

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2025

OR

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

For the transition period from ___ to ___

Commission File Number 001-37553

REGENXBIO Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware

(State or other jurisdiction of
incorporation or organization)

**9804 Medical Center Drive
Rockville, MD**

(Address of principal executive offices)

47-1851754

(I.R.S. Employer
Identification No.)

20850

(Zip Code)

(240) 552-8181

(Registrant's telephone number, including area code)

Not Applicable

(Former name, former address and former fiscal year, if changed since last report)

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

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

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

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

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

Large accelerated filer

Non-accelerated filer

Accelerated filer

Smaller reporting company

Emerging growth company

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

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

As of July 31, 2025, there were 50,514,216 shares of the registrant's common stock, par value \$0.0001 per share, issued and outstanding.

REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2025

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INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “anticipate,” “assume,” “believe,” “continue,” “could,” “design,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “objective,” “plan,” “position,” “potential,” “predict,” “project,” “seek,” “should,” “will,” “would” or variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations, estimates and assumptions and analyses in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- our ability to establish and maintain development partnerships, including our collaboration with AbbVie to develop and commercialize ABBV-RGX-314 and our collaboration with Nippon Shinyaku to develop and commercialize RGX-121 and RGX-111;
- our ability to obtain and maintain regulatory approval of our product candidates and the labeling for any approved products;
- the timing of enrollment, commencement, completion and the success of our AAVIATE[®], AFFINITY BEYOND[®], AFFINITY DUCHENNE[®], ALTITUDE[®], ASCENT[™], ATMOSPHERE[®], CAMPSITE[®] and other clinical trials;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain, maintain and enforce intellectual property protection for our product candidates and technology, and defend against third-party intellectual property-related claims;
- our expectations regarding the development and commercialization of product candidates currently being developed by third parties that utilize our technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our products that are approved;
- our expectations regarding our expenses and revenue;
- our expectations regarding our need for additional financing and our ability to obtain additional financing;
- our expectations regarding the outcome of legal proceedings;
- our expectations regarding regulatory developments in the United States and foreign countries;
- the impact of any government-imposed tariffs or other trade barriers on cost of goods and services, particularly related to partnered product candidates; and
- changes in the financial markets and banking system that may affect the availability and terms on which we may obtain financing and our ability to accurately predict how long our existing cash resources will be sufficient to fund our anticipated operating expenses.

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You should carefully read the factors discussed in the sections titled “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” and the factors discussed elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2024 and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance, and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this Quarterly Report on Form 10-Q. Except as required by law, we disclaim any duty to update any forward-looking statements, whether as a result of new information, future events or otherwise.

Available Information

Our principal offices are located at 9804 Medical Center Drive, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. The information contained in, or that can be accessed through, our website is not a part of, or incorporated by reference in, this Quarterly Report on Form 10-Q. We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. You may obtain any reports, proxy and information statements, and other information that we file electronically with the SEC at www.sec.gov.

You also may view and download copies of our SEC filings free of charge at our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and is not considered part of, this Quarterly Report on Form 10-Q. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our business. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

As used in this Quarterly Report on Form 10-Q, the terms “REGENXBIO,” “we,” “us,” “our” or the “Company” mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

AAVIATE, AFFINITY BEYOND, AFFINITY DUCHENNE, ALTITUDE, ATMOSPHERE, CAMPSITE, NAV, NAVXpress, NAVXcell, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective holders.

PART I – FINANCIAL INFORMATION
Item 1. Financial Statements.

REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share data)

	<u>June 30, 2025</u>	<u>December 31, 2024</u>
Assets		
Current assets		
Cash and cash equivalents	\$ 79,558	\$ 57,526
Marketable securities	243,740	177,161
Accounts receivable	20,199	20,473
Prepaid expenses	10,776	9,067
Other current assets	19,479	13,774
Total current assets	<u>373,752</u>	<u>278,001</u>
Marketable securities	40,296	10,179
Accounts receivable	1,584	474
Property and equipment, net	111,017	117,589
Operating lease right-of-use assets	50,469	53,716
Restricted cash	2,030	2,030
Other assets	1,879	4,000
Total assets	<u>\$ 581,027</u>	<u>\$ 465,989</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 21,655	\$ 22,798
Accrued expenses and other current liabilities	35,557	38,070
Deferred revenue	13,977	115
Operating lease liabilities	8,049	7,902
Royalty monetization liabilities	40,302	34,309
Total current liabilities	<u>119,540</u>	<u>103,194</u>
Deferred revenue	23,804	—
Operating lease liabilities	69,647	74,131
Royalty monetization liabilities	153,693	25,378
Other liabilities	664	3,635
Total liabilities	<u>367,348</u>	<u>206,338</u>
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at June 30, 2025 and December 31, 2024	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at June 30, 2025 and December 31, 2024; 50,389 and 49,549 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	1,211,361	1,192,536
Accumulated other comprehensive loss	(750)	(741)
Accumulated deficit	(996,937)	(932,149)
Total stockholders' equity	<u>213,679</u>	<u>259,651</u>
Total liabilities and stockholders' equity	<u>\$ 581,027</u>	<u>\$ 465,989</u>

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except per share data)

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2025</u>	<u>2024</u>	<u>2025</u>	<u>2024</u>
Revenues				
License and royalty revenue	\$ 18,465	\$ 21,846	\$ 105,514	\$ 37,190
Service revenue	2,894	449	4,857	727
Total revenues	<u>21,359</u>	<u>22,295</u>	<u>110,371</u>	<u>37,917</u>
Operating Expenses				
Cost of license and royalty revenues	5,209	10,579	8,645	14,862
Research and development	59,500	48,869	112,587	103,713
General and administrative	19,883	18,855	40,230	37,146
Impairment of long-lived assets	—	—	—	2,101
Other operating expenses (income)	45	29	60	(5)
Total operating expenses	<u>84,637</u>	<u>78,332</u>	<u>161,522</u>	<u>157,817</u>
Loss from operations	<u>(63,278)</u>	<u>(56,037)</u>	<u>(51,151)</u>	<u>(119,900)</u>
Other Income (Expense)				
Interest income from licensing	21	29	46	66
Investment income	3,379	3,468	5,880	5,937
Interest expense	(10,993)	(449)	(19,563)	(2,422)
Total other income (expense)	<u>(7,593)</u>	<u>3,048</u>	<u>(13,637)</u>	<u>3,581</u>
Net loss	<u>\$ (70,871)</u>	<u>\$ (52,989)</u>	<u>\$ (64,788)</u>	<u>\$ (116,319)</u>
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	12	963	(9)	2,163
Total other comprehensive income (loss)	<u>12</u>	<u>963</u>	<u>(9)</u>	<u>2,163</u>
Comprehensive loss	<u>\$ (70,859)</u>	<u>\$ (52,026)</u>	<u>\$ (64,797)</u>	<u>\$ (114,156)</u>
Net loss per share, basic and diluted	<u>\$ (1.38)</u>	<u>\$ (1.05)</u>	<u>\$ (1.26)</u>	<u>\$ (2.41)</u>
Weighted-average common shares outstanding, basic and diluted	<u>51,483</u>	<u>50,601</u>	<u>51,423</u>	<u>48,167</u>

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands)

	Three Months Ended June 30, 2025						
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	
	Shares	Amount					
Balances at March 31, 2025	50,117	\$ 5	\$ 1,201,020	\$ (762)	\$ (926,066)	\$ 274,197	
Vesting of restricted stock units, net of tax	52	—	—	—	—	—	
Exercise of stock options, net of tax	21	—	78	—	—	78	
Issuance of warrants, net of transaction costs	—	—	1,610	—	—	1,610	
Exercise of pre-funded warrants	199	—	—	—	—	—	
Stock-based compensation expense	—	—	8,653	—	—	8,653	
Unrealized gain on available-for-sale securities, net	—	—	—	12	—	12	
Net loss	—	—	—	—	(70,871)	(70,871)	
Balances at June 30, 2025	<u>50,389</u>	<u>\$ 5</u>	<u>\$ 1,211,361</u>	<u>\$ (750)</u>	<u>\$ (996,937)</u>	<u>\$ 213,679</u>	

	Three Months Ended June 30, 2024						
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	
	Shares	Amount					
Balances at March 31, 2024	49,043	\$ 5	\$ 1,162,267	\$ (3,229)	\$ (768,377)	\$ 390,666	
Vesting of restricted stock units, net of tax	39	—	—	—	—	—	
Exercise of stock options, net of tax	38	—	192	—	—	192	
Exercise of pre-funded warrants	197	—	—	—	—	—	
Stock-based compensation expense	—	—	9,435	—	—	9,435	
Unrealized gain on available-for-sale securities, net	—	—	—	963	—	963	
Net loss	—	—	—	—	(52,989)	(52,989)	
Balances at June 30, 2024	<u>49,317</u>	<u>\$ 5</u>	<u>\$ 1,171,894</u>	<u>\$ (2,266)</u>	<u>\$ (821,366)</u>	<u>\$ 348,267</u>	

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands)

	Six Months Ended June 30, 2025					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2024	49,549	\$ 5	\$ 1,192,536	\$ (741)	\$ (932,149)	\$ 259,651
Vesting of restricted stock units, net of tax	533	—	(510)	—	—	(510)
Exercise of stock options, net of tax	61	—	228	—	—	228
Issuance of common stock under employee stock purchase plan	47	—	307	—	—	307
Issuance of warrants, net of transaction costs	—	—	1,610	—	—	1,610
Exercise of pre-funded warrants	199	—	—	—	—	—
Stock-based compensation expense	—	—	17,190	—	—	17,190
Unrealized loss on available-for-sale securities, net	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(64,788)	(64,788)
Balances at June 30, 2025	<u>50,389</u>	<u>\$ 5</u>	<u>\$ 1,211,361</u>	<u>\$ (750)</u>	<u>\$ (996,937)</u>	<u>\$ 213,679</u>
	Six Months Ended June 30, 2024					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2023	44,046	\$ 4	\$ 1,021,214	\$ (4,429)	\$ (705,047)	\$ 311,742
Vesting of restricted stock units, net of tax	309	—	(910)	—	—	(910)
Exercise of stock options, net of tax	173	—	1,076	—	—	1,076
Issuance of common stock under employee stock purchase plan	27	—	411	—	—	411
Issuance of common stock and pre-funded warrants upon public offering, net of transaction costs of \$534	4,565	1	131,066	—	—	131,067
Exercise of pre-funded warrants	197	—	—	—	—	—
Stock-based compensation expense	—	—	19,037	—	—	19,037
Unrealized gain on available-for-sale securities, net	—	—	—	2,163	—	2,163
Net loss	—	—	—	—	(116,319)	(116,319)
Balances at June 30, 2024	<u>49,317</u>	<u>\$ 5</u>	<u>\$ 1,171,894</u>	<u>\$ (2,266)</u>	<u>\$ (821,366)</u>	<u>\$ 348,267</u>

