyber-attacks more quickly and at greater scale. In addition, the number and frequency of cybersecurity events globally may be heightened during times of geopolitical tension or instability between countries, including, for example, the ongoing conflicts in Eastern Europe and the Middle East, as well as related political or economic responses and counter-responses by various global actors.

Cybersecurity related events could include the deployment or use of harmful malware or malicious code, denial-of-service attacks, credential stuffing attacks, credential harvesting attacks, social engineering attacks (including deep fakes and phishing/vishing attacks), ransomware and other means to affect, as applicable, the confidentiality, privacy, security, integrity or availability of our data as well as information systems and infrastructure (including that of third parties with whom we work). Our current, past and prospective business partners face similar risks and any security breaches of their systems (or those of third parties upon which they rely) could adversely affect our security posture. A security breach or a violation of obligations related to privacy or cybersecurity (including perceived breaches or violations) could result in any of the following, any of which could disrupt our business and result in increased costs or loss of revenue:

- harm our reputation;
- delay progress on the development of our medicines;
- compel us to comply with applicable security or data breach notification obligations (including laws);
- result in the diversion of monetary funds and other company resources;
- subject us to financial or other penalties, regulatory investigations or actions, including mandatory and costly corrective actions, and otherwise subject us to litigation or other liabilities; and
- require us to verify the correctness of database contents.

Risk mitigation strategies such as liability limitations in our contracts and insurance coverage may prove inadequate if there is a security breach or privacy violation. We do not maintain cyber insurance. While we have invested, and continue to invest, in the protection of our data and information technology systems and infrastructure as well as our compliance with relevant privacy and cybersecurity obligations, our efforts may not be successful. Non-compliance with relevant data protection obligations or a failure to secure our data, information technology systems or infrastructure could adversely affect our business and operations and result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm to us.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our medicines and the diseases our therapies are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear and create uncertainty and risk of noncompliance with regulations applicable to our business. Despite our efforts to provide guidelines and training to our employees regarding social media use and monitor social media communications, there is risk that the unauthorized use of social media by our employees to communicate about our products or business, or any inadvertent disclosure of material, nonpublic information through these means, may result in violations of applicable laws and regulations, which may result in significant legal and financial exposure and reputational damages that could have a material adverse impact on our business. Furthermore, negative posts or comments about us or our medicines on social media could seriously damage our reputation, brand image and goodwill. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business.

We use artificial intelligence in certain aspects of our business, and challenges with properly managing its use could adversely affect our business.

We incorporate artificial intelligence, or AI, solutions into certain aspects of our business, and applications of AI may become important in our operations over time. Our competitors or other third parties may incorporate AI into their businesses more quickly or more successfully than us, which could impair our ability to compete effectively and adversely affect our results of operations.

There are also significant risks involved in developing and deploying AI, and there can be no assurance that the usage of AI will enhance our medicines or the discovery or development of our product candidates or be beneficial to our business, including our efficiency or profitability. It is also uncertain how various laws will apply to content generated by AI. Various government authorities have proposed or enacted laws governing the development and use of AI technologies. We are subject to the risks of new or enhanced governmental or regulatory scrutiny, litigation, or other legal liability, ethical concerns, negative consumer perceptions as to automation and AI, or other complications that could adversely affect our business, reputation, or financial results.

Risks related to our securities and the global credit markets

If we do not progress in our programs as anticipated, the price of our securities could decrease.

For planning purposes, we estimate and may disclose the timing of a variety of clinical, regulatory and other milestones, such as when we anticipate a certain medicine will enter clinical trials, when we anticipate disclosing clinical data, when we anticipate completing a clinical study, when we anticipate filing an application for, or obtaining, marketing authorization, or when we or our partners plan to commercially launch a medicine. We base our estimates on present facts and a variety of assumptions, many of which are outside of our control. If we do not achieve milestones in accordance with our or our investors' or securities analysts' expectations, including milestones related to our commercial medicines and medicines in development, the price of our securities could decrease. In addition, our share price may be dependent upon the valuations and recommendations of the analysts who cover our business. If our results do not meet these analysts' forecasts, the expectations of our investors or the financial guidance we provide to investors in any period, the market price of our common stock could decline. Our ability to meet analysts' forecasts, investors' expectations and our financial guidance is substantially dependent on our ability to maintain or increase sales of our commercial medicines, both partnered and unpartnered.

If the price of our securities continues to be highly volatile, this could make it harder to liquidate your investment and could increase your risk of suffering a loss.

The market price of our common stock, like that of the securities of many other biopharmaceutical companies, has been and is likely to continue to be highly volatile. These fluctuations in our common stock price may significantly affect the trading price of our securities. During the 12 months preceding September 30, 2026, the closing market price of our common stock ranged from $86.50 to $43.81 per share. Many factors can affect the market price of our securities, including, for example, fluctuations in our operating results, financing transactions, announcements related to collaborations, clinical study results, technological innovations or new products being developed by us or our competitors, the commercial success of our approved medicines, governmental regulation, marketing authorizations, changes in payers' reimbursement policies, developments in patent or other proprietary rights and public concern regarding the safety of our medicines.

Broad market factors may materially harm the market price of our common stock irrespective of our operating performance. For example, events such as recent tariffs announcements and the ongoing conflicts in Eastern Europe and the Middle East have caused disruptions of global financial markets and resulted in increased volatility in the trading price of our common stock. In addition, industry factors may materially harm the market price of our common stock. Nasdaq, and the market for biotechnology companies in particular, have historically experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of the particular companies affected. The trading prices and valuations of these stocks, and of ours, may not be predictable. A loss of investor confidence in the market for biotechnology or pharmaceutical stocks or the stocks of other companies that investors perceive to be similar to us, the opportunities in the biotechnology and pharmaceutical market or the stock market in general, could depress our stock price regardless of our business, prospects, financial conditions or results of operations.

Negative conditions in the global economy, credit markets and financial services and other industries may adversely affect our business, financial condition or stock price.*

The global economy, credit and financial markets have experienced extreme volatility and disruptions recently, including as a result of tariffs announcements and the ongoing conflicts in Eastern Europe and the Middle East. These disruptions can result in severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, increases in inflation rates, disruptions of the global supply chain and energy markets and uncertainty about economic stability. Any such volatility and disruptions may have adverse consequences on us or the third parties on whom we rely. There can be no assurance that further deterioration in the global economy, credit and financial markets and confidence in economic conditions will not occur. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our operations, growth plans, financial performance or stock price. In addition, our insurance carriers and insurance policies covering all aspects of our business may become financially unstable or may not be sufficient to cover any or all of our losses and may not continue to be available to us on acceptable terms, or at all.

A variety of risks associated with operating our business and marketing our medicines internationally, including possible unfavorable regulatory, pricing and reimbursement, political, tax and labor conditions, could adversely affect our business. We or our partners are commercializing several medicines in countries outside the U.S. Because we have international operations, we are subject to numerous risks associated with international business activities, including:

- compliance with differing or unexpected regulatory requirements for our medicines and foreign employees;
- complexities associated with managing multiple payer reimbursement regimes, government payers or patient self-pay systems;
- difficulties in staffing and managing foreign operations;
- in certain circumstances, increased dependence on the commercialization efforts and regulatory compliance of third-party distributors or strategic partners;
- foreign government taxes, regulations and permit requirements;
- U.S. and foreign government tariffs, trade and export restrictions, price and exchange controls and other regulatory requirements;
- U.S. restrictions on the use of certain foreign biotech equipment and service providers, such as those set forth in the BIOSECURE Act;
- anti-corruption laws, including the Foreign Corrupt Practices Act, or the FCPA, and its equivalent in foreign jurisdictions;
- economic weakness, including inflation, natural disasters, war, acts of terrorism, political instability or public health issues or health epidemics, in particular foreign countries or globally;
- fluctuations in currency exchange rates, which could result in increased operating expenses and reduced revenue, and other obligations related to doing business in another country;
- the potential for a local seller, faced with higher local prices, importing medicines from an international market with lower prices rather than buying such medicines locally, which is referred to as parallel importation;
- compliance with tax, employment, privacy, immigration and labor laws, regulations and restrictions for employees living or traveling abroad;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.; and
- changes in diplomatic and trade relationships.

Our business activities outside of the U.S. are subject to the FCPA and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we operate, including the United Kingdom's Bribery Act 2010. In many other countries, the healthcare providers who prescribe pharmaceuticals are employed by their government, and the purchasers of pharmaceuticals are government entities; therefore, any dealings with these prescribers and purchasers may be subject to regulation under the FCPA. There is no certainty that all employees and third-party business partners (including our contract research organizations, contract manufacturing organizations, distributors, wholesalers, agents, contractors and other partners) will comply with anti-bribery laws. Importantly, we do not control the actions of manufacturers and other third-party agents, although we may be liable for their actions. Violation of these laws may result in civil or criminal sanctions, which could include monetary fines, criminal penalties, and disgorgement of past profits, which could have an adverse impact on our business and financial condition.

Provisions in our certificate of incorporation, bylaws and convertible notes documents and Delaware law may prevent stockholders from receiving a premium for their shares.*

Our certificate of incorporation provides for classified terms for the members of our board of directors. Our certificate also includes a provision that requires at least 66 2/3 percent of our voting stockholders to approve a merger or certain other business transactions with, or proposed by, any holder of 15 percent or more of our voting stock, except in cases where certain directors approve the transaction or certain minimum price criteria and other procedural requirements are met.

Our certificate of incorporation also requires that any action required or permitted to be taken by our stockholders must be taken at a duly called annual or special meeting of stockholders and may not be taken by written consent. In addition, only our board of directors, chairperson of the board or chief executive officer can call special meetings of our stockholders. We have in the past, and may in the future, implement a stockholders' rights plan, also called a poison pill, which could make it uneconomical for a third party to acquire our company on a hostile basis. In addition, our board of directors has the authority to fix the rights and preferences of, and issue shares of preferred stock, which may have the effect of delaying or preventing a change in control of our company without action by our stockholders.

The provisions of our convertible senior notes could make it more difficult or more expensive for a third party to acquire us. Upon the occurrence of certain transactions constituting a fundamental change, holders of the notes will have the right, at their option, to require us to repurchase all of their notes or a portion of their notes, which may discourage certain types of transactions in which our stockholders might otherwise receive a premium for their shares over the then-current market prices. As of September 30, 2026, we had two outstanding convertible senior notes, our 0% Notes due 2030, which mature in December 2030, and our 1.75% Notes due 2028, which mature in June 2028.

These provisions, as well as Delaware law, including Section 203 of the Delaware General Corporation Law, and other of our agreements, may discourage certain types of transactions in which our stockholders might otherwise receive a premium for their shares over then-current market prices, and may limit the ability of our stockholders to approve transactions that they think may be in their best interests.

Future sales of our common stock in the public market could adversely affect the trading price of our securities.*

Future sales of substantial amounts of our common stock in the public market, or the perception that such sales could occur, could adversely affect trading prices of our securities. For example, we may issue approximately 18.6 million shares of our common stock upon conversion of our 0% Notes due 2030 and 1.75% Notes due 2028.

Risks related to compliance with laws

Our operations are subject to extensive legal and regulatory requirements affecting the health care industry.

Our operations are subject to extensive legal and regulatory requirements affecting the health care industry, including federal, state, and foreign fraud and abuse (including anti-kickback laws and false claims laws), transparency laws, such as the federal Sunshine Act, and health information privacy and security laws, which are subject to change at any time. It is possible that government authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. Penalties for violations of applicable healthcare laws and regulations may include significant civil, criminal and administrative penalties, damages, disgorgement, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and additional reporting requirements and oversight if we enter into a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws. In addition, violations may also result in reputational harm, diminished profits and future earnings.

Because we use biological materials, hazardous materials, chemicals and radioactive compounds, if we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development and manufacturing activities involve the use of potentially harmful biological materials as well as materials, chemicals and various radioactive compounds that could be hazardous to human health and safety or the environment. We store most of these materials and various wastes resulting from their use at our facilities in Carlsbad, California pending ultimate use and disposal. We cannot completely eliminate the risk of contamination, which could cause:

- interruption of our research, development and manufacturing efforts;
- injury to our employees and others;
- environmental damage resulting in costly clean up; and
- liabilities under federal, state and local laws and regulations governing health and human safety, as well as the use, storage, handling and disposal of these materials and resultant waste products.

In such an event, we may be held liable for any resulting damages, and any liability could exceed our resources. Although we carry insurance for pollution liability in amounts and types that we consider commercially reasonable, the coverage or coverage limits of our insurance policies may not be adequate. If our losses exceed our insurance coverage, our financial condition would be adversely affected.

Our business is subject to changing regulations for corporate governance and public disclosure that has increased both our costs and the risk of noncompliance.

Each year we are required to evaluate our internal control systems to allow management to report on, and our Independent Registered Public Accounting Firm to attest to, our internal controls as required by Section 404 of the Sarbanes-Oxley Act. As a result, we continue to incur additional expenses and divert our management's time to comply with these regulations. In addition, if we cannot continue to comply with the requirements of Section 404 in a timely manner, we might be subject to sanctions or investigation by regulatory authorities, such as the SEC, the Public Company Accounting Oversight Board, or PCAOB, or The Nasdaq Global Select Market. Any such action could adversely affect our financial results and the market price of our common stock.

The SEC and other regulators have continued to adopt new rules and regulations and make additional changes to existing regulations that require our compliance. In July 2010, the Dodd-Frank Wall Street Reform and Protection Act, or the Dodd-Frank Act, was enacted, and in August 2022, the SEC adopted additional rules and regulations under the Dodd-Frank Act related to "say on pay" and proxy access. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which has and may in the future lead to additional compliance costs and impact the manner in which we operate our business.

Risks related to taxes

Our ability to use our net operating loss carryovers and certain other tax attributes may be limited.

Under the Internal Revenue Code of 1986, as amended, or the Code, a corporation is generally allowed a deduction for net operating losses, or NOLs, carried over from a prior taxable year. Under the Code, we can carry forward our NOLs to offset our future taxable income, if any, until such NOLs are used or expire. The same is true of other unused tax attributes, such as tax credits.

Under the current U.S. federal income tax law, U.S. federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such U.S. federal NOLs is limited to 80 percent of taxable income.

In addition, under Sections 382 and 383 of the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percentage-point cumulative change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our NOL carryforwards or other tax attributes is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. As a result of our merger with Akcea Therapeutics, Inc. in 2020, or the Akcea Merger, we are subject to the separate return limitation year, or SRLY, rules. Under the SRLY rules, our utilization of Akcea's pre-merger NOL and tax credit carryforwards is limited to the amount of income that Akcea contributes to our consolidated taxable income. The Akcea pre-merger tax attributes cannot be used to offset any of the income that Ionis contributes to our consolidated taxable income. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, in June 2024, California enacted Senate Bill 167, or SB 167, which, with certain exceptions, suspends the ability to use California net operating losses to offset California income and limits the ability to use California business tax credits to offset California taxes, for taxable years beginning after 2023 and before 2027.

Our future taxable income could be impacted by changes in tax laws, regulations and treaties.

A change in tax laws, treaties or regulations, or their interpretation, of any country in which we operate could materially affect us.

We could be subject to additional tax liabilities.

We are subject to U.S. federal, state, local and foreign income taxes, sales taxes in the U.S., withholding taxes and transaction taxes in foreign jurisdictions. Significant judgment is required in evaluating our tax positions and our worldwide provision for taxes. During the ordinary course of business, there are many activities and transactions for which the ultimate tax determination is uncertain. In addition, our tax obligations and effective tax rates could be adversely affected by changes in the relevant tax, accounting and other laws, regulations, principles and interpretations, including those relating to income tax nexus, by recognizing tax losses or lower than anticipated earnings in jurisdictions where we have lower statutory rates and higher than anticipated earnings in jurisdictions where we have higher statutory rates, by changes in foreign currency exchange rates, or by changes in the valuation of our deferred tax assets and liabilities. In particular, our tax obligations and effective tax rate in the jurisdictions in which we conduct business could increase in the future as a result of the base erosion and profit shifting, or BEPS, project led by the Organization for Economic Co-operation and Development, or OECD, and other initiatives led by the OECD or the European Commission. The OECD is leading work on an iteration of the BEPS project based on two "pillars" (subject to certain revenue thresholds), involving, among other measures, the reallocation of taxing rights in respect of certain multinational enterprises above a fixed profit margin to the jurisdictions in which they carry on business (referred to as "Pillar One") and imposition of a minimum effective corporate tax rate on certain multinational enterprises (referred to as "Pillar Two"). A number of countries in which we conduct business have enacted, or are in the process of enacting, core elements of the Pillar Two rules (with further provisions expected to be enacted in the future). Based on the minimum revenue thresholds we currently expect to fall within the scope of these requirements in the near term. The OECD has issued (and is expected to continue to issue further) administrative guidance providing transition and safe harbor rules in relation to the implementation of the Pillar Two proposal. For example, in January 2026, the OECD issued administrative guidance providing for, among other things, a side-by-side framework intended to limit the application of Pillar Two top-up taxes to U.S.-parented groups; however, the ultimate impact will depend on jurisdictional adoption and further guidance. We are monitoring developments and evaluating the potential impacts of the Pillar Two rules, including on our effective tax rates, and considering our eligibility to qualify for these safe harbor rules (including under the proposed "side-by-side" arrangement).