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	Six Months Ended June 30,	
	2025	2024
Cash flows from operating activities		
Net loss	\$ (64,788)	\$ (116,319)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	17,190	19,037
Depreciation and amortization	7,860	8,231
Net accretion of discounts on marketable debt securities	(2,427)	(1,711)
Impairment of long-lived assets	—	2,101
Non-cash interest expense	2,490	(27)
Other non-cash adjustments	12	(71)
Changes in operating assets and liabilities		
Accounts receivable	(790)	2,284
Prepaid expenses	(1,709)	4,499
Other current assets	(5,593)	(2,970)
Operating lease right-of-use assets	3,306	3,011
Other assets	2,121	1,861
Accounts payable	(1,117)	(6,454)
Accrued expenses and other current liabilities	(2,567)	(7,551)
Deferred revenue	37,666	(127)
Operating lease liabilities	(4,396)	(4,023)
Other liabilities	(2,971)	(2,723)
Net cash used in operating activities	(15,713)	(100,952)
Cash flows from investing activities		
Purchases of marketable debt securities	(230,298)	(137,718)
Maturities of marketable debt securities	136,020	151,664
Purchases of property and equipment	(1,415)	(1,031)
Net cash provided by (used in) investing activities	(95,693)	12,915
Cash flows from financing activities		
Proceeds from exercise of stock options	228	1,076
Taxes paid related to net settlement of stock-based awards	(510)	(910)
Proceeds from issuance of common stock under employee stock purchase plan	307	411
Proceeds from public offering of common stock and pre-funded warrants, net of issuance costs	—	131,352
Offering expenses related to at-the-market offering programs	(259)	(138)
Proceeds from issuance of royalty bond and warrants, net of transaction costs	144,526	—
Repayments under royalty monetization liabilities, net of interest	(10,854)	(20,511)
Net cash provided by financing activities	133,438	111,280
Net increase in cash and cash equivalents and restricted cash	22,032	23,243
Cash and cash equivalents and restricted cash		
Beginning of period	59,556	36,552
End of period	\$ 81,588	\$ 59,795

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

REGENXBIO INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

1. Nature of Business

REGENXBIO Inc. (the Company) is a clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's investigational gene therapies use adeno-associated virus (AAV) vectors from its proprietary gene delivery platform (NAV Technology Platform). The NAV[®] Technology Platform consists of exclusive rights to a large portfolio of proprietary AAV vectors. The Company has developed a broad pipeline of gene therapy product candidates using the NAV Technology Platform as a one-time treatment to address an array of diseases. In addition to its internal product development efforts, the Company also selectively licenses the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies (NAV Technology Licensees). As of June 30, 2025, the NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma[®], and in the preclinical and clinical development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity

The Company has incurred cumulative losses since inception and as of June 30, 2025, had generated an accumulated deficit of \$996.9 million. The Company's ability to transition to recurring profitability is dependent upon achieving a level of revenues adequate to support its cost structure, which depends heavily on the successful development, approval and commercialization of its product candidates. The Company may never achieve recurring profitability, and unless and until it does, will continue to need to raise additional capital through equity offerings, licensing and collaboration arrangements, or other non-dilutive financings. There is no assurance that the Company will be able to raise sufficient capital or obtain financing on favorable terms, or at all. The Company's ability to continue as a going concern may be impacted by the development and potential commercialization of its product candidates and its ability to raise additional capital to fund its operations. If the Company is unable to raise capital sufficient to meet its working capital needs in the future, it may be forced to delay expenditures, reduce the scope of its development activities or make other changes to its operating plans. As of June 30, 2025, the Company had cash, cash equivalents and marketable securities of \$363.6 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). The interim unaudited consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2024 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on March 13, 2025. Certain information and footnote disclosures required by GAAP, which are normally included in the Company's annual consolidated financial statements, have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which are normal and recurring in nature, necessary for a fair statement of the results of operations for the periods presented.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2024, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2024.

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Management bases its estimates on historical experience and various other factors that it

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believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates. Estimates are used in the following areas, among others: revenue recognition, the allowance for credit losses, accrued research and development expenses and other accrued liabilities, stock-based compensation expense, interest expense under royalty monetization liabilities, income taxes and fair value measurements.

Reclassifications

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation, including separate presentation of service revenue in the statements of operations and comprehensive loss. As a result of the Company's collaboration and license agreement with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) effective in March 2025, the Company has modified the presentation of its revenues and now presents service revenues separately from license and royalty revenues. The modified presentation has been applied retrospectively to all prior periods presented. The reclassifications have no effect on previously reported financial position, results of operations and cash flows.

Restricted Cash

Restricted cash consists of deposits held at financial institutions that are used to collateralize irrevocable letters of credit required under the Company's lease agreements and certain other agreements with third parties. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	As of June 30,	
	2025	2024
Cash and cash equivalents	\$ 79,558	\$ 57,765
Restricted cash	2,030	2,030
Total cash and cash equivalents and restricted cash	\$ 81,588	\$ 59,795

Accounts Receivable

Accounts receivable consist of consideration due to the Company resulting from its agreements with customers. Accounts receivable include amounts invoiced to customers as well as rights to consideration which have not yet been invoiced, including unbilled royalties and services, and for which payment is conditional solely upon the passage of time. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any accounts receivable from the licensee which are not contractually payable to the Company are charged off as a reduction of revenue in the period of the termination. Accounts receivable which are not expected to be received by the Company within 12 months from the reporting date are stated net of a discount to present value and recorded as non-current assets on the consolidated balance sheets. The present value discount is recognized as a reduction of revenue in the period in which the accounts receivable are initially recorded and is accreted as interest income from licensing over the term of the receivables.

Accounts receivable are stated net of an allowance for credit losses, if deemed necessary based on the Company's evaluation of collectability and potential credit losses. Management assesses the collectability of its accounts receivable using the specific identification of account balances, and considers the credit quality and financial condition of its significant customers, historical information regarding credit losses and the Company's evaluation of current and expected future economic conditions. If necessary, an allowance for credit losses is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. Accounts receivable balances are written off against the allowance for credit losses when the potential for collectability is considered remote. The Company did not record an allowance for credit losses on its accounts receivable as of June 30, 2025 and December 31, 2024.

Impairment of Long-lived Assets

The Company's long-lived assets consist primarily of property and equipment and operating lease right-of-use assets. The Company evaluates its long-lived assets for impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to estimated future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. Please refer to Note 5 and Note 6 for further information on impairment of long-lived assets.

Fair Value Measurements

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. Accounting Standards Codification (ASC) 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for assets and liabilities categorized in Level 3. The level within the fair value hierarchy of an asset or liability measured at fair value is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 financial instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third-party pricing providers or other market observable data. Please refer to Note 4 for further information on the Company's fair value measurements.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of ASC 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of ASC 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

The Company licenses its NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of its product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the Company's NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration payable to the Company under its license and collaboration agreements may

include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

The Company evaluates its agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company identifies the various transactions with the counterparty and determines if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, the Company applies the five-step model as described in its revenue recognition policies.

The Company's license and collaboration agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each agreement, the Company determines the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to the Company. For each license granted, the Company evaluates these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options.

Performance obligations under the Company's license and collaboration agreements may include (i) the delivery of intellectual property licenses, (ii) development and manufacturing services to be performed by the Company related to licensed products and (iii) options granted to purchase additional goods and services, to the extent the options convey material rights. At the inception of each license agreement which contains performance obligations for development or other services, the Company evaluates whether the license is distinct from the services, which requires judgment. In making this determination, the Company considers, among other things, the stage of development of the licensed products and whether the services will significantly impact further development of the licensed products. If it is determined that the license is not distinct from the services, the license is combined with the services into a single performance obligation. Agreements may provide licensees and collaborators with options to purchase additional goods or other services, including options to purchase commercial supply of licensed products. Options are evaluated at the inception of the agreement to determine whether they provide material rights to the customer. In making this determination, the Company considers whether the options are priced at an incremental discount to the standalone selling price of the underlying goods or services, in which case the option is considered to be a material right. Material rights are accounted for as separate performance obligations under the current arrangement.

The Company evaluates the transaction price of its license and collaboration agreements at contract inception and at each reporting date. The transaction price includes the fixed consideration payable to the Company over the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the agreements may include up-front and annual fees payable over the contract term and fixed fees for development and other services performed by the Company. Variable consideration under the agreements may include development and sales-based milestone payments, payments for development and other services performed by the Company, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by the customer is excluded from the transaction price and not accounted for as part of the arrangement until the option is exercised.

The transaction price of the Company's license and collaboration arrangements is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Variable consideration payable based on services performed by the Company is allocated directly to the performance obligation for such services. Consideration allocated to performance obligations for the delivery of intellectual property licenses is recognized as license and royalty revenue in full upon the delivery of the license. Consideration allocated to performance obligations for development and manufacturing services is recognized as service revenue as the services are performed by the Company. Consideration allocated to performance obligations for material rights to purchase additional goods and services is recognized as revenue upon the satisfaction of the performance obligations underlying the optional goods and services purchased by the customer. Service revenue is recognized using a measure of progress that best reflects the pattern of satisfaction of the performance obligations. At each reporting date, the Company re-evaluates the measure of progress and adjusts service revenue on a cumulative catch-up basis to reflect its best estimate of the services performed to date versus the total services to be performed under the arrangement.

Development milestone payments are payable to the Company upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of each outstanding development milestone and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. The increase to the transaction price as a result of any such adjustments is then allocated to the underlying performance obligations in a manner similar to the allocation of the initial transaction price and, to the extent the performance obligations are satisfied, recognized as revenue on a cumulative catch-up basis in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as license and royalty revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under the Company's license agreement with Novartis Gene Therapies, Inc. (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the development and commercialization of treatments for spinal muscular atrophy (SMA). The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

The Company receives payments from licensees and collaborators based on the billing schedules established in the associated agreements. Amounts recognized as revenue which have not yet been received from the customer are recorded as accounts receivable when the Company's rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from customers are recorded as contract assets when the Company's rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets if the consideration is expected to be realized within 12 months from the reporting date, or as other assets if the consideration is expected to be realized in periods beyond 12 months from the reporting date. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of revenue in the period of the termination. Amounts received by the Company prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations by the Company. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting the weighted-average common shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of computing both basic and diluted net loss per share, pre-funded warrants are considered outstanding shares upon issuance because the underlying shares may be issued for nominal consideration and are exercisable after the original issuance date. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation of diluted net loss per share if their effect would be anti-dilutive.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

The Company did not adopt any new accounting standards during the three and six months ended June 30, 2025 and 2024 which had a material impact on the consolidated financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In December 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*, which enhances the disclosure of an entity's effective tax rate reconciliation and requires the disclosure of income taxes paid to be disaggregated by jurisdiction. The standard is effective for the Company for annual periods beginning January 1, 2025. The Company is currently in the process of evaluating the impact of this standard on its consolidated financial statements and related disclosures.

In November 2024, the FASB issued ASU 2024-03, *Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses*. The new standard requires disclosures about specific types of expenses included in the expense captions presented on the face of the income statement as well as disclosures about selling expenses. In January 2025, the FASB issued ASU 2025-01, which clarifies the effective date of ASU 2024-03 with respect to interim periods. The standard is effective for the Company for annual periods beginning January 1, 2027 and interim periods beginning January 1, 2028, with early adoption permitted. The standard may be applied either prospectively to financial statements issued for reporting periods after the effective date or retrospectively to any or all prior periods presented in the financial statements. The Company is currently in the process of evaluating the impact of this standard on its consolidated financial statements and related disclosures.