We may be audited in various jurisdictions, and such jurisdictions may assess additional income, sales and value-added or other taxes against us. In addition, we are currently under examination by the Internal Revenue Service for tax year 2023. Although we believe our tax estimates are reasonable, the final determination of any tax audits or litigation could be materially different from our historical tax provisions and accruals, which could have a material adverse effect on our operating results or cash flows in the period for which a determination is made.

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

Not applicable.

ITEM 3. DEFAULT UPON SENIOR SECURITIES

Not applicable.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Trading Plans

During the quarter ended September 30, 2026, our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act), or Section 16 officers and directors, did not adopt or terminate contracts, instructions or written plans for the purchase or sale of our securities.

ITEM 6. EXHIBITS

a. Exhibits

Exhibit Number	Description of Document
10.1	Form of Global Restricted Stock Unit Agreement for restricted stock units granted under the Amended and Restated Ionis Pharmaceuticals, Inc. 2011 Equity Incentive Plan.
10.2	Form of Global Option Agreement for stock options granted under the Amended and Restated Ionis Pharmaceuticals, Inc. 2011 Equity Incentive Plan.
10.3	UK Sub-Plan to the Amended and Restated Ionis Pharmaceuticals, Inc. 2011 Equity Incentive Plan.
31.1	Certification by Chief Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as amended.
31.2	Certification by Chief Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as amended.
32.1*	Certification Pursuant to 18 U.S.C. Section 1350 as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101	The following financial statements from the Ionis Pharmaceuticals, Inc. Quarterly Report on Form 10-Q for the quarter ended September 30, 2026, formatted in Inline Extensible Business Reporting Language (iXBRL): (i) condensed consolidated balance sheets, (ii) condensed consolidated statements of operations, (iii) condensed consolidated statements of comprehensive loss, (iv) condensed consolidated statements of stockholders' equity, (v) condensed consolidated statements of cash flows and (vi) notes to condensed consolidated financial statements (detail tagged).
104	Cover Page Interactive Data File (formatted in iXBRL and included in exhibit 101).

* This certification is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

Signatures	Title	Date
/s/ BRETT P. MONIA Brett P. Monia, Ph.D.	Director and Chief Executive Officer (Principal executive officer)	November 9, 2026
/s/ ELIZABETH L. HOUGEN Elizabeth L. Hougen	Executive Vice President, Finance and Chief Financial Officer (Principal financial and accounting officer)	November 9, 2026

IONIS PHARMACEUTICALS, INC.
AMENDED AND RESTATED 2011
EQUITY INCENTIVE PLAN

GLOBAL RESTRICTED STOCK UNIT AGREEMENT

Pursuant to the Restricted Stock Unit Grant Notice ("***Grant Notice***") and this Restricted Stock Unit Agreement (the definition of which shall include, if applicable, the non-U.S. terms and conditions set forth in the appendix to this Restricted Stock Unit Agreement (the "***Appendix***")) and in consideration of your services, Ionis Pharmaceuticals, Inc. (the "***Company***") has awarded you a Restricted Stock Unit Award (the "***Award***") under its Amended and Restated 2011 Equity Incentive Plan (the "***Plan***"). Your Award is granted to you effective as of the Date of Grant set forth in the Grant Notice for this Award. This Restricted Stock Unit Award Agreement shall be deemed to be agreed to by the Company and you upon the earlier of (i) signing (or electronic acceptance) by you of the Restricted Stock Unit Grant Notice to which it is attached, and (ii) your receipt of shares of Common Stock under this Restricted Stock Unit Agreement. Capitalized terms not explicitly defined in this Restricted Stock Unit Agreement shall have the same meanings given to them in the Plan or the Grant Notice, as applicable. In the event of any conflict between the terms in this Restricted Stock Unit Agreement and the Plan, the terms of the Plan shall control. The details of your Award, in addition to those set forth in the Grant Notice and the Plan, are as follows.

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1. GRANT OF THE AWARD. This Award represents the right to be issued on a future date the number of shares of the Company's Common Stock that is equal to the number of stock units indicated in the Grant Notice (the "***Stock Units***"). As of the Date of Grant, the Company will credit to a bookkeeping account maintained by the Company for your benefit (the "***Account***") the number of Stock Units subject to the Award. This Award was granted in consideration of your services to the Company. Except as otherwise provided herein, you will not be required to make any payment to the Company (other than past and future services to the Company) with respect to your receipt of the Award, the vesting of the Stock Units or the delivery of the Common Stock to be issued in respect of the Award.

2. VESTING.

(a) In General. Subject to the limitations contained herein, your Award will vest, if at all, in accordance with the vesting schedule provided in the Grant Notice, provided that vesting will cease upon the termination of your Continuous Service. Upon such termination of your Continuous Service, the Stock Units credited to the Account that were not vested on the date of such termination will be forfeited at no cost to the Company and you will have no further right, title or interest in the Stock Units or the shares of Common Stock to be issued in respect of the Award.

3. **Number of Shares**.

(a) The number of Stock Units subject to your Award may be adjusted from time to time for Capitalization Adjustments, as provided in the Plan.

(b) Any additional Stock Units that become subject to the Award pursuant to this Section 3 and Section 7, if any, shall be subject, in a manner determined by the Board, to the same forfeiture restrictions, restrictions on transferability, and time and manner of delivery as applicable to the other Stock Units covered by your Award.

(c) Notwithstanding the provisions of this Section 3, no fractional shares or rights for fractional shares of Common Stock shall be created pursuant to this Section 3. The Board shall, in its discretion, determine an equivalent benefit for any fractional shares or fractional shares that might be created by the adjustments referred to in this Section 3.

4. **Securities Law Compliance**. You may not be issued any shares in respect of your Award unless either (i) the shares are registered under the Securities Act; or (ii) the Company has determined that such issuance would be exempt from the registration requirements of the Securities Act. Your Award also must comply with other applicable laws and regulations governing the Award, and you will not receive such shares if the Company determines that such receipt would not be in material compliance with such laws and regulations.

5. **Transfer Restrictions**. Prior to the time that shares of Common Stock have been delivered to you, you may not transfer, pledge, sell or otherwise dispose of this Award or the shares issuable in respect of your Award, except as expressly provided in this Section 5. For example, you may not use shares that may be issued in respect of your Award as security for a loan. The restrictions on transfer set forth herein will lapse upon delivery to you of shares in respect of your vested Award.

(a) **Death**. Your Award is transferable by will and by the laws of descent and distribution. In addition, upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form provided by or otherwise satisfactory to the Company and any broker designated by the Company to effect transactions under the Plan, designate a third party who, in the event of your death, shall thereafter be entitled to receive any distribution of Common Stock or other consideration to which you were entitled at the time of your death pursuant to this Agreement. In the absence of such a designation, your executor or administrator of your estate shall be entitled to receive, on behalf of your estate, such Common Stock or other consideration.

(b) **Certain Trusts**. Upon receiving written permission from the Board or its duly authorized designee, you may transfer your Award to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the Award is held in the trust, provided that you and the trustee enter into transfer and other agreements required by the Company.

(c) **Domestic Relations Orders**. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your Award or your right to receive the distribution of Common Stock or other consideration thereunder, pursuant to a domestic relations order that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any division of this Award with the Company prior to finalizing the domestic relations order to help ensure the required information is contained within the domestic relations order.

6. Date of Issuance.

(a) If the Award is exempt from application of Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively **"*Section 409A*"**), the Company will deliver to you a number of shares of the Company's Common Stock equal to the number of vested Stock Units subject to your Award, including any additional Stock Units received pursuant to Section 3 above that relate to those vested Stock Units on the applicable vesting date(s). However, if a scheduled delivery date falls on a date that is not a business day, such delivery date shall instead fall on the next following business day. Notwithstanding the foregoing, in the event that (i) you are subject to the Company's policy permitting officers and directors to sell shares only during certain "window" periods, in effect from time to time (the "***Policy***") or you are otherwise prohibited from selling shares of the Company's Common Stock in the public market and any shares covered by your Award are scheduled to be delivered on a day (the "***Original Distribution Date***") that does not occur during an open "window period" applicable to you or a day on which you are permitted to sell shares of the Company's common stock pursuant to a written plan that meets the requirements of Rule 10b5-1 under the Exchange Act, in each case as determined by the Company in accordance with the Policy, or does not occur on a date when you are otherwise permitted to sell shares of the Company's common stock on the open market, and (ii) the Company elects not to satisfy the tax and/or social security withholding obligations by withholding shares from your distribution, then such shares shall not be delivered on such Original Distribution Date and shall instead be delivered on the first business day of the next occurring open "window period" applicable to you pursuant to such policy (regardless of whether you are still providing continuous services at such time) or the next business day when you are not prohibited from selling shares of the Company's Common Stock in the open market, but in no event later than the fifteenth day of the third calendar month of the calendar year following the calendar year in which the shares covered by the Award vest. Delivery of the shares pursuant to the provisions of this Section 6(a) is intended to comply with the requirements for the short-term deferral exemption available under Treasury Regulation 1.409A-1(b)(4) and shall be construed and administered in such manner. The form of such delivery of the shares (*e.g.*, a stock certificate or electronic entry evidencing such shares) shall be determined by the Company.