3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist solely of available-for-sale debt securities (in thousands):

	<u>Amortized Cost</u>	<u>Unrealized Gains</u>	<u>Unrealized Losses</u>	<u>Fair Value</u>
June 30, 2025				
U.S. government and agency securities	\$ 132,032	\$ 17	\$ (30)	\$ 132,019
Certificates of deposit	245	—	—	245
Corporate bonds	151,649	149	(26)	151,772
	<u>\$ 283,926</u>	<u>\$ 166</u>	<u>\$ (56)</u>	<u>\$ 284,036</u>
December 31, 2024				
U.S. government and agency securities	\$ 44,281	\$ 11	\$ (77)	\$ 44,215
Certificates of deposit	1,466	—	(4)	1,462
Corporate bonds	141,474	234	(45)	141,663
	<u>\$ 187,221</u>	<u>\$ 245</u>	<u>\$ (126)</u>	<u>\$ 187,340</u>

As of June 30, 2025 and December 31, 2024, no available-for-sale debt securities had remaining maturities greater than two years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of June 30, 2025 and December 31, 2024, the balance in accumulated other comprehensive loss consisted solely of unrealized gains and losses on available-for-sale debt securities, net of reclassification adjustments for realized gains and losses and income tax effects. The Company uses the aggregate portfolio approach to release the tax effects of unrealized gains and losses on available-for-sale debt securities in accumulated other comprehensive loss. Realized gains and losses from the sale or maturity of marketable securities are based on the specific identification method and are included in results of operations as investment income. The Company did not recognize any realized gains or losses on available-for-sale securities during the three and six months ended June 30, 2025 and 2024, and no income tax effects or reclassification adjustments were recorded in accumulated other comprehensive loss during the periods.

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The following tables present the fair values and unrealized losses of available-for-sale debt securities held by the Company in an unrealized loss position for less than 12 months and 12 months or greater (in thousands):

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
June 30, 2025						
U.S. government and agency securities	\$ 81,230	\$ (28)	\$ 2,498	\$ (2)	\$ 83,728	\$ (30)
Certificates of deposit	—	—	245	—	245	—
Corporate bonds	41,726	(26)	—	—	41,726	(26)
	<u>\$ 122,956</u>	<u>\$ (54)</u>	<u>\$ 2,743</u>	<u>\$ (2)</u>	<u>\$ 125,699</u>	<u>\$ (56)</u>
December 31, 2024						
U.S. government and agency securities	\$ —	\$ —	\$ 22,917	\$ (77)	\$ 22,917	\$ (77)
Certificates of deposit	—	—	1,221	(4)	1,221	(4)
Corporate bonds	21,317	(45)	—	—	21,317	(45)
	<u>\$ 21,317</u>	<u>\$ (45)</u>	<u>\$ 24,138</u>	<u>\$ (81)</u>	<u>\$ 45,455</u>	<u>\$ (126)</u>

As of June 30, 2025, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 41 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and based on the credit quality of the issuers and low severity of each unrealized loss position relative to its amortized cost basis, the Company did not identify any credit losses associated with its available-for-sale debt securities. The Company did not record an allowance for credit losses on its available-for-sale debt securities as of June 30, 2025 or December 31, 2024, and no impairment or credit losses on available-for-sale debt securities were recorded during the three and six months ended June 30, 2025 and 2024.

4. Fair Value Measurements

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the fair value hierarchy discussed in Note 2 (in thousands):

	Level 1	Level 2	Level 3	Total
June 30, 2025				
Cash equivalents:				
Money market mutual funds	\$ —	\$ 44,975	\$ —	\$ 44,975
U.S. government and agency securities	—	9,969	—	9,969
Total cash equivalents	—	54,944	—	54,944
Marketable securities:				
U.S. government and agency securities	—	132,019	—	132,019
Certificates of deposit	—	245	—	245
Corporate bonds	—	151,772	—	151,772
Total marketable securities	—	284,036	—	284,036
Total cash equivalents and marketable securities	<u>\$ —</u>	<u>\$ 338,980</u>	<u>\$ —</u>	<u>\$ 338,980</u>

	Level 1	Level 2	Level 3	Total
December 31, 2024				
Cash equivalents:				
Money market mutual funds	\$ —	\$ 43,895	\$ —	\$ 43,895
U.S. government and agency securities	—	2,498	—	2,498
Total cash equivalents	—	46,393	—	46,393
Marketable securities:				
U.S. government and agency securities	—	44,215	—	44,215
Certificates of deposit	—	1,462	—	1,462
Corporate bonds	—	141,663	—	141,663
Total marketable securities	—	187,340	—	187,340
Total cash equivalents and marketable securities	\$ —	\$ 233,733	\$ —	\$ 233,733

Management estimates that the carrying values of its current accounts receivable, other current assets, accounts payable, accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Certain accounts receivable which contain non-current portions and certain non-current payables reported as other liabilities are recorded at their present values using a discount rate that is based on prevailing market rates on the date the amounts were initially recorded. Management does not believe there have been any significant changes in market conditions or credit quality that would cause the discount rates initially used to be materially different from those that would be used as of June 30, 2025 to determine the present value of these instruments. Accordingly, management estimates that the carrying values of its non-current accounts receivable and other liabilities approximate the fair value of those instruments. Management estimates that the carrying values of its royalty monetization liabilities approximate fair value. As discussed in Note 7, the carrying values of royalty monetization liabilities are based on the Company's estimate of future royalties, milestones and other consideration to be paid over the life of the arrangement, which are considered Level 3 inputs, as well as any remaining repayment obligations upon maturity of the instruments.

Long-lived assets, if determined to be impaired, are measured at fair value on a nonrecurring basis using Level 3 inputs. Please refer to Note 6 for further information on nonrecurring fair value measurements of long-lived assets during the three and six months ended June 30, 2025 and 2024.

5. Property and Equipment, Net

Property and equipment, net consists of the following (in thousands):

	June 30, 2025	December 31, 2024
Laboratory and manufacturing equipment	\$ 77,765	\$ 77,141
Computer equipment and software	4,743	4,244
Furniture and fixtures	7,032	7,031
Leasehold improvements	101,533	101,465
Total property and equipment	191,073	189,881
Accumulated depreciation and amortization	(80,056)	(72,292)
Property and equipment, net	\$ 111,017	\$ 117,589

In March 2024, the Company entered into an agreement to sublease its office facilities in New York, New York. In connection with the sublease, the Company recorded impairment of property and equipment of \$0.7 million in the first quarter of 2024 related to furniture and fixtures and leasehold improvements located at the subleased facility. Please refer to Note 6 for further information regarding the sublease agreement and associated impairment of long-lived assets.

6. Leases

New York Lease and Sublease

In May 2016, the Company entered into an operating lease for office space in New York, New York (the New York Lease), which has since been amended to include additional office space and extend the term of the lease. The lease term commenced in July 2016 and expires in April 2027.

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In March 2024, the Company entered into an agreement to sublease its office space under the New York Lease (the New York Sublease) to a third-party subtenant. The sublease term commenced in April 2024 and expires in April 2027 concurrent with the expiration of the New York Lease. Monthly payments under the New York Sublease commenced in July 2024 and escalate annually in accordance with the sublease agreement. As of June 30, 2025, total undiscounted future minimum lease payments to be received by the Company over the term of the New York Sublease were \$0.9 million. The Company recognized sublease income under the New York Sublease of \$0.1 million and \$0.2 million during the three and six months ended June 30, 2025, respectively, and \$0.1 million during the three and six months ended June 30, 2024.

The New York Sublease is classified as an operating lease and the Company was not relieved of its primary obligation under the New York Lease. The Company continues to account for the New York Lease as it did prior to the commencement of the sublease.

As a result of the New York Sublease, the Company determined an impairment indicator was present as of March 31, 2024 related to the long-lived asset group subject to the sublease, which included the right-of-use asset under the New York Lease, leasehold improvements and other property and equipment allocable to the New York Sublease. The Company concluded the carrying value of the asset group as of March 31, 2024 was not recoverable, as it exceeded the sum of the estimated undiscounted cash flows to be generated by the assets over their remaining lives. The Company estimated the fair value of the asset group as of March 31, 2024 using a discounted cash flow method, which incorporated unobservable inputs including the net identifiable cash flows over the term of the New York Sublease and an estimated borrowing rate of a market participant subtenant. The estimated fair value of the asset group as of March 31, 2024 represents a Level 3 nonrecurring fair value measurement. The Company concluded the carrying value of the asset group of \$3.4 million exceeded its estimated fair value of \$1.3 million as of March 31, 2024. As such, the Company recognized impairment losses of \$2.1 million during the three months ended March 31, 2024 on the long-lived asset group associated with the New York Sublease. The impairment losses were allocated to the various assets within the long-lived asset group based on their relative carrying values and consisted of \$1.4 million recorded to the right-of-use assets and \$0.7 million recorded to property and equipment. No impairment losses on long-lived assets were recorded during the three and six months ended June 30, 2025 and the three months ended June 30, 2024.

7. Royalty Monetization Liabilities

Royalty monetization liabilities are accounted for as debt and consist of the following (in thousands):

	June 30, 2025	December 31, 2024
2020 Royalty Purchase Agreement	\$ 48,231	\$ 59,687
2025 Royalty Bond	145,764	—
Total	<u>\$ 193,995</u>	<u>\$ 59,687</u>
Current portion of royalty monetization liabilities	\$ 40,302	\$ 34,309
Non-current portion of royalty monetization liabilities	153,693	25,378
Total	<u>\$ 193,995</u>	<u>\$ 59,687</u>

2020 Royalty Purchase Agreement

In December 2020, the Company entered into a royalty purchase agreement (the 2020 Royalty Purchase Agreement) with entities managed by Healthcare Royalty Management, LLC (collectively and with other affiliated entities, HCR). Under the 2020 Royalty Purchase Agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement with Novartis Gene Therapies (the Novartis License), including \$4.0 million of royalty payments received by the Company in the fourth quarter of 2020. In consideration for these rights, HCR paid the Company \$200.0 million (the Purchase Price), less \$4.0 million representing the payment of the royalties received in the fourth quarter of 2020 to HCR. Beginning upon the effective date of the 2020 Royalty Purchase Agreement, Zolgensma royalty payments, up to a specified threshold, shall be paid to HCR, net of upstream royalties payable by the Company to certain licensors in accordance with existing license agreements.

Pursuant to the 2020 Royalty Purchase Agreement, the total amount of royalty payments to be paid to HCR was subject to an increasing cap (the Cap Amount) equal to (i) \$260.0 million applicable for the period from the effective date of the 2020 Royalty Purchase Agreement through November 7, 2024 (the First Cap Amount), and (ii) \$300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the Novartis License (the Second Cap Amount). If, on or prior to the defined dates for each Cap Amount, the total amount of royalty payments paid to HCR equals or exceeds the Cap Amount applicable to such date, the 2020 Royalty Purchase Agreement will automatically terminate. The First Cap Amount was not achieved prior to November 7, 2024, therefore the 2020 Royalty Purchase Agreement will remain in effect until the achievement of the Second Cap

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Amount or the termination of the Novartis License, if earlier. The Company has no obligation to repay any amounts to HCR under the 2020 Royalty Purchase Agreement if future Zolgensma royalty payments are not sufficient to achieve the applicable Cap Amount prior to the termination of the Novartis License.

The Company has a call option to repurchase its rights to the royalties under the 2020 Royalty Purchase Agreement for a repurchase price equal to, as of the option exercise date, \$300.0 million minus the total amount of royalty payments paid to HCR.

The proceeds received from HCR under the 2020 Royalty Purchase Agreement of \$196.0 million were recorded as a liability, net of transaction costs of \$3.5 million, which is amortized over the estimated life of the arrangement using the effective interest method. In order to determine the amortization of the liability, the Company is required to estimate the total amount of future royalty payments to be paid to HCR, subject to the Cap Amount, over the life of the arrangement. The total amount of royalty payments paid to HCR, less the net proceeds received by the Company of \$192.5 million, is recorded as interest expense over the life of the arrangement using the effective interest method. Due to its continuing involvement in the Novartis License, the Company continues to recognize royalty revenue on net sales of Zolgensma and records the royalty payments to HCR as a reduction of the liability when paid. As such payments are made to HCR, the balance of the liability will be effectively repaid over the life of the arrangement.

The Company estimates the effective interest rate used to record interest expense under the 2020 Royalty Purchase Agreement based on its estimate of future royalty payments to be paid HCR. At each reporting date, the Company reassesses its estimate of total future royalty payments to be paid to HCR at the applicable Cap Amount, and prospectively adjusts the effective interest rate and amortization of the liability as necessary. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the royalty payments paid to HCR and changes in the Company's forecasted royalties. The estimated interest rate in effect as of June 30, 2025 and December 31, 2024 was 71.4% and 65.5%, respectively, which was based on the amortized balance of the liability and the estimated remaining royalties to be paid to HCR under the arrangement. This interest rate is subject to adjustments in the future based on actual royalties paid to HCR and changes in the royalty forecast. As of June 30, 2025, the estimated effective interest rate over the life of the 2020 Royalty Purchase Agreement, taking into account actual royalties paid to date and the estimated remaining royalties to be paid under the arrangement, was 16.2%.

The following table presents the changes in the royalty monetization liability under the 2020 Royalty Purchase Agreement with HCR (in thousands):

	2020 Royalty Purchase Agreement
Balance at December 31, 2024	\$ 59,687
Zolgensma royalties paid to HCR	(27,928)
Interest expense recognized	16,472
Balance at June 30, 2025	48,231
Current portion	(36,996)
Non-current portion	\$ 11,235

2025 Royalty Bond

In May 2025, the Company entered into a loan agreement with HCR pursuant to which HCR will provide the Company with an aggregate limited recourse loan of up to \$250.0 million (the 2025 Royalty Bond). The 2025 Royalty Bond is disbursable to the Company in three tranches, with \$150.0 million funded on the closing date in May 2025, \$50.0 million available to be funded if sales of a specified products exceed a specified sales threshold prior to December 31, 2026, and \$50.0 million available to be funded if both parties exercise an option in 2027. Loan proceeds under the 2025 Royalty Bond are funded to the Company net of an original issue discount of 2.25% and reimbursement of certain expenses to HCR. Proceeds received by the Company from the initial funding tranche of the 2025 Royalty Bond in May 2025, net of discounts and transaction costs, were \$144.5 million.

Prior to the maturity date, interest and principal under the 2025 Royalty Bond shall be paid quarterly to HCR solely from proceeds received from certain specified royalties, milestone payments, license fees and other consideration payable to the Company under specified license agreements (collectively, the Royalty Interest), including (i) the Novartis License for Zolgensma, (ii) the collaboration and license agreement with Nippon Shinyaku for RGX-121 and RGX-111, and (iii) NAV Technology Platform license agreements with Rocket Pharmaceuticals, Inc. and Ultragenyx Pharmaceutical Inc. Zolgensma royalties under the Novartis License shall only be included in the Royalty Interest after full repayment of the applicable Cap Amount under the 2020 Royalty Purchase Agreement with HCR. The Royalty Interest excludes, and the Company retains the rights to, certain other consideration payable under the license agreements including certain milestone payments, license fees and reimbursement of certain costs as applicable. The Royalty Interest is payable to HCR net of upstream royalty and sublicense fee obligations payable by the Company to applicable licensors. The 2025 Royalty Bond is collateralized by a security interest and lien on the Royalty Interest.

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The 2025 Royalty Bond bears interest at a rate of 9.75% plus the 3-month secured overnight financing rate as administered by the Federal Reserve Bank of New York (SOFR), with a minimum interest rate of 14.0%. Interest payments are due quarterly using proceeds received under the Royalty Interest. At each payment date, any proceeds received under the Royalty Interest in excess of the interest payment due will be applied to outstanding principal. If the proceeds received under the Royalty Interest are insufficient to pay the interest due, unpaid interest will accrue to the principal balance.

The 2025 Royalty Bond matures in May 2035, subject to potential extension, unless repaid in full at an earlier date. The maturity date may be extended by two years to May 2037 subject to a potential patent term extension of a specific patent. Upon maturity, the outstanding principal and interest shall be due and payable to HCR. Additionally, upon repayment in full prior to the maturity date, or at the maturity date, the Company shall pay to HCR an additional amount equal to 5.0% of the total outstanding principal as of the applicable determination date. Other than through the payment of proceeds received under the Royalty Interest, the 2025 Royalty Bond may not be prepaid prior to maturity.

In connection with the loan agreement for the 2025 Royalty Bond, the Company also issued HCR warrants to purchase 268,096 shares of its common stock at an exercise price per share of \$14.92 (the May 2025 Warrants). The May 2025 Warrants are exercisable upon issuance and expire 10 years from the closing date of the 2025 Royalty Bond. The Company evaluated the May 2025 Warrants and concluded the warrants are indexed to the Company's common stock and meet the criteria to be classified as equity. The net proceeds received by the Company under the loan agreement of \$144.5 million were allocated between the 2025 Royalty Bond and the May 2025 Warrants based on their relative fair values. The fair value of the 2025 Royalty Bond was determined based on the carrying amount of the loan on the closing date. The fair value of the May 2025 Warrants was determined using a Black-Scholes option-pricing model on the closing date, resulting in an estimated fair value of the warrants of \$1.7 million. Based on the relative fair values of these instruments, \$1.6 million of the net proceeds were allocated to the warrants and recorded as additional paid-in capital. The net proceeds allocated to the 2025 Royalty Bond were \$142.9 million, resulting in a total debt discount of \$7.1 million which is recorded as a reduction of the carrying value of the debt and will be amortized as interest expense over the life of the 2025 Royalty Bond.

The effective interest rate of the 2025 Royalty Bond is partially estimated based on the Company's estimate of future payments under the Royalty Interest to be paid HCR. At each reporting date, the Company reassesses its estimate of total future payments to HCR under the arrangement and prospectively adjusts the effective interest rate and amortization of the debt and associated discount as necessary. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the Royalty Interest payments to HCR, changes in the Company's forecast and fluctuations in the variable interest rate. The estimated effective interest rate in effect as of June 30, 2025 was 15.2%, which was based on the amortized balance of the liability, the estimated remaining payments to HCR under the arrangement and the current coupon rate in effect at the end of the period.

The following table presents the changes in the royalty monetization liability under the 2025 Royalty Bond with HCR (in thousands):

	<u>2025 Royalty Bond</u>
Balance at December 31, 2024	\$ —
Proceeds from royalty bond, net of discount and issuance costs	142,883
Unpaid interest accrued to principal	2,668
Amortization of debt discount and issuance costs	213
Balance at June 30, 2025	145,764
Current portion	(3,306)
Non-current portion	<u>\$ 142,458</u>

8. Commitments and Contingencies

GlaxoSmithKline

In March 2009, the Company entered into a license agreement, which was amended in April 2009 (as amended, the GSK License), with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents underlying the Company's NAV Technology Platform which are owned by The Trustees of the University of Pennsylvania (Penn) and exclusively licensed to GSK. Pursuant to the GSK License, the Company is obligated to pay GSK royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse GSK for certain costs incurred related to the maintenance of the licensed patents. The Company was also obligated to pay \$1.5 million to GSK upon the achievement of various milestones, all of which have been achieved and paid.

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In March 2022, the Company entered into a letter agreement (the Penn Letter Agreement) with Penn to buy out the Company's obligation to pay sublicense fees under its license agreement with Penn. In connection with the execution of the Penn Letter Agreement in March 2022, the Company's royalty obligations under the GSK License were assigned by GSK to Penn. Beginning upon the effective date of the Penn Letter Agreement in March 2022, any royalties payable by the Company under the GSK License shall be paid to Penn rather than GSK. The Company remains obligated to pay GSK sublicense fees and reimbursement of certain patent maintenance costs in accordance with the GSK License.

Expenses incurred by the Company related to the GSK License were recorded as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Cost of license and royalty revenues:				
Royalties on net sales of Zolgensma	\$ 5,204	\$ 10,570	\$ 7,786	\$ 14,838
Other	5	9	243	24
Total cost of license and royalty revenues	5,209	10,579	8,029	14,862
General and administrative	53	82	107	77
	<u>\$ 5,262</u>	<u>\$ 10,661</u>	<u>\$ 8,136</u>	<u>\$ 14,939</u>

As of June 30, 2025 and December 31, 2024, the Company had recorded \$4.9 million and \$6.3 million, respectively, payable under the GSK License.

The Company has been notified of a potential dispute with GSK over the amount of sublicense fees paid by the Company to GSK under the GSK License. GSK claims there has been a significant underpayment by the Company as they are entitled to a sublicense payment on all amounts received by the Company from sublicensees, including royalties, and not just amounts received for GSK's sublicensed patents. The Company disagrees with GSK's interpretation of the GSK License. The Company does not believe that a loss is probable, and no reasonable range of loss is estimable, related to this matter. No liabilities related to this matter were recorded as of June 30, 2025 and December 31, 2024.

9. Capitalization

March 2024 Public Offering and Pre-funded Warrants

In March 2024, the Company completed a public offering of 4,565,260 shares of its common stock at a price of \$23.00 per share and 1,521,740 pre-funded warrants (the March 2024 Pre-funded Warrants) to purchase shares of its common stock at a price of \$22.9999 per pre-funded warrant, which equaled the public offering price per share of the common stock less the \$0.0001 exercise price of each pre-funded warrant. The aggregate net proceeds received by the Company from the offering were \$131.1 million, net of underwriting discounts and commissions and offering expenses.

The rights and privileges of the March 2024 Pre-funded Warrants are set forth in the warrant agreement between the Company and each of the respective warrant holders. The March 2024 Pre-funded Warrants are exercisable at the option of the warrant holder at any time and do not expire. However, as set forth in the warrant agreements with each holder, the number of pre-funded warrants that may be exercised at any given time may be limited if, upon exercise, the warrant holder and any of its affiliates would beneficially own more than 9.99% of the Company's common stock, or have voting power of more than 9.99% of the Company's common stock. The limitation threshold may be increased or decreased by the warrant holder, with advance notice to the Company, to any other percentage not less than 4.99% nor in excess of 19.99%. The March 2024 Pre-funded Warrants do not provide any of the rights or privileges provided by the Company's common stock, including any voting rights, until the pre-funded warrants are exercised and settled in underlying shares of common stock.

The Company evaluated the March 2024 Pre-funded Warrants and concluded the warrants are indexed to the Company's common stock, meet the criteria to be classified as equity and are not subject to remeasurement. The proceeds received from the issuance of the pre-funded warrants were recorded as additional paid-in capital. The Company issued 199,300 shares of common stock upon the exercise of March 2024 Pre-funded Warrants during the three and six months ended June 30, 2025, and 197,000 shares of common stock upon the exercise of March 2024 Pre-funded Warrants during the three and six months ended June 30, 2024. As of June 30, 2025, 1,125,440 of the March 2024 Pre-funded Warrants remained outstanding.