(b) The provisions of this Section 6(b) are intended to apply if the Award is subject to Section 409A because of the terms of a severance arrangement or other agreement between you and the Company, if any, that provides for acceleration of vesting of the Award upon your separation from service (as such term is defined in section 409A(a)(2)(A)(i) of the Code and applicable guidance thereunder ("***Separation From Service***") and such severance benefit does not satisfy the requirements for an exemption from application of Section 409A provided under Treasury Regulations Sections 1.409A-1(b)(4) or 1.409A-1(b)(9) ("***Non-Exempt Severance Arrangement***"). If the Award is subject to and not exempt from application of Section 409A due to application of a Non-Exempt Severance Arrangement, the following provisions in this Section 6(b) shall supersede anything to the contrary in Section 6(a).

(i) If the Award vests in ordinary course during your Continuous Service in accordance with the vesting schedule set forth in the Grant Notice, in no event will the shares to be issued in respect of your Award be issued any later than the later of: (i) December 31st of the calendar year that includes the applicable vesting date, or (ii) the 60th day that follows the applicable vesting date.

(ii) If the Award accelerates vesting under the terms of your Non-Exempt Severance Arrangement in connection with your Separation From Service, and such vesting acceleration provisions of your Non-Exempt Severance Arrangement were in effect as of the date of grant of the Award and therefore part of the terms of the Award as of the date of grant, then the shares will be earlier issued in respect of your Award upon your Separation From Service in accordance with the terms of the Non-Exempt Severance Arrangement, but in no event later than the 60th day that follows the date of your Separation From Service. However, if at the time the shares would otherwise be issued you are subject to the distribution limitations contained in section 409A of the Code applicable to "specified employees" as defined in section 409A(a)(2)(B)(i) of the Code and applicable guidance thereunder, such share issuances shall not be made before the date which is six months following the date of your Separation From Service, or, if earlier, the date of your death that occurs within such six month period.

(iii) If the Award accelerates vesting under the terms of your Non-Exempt Severance Arrangement in connection with your Separation From Service, and such vesting acceleration provisions of your Non-Exempt Severance Arrangement were not in effect as of the date of grant of the Award and therefore not a part of the terms of the Award on the date of grant, then such acceleration of vesting of the Award shall not accelerate the issuance date of the shares, but the shares shall instead be issued on the same schedule as set forth on the Grant Notice as if they had vested in ordinary course during your Continuous Service, notwithstanding the vesting acceleration of the Award. Such issuance schedule is intended to satisfy the requirements of payment on a specified date or pursuant to a fixed schedule, as provided under Treas. Reg. 1.409A-3(a)(4).

(c) The provisions in this Agreement for delivery of the shares in respect of the Award are intended either to comply with the requirements of Section 409A or to provide a basis for exemption from such requirements so that the delivery of the shares will not trigger the additional tax imposed under Section 409A, and any ambiguities herein will be so interpreted.

7. **Dividends**. You shall be entitled to receive payments equal to any cash dividends and other distributions paid with respect to a corresponding number of shares to be issued in respect of the Stock Units covered by your Award, provided that if any such dividends or distributions are paid in shares, the Fair Market Value of such shares shall be converted into additional Stock Units covered by the Award, and further provided that such additional Stock Units shall be subject to the same forfeiture restrictions and restrictions on transferability as apply to the Stock Units subject to the Award with respect to which they relate.

8. **Restrictive Legends**. The shares issued in respect of your Award shall be endorsed with appropriate legends determined by the Company.

9. AWARD NOT A SERVICE CONTRACT.

(a) Your Continuous Service with the Company or an Affiliate is not for any specified term and may be terminated by you or by the Company or an Affiliate at any time, for any reason, with or without cause and with or without notice. Nothing in this Restricted Stock Unit Agreement (including, but not limited to, the vesting of your Award pursuant to the schedule set forth in Section 2 herein or the issuance of the shares in respect of your Award), the Plan or any covenant of good faith and fair dealing that may be found implicit in this Restricted Stock Unit Agreement or the Plan shall: (i) confer upon you any right to continue in the employ of, or affiliation with, the Company or an Affiliate; (ii) constitute any promise or commitment by the Company or an Affiliate regarding the fact or nature of future positions, future work assignments, future compensation or any other term or condition of employment or affiliation; (iii) confer any right or benefit under this Restricted Stock Unit Agreement or the Plan unless such right or benefit has specifically accrued under the terms of this Agreement or Plan; or (iv) deprive the Company of the right to terminate you at will and without regard to any future vesting opportunity that you may have.

(b) By accepting this Award, you acknowledge and agree that the right to continue vesting in the Award pursuant to the schedule set forth in Section 2 is earned only by continuing as an employee, director or consultant at the will of the Company (not through the act of being hired, being granted this Award or any other award or benefit) and that the Company has the right to reorganize, sell, spin-out or otherwise restructure one or more of its businesses or Affiliates at any time or from time to time, as it deems appropriate (a "***reorganization***"). You further acknowledge and agree that such a reorganization could result in the termination of your Continuous Service, or the termination of Affiliate status of your employer and the loss of benefits available to you under this Restricted Stock Unit Agreement, including but not limited to, the termination of the right to continue vesting in the Award. You further acknowledge and agree that this Restricted Stock Unit Agreement, the Plan, the transactions contemplated hereunder and the vesting schedule set forth herein or any covenant of good faith and fair dealing that may be found implicit in any of them do not constitute an express or implied promise of continued engagement as an employee or consultant for the term of this Agreement, for any period, or at all, and shall not interfere in any way with your right or the Company's right to terminate your Continuous Service at any time, with or without cause and with or without notice.

10. WITHHOLDING OBLIGATIONS.

(a) On or before the time you receive a distribution of the shares subject to your Award, or at any time thereafter as requested by the Company, you hereby authorize any required withholding from the Common Stock issuable to you and/or otherwise agree to make adequate provision in cash for any sums required to satisfy the federal, state, local and foreign tax and social security withholding obligations of the Company or any Affiliate which arise in connection with your Award (the "***Withholding Taxes***"). Additionally, the Company may, in its sole discretion, satisfy all or any portion of the Withholding Taxes obligation relating to your Award by any of the following means or by a combination of such means: (i) withholding from any compensation otherwise payable to you by the Company or any Affiliate; (ii) causing you to tender a cash payment; or (iii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to you in connection with the Award with a Fair Market Value (measured as of the date shares of Common Stock are issued to pursuant to Section 6) equal to the amount of such Withholding Taxes; provided, however, that the number of such shares of Common Stock so withheld shall not exceed the amount necessary to satisfy the Company's required tax withholding obligations using the maximum statutory withholding rates for federal, state, local and foreign tax and social security purposes, including payroll taxes, that are applicable to supplemental taxable income.

(b) Unless the tax and social security withholding obligations of the Company and/or any Affiliate are satisfied, the Company shall have no obligation to deliver to you any Common Stock.

(c) In the event the Company's obligation to withhold arises prior to the delivery to you of Common Stock or it is determined after the delivery of Common Stock to you that the amount of the Company's withholding obligation was greater than the amount withheld by the Company, you agree to indemnify and hold the Company and any Affilaite harmless from any failure by the Company to withhold the proper amount.

(d) If specified in your Grant Notice and permitted by the Company, you may direct the Company to withhold shares of Common Stock with a Fair Market Value (measured as of the date shares of Common Stock are issued pursuant to Section 6) equal to the amount of such Withholding Taxes; provided, however, that the number of such shares of Common Stock so withheld shall not exceed the amount necessary to satisfy the required tax and social security obligations using the maximum statutory withholding rates for federal, state, local and foreign tax purposes, including payroll taxes, that are applicable to supplemental taxable income.

11. **Unsecured Obligation**. Your Award is unfunded, and as a holder of a vested Award, you shall be considered an unsecured creditor of the Company with respect to the Company's obligation, if any, to issue shares pursuant to this Agreement. You shall not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this Agreement until such shares are issued to you pursuant to Section 6 of this Agreement. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this Agreement, and no action taken pursuant to its provisions, shall create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

12. **Other Documents**. You hereby acknowledge receipt or the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus. In addition, you acknowledge receipt of the Company's insider-trading policy and agree that you may sell shares only in compliance with such policy, in effect from time to time.

13. **Notices**. Any notices provided for in your Award or the Plan shall be given in writing and shall be deemed effectively given upon receipt or, in the case of notices delivered by the Company to you, five days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company. Notwithstanding the foregoing, the Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this Award by electronic means or to request your consent to participate in the Plan by electronic means. You hereby consent to receive such documents by electronic delivery and, if requested, to agree to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

14. MISCELLANEOUS.

(a) The rights and obligations of the Company under your Award shall be transferable to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by the Company's successors and assigns. Your rights and obligations under your Award may only be assigned with the prior written consent of the Company.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award, and fully understand all provisions of your Award.

(d) This Agreement shall be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Agreement shall be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

15. GOVERNING PLAN DOCUMENT. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your Award, and is further subject to all interpretations, amendments, rules and regulations which may from time to time be promulgated and adopted pursuant to the Plan. Except as expressly provided herein, in the event of any conflict between the provisions of your Award and those of the Plan, the provisions of the Plan shall control except as expressly amended or overridden in this Agreement.

16. SEVERABILITY. If all or any part of this Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity shall not invalidate any portion of this Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

17. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Award subject to this Agreement shall not be included as compensation, earnings, salaries, or other similar terms used when calculating the Employee's benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company's or any Affiliate's employee benefit plans.

18. **Choice of Law**. The interpretation, performance and enforcement of this Agreement will be governed by the law of the state of California without regard to such state's conflicts of laws rules.

19. **Amendment**. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by you and by a duly authorized representative of the Company. Notwithstanding the foregoing, this Agreement may be amended solely by the Board by a writing which specifically states that it is amending this Agreement, so long as a copy of such amendment is delivered to you, and provided that no such amendment adversely affecting your rights hereunder may be made without your written consent. Without limiting the foregoing, the Board reserves the right to change, by written notice to you, the provisions of this Agreement in any way it may deem necessary or advisable to carry out the purpose of the grant as a result of any change in applicable laws or regulations or any future law, regulation, ruling, or judicial decision, provided that any such change shall be applicable only to rights relating to that portion of the Award which is then subject to restrictions as provided herein.

20. **Appendix**. Notwithstanding any provisions in this Agreement, if you are resident and/or work outside the U.S., your Award shall be subject to the general non-U.S. provisions, and the special terms and conditions for your country of residence and/or work, each as set forth in the Appendix attached to this Agreement which, where applicable, shall prevail in the event of conflict between such terms and conditions and the terms of this Agreement, and/or the Plan. Moreover, if you relocate to one of the countries included therein, the terms and conditions for such country will apply to you to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Agreement.

APPENDIX

This Appendix includes general terms and conditions applicable to you and your Award if you reside and/or work outside the U.S., and special terms and conditions applicable to you if you reside and/or work in the countries below. These terms and conditions are in addition to those set forth in the Restricted Stock Unit Award Agreement. Any capitalized term used in this Appendix without definition shall have the meaning ascribed to such term in the Plan, the Grant Notice, or the Restricted Stock Unit Award Agreement, as applicable.

This Appendix also includes information relating to exchange control, foreign asset and/or account reporting, and other issues of which you should be aware with respect to your participation in the Plan. Such laws are often complex and change frequently. As a result, the Company strongly recommends that you not rely on the information herein as the only source of information relating to the consequences of participation in the Plan because the information may be out of date at the time your Award vests or shares of Common Stock acquired under the Plan are sold.

In addition, the information is general in nature and may not apply to your particular situation, and the Company is not in a position to assure you of any particular result. Accordingly, you should seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, are considered a resident of another country or transfer employment and/or change residency to another country after the Award has been granted, the terms and conditions and the information contained herein may not be applicable to you. The Company shall, in its sole discretion, determine to what extent the terms and conditions included herein will apply to you in such circumstances. Any references to the "employer" in this Appendix shall include any entity engaging your services.

GENERAL NON-U.S. PROVISIONS

1. **TRANSFERABILITY**. Notwithstanding the terms of Section 5 of the Restricted Stock Unit Award Agreement, your Award is not transferable, except to your personal representative on your death.

2. **NATURE OF GRANT**. By accepting the Award, you acknowledge, understand and agree that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or terminated by the Company at any time, to the extent permitted under the Plan;

(b) the grant of your Award is voluntary and occasional and does not create any contractual or other right to receive future grants of Awards (whether on the same or different terms), or benefits in lieu of Awards, even if Awards have been granted in the past;

(c) your Award and any shares of Common Stock acquired under the Plan on settlement of your Award, and the income and value of same, are not part of normal or expected compensation for any purpose, including, without limitation, calculating any severance, resignation, termination, vacation, redundancy, dismissal, end-of-service payments, bonuses, long-service awards, pension or retirement or welfare benefits or similar payments;

(d) the future value of the shares of Common Stock underlying the Award is unknown, indeterminable, and cannot be predicted with certainty;

(e) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of the Award or of any amounts due to you pursuant to the vesting of the Award or the subsequent sale of any shares of Common Stock received;

(f) notwithstanding anything to the contrary in the Plan, for the purposes of the Award, your Continuous Service will be considered terminated as of the date you are no longer actively providing services to the Company or any Affiliate (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any), provided that, unless otherwise expressly provided in this Agreement or determined by the Company, the vesting of your Award will not continue during any notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or are otherwise providing services, or the terms of your employment or service agreement, if any (regardless, in each case, of whether or not you are providing services to the Company or one of its Affiliates during such notice period, garden leave period, or similar period); and the Board shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the Award (including whether you may still be considered to be providing services while on a leave of absence); and

(g) no claim or entitlement to compensation or damages shall arise from forfeiture of this Award resulting from the termination of your Continuous Service (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment or service agreement, if any), and in consideration of the grant of this Award to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Affiliate, waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

3. **DATA PRIVACY**.

(a) To the extent that the processing of your personal data by the Company and its Affiliates under and/or in connection with this Agreement falls within the territorial scope of (i) Regulation (EU) 2016/679 of the European Parliament and of the Council of 27th April 2016 (the "***EU GDPR***"), (ii) the EU GDPR as it forms part of UK law by virtue of section 3 of the European Union (Withdrawal) Act 2018, as amended (the "***UK GDPR***"), and/or (iii) equivalent legislation and/or legislation implementing and/or supplementing the EU GDPR or UK GDPR in any member state of the European Economic Area or the UK or Switzerland, Company and/or its Affiliates will carry out such processing in accordance with their EEA/UK privacy notice from time to time in force, the latest version of which has been provided to you.

(b) Except where (a) above applies, you explicitly and unambiguously acknowledge and consent to the collection, use, transfer and other processing of your personal data as described in this paragraph (b) by the Company and its Affiliates for the purpose of implementing, administering and managing your participation in the Plan. You understand that the Company and its Affiliates hold certain personal data about you, including, but not limited to, your name, home address, telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held by you in the Company, details of all Awards or any other entitlement to shares of Common Stock awarded, cancelled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan. You understand that this personal data may be transferred to any third parties assisting in the implementation, administration and management of the Plan.

4. **No Advice Regarding Grant**. The Company is not providing any tax, legal or financial advice, nor is the Company making any recommendations regarding your participation in the Plan, or your acquisition or sale of the underlying shares of Common Stock. You should consult with your own personal tax, legal and financial advisors regarding your participation in the Plan before taking any action.

5. **Language**. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor who is sufficiently proficient in English, so as to allow you to understand the terms and conditions of this Agreement. If you have received this Agreement, or any other document related to this Award and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

6. **Foreign Asset/Account, Exchange Control and Tax Reporting**. You may be subject to foreign asset/account, exchange control and/or tax reporting requirements as a result of the acquisition, holding and/or transfer of shares of Common Stock or cash (including dividends and the proceeds arising from the sale of shares of Common Stock) derived from your participation in the Plan in, to and/or from a brokerage/bank account or legal entity located outside your country. The applicable laws in your country may require that you report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

7. **Applicable Law**. In the event applicable laws prevent or hinder the consummation of the actions and transactions contemplated in this Agreement or the Plan, the Company may in its sole discretion agree to vary the terms of the Plan and/or this Agreement so that you receive substantially the same economic result as contemplated herein, such as through a cash bonus or phantom stock.

COUNTRY-SPECIFIC PROVISIONS

Germany

Sole Contact and Contractual Partner Information. Please note that the Award, the Grant Notice, the Agreement, the Appendix and your participation in the Plan do not create any claims against the Affiliate of the Company you are employed by/your German employer either directly or indirectly. To be clear: your sole contact and sole contractual partner regarding the Plan and the Award is the Company and they are not part of your contractual salary.

Exchange Control Information. Cross-border payments in excess of €50,000 must be reported monthly to the German Federal Bank (*Bundesbank*). In case of payments in connection with securities (including proceeds realized upon the sale of shares of Common Stock or the receipt of dividends), the report must be made by the 7th working day of the month following the month in which the payment was received. The report must be filed electronically and the form of report ("*Allgemeine Meldeportal Statistik*") can be accessed via the Bundesbank's website (www.bundesbank.de) in both German and English. You are responsible for making this report.