May 2025 Warrants

In May 2025, in connection with issuance of the 2025 Royalty Bond, the Company issued to HCR the May 2025 Warrants to purchase 268,096 shares of its common stock at an exercise price per share of \$14.92. The May 2025 Warrants are exercisable upon issuance and have a contractual term of 10 years. The Company evaluated the May 2025 Warrants and concluded the warrants are indexed to the Company's common stock, meet the criteria to be classified as equity and are not subject to remeasurement. The Company allocated \$1.6 million of the net proceeds from the 2025 Royalty Bond to the issuance of the May 2025 Warrants, which were recorded as additional paid-in capital. Please refer to Note 7 for further information on the May 2025 Warrants issued in connection with the 2025 Royalty Bond. As of June 30, 2025, none of the May 2025 Warrants had been exercised and 268,096 of the May 2025 Warrants remained outstanding.

At-the-Market Offering Program

In December 2024, the Company entered into a Sales Agreement with Leerink Partners LLC (Leerink) pursuant to which the Company may offer and sell shares of its common stock having an aggregate offering price of up to \$150.0 million from time to time through Leerink, acting as the Company's sales agent (the Leerink ATM Program). As of June 30, 2025, no shares of common stock had been sold under the Leerink ATM Program.

10. License and Collaboration Agreements

License and Collaboration Revenues

As of June 30, 2025, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma, and in the development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates. Consideration payable to the Company under its license and collaboration agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators. Sublicense fees vary by license and range from a mid-single digit percentage to a low-double digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single digit percentage to a low double-digit percentage of net sales by licensees.

Revenues earned under license and collaboration agreements consisted of the following (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
License and royalty revenue:				
Zolgensma royalties	\$ 18,423	\$ 21,763	\$ 35,416	\$ 36,977
Nippon Shinyaku licenses	—	—	69,979	—
Other license and royalty revenue	42	83	119	213
Total license and royalty revenue	18,465	21,846	105,514	37,190
Service revenue:				
Nippon Shinyaku services	2,720	—	4,494	—
Other service revenue	174	449	363	727
Total service revenue	2,894	449	4,857	727
Total revenues	\$ 21,359	\$ 22,295	\$ 110,371	\$ 37,917

Outstanding development milestone payments are evaluated each reporting period and are only included in the transaction price of each license to the extent the milestones are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as royalty revenue in the period of achievement. As of June 30, 2025, the Company's license and collaboration agreements contained unachieved milestones which could result in aggregate milestone payments to the Company of up to \$2.18 billion, including (i) \$548.2 million upon the commencement of various stages of clinical trials, (ii) \$106.3 million upon the submission of regulatory approval filings or upon regulatory approval of licensed products and (iii) \$1.53 billion upon the achievement of specified sales targets for licensed products, including milestones payable upon the first commercial sale of licensed products. To the extent the milestone payments are realized by the Company, the Company may be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of these milestones is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

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Accounts Receivable, Contract Assets and Deferred Revenue

The following table presents the balances of the Company's accounts receivable, contract assets and deferred revenue, as well as other information regarding revenue recognized, during the periods presented (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Accounts receivable, net, current and non-current:				
Beginning of period	\$ 19,400	\$ 16,351	\$ 20,947	\$ 25,491
End of period	\$ 21,783	\$ 23,273	\$ 21,783	\$ 23,273
Contract assets:				
Beginning of period	\$ 44	\$ —	\$ 239	\$ —
End of period	\$ 28	\$ 194	\$ 28	\$ 194
Deferred revenue, current and non-current:				
Beginning of period	\$ 39,885	\$ 13	\$ 115	\$ 148
End of period	\$ 37,781	\$ 21	\$ 37,781	\$ 21
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	\$ 2,104	\$ 13	\$ 115	\$ 148
Performance obligations satisfied in previous periods	\$ 18,425	\$ 21,768	\$ 35,420	\$ 37,037

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma royalties.

As of June 30, 2025, the Company had recorded deferred revenue of \$37.8 million which represents consideration received or unconditionally due from licensees and collaboration partners for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations as of June 30, 2025 consisted of (i) development services to be performed related to licensed products, which will be satisfied as the services are performed, and (ii) material rights granted to purchase commercial supply of licensed products, which will be satisfied upon delivery of the commercial supply. As of June 30, 2025, the aggregate transaction price of the Company's license and collaboration agreements allocated to performance obligations not yet satisfied or partially satisfied was \$43.4 million, primarily associated with the Company's collaboration and license agreement with Nippon Shinyaku, the substantial majority of which is expected to be satisfied over a period of approximately five years.

Accounts receivable consisted of the following (in thousands):

	June 30, 2025	December 31, 2024
Current accounts receivable:		
Billed to customers	\$ 60	\$ 65
Unbilled Zolgensma royalties	18,870	20,106
Unbilled Nippon Shinyaku services	1,127	—
Other unbilled	142	302
Current accounts receivable	20,199	20,473
Non-current accounts receivable:		
Unbilled Nippon Shinyaku services	1,127	—
Other unbilled	457	474
Non-current accounts receivable	1,584	474
Total accounts receivable	\$ 21,783	\$ 20,947

Zolgensma License with Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement (as amended, the Novartis License) with Novartis Gene Therapies. Under the Novartis License, the Company granted Novartis Gene Therapies an exclusive, worldwide commercial license, with rights to sublicense, to the NAV Technology Platform, as well as other certain rights, for the treatment of SMA in humans by *in vivo* gene therapy. In 2019, Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the Novartis License. In accordance with the Novartis License, the Company receives royalties on net sales of Zolgensma.

The Company recognized the following amounts under the Novartis License (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Zolgensma royalties	\$ 18,423	\$ 21,763	\$ 35,416	\$ 36,977
Total license and royalty revenue	\$ 18,423	\$ 21,763	\$ 35,416	\$ 36,977
Interest income from licensing	\$ 10	\$ 6	\$ 21	\$ 13

As of June 30, 2025 and December 31, 2024, the Company had recorded total accounts receivable of \$19.2 million and \$20.4 million, respectively, from Novartis Gene Therapies under the Novartis License, which consisted primarily of Zolgensma royalties receivable. The Zolgensma royalties receivable recorded as of June 30, 2025 included \$14.1 million expected to be paid to HCR in accordance with the 2020 Royalty Purchase Agreement discussed in Note 7. The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

Collaboration Agreements

AbbVie Collaboration and License Agreement

In September 2021, the Company entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314, the Company's product candidate for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal diseases (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement became effective in November 2021.

Pursuant to the AbbVie Collaboration Agreement, the Company granted AbbVie a co-exclusive license to develop and commercialize ABBV-RGX-314 in the United States and an exclusive license to develop and commercialize ABBV-RGX-314 outside the United States. The Company and AbbVie will collaborate to develop ABBV-RGX-314 in the United States, and AbbVie will be responsible for the development of ABBV-RGX-314 in specified markets outside the United States. Through December 31, 2022, the Company was responsible for the development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the parties shared the additional development expenses related to ABBV-RGX-314. Beginning on January 1, 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of ABBV-RGX-314 for commercial supply outside the United States. Manufacturing expenses will be allocated between the parties in accordance with the terms of the AbbVie Collaboration Agreement and mutually agreed supply agreements. If requested by AbbVie, the Company will manufacture up to a specified portion of ABBV-RGX-314 for commercial supply outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of ABBV-RGX-314 globally, and the Company will participate in U.S. commercialization efforts as provided under a commercialization plan determined in accordance with the agreement. The Company and AbbVie will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of ABBV-RGX-314.

In consideration for the rights granted under the AbbVie Collaboration Agreement, AbbVie paid the Company an up-front fee of \$370.0 million upon the effective date of the agreement in November 2021 and is required to pay to the Company up to \$1.38 billion upon the achievement of specified development and sales-based milestones, of which \$562.5 million are based on development milestones and \$820.0 million are sales-based milestones. AbbVie is also required to pay to the Company tiered royalties on net sales of ABBV-RGX-314 outside the United States at percentages in the mid-teens to low twenties, subject to specified offsets and reductions.

The Company applied the requirements of ASC 606 to the AbbVie Collaboration Agreement for the units of account in which AbbVie was deemed to be a customer. The Company determined that there is only one material performance obligation under the agreement for the delivery of the intellectual property license to develop and commercialize ABBV-RGX-314 globally. The intellectual property licensed to AbbVie includes the rights to certain patents, data, know-how and other rights developed and owned by the Company, as well as other intellectual property rights exclusively licensed by the Company from various third parties. As of June 30, 2025 and December 31, 2024, the transaction price of the AbbVie Collaboration Agreement was \$370.0 million, which consisted solely of the up-front payment received from AbbVie in November 2021. The \$370.0 million transaction price was fully recognized as revenue upon the delivery of the license to AbbVie in November 2021. Variable consideration under the AbbVie Collaboration Agreement, which has been excluded from the transaction price, includes \$562.5 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement. Additionally, the transaction price excludes sales-based milestone payments of \$820.0 million and royalties on net sales of ABBV-RGX-314 outside the United States. Development milestones will be added to the transaction price and recognized as revenue upon achievement, or if deemed probable of achievement. In accordance with the sale- or usage-based royalty exception under ASC 606, royalties on net sales and sales-based milestones will be recognized as revenue in the period the underlying sales occur or milestones are achieved. There were no changes in the transaction price of the AbbVie Collaboration Agreement, and no revenue was recognized, during the three and six months ended June 30, 2025 and 2024.

The Company applied the requirements of ASC 808 to the AbbVie Collaboration Agreement for the units of account which were deemed to be a collaborative arrangement. Both the Company and AbbVie will perform various activities related to the development, manufacturing and commercialization of ABBV-RGX-314 in the United States. Development costs are shared between the parties in accordance with the terms of the AbbVie Collaboration Agreement, and the parties will share equally in the net profits and losses derived from sales of ABBV-RGX-314 in the United States. The Company accounts for payments to and from AbbVie for the sharing of development and commercialization costs in accordance with its accounting policy for collaborative arrangements. Amounts owed to AbbVie for the Company's share of development costs or commercialization costs incurred by AbbVie are recorded as research and development expense or general and administrative expense, respectively, in the period the costs are incurred. Amounts owed to the Company for AbbVie's share of development costs or commercialization costs incurred by the Company are recorded as a reduction of research and development expense or general and administrative expense, respectively, in the period the costs are incurred. At the end of each reporting period, the Company records a net amount due to or from AbbVie as a result of the cost-sharing arrangement. As of June 30, 2025 and December 31, 2024, the Company had recorded \$17.0 million and \$11.3 million, respectively, due from AbbVie for net reimbursement of costs incurred for activities performed under AbbVie Collaboration Agreement, which was included in other current assets on the consolidated balance sheets.