Foreign Asset/Account Reporting Information. German residents holding shares of Common Stock must notify their local tax office if the acquisition of shares of Common Stock leads to a so-called qualified participation at any point during the calendar year. A qualified participation is attained only in the unlikely event (i) you own at least 1% of the Company and the value of the shares of Common Stock acquired exceeds €150,000, or (ii) you hold shares of Common Stock exceeding 10% of the total capital of the Company.

Securities Disclaimer. The participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Directive as implemented in Germany.

Ireland

Securities Disclaimer. Neither the offer nor the award of Stock Units, nor the subsequent issue of shares thereunder, to Irish resident Employees, Directors or Consultants will trigger a requirement to produce a prospectus under Irish securities laws given that such offer or award is made to fewer than 150 such persons in Ireland (being a *de minimus* exemption which may be availed of). This Agreement is not intended to be and is not a prospectus for the purposes of Regulation (EU) 2017/1129 of the European Parliament and of the Council of 14 June 2017 or the European Union (Prospectus) Regulations 2019 of Ireland (as amended). This Award Agreement has not been approved by any competent authority for the purposes of Regulation (EU) 2017/1129 of the European Parliament and of the Council of 14 June 2017.

Director and Company Secretary Notification Requirement. If you are a director (including a shadow director or a de facto director) or a company secretary of an Irish Affiliate of the Company whose interest in the Company represent more than 1% of the nominal value of the Company's voting share capital, you are subject to certain notification requirements under Part 5, Chapter 5 of the Irish Companies Act 2014. In such a case, you must notify the relevant Irish subsidiary in writing, within five or eight days, depending on the circumstances involved, of receiving or disposing of an interest in the Company (including the grant of the Stock Units and any shares of Common Stock in the Company acquired on vesting of the Stock Units) and the number and class of shares or rights to which the interest relates. This notification requirement also applies with respect to the interests of your spouse, civil partner and / or children under the age of 18 (whose interests will also be attributed to you).

Nature of Grant. As a supplement to section 2 of the General Non-U.S. Provisions in this Appendix, you acknowledge:

(i) that you shall not be entitled to (and hereby waive) any compensation for the loss of any rights or benefits under any replacement or successor plan or award agreement.

(ii) that your participation in the Plan and Award Agreement shall not form part of your terms and conditions of employment (either expressly or impliedly).

Employment Rights. Rights under the Plan or Agreement are not pensionable.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Choice of Law clause in the Plan and the Agreement and accept that Irish law does not apply and that Irish courts do not have any jurisdiction in regard to any claims under the Plan or the Agreement.

United Kingdom

U.K. Sub-Plan. If you are an Employee, including a Director who is an Employee, your Stock Units are granted under, and shall be subject to the terms of, the U.K. Sub-Plan to the Plan.

No Cash Settlement. Notwithstanding any provision of the Plan or the Agreement, your Award may not be settled in cash.

Award Not a Service Contract. The following supplements Section 2 of the General Non-U.S. Provisions in this Appendix:

"You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your Award, or from the loss on diminution in value of any rights or entitlements in connection with the Plan."

Withholding Obligations. The following supplements Section 10 of the Agreement:

(e) As a condition of the vesting of your Award, you therefore unconditionally and irrevocably agree:

(i) to place the Company in funds and indemnify the Company in respect of (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; and (2) all liability to employee national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs, which arises as a consequence of or in connection with your Award (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you following vesting as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any Affiliate.

Withholding Obligations. By accepting the grant of the Award you acknowledge and agree that your option and any shares of Common Stock issued on settlement of the Award may be subject to clawback in accordance with any applicable policy of the Company from time to time.

EXHIBIT 10.2

Ionis Pharmaceuticals, Inc.
Amended and Restated 2011
Equity Incentive Plan

GLOBAL OPTION AGREEMENT
(NONSTATUTORY STOCK OPTION)

FOR OPTIONS GRANTED AFTER JANUARY 1, 2022

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement (the definition of which shall include, if applicable, the non-U.S. terms and conditions set forth in the appendix to this Option Agreement (the "***Appendix***")), Ionis Pharmaceuticals, Inc. (the "***Company***") has granted you an option under its Amended and Restated 2011 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. Capitalized terms not explicitly defined in this Option Agreement but defined in the Plan shall have the same definitions as in the Plan.

The details of your option are as follows:

1. **VESTING**. Subject to the limitations contained herein, your option will vest as provided in your Grant Notice, provided that vesting will cease upon the termination of your Continuous Service.

2. **NUMBER OF SHARES AND EXERCISE PRICE**. The number of shares of Common Stock subject to your option and your exercise price per share referenced in your Grant Notice may be adjusted from time to time for Capitalization Adjustments.

3. **Exercise Restriction for Non-Exempt Employees**. In the event that you are an Employee eligible for overtime compensation under the Fair Labor Standards Act of 1938, as amended (*i.e.*, a "***Non-Exempt Employee***"), you may not exercise your option until you have completed at least six months of Continuous Service measured from the Date of Grant specified in your Grant Notice, notwithstanding any other provision of your option.

4. **METHOD OF PAYMENT**. Payment of the exercise price is due in full upon exercise of all or any part of your option. You may elect to make payment of the exercise price in cash or by check or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds.

(b) Subject to the consent of the Company at the time of exercise, by a " net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of your option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; provided, however, that the Company shall accept a cash or other payment from you to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued; provided further, however, that shares of Common Stock will no longer be outstanding under your option and will not be exercisable thereafter to the extent that (1) shares are used to pay the exercise price pursuant to the "net exercise," (2) shares are delivered to you as a result of such exercise, and (3) shares are withheld to satisfy tax withholding obligations.

5. **Whole Shares**. You may exercise your option only for whole shares of Common Stock.

6. **Securities Law Compliance**. Notwithstanding anything to the contrary contained herein, you may not exercise your option unless the shares of Common Stock issuable upon such exercise are then registered under the Securities Act or, if such shares of Common Stock are not then so registered, the Company has determined that such exercise and issuance would be exempt from the registration requirements of the Securities Act. The exercise of your option also must comply with other applicable laws and regulations governing your option, and you may not exercise your option if the Company determines that such exercise would not be in material compliance with such laws and regulations.

7. **Term**. You may not exercise your option before the commencement or after the expiration of its term. The term of your option commences on the Date of Grant and expires upon the earliest of the following:

(a) three months after the termination of your Continuous Service for any reason other than upon your Disability, retirement or death; provided, however, that if during any part of such three month period your option is not exercisable solely because of the condition set forth in the section above relating to "Securities Law Compliance," your option shall not expire until the earlier of the Expiration Date or until it shall have been exercisable for an aggregate period of three months after the termination of your Continuous Service; and if (i) you are a Non-Exempt Employee, (ii) your Continuous Service terminates within six months after the Date of Grant specified in your Grant Notice, and (iii) you have vested in a portion of your option at the time of your termination of Continuous Service, your option shall not expire until the earlier of (x) the later of (A) the date that is seven months after the Date of Grant specified in your Grant Notice or (B) the date that is three months after the termination of your Continuous Service, or (y) the Expiration Date;

(b) 12 months after the termination of your Continuous Service due to your Disability;

(c) 18 months after the termination of your Continuous Service due to your retirement; provided that (i) you were over the age of 55 at the time of such retirement and (ii) you had been providing Continuous Service to the Company as an Employee, Director or Consultant (or any combination thereof) for a continuous and uninterrupted period of at least five years prior to such retirement;

(d) 18 months after your death if you die during your Continuous Service;

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10^{th}) anniversary of the Date of Grant.

Notwithstanding the foregoing, if you die during the period provided in Section 7(a), 7(b) or 7(c) above, the term of your option shall not expire until the earlier of 18 months after your death, the Expiration Date indicated in your Grant Notice, or the day before the tenth anniversary of the Date of Grant.

8. EXERCISE.

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so permits) during its term by delivering a Notice of Exercise (in a form designated by the Company) together with the exercise price to the Secretary of the Company, or to such other person as the Company may designate, during regular business hours, together with such additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax withholding obligation of the Company arising by reason of (1) the exercise of your option, (2) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (3) the disposition of shares of Common Stock acquired upon such exercise.

(c) If (i) you have not exercised your option in full by the 10th business day prior to the expiration of this option, (ii) at such time you are a current employee of the Company or were an employee of the Company within the previous 90 days, (iii) you have not otherwise instructed the broker to exercise this option prior to expiry under a separate Company-approved Rule 10b5-1 Plan, and (iv) the prevailing market price of the Common Stock exceeds the Exercise Price per share of this option, then you authorize the Company's external stock plan administrator ("***Administrator***") to, in its sole discretion, without obligation, and in compliance with all applicable legal conditions and restrictions, exercise this option on your behalf and sell the number of whole shares of Common Stock having a Fair Market Value sufficient to cover the aggregate exercise price plus the minimum amount of tax required to be withheld by law (such number of shares to be determined by the Company as of the date of exercise), and to remit payment of such exercise price and withholding amounts to the Company. In addition, you authorize the Company to, in its sole discretion and in compliance with all applicable legal conditions and restrictions, take any action it deems necessary or appropriate to effect such an exercise and sale.

Notwithstanding the foregoing, the Company may, in its sole discretion, without obligation, and in lieu of the automatic exercise and sale set forth above, effect a "net exercise" arrangement to cover the aggregate exercise price and minimum amount of the tax to be withheld by the Company by law, each as further described in Section 5(c)(iv) and Section 8(f) of the Plan.

You will take all necessary and appropriate actions to effect the exercise and sale under this section, including but not limited to completing any exercise forms or authorizations. You hereby authorize and appoint each of Administrator and the Company's Corporate Secretary to serve, individually or collectively, as your agent and attorney-in-fact and, in accordance with the terms of this section, to effect the exercise and sale of your option. You will pay the Company an administrative fee of $100 in connection with any exercise or sale under this section.

This Section 8(c) is intended to meet the requirements of, and comply with, Rule 10b5-1 (c) under the Exchange Act.

9. **Transferability**. Your option is not transferable, except (1) by will or by the laws of descent and distribution, (2) pursuant to a domestic relations order, (3) with the prior written approval of the Company, by instrument to an *inter vivos* or testamentary trust, in a form accepted by the Company, in which the option is to be passed to beneficiaries upon the death of the trustor (settlor) and (4) with the prior written approval of the Company, by gift, in a form accepted by the Company, to a permitted transferee under Rule 701 of the Securities Act.

10. **Option not a Service Contract**. Your option is not an employment or service contract, and nothing in your option shall be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in your option shall obligate the Company or an Affiliate, their respective stockholders, Boards of Directors, Officers or Employees to continue any relationship that you might have as a Director or Consultant f or the Company or an Affiliate.

11. **Withholding Obligations**.

(a) At the time you exercise your option, in whole or in part, or at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "cashless exercise" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax and social security withholding obligations of the Company or an Affiliate (or your employer, if different), if any, which arise in connection with your option.

(b) Upon your request and subject to approval by the Company, in its sole discretion, and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of your option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the maximum amount of tax and social security required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). If the date of determination of any tax withholding obligation is deferred to a date later than the date of exercise of your option, share withholding pursuant to the preceding sentence shall not be permitted unless you make a proper and timely election under Section 83(b) of the Code, covering the aggregate number of shares of Common Stock acquired upon such exercise with respect to which such determination is otherwise deferred, to accelerate the determination of such tax withholding obligation to the date of exercise of your option. Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax and social security withholding obligations of the Company and/or any Affiliate (or your employer, if different) are satisfied. Accordingly, you may not be able to exercise your option when desired even though your option is vested, and the Company shall have no obligation to issue a certificate for such shares of Common Stock unless such obligations are satisfied.

12. **Tax Consequences**. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax and social security liabilities. You shall not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax or social security liabilities arising from your option or your other compensation. In particular, you acknowledge that this option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option.

13. **Notices**. Any notices provided for in your option or the Plan shall be given in writing and shall be deemed effectively given (i) upon receipt, (ii) in the case of notices delivered by mail by the Company to you, five days after deposit in the mail, postage prepaid, addressed to you at the last address you provided to the Company, or (iii) in the case of notices delivered by courier by the Company to you, one day after deposit with an internationally recognized overnight courier, specifying next day delivery, addressed to you at the last address you provided to the Company, with written verification of receipt.

14. **Governing Plan Document**. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. In the event of any conflict between the provisions of your option and those of the Plan, the provisions of the Plan shall control except as expressly amended or overridden in this Option Agreement.

15. **Appendix**. Notwithstanding any provisions in this Option Agreement, if you are resident and/or work outside the U.S., your option shall be subject to the general non-U.S. provisions, and the special terms and conditions for your country of residence and/or work, each as set forth in the Appendix attached to this Option Agreement which, where applicable, shall prevail in the event of conflict between such terms and conditions and the terms of this Option Agreement, and/or the Plan. Moreover, if you relocate to one of the countries included therein, the terms and conditions for such country will apply to you to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Option Agreement.

APPENDIX

This Appendix includes general terms and conditions applicable to you and your option if you reside and/or work outside the U.S., and special terms and conditions applicable to you if you reside and/or work in the countries below. These terms and conditions are in addition to those set forth in the Option Agreement. Any capitalized term used in this Appendix without definition shall have the meaning ascribed to such term in the Plan, the Grant Notice, or the Option Agreement, as applicable.

This Appendix also includes information relating to exchange control, foreign asset and/or account reporting, and other issues of which you should be aware with respect to your participation in the Plan. Such laws are often complex and change frequently. As a result, the Company strongly recommends that you not rely on the information herein as the only source of information relating to the consequences of participation in the Plan because the information may be out of date at the time your option vest, your exercise it, or shares of Common Stock acquired under the Plan are sold.

In addition, the information is general in nature and may not apply to your particular situation, and the Company is not in a position to assure you of any particular result. Accordingly, you should seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, are considered a resident of another country or transfer employment and/or change residency to another country after the Award has been granted, the terms and conditions and the information contained herein may not be applicable to you. The Company shall, in its sole discretion, determine to what extent the terms and conditions included herein will apply to you in such circumstances. Any references to the "employer" in this Appendix shall include any entity engaging your services.

GENERAL NON-U.S. PROVISIONS

1. **TRANSFERABILITY**. Notwithstanding the terms of Section 9 of the Agreement, your option is not transferable, except to your personal representative on your death, and is exercisable during your life only by you or by your personal representative after your death.

2. **OPTION NOT A SERVICE CONTRACT**. By accepting your option, you acknowledge, understand and agree that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or terminated by the Company at any time, to the extent permitted under the Plan;

(b) the grant of your option is voluntary and occasional and does not create any contractual or other right to receive future grants of options (whether on the same or different terms), or benefits in lieu of options, even if options have been granted in the past;

(c) your options and any shares of Common Stock acquired under the Plan on exercise of your options, and the income and value of same, are not part of normal or expected compensation for any purpose, including, without limitation, calculating any severance, resignation, termination, vacation, redundancy, dismissal, end-of-service payments, bonuses, long-service awards, pension or retirement or welfare benefits or similar payments;

(d) the future value of the shares of Common Stock underlying the option is unknown, indeterminable, and cannot be predicted with certainty;

(e) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of your options or of any amounts due to you pursuant to the exercise of your option or the subsequent sale of any shares of Common Stock received;

(f) notwithstanding anything to the contrary in the Plan, for the purposes of the option, your Continuous Service will be considered terminated as of the date you are no longer actively providing services to the Company or one of its Affiliates (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or are otherwise providing services, or the terms of your employment or service agreement, if any), provided that, unless otherwise expressly provided in this Option Agreement or determined by the Company, the vesting of your option will not continue during any notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or where you are otherwise providing services, or the terms of your employment or service agreement, if any (regardless, in each case, of whether or not you are providing services to the Company or one of its Affiliates during such notice period, garden leave period, or similar period); and the Board shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the option (including whether you may still be considered to be providing services while on a leave of absence); and

(g) no claim or entitlement to compensation or damages shall arise from forfeiture of this option resulting from the termination of your Continuous Service (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or are otherwise providing services, or the terms of your employment or service agreement, if any), and in consideration of the grant of this option to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Affiliate, waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

3. **No Advice Regarding Grant**. The Company is not providing any tax, legal or financial advice, nor is the Company making any recommendations regarding your participation in the Plan, or your acquisition or sale of the underlying shares of Common Stock. You should consult with your own personal tax, legal and financial advisors regarding your participation in the Plan before taking any action.

4. **Data Privacy**.

(a) To the extent that the processing of your personal data by the Company or its Affiliates under and/or in connection with this Option Agreement falls within the territorial scope of (i) Regulation (EU) 2016/679 of the European Parliament and of the Council of 27th April 2016 (the "***EU GDPR***"), (ii) the EU GDPR as it forms part of UK law by virtue of section 3 of the European Union (Withdrawal) Act 2018, as amended (the "***UK GDPR***"), and/or (iii) equivalent legislation and/or legislation implementing and/or supplementing the EU GDPR or UK GDPR in any member state of the European Economic Area or the UK or Switzerland, Company and/or its Affiliates will carry out such processing in accordance with their EEA/UK privacy notice from time to time in force, the latest version of which has been provided to you.

(b) Except where (a) above applies, you explicitly and unambiguously acknowledge and consent to the collection, use, transfer and other processing of your personal data as described in this paragraph (b) by the Company and its Affiliates for the purpose of implementing, administering and managing your participation in the Plan. You understand that the Company and its Affiliates hold certain personal data about you, including, but not limited to, your name, home address, telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held by you in the Company, details of all options or any other entitlement to shares of Common Stock awarded, cancelled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan. You understand that this personal data may be transferred to any third parties assisting in the implementation, administration and management of the Plan.