The Company recognized the following amounts under the AbbVie Collaboration Agreement (in thousands):

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2025</u>	<u>2024</u>	<u>2025</u>	<u>2024</u>
Net cost reimbursement to (from) AbbVie included in:				
Research and development expense	\$ (17,056)	\$ (21,271)	\$ (31,737)	\$ (46,248)
General and administrative expense	551	425	1,148	827
Total net cost reimbursement to (from) AbbVie	<u>\$ (16,505)</u>	<u>\$ (20,846)</u>	<u>\$ (30,589)</u>	<u>\$ (45,421)</u>

In August 2025, the Company and AbbVie entered into an amendment to the AbbVie Collaboration Agreement which modifies the development plan and milestone payment structure for the ABBV-RGX-314 DR program. Under the amendment, the Company will conduct the first registration enabling trial for DR suprachoroidal (SCS) treatment as a combined Phase IIb/III trial performed in two parts (Part 1 and Part 2), and AbbVie will conduct the second registration enabling trial as a separate, standalone Phase III trial. In lieu of the \$200.0 million milestone due to the Company under the original AbbVie Collaboration Agreement upon first patient dosed in the first registration enabling trial for DR SCS treatment, AbbVie will pay the Company \$100.0 million upon first patient dosed in the Phase IIb/III trial for DR SCS treatment and an additional \$100.0 million upon first patient dosed in the subsequent Phase III trial. Also pursuant to the amendment, AbbVie will lead a new Phase IIb randomized controlled study (the ACHIEVE Study) to assess the injection burden, adverse events, change in disease activity, and long-term preservation of visual acuity of ABBV-RGX-314 in adult participants with neovascular AMD. The Company will be responsible for its development expenses to conduct Part 1 of the Phase IIb/III trial for DR and the parties will share the development expenses related to Part 2 of the Phase IIb/III trial and the subsequent Phase III trial for DR in accordance with the existing terms of the AbbVie Collaboration Agreement. AbbVie will be responsible for all development expenses related to the ACHIEVE Study.

Nippon Shinyaku Collaboration and License Agreement

In January 2025, the Company entered into a collaboration and license agreement with Nippon Shinyaku for the development and commercialization of RGX-121, the Company's product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), and RGX-111, the Company's product candidate for the treatment of Mucopolysaccharidosis Type I (MPS I) (the Nippon Shinyaku Collaboration Agreement). The Nippon Shinyaku Collaboration Agreement became effective in March 2025.

Pursuant to the Nippon Shinyaku Collaboration Agreement, the Company granted Nippon Shinyaku a license to develop and exclusively commercialize RGX-121 and RGX-111 in the United States and certain countries in Asia. The Company is responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. The Company is responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement and mutually agreed supply agreements. Nippon Shinyaku will be responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. The Company reserves the right to develop and commercialize RGX-121 and RGX-111 in countries outside the licensed territories. The Nippon Shinyaku Collaboration Agreement contains provisions for termination, including termination for convenience by Nippon Shinyaku.

In consideration for the rights granted and services to be performed under the Nippon Shinyaku Collaboration Agreement, Nippon Shinyaku paid the Company an up-front fee of \$110.0 million upon the effective date of the agreement in March 2025 and is required to pay to the Company up to \$700.0 million upon the achievement of specified development and sales-based milestones, of which \$40.0 million are based on development milestones and \$660.0 million are sales-based milestones. Nippon Shinyaku is also required to pay to the Company double-digit royalties on net sales of RGX-121 and RGX-111 in the licensed territories, subject to specified offsets and reductions. The Company retains all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111.

The Company evaluated its various commitments under the Nippon Shinyaku Collaboration Agreement and identified the distinct units of account under the arrangement. For each of the distinct units of account identified, the Company determined whether the transactions should be accounted for as a contract with a customer within the scope of ASC 606 or as a collaborative arrangement within the scope of ASC 808. The Company concluded that each of the distinct units of account identified should be accounted for as revenue under ASC 606, as Nippon Shinyaku is deemed to be a customer for each of the various transactions. The Company identified the following material performance obligations under the agreement: (i) delivery of intellectual property licenses to develop and commercialize RGX-121 and RGX-111 in the United States and Asia territories, (ii) development services for RGX-121 and RGX-111 in the United States, including manufacturing of clinical supply and commercial supply prior to regulatory approval, and (iii) material rights granted to Nippon Shinyaku to purchase commercial supply for sales in licensed territories.

The intellectual property licensed to Nippon Shinyaku includes the rights to certain patents, data, know-how and other rights developed and owned by the Company, as well as other intellectual property rights exclusively licensed by the Company from various third parties. In determining the distinct performance obligations under the agreements, the Company concluded that the licenses granted to Nippon Shinyaku to develop and commercialize RGX-121 and RGX-111 are distinct from the other goods and services promised under the agreement, as Nippon Shinyaku can benefit from the license on a standalone basis and, based on the stage of development of the product candidates, the underlying licensed products and know-how are not expected to be significantly modified as a result of other goods and services promised under the agreement. The Company evaluated all options granted to Nippon Shinyaku under the agreement to determine whether the options represent material rights. Management concluded the options to purchase commercial supply convey material rights granted to Nippon Shinyaku, and therefore are accounted for as separate performance obligations under the current arrangement. The Company identified various promises under the Nippon Shinyaku Collaboration Agreement which were determined to be immaterial in the context of the contract and will not be accounted for as separate performance obligations.

As of June 30, 2025, the transaction price of the Nippon Shinyaku Collaboration Agreement included fixed consideration of \$110.0 million for the up-front payment and variable consideration of \$6.9 million for estimated reimbursable manufacturing costs which are deemed not to be constrained. Variable consideration which has been excluded from the transaction price includes \$40.0 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement, and other reimbursable manufacturing costs which are contingent on events occurring that are outside the Company's control. The transaction price also excludes sales-based milestone payments of \$660.0 million and royalties on net sales of RGX-121 and RGX-111 in the United States and Asia territories. Development milestones will be added to the transaction price upon achievement, or if deemed probable of achievement, and other variable consideration may be added to the transaction price in the future as uncertainties regarding payment of the consideration are resolved. In accordance with the sale- or usage-based royalty exception under ASC 606, royalties on net sales and sales-based milestones will be recognized as revenue in the period the underlying sales occur or milestones

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are achieved. There were no changes in the fixed transaction price of the Nippon Shinyaku Collaboration Agreement between the effective date of the agreement and June 30, 2025.

The fixed transaction price of the Nippon Shinyaku Collaboration Agreement of \$110.0 million was allocated to the various performance obligations based on their relative standalone selling prices, which requires significant judgment. The selling prices of the intellectual property licenses were determined based on discounted cash flow models for each of the licensed products in the respective licensed territories and were adjusted for the probability of developmental, regulatory and commercial success. Significant assumptions and judgments were required to estimate the future cash flows, discount rates and probabilities of success for each of the licensed products and territories. The selling prices of development services and commercial supply were determined based on the expected cost plus a reasonable margin. The selling prices of the material rights to purchase commercial supply were determined based on the incremental discount to the standalone selling prices of the commercial supply and were adjusted to take into account the likelihood of exercise of the material rights. Significant assumptions and judgments were required to estimate the future costs of development and manufacturing, an appropriate margin for such services, and the likelihood of exercise of the material rights to purchase commercial supply based on anticipated demand and risks. The \$6.9 million of variable consideration included in the transaction price is allocated directly to performance obligations for development services related to the manufacturing of commercial supply prior to regulatory approval since the consideration is directly associated with reimbursement of such costs. Consideration contingent upon the future exercise of options to purchase commercial supply is excluded from the transaction price until exercised.

The portion of the \$110.0 million fixed transaction price allocated to the delivery of the intellectual property licenses was recognized as license and royalty revenue upon the delivery of the license to Nippon Shinyaku in March 2025. The portion of the fixed transaction price allocated to development services will be recognized as service revenue as the services are performed using an input method based on costs incurred versus total estimated costs to perform the services, which is re-assessed at each reporting date. The portion of the fixed transaction price allocated to material rights to purchase commercial supply will be recognized as revenue proportionally with the total expected commercial supply revenue expected to be recognized under the arrangement, which is re-assessed at each reporting date. Commercial supply revenue will be recognized as revenue upon delivery to Nippon Shinyaku, or otherwise upon transfer of control to Nippon Shinyaku as defined in the associated supply agreements.

The Company recognized the following amounts under the Nippon Shinyaku Collaboration Agreement (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
License and royalty revenue	\$ —	\$ —	\$ 69,979	\$ —
Service revenue	2,720	—	4,494	—
Total revenues	\$ 2,720	\$ —	\$ 74,473	\$ —

As of June 30, 2025, the Company had recorded total accounts receivable of \$2.3 million for reimbursement of manufacturing-related development costs and other services under the Nippon Shinyaku Collaboration Agreement, of which \$1.1 million was included in current assets and \$1.1 million was included in non-current assets. As of June 30, 2025, the Company had recorded total deferred revenue of \$37.8 million for development services and material rights which have not yet been satisfied under the Nippon Shinyaku Collaboration Agreement, of which \$14.0 million was included in current liabilities and \$23.8 million was included in non-current liabilities.

11. Stock-based Compensation

Effective in January 2025, an additional 1,981,975 shares were authorized for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). The 2015 Plan expired in June 2025, upon which no further awards may be issued under the plan. Any awards outstanding under the 2015 Plan as of its expiration shall remain outstanding and effective pursuant to the contractual terms of the awards. Additionally, as of June 30, 2025, no awards remained outstanding or available for issuance under the 2014 Stock Plan (the 2014 Plan), the predecessor plan to the 2015 Plan.

In May 2025, the Company adopted the 2025 Equity Incentive Plan (the 2025 Plan), which replaced the 2015 Plan upon its expiration in June 2025. The total number of shares of common stock authorized for issuance under the 2025 Plan upon its adoption was 5,500,000. The number of shares authorized for issuance under the 2025 Plan shall automatically increase for any shares of common stock underlying awards outstanding under the 2015 Plan, as of the adoption date of the 2025 Plan, which are not issued due to forfeiture, expiration, termination or cancellation of the award. Shares of common stock that are withheld, tendered, or otherwise not issued in connection with the settlement of awards outstanding under the 2015 Plan do not increase the number of shares authorized for issuance under the 2025 Plan. As of June 30, 2025, the total number of shares of common stock reserved for issuance under the 2025 Plan and the 2015 Plan was 20,311,344, of which 5,519,192 remained available for future grants under the 2025 Plan.

The 2025 Plan and 2015 Plan provide for the issuance of stock options, stock appreciation rights, restricted and unrestricted stock and unit awards, and performance cash awards to employees, members of the Board of Directors and consultants of the Company. Stock options generally expire 10 years following the date of grant. Options typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Stock options have an exercise price at least equal to the estimated fair value of the Company's common stock on the date of grant. Restricted stock units typically vest over a four-year period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Upon vesting, restricted stock units are settled in common stock of the Company. Awards granted under the 2025 Plan generally have a minimum vesting requirement of one year from the grant date.

Shares of common stock underlying awards granted under the 2025 Plan which are not issued due to forfeiture, expiration, termination or cancellation of the award are added to the number of shares of common stock available for issuance under the 2025 Plan, except for shares that are withheld, tendered or otherwise not issued in connection with the settlement of the award. Shares available for issuance under the 2025 Plan and 2015 Plan may be either authorized but unissued shares of the Company's common stock or common stock reacquired by the Company and held in treasury. The 2025 Plan expires in May 2035, 10 years from its adoption date, unless earlier terminated.

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type was as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Stock options	\$ 4,778	\$ 5,940	\$ 9,485	\$ 11,918
Restricted stock units	3,713	3,334	7,381	6,781
Employee stock purchase plan	162	161	324	338
	<u>\$ 8,653</u>	<u>\$ 9,435</u>	<u>\$ 17,190</u>	<u>\$ 19,037</u>

As of June 30, 2025, the Company had \$65.6 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.5 years.

The Company recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Research and development	\$ 3,989	\$ 4,699	\$ 7,936	\$ 9,592
General and administrative	4,664	4,736	9,254	9,445
	<u>\$ 8,653</u>	<u>\$ 9,435</u>	<u>\$ 17,190</u>	<u>\$ 19,037</u>

Stock Options

The following table summarizes stock option activity under the Company's equity incentive plans (in thousands, except per share data):

	Shares	Weighted-average Exercise Price	Weighted-average Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2024	9,994	\$ 29.48	5.9	\$ 240
Granted	2,424	\$ 8.02		
Exercised	(61)	\$ 3.76		
Cancelled or forfeited	(547)	\$ 29.87		
Outstanding at June 30, 2025	11,810	\$ 25.19	6.5	\$ 737
Exercisable at June 30, 2025	7,289	\$ 32.83	5.0	\$ —
Vested and expected to vest at June 30, 2025	11,810	\$ 25.19	6.5	\$ 737

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at the dates reported.

The weighted-average grant date fair value per share of options granted during the six months ended June 30, 2025 was \$4.96. During the six months ended June 30, 2025, the total number of stock options exercised was 60,554, resulting in total proceeds of \$0.2 million. The total intrinsic value of options exercised during the six months ended June 30, 2025 was \$0.3 million.

Restricted Stock Units

The following table summarizes restricted stock unit activity under the Company's equity incentive plans (in thousands, except per share data):

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2024	2,042	\$ 19.95
Granted	1,607	\$ 8.56
Vested	(598)	\$ 22.49
Forfeited	(69)	\$ 18.16
Unvested balance at June 30, 2025	2,982	\$ 13.35

The total intrinsic value of restricted stock units vested during the six months ended June 30, 2025 was \$4.7 million.

Employee Stock Purchase Plan

As of June 30, 2025, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 866,258 remained available for future issuance. During the six months ended June 30, 2025, 46,706 shares of common stock were issued under the 2015 ESPP.

12. Income Taxes

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of June 30, 2025 and December 31, 2024. Based on the Company's history of operating losses, and other relevant facts and circumstances, the Company concluded that it was more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company provided a full valuation allowance for its net deferred tax assets as of June 30, 2025 and December 31, 2024.

In July 2025, the One Big Beautiful Bill Act (OBBBA) was enacted into law. The OBBBA amends U.S. tax laws, including provisions related to bonus depreciation and deductions for research and development expenses. The Company is currently evaluating the impact of the OBBBA on its consolidated financial statements.

13. Net Loss Per Share

Since the Company incurred net losses for the three and six months ended June 30, 2025 and 2024, common stock equivalents were excluded from the calculation of diluted net loss per share for such periods as their effect would be anti-dilutive. Accordingly, basic and diluted net loss per share were the same for such periods. The weighted-average number of common shares outstanding used in the basic and diluted net loss per share calculations includes the weighted-average effect of pre-funded warrants to purchase shares of the Company's common stock, as the pre-funded warrants are exercisable at any time for nominal cash consideration. The following potentially dilutive common stock equivalents outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

	Three and Six Months Ended June 30,	
	2025	2024
Stock options issued and outstanding	11,810	9,638
Unvested restricted stock units outstanding	2,982	1,668
Employee stock purchase plan	111	78
Warrants outstanding	268	—
	15,171	11,384

14. Segment Information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker (CODM), or decision-making group, in making decisions on how to allocate resources and assess performance. The Company's CODM, its Chief Executive Officer, views the Company's operations and manages the business as one operating segment focused on the development and commercialization of gene therapies to treat an array of diseases. The determination of a single operating segment is consistent with the consolidated financial information regularly provided to the CODM

The CODM reviews and evaluates consolidated net income (loss) for purposes of assessing performance, making operating decisions and allocating resources. The CODM uses net income (loss) to assess performance versus operating budgets and in the preparation of near-term and long-range operating plans to inform decisions on resource and capital allocation. The CODM reviews consolidated cash, cash equivalents and marketable securities as a measure of segment assets. As of June 30, 2025 and December 31, 2024, the Company's cash, cash equivalents and marketable securities were \$363.6 million and \$244.9 million, respectively.

The following table presents information about the Company's segment revenues, significant segment expenses regularly provided to the CODM, other segment items and consolidated net income (loss) (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenues	\$ 21,359	\$ 22,295	\$ 110,371	\$ 37,917
Less:				
Cost of license and royalty revenues	5,209	10,579	8,645	14,862
Research and development expense				
Personnel	18,047	16,039	36,689	33,099
Direct development and support (a)	31,041	21,326	55,060	47,359
Facilities	2,753	3,013	5,521	5,930
Stock-based compensation	3,989	4,699	7,936	9,592
Depreciation and amortization	3,670	3,792	7,381	7,733
Total research and development expense	59,500	48,869	112,587	103,713
General and administrative expense				
Personnel	5,524	5,075	11,488	10,393
Other general and administrative (b)	8,201	7,470	16,420	14,462
Facilities	1,259	1,315	2,589	2,348
Stock-based compensation	4,664	4,736	9,254	9,445
Depreciation and amortization	235	259	479	498
Total general and administrative expense	19,883	18,855	40,230	37,146
Other segment items (c)	(7,638)	3,019	(13,697)	1,485
Net loss	\$ (70,871)	\$ (52,989)	\$ (64,788)	\$ (116,319)

- (a) Direct development and support includes external goods and services for the development of product candidates and early-stage research activities, laboratory costs, consulting, development cost reimbursement to and from collaborators and other expenses in support of research and development activities.
- (b) Other general and administrative expenses include professional and administrative services, consulting, commercial cost reimbursement to and from collaborators and other corporate overhead expenses.
- (c) Other segment items include impairment of long-lived assets, other operating expenses (income), interest income from licensing, investment income and interest expense.

The Company's interest income included interest income from licensing as presented in the consolidated statements of operations and comprehensive loss, as well as interest income from investments of \$3.4 million and \$5.9 million during the three and six months ended June 30, 2025, respectively, and \$3.5 million and \$5.9 million during the three and six months ended June 30, 2024, respectively, which is included within investment income in the consolidated statements of operations and comprehensive loss.

The substantial majority of the Company's assets reside in the United States.

15. Supplemental Disclosures

Other Current Assets

Other current assets consisted of the following (in thousands):

	June 30, 2025	December 31, 2024
Net cost reimbursement due from AbbVie	\$ 16,989	\$ 11,304
Accrued interest on investments	1,287	1,094
Other	1,203	1,376
	<u>\$ 19,479</u>	<u>\$ 13,774</u>

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	June 30, 2025	December 31, 2024
Accrued personnel costs	\$ 13,801	\$ 17,607
Accrued external research and development expenses	11,418	8,998
Accrued sublicense fees and royalties	7,548	8,658
Accrued external general and administrative expenses	2,015	2,002
Accrued purchases of property and equipment	39	156
Other	736	649
	<u>\$ 35,557</u>	<u>\$ 38,070</u>

Supplemental Disclosures of Non-cash Investing and Financing Activities

Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities were \$0.2 million as of June 30, 2025, a net decrease of \$0.1 million from December 31, 2024, and \$0.1 million as of June 30, 2024, a net decrease of \$0.3 million from December 31, 2023.

During the six months ended June 30, 2025, accrued interest of \$2.7 million was converted to principal balance under the 2025 Royalty Bond.

Issuance costs related to equity offerings and warrants included in accounts payable and accrued expenses and other current liabilities were \$0.1 million and \$0.3 million as of June 30, 2025 and 2024, respectively.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2024, which we filed with the SEC on March 13, 2025. In addition, you should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2024 for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our investigational gene therapies are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, gene therapy could potentially alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects.

Overview of Product Candidates

We have developed a broad pipeline of gene therapy programs using our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) as a one-time treatment to address an array of diseases. Our lead programs and product candidates are described below:

- **ABBV-RGX-314:** We are developing ABBV-RGX-314 (surabgene lomparvovec, sura-vec) in collaboration with AbbVie as a potential one-time treatment for chronic retinal conditions that cause total or partial vision loss, including wet age-related macular degeneration (wet AMD) and diabetic retinopathy (DR). ABBV-RGX-314 is currently being evaluated in multiple clinical trials, including two pivotal trials (ATMOSPHERE and ASCENT), one Phase II bridging study, one long-term follow-up study and a fellow eye sub-study in patients with wet AMD, all utilizing subretinal delivery. Additionally, two Phase II clinical trials in patients with wet AMD (AAVIATE) and DR (ALTITUDE) are ongoing along with two corresponding long-term follow-up studies, all utilizing in-office suprachoroidal delivery. Within the Phase II study in DR, we are also evaluating ABBV-RGX-314 in diabetic macular edema (DME). ABBV-RGX-314 uses the NAV[®] AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF). We have licensed certain exclusive rights to the SCS Microinjector[®] from Clearside Biomedical, Inc. (Clearside) to deliver gene therapy treatments to the suprachoroidal space of the eye.

Wet AMD

Subretinal Delivery

Enrollment continues to be on track in the ATMOSPHERE[®] and ASCENT[™] pivotal trials for the treatment of patients with wet AMD using subretinal delivery. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Topline data from these trials are expected to be shared in 2026.

Suprachoroidal Delivery

The AAVIATE[®] trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 for the treatment of wet AMD. As of July 29, 2024, ABBV-RGX-314 at dose level 3 with short course prophylactic steroid eye drops continues to be well tolerated with no drug-related serious adverse events (SAEs) and no cases of intraocular inflammation, endophthalmitis, vasculitis, retinal artery occlusion, choroidal effusion or hypotony. Mild episcleritis occurred in three patients, all resolved and completed treatment with topical steroids. There were no cases of elevated intraocular pressure. Based on this favorable safety profile, the Phase II AAVIATE trial continues to enroll a new cohort to evaluate ABBV-RGX-314 at dose level 4 (1.5x10¹² GC/eye). Patients in this cohort will also receive short course prophylactic steroid eye drops.

DR and DME

The ALTITUDE[®] trial is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of ABBV-RGX-314 using suprachoroidal delivery for the treatment of DR. In November 2023, we announced data showing ABBV-RGX-314 was well tolerated at dose levels 1 and 2 and positive signals of efficacy, including 20.8% of patients exhibiting >2-step Diabetic Retinopathy Severity Scale (DRSS) improvement without additional DR treatment at one year.

In August 2025, we announced new data from the ALTITUDE trial and plans to initiate a pivotal program. New ALTITUDE trial data demonstrate a durable safety and efficacy profile observed in patients with non-proliferative DR through two years with a single, in-office injection. As of June 9, 2025, ABBV-RGX-314 was well tolerated at dose levels 1, 2 and 3, with no drug-related serious adverse events. No intraocular inflammation was observed through two years at dose level 3 (1.0x10¹² GC/eye) (n=15) with short-course topical prophylactic steroids. In August 2025, we and AbbVie executed an amendment to our collaboration agreement and announced plans to initiate a pivotal two-part placebo-controlled Phase IIb/III trial, with the primary endpoint being ≥2-step DRSS improvement at one year. Site selection for the Phase IIb/III trial is in progress.

The ALTITUDE trial includes a new cohort of patients with center-involved DME evaluating ABBV-RGX-314 at dose level 4. Enrollment completed in this cohort in June 2025. DME is a vision-threatening complication of DR; an estimated 34 million people globally have DME. Patients will receive a one-time, in-office injection of ABBV-RGX-314 at dose level 4 (1.5x10¹² GC/eye) with short course prophylactic steroid eye drops.