5. **Language**. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor who is sufficiently proficient in English, so as to allow you to understand the terms and conditions of this Option Agreement. If you have received this Option Agreement, or any other document related to your option and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

6. **Foreign Asset/Account, Exchange Control and Tax Reporting**. You may be subject to foreign asset/account, exchange control and/or tax reporting requirements as a result of the acquisition, holding and/or transfer of shares of Common Stock or cash (including dividends and the proceeds arising from the sale of shares of Common Stock) derived from your participation in the Plan in, to and/or from a brokerage/bank account or legal entity located outside your country of residence. The applicable laws in your country of residence may require that you report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country of residence through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

7. **Applicable Law**. In the event applicable laws prevent or hinder the consummation of the actions and transactions contemplated in this Option Agreement or the Plan, the Company may in its sole discretion agree to vary the terms of the Plan and/or this Option Agreement so that you receive substantially the same economic result as contemplated herein, such as through a cashless sell to cover exercise (provided that at the time of exercise the shares of Common Stock are publicly traded or otherwise liquid), a cash bonus or phantom stock.

8. **Choice of Law**. The provisions of the Plan relating to choice of law shall apply to this Option Agreement and the option.

COUNTRY-SPECIFIC PROVISIONS

Germany

Securities Disclaimer. The participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Regulation, as amended.

Exchange Control Information. Cross-border payments in excess of €50,000 must be reported monthly to the German Federal Bank (*Bundesbank*). In the event that you make or receive a payment in excess of this amount, you are required to report the payment to Bundesbank electronically using the "General Statistics Reporting Portal" ("*Allgemeines Meldeportal Statistik*") available via Bundesbank's website (www.bundesbank.de) in both German and English. You are responsible for making this report.

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is 31 July of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

Ireland

Director Notification Requirement. If you are a director, shadow director, or secretary of an Irish Affiliate of the Company, pursuant to Part 5, Chapter 5 of the Companies Act 2014, you must notify that Affiliate in writing when receiving or disposing of an interest of any kind in the shares or debentures of the Company (*e.g.*, the option, shares of Common Stock), or when becoming aware of the event giving rise to the notification requirement, or when becoming a director or secretary if such an interest exists at the time. This notification requirement also applies with respect to the interests of a spouse, civil partner or minor children (whose interests will be attributed to the director, shadow director, or secretary). The foregoing requirements apply only if your securities represent one percent (1%) or more of the Company.

Optionholder Self-Assessment. The following section supplements Section 11 of the Option Agreement (Withholding Obligations):

> "For options exercised on or after 1 January 2024, it is your employer's responsibility to operate withholding tax on the exercise of your options and account to the Irish Revenue Commissioners for any tax in connection with the exercise of the options, under the PAYE system."

United Kingdom

U.K. Sub-Plan. If you are an Employee, including a Director who is an Employee, your option is granted under, and shall be subject to the terms of, the U.K. Sub-Plan to the Plan.

Option Not A Service Contract. The following supplements Section 2 of the General Non-U.S. Provisions:

> "You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your option, or from the loss or diminution in value of any rights or entitlements in connection with the Plan."

Withholding Obligations. The following supplements Section 11 of the Option Agreement:

"(d) As a condition of the vesting of your option, you unconditionally and irrevocably agree:

(i) to place the Company in funds and indemnify the Company in respect of (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; and (2) all liability to employee national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs, which arises as a consequence of or in connection with the exercise of your option (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you following exercise as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any Affiliate (or your employer, if different)."

Withholding Obligations. By accepting the grant of the option you acknowledge and agree that your option and any shares of Common Stock issued on exercise of your option may be subject to clawback in accordance with any applicable policy of the Company from time to time.

UK SUB-PLAN

TO THE AMENDED AND RESTATED IONIS PHARMACEUTICALS, INC. 2011 EQUITY INCENTIVE PLAN

This sub-plan (the "***UK Sub-Plan***") to the Amended and Restated Ionis Pharmaceuticals, Inc. 2011 Equity Incentive Plan (the "***Plan***") governs the grant of Stock Awards to United Kingdom Employees and has been adopted in accordance with Section 2(b)(x) of the Plan. The UK Sub-Plan incorporates all the provisions of the Plan except as modified in accordance with the provisions of this UK Sub-Plan. Capitalized terms used but not defined herein shall have the meaning set forth in the Plan.

Stock Awards granted pursuant to the UK Sub-Plan are granted pursuant to an "**employees**' **share scheme**" for the purposes of the (UK) Financial Services and Markets Act 2000.

For the purposes of the UK Sub-Plan, the provisions of the Plan shall operate subject to the following modifications:

1. Eligibility

Only Employees (including Directors who are Employees) may be granted Stock Awards under the UK Sub-Plan, and Sections 1 and 4(a) of the Plan shall be read and construed to take effect accordingly.

For the purposes of the UK Sub-Plan, the following definition of an "***Employee***" shall replace the definition in the Plan:

> "***Employee***" means any individual employed directly by the Company or a subsidiary thereof (as defined in section 1159 of the (UK) Companies Act 2006), provided that service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered as an "Employee" for the purposes hereof.

2. Restrictions on Transfer

A Stock Award shall be personal to the Participant to whom it is granted and shall not be capable of being transferred, assigned or charged except that a Participant's Stock Award may be transmitted to the Participant's personal representatives on their death. Participants may not designate a third party to be a beneficiary of their Stock Award after their death.

In order to give effect to this provision, Section 5(e) of the Plan shall be read and construed accordingly.

3. No Employment or Other Service Rights

The following additional wording shall be included at the end of Section 8(d) of the Plan:

"The grant of a Stock Award will not form part of the Participant's entitlement to remuneration or benefits pursuant to their contract of employment nor does the existence of a contract of employment between a person and the Company or any Affiliate give any right or expectation that a Stock Award will be granted to them. The rights and obligations of a Participant under the terms of their contract of employment with the Company or any Affiliate shall not be affected by the grant of a Stock Award. A Participant waives all and any rights to compensation or damages under the Plan in consequence of the termination of the Participant's office or employment with the Company or an Affiliate for any reason (including, without limitation, any breach of contract by their employer)."

4. UK Withholding Obligations

Section 8 of the Plan shall include the following additional section:

"(l) **UK Tax Withholding**. A Participant shall, unconditionally and irrevocably agree as a condition of the vesting of their Stock Award:

(i) to place the Company in funds and indemnify the Company in respect of (A) all liability to UK income tax which the Company is liable to account for on behalf of the Participant directly to HM Revenue & Customs; (B) all liability to national insurance contributions which the Company is liable to account for on behalf of the Participant to HM Revenue & Customs (including secondary class 1 (employer's) national insurance contributions for which the Participant is liable and agrees to bear); and (C) all liability to national insurance contributions for which the Company is liable which arises as a consequence of or in connection with the vesting or exercise of the Stock Award (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to the Participant following exercise or vesting (as the case may be) of their Stock Award as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to the Participant (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to the Participant; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this Section.

References in this Section to "the Company" shall, if applicable, be construed as also referring to any Affiliate."

5. Data Protection

Section 8 of the Plan shall include the following additional section:

"(m) **Data Protection**. For the purposes of operating the Plan in the United Kingdom, the Company will collect and process information relating to the Participant in accordance with the privacy notice from time to time in force."

EXHIBIT 31.1

CERTIFICATION

I, Brett P. Monia, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ionis Pharmaceuticals, Inc.;

2. Based on my knowledge, this quarterly 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 quarterly report;

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

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

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

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

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of 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.

Dated: November 9, 2026

/s/ BRETT P. MONIA
Brett P. Monia, Ph.D.
Chief Executive Officer

EXHIBIT 31.2

CERTIFICATION

I, Elizabeth L. Hougen, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ionis Pharmaceuticals, Inc.;

2. Based on my knowledge, this quarterly 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 quarterly report;

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

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

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

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

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of 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.

Dated: November 9, 2026

/s/ ELIZABETH L. HOUGEN
Elizabeth L. Hougen
Chief Financial Officer

Exhibit 32.1

CERTIFICATION

Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, Brett P. Monia, the Chief Executive Officer of Ionis Pharmaceuticals, Inc., (the "Company"), and Elizabeth L. Hougen, the Chief Financial Officer of the Company, each hereby certifies that, to the best of his or her knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2026, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition of the Company at the end of the period covered by the Periodic Report and the results of operations of the Company for the period covered by the Periodic Report.

Dated: November 9, 2026

/s/ BRETT P. MONIA
Brett P. Monia, Ph.D.
Chief Executive Officer

/s/ ELIZABETH L. HOUGEN
Elizabeth L. Hougen
Chief Financial Officer

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