- **RGX-202:** We are developing RGX-202 as an investigational AAV therapeutic for the treatment of Duchenne muscular dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

AFFINITY DUCHENNE[®] is a multicenter, open-label Phase I/II/III trial to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous dose of RGX-202 in patients with Duchenne aged one and older. The initiation of the pivotal study, which is expected to enroll approximately 30 patients in the U.S. and Canada, as well as positive interim safety and efficacy data from the Phase I/II portion of the study were announced in November 2024. These data included positive biomarker data from the first nine patients, which demonstrated consistent, robust microdystrophin and transduction, as well as positive initial functional data. Subsequent findings were presented in March 2025 at the 2025 Muscular Dystrophy Association Clinical & Scientific Conference and in June 2025 via a Company webcast. In sum, these data were positive and demonstrate potential for RGX-202 to serve as a differentiated gene therapy for Duchenne. As of May 2025, we had reported positive microdystrophin data on 12 patients and positive initial functional data from five patients. We also reported a favorable safety profile with no serious adverse events or adverse events of special interest observed (n=13).

As of May 2025, the pivotal study was beyond 50% enrolled. In August 2025, we announced expectations to complete enrollment by October 2025, earlier than previous guidance of year-end 2025. Upon completion of enrollment in the pivotal trial, we expect to continue enrollment to support a planned confirmatory trial. We expect to share topline data in the first half of 2026 and submit a Biologics License Application (BLA) under the accelerated approval pathway in mid-2026.

We are also recruiting patients in the AFFINITY BEYOND[®] trial, an observational screening study. The primary objective is to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

- **RGX-121:** We are developing RGX-121 (clemidsogene lanparvovec) in collaboration with Nippon Shinyaku in the United States and certain countries in Asia as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome, using the NAV AAV9 vector to deliver the gene that encodes the iduronate-2-sulfatase enzyme.

In February 2024, we announced that, in the pivotal phase of the Phase I/II/III CAMPSIITE[®] trial, RGX-121 achieved its primary endpoint, a reduction in cerebrospinal fluid Heparan sulfate levels of D2S6, a biomarker indicative of brain disease activity, with statistical significance. In September 2024, we announced positive data from the pivotal dose level of RGX-121 demonstrating long-term systemic effect. We plan to use levels of cerebrospinal fluid Heparan sulfate D2S6 as a surrogate endpoint reasonably likely to predict clinical benefit for accelerated approval. A BLA for RGX-121 seeking accelerated approval was submitted to the FDA in March 2025. In May 2025, the FDA granted priority review of the BLA and a Prescription Drug User Fee Act (PDUFA) target action date of November 9, 2025 was assigned. A mid-cycle

meeting and pre-license and Bioresearch Monitoring inspections were successfully completed in July and August 2025, respectively.

Potential approval of the BLA for RGX-121 could result in receipt of a Rare Pediatric Disease Priority Review Voucher in 2025, assuming the statutory criteria are met. If approved, RGX-121 would be the first approved gene therapy and one-time treatment for MPS II.

- **RGX-111:** We are developing RGX-111 in collaboration with Nippon Shinyaku in the United States and certain countries in Asia as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type I (MPS I), also known as Hurler syndrome, using the NAV AAV9 vector to deliver the IDUA gene.

In November 2023, future development of RGX-111 was halted as a result of a strategic pipeline prioritization and corporate restructuring. Prior to that announcement, RGX-111 demonstrated to be well tolerated and indicated encouraging biomarker and neurodevelopmental results in a Phase I/II study. Efforts to continue development of RGX-111 as part of the strategic partnership with Nippon Shinyaku are ongoing.

AbbVie Collaboration for ABBV-RGX-314

In September 2021, we entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314 (the AbbVie Collaboration Agreement). Pursuant to the AbbVie Collaboration Agreement, both we and AbbVie are active participants in the development of ABBV-RGX-314 and development expenses are shared between the parties in accordance with the agreement. The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the global commercialization of ABBV-RGX-314. We received an up-front fee of \$370.0 million from AbbVie upon the effective date of the AbbVie Collaboration Agreement in November 2021, and we are eligible to receive up to \$1.38 billion from AbbVie upon the achievement of specified development and sales-based milestones. Additionally, the parties will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States, and we are eligible to receive tiered royalties on net sales by AbbVie of ABBV-RGX-314 outside the United States. For additional information regarding the AbbVie Collaboration Agreement, please refer to Note 10, “License and Collaboration Agreements—AbbVie Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

In August 2025, we and AbbVie entered into an amendment to the AbbVie Collaboration Agreement which modifies the development plan and milestone payment structure for the ABBV-RGX-314 DR program. Under the amendment, we will conduct the first registration enabling trial for DR suprachoroidal (SCS) treatment as a combined Phase IIb/III trial performed in two parts (Part 1 and Part 2), and AbbVie will conduct the second registration enabling trial as a separate, standalone Phase III trial. In lieu of the \$200.0 million milestone due under the original AbbVie Collaboration Agreement upon first patient dosed in the first registration enabling trial for DR SCS treatment, AbbVie will pay us \$100.0 million upon first patient dosed in the Phase IIb/III trial for DR SCS treatment and an additional \$100.0 million upon first patient dosed in the subsequent Phase III trial. Also pursuant to the amendment, AbbVie will lead a new Phase IIIb randomized controlled study (the ACHIEVE Study) to assess the injection burden, adverse events, change in disease activity, and long-term preservation of visual acuity of ABBV-RGX-314 in adult participants with neovascular AMD. We will be responsible for our development expenses to conduct Part 1 of the Phase IIb/III trial for DR and the parties will share the development expenses related to Part 2 of the Phase IIb/III trial and the subsequent Phase III trial for DR in accordance with the existing terms of the AbbVie Collaboration Agreement. AbbVie will be responsible for all development expenses related to the ACHIEVE Study.

Nippon Shinyaku Collaboration for RGX-121 and RGX-111

In January 2025, we entered into a collaboration and license agreement with Nippon Shinyaku Co., Ltd. (Nippon Shinyaku) for the development and commercialization of RGX-121 and RGX-111 (the Nippon Shinyaku Collaboration Agreement). Pursuant to the Nippon Shinyaku Collaboration Agreement, we are responsible for the development of RGX-121 and RGX-111 in the United States, and Nippon Shinyaku is responsible for development in licensed territories outside the United States. We are responsible for the manufacturing of RGX-121 and RGX-111 for clinical development and commercial supply, and manufacturing expenses will be allocated between the parties in accordance with the terms of the Nippon Shinyaku Collaboration Agreement. Nippon Shinyaku is responsible, at its sole cost, for the commercialization of RGX-121 and RGX-111 in the licensed territories. Under the terms of the Nippon Shinyaku Collaboration Agreement, we received an up-front payment of \$110.0 million from Nippon Shinyaku following the effective date of the agreement in March 2025 and are eligible to receive up to \$700.0 million from Nippon Shinyaku upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions. We retain all rights to, and any proceeds related to the sale of, any priority review vouchers that may be issued upon the potential approvals of RGX-121 and RGX-111.

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We recognized \$74.5 million of revenue under the Nippon Shinyaku Collaboration Agreement in the first half of 2025. For additional information regarding the agreement with Nippon Shinyaku, please refer to Note 10, “License and Collaboration Agreements—Nippon Shinyaku Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

In May 2025, we entered into a loan agreement with entities managed by Healthcare Royalty Management, LLC (collectively and with other affiliated entities, HCR). Pursuant to the terms of the loan agreement, future royalties, sales-based milestone payments and certain development milestone payments earned under the Nippon Shinyaku Collaboration Agreement, along with consideration earned under various other NAV Technology Platform license agreements, shall be used to repay principal and interest owed to HCR. For additional information regarding the May 2025 loan agreement with HCR, please refer to Note 7, “Royalty Monetization Liabilities—2025 Royalty Bond” to the accompanying unaudited consolidated financial statements.

NAV Technology Licensing Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform and other intellectual property rights to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of June 30, 2025, our NAV Technology Platform was being applied in one commercial product, Zolgensma[®], and the preclinical and clinical development of a number of other licensed products. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform and creating additional revenue opportunities.

Financial Overview

Revenues

Our revenues to date have consisted primarily of revenue generated from the licensing of our NAV Technology Platform and other intellectual property rights to NAV Technology Licensees and collaborators. We have not generated any revenues from commercial sales of our own products. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval and adequate labeling, our ability to generate future revenues will be materially compromised.

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of our product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration to the Company under our license and collaboration agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

Future revenues under our license and collaboration arrangements are dependent on the successful development and commercialization of licensed products, which is uncertain, and revenues may fluctuate significantly from period to period. Additionally, we may never receive consideration under our license or collaboration agreements that is contemplated on optional goods and services, development and sales-based milestones, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments. Our revenues are concentrated among a low number of licensees and collaborators and the arrangements are terminable at the option of the counterparty. The termination of our license and collaborations arrangements may materially impact the amount of revenue we recognize in future periods.

Zolgensma Royalties

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is marketed by Novartis Gene Therapies, Inc. (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the treatment of spinal muscular atrophy (SMA). Zolgensma is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA using the NAV Technology Platform.

Operating Expenses

Our operating expenses consist primarily of cost of license and royalty revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, wages, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee or using other reasonable allocation methodologies.

Cost of License and Royalty Revenues

Our cost of license and royalty revenues consists primarily of upstream fees due to our licensors as a result of revenue generated from the licensing of our NAV Technology Platform and other intellectual property rights, including sublicense fees and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from licensees and are recognized in the period that the underlying license revenue is recognized. Royalties are based on a percentage of net sales of licensed products by licensees and are recognized in the period that the underlying sales occur. Future costs of revenues are uncertain due to the nature of our license agreements and significant fluctuations in cost of license and royalty revenues may occur from period to period.

Research and Development Expense

Our research and development expenses consist primarily of:

- salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for our scientific personnel and others performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- fees paid to consultants and other third-parties who support our product candidate development;
- other costs in seeking regulatory approval of our product candidates; and
- direct costs and allocated costs related to laboratories and facilities, depreciation expense, information technology and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities, as well as associated milestone payments, are charged to research and development expense as incurred if the technology licensed has no alternative future use.

We expect to continue to incur significant research and development expenses for the foreseeable future as we continue the development of our product candidates and engage in early research and development for prospective product candidates and new technologies. The following table summarizes our research and development expenses incurred during the three and six months ended June 30, 2025 and 2024 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024*	2025	2024*
Direct Expenses				
ABBV-RGX-314	\$ 12,691	\$ 9,483	\$ 21,254	\$ 18,627
RGX-202	4,929	2,295	9,437	5,329
RGX-121	2,863	3,505	6,337	7,835
Other product candidates	2,508	1,081	3,299	3,434
Total direct expenses	22,991	16,364	40,327	35,225
Unallocated Expenses				
Platform and early research	8,050	4,962	14,733	12,134
Personnel	18,047	16,039	36,689	33,099
Facilities	2,753	3,013	5,521	5,930
Stock-based compensation	3,989	4,699	7,936	9,592
Depreciation and amortization	3,670	3,792	7,381	7,733
Total unallocated expenses	36,509	32,505	72,260	68,488
Total research and development	\$ 59,500	\$ 48,869	\$ 112,587	\$ 103,713

* Certain amounts reported in prior years have been reclassified to conform to the current year's presentation.

Direct expenses related to the development of ABBV-RGX-314 include \$17.1 million and \$31.7 million for the three and six months ended June 30, 2025, respectively, and \$21.3 million and \$46.2 million for the three and six months ended June 30, 2024, respectively, in net cost reimbursement from AbbVie under our eye care collaboration, which were recorded as a reduction of research and development expenses. In addition to reimbursement of direct development expenses, net cost reimbursement from AbbVie includes reimbursement of personnel and overhead costs attributable to the development of ABBV-RGX-314, the underlying costs of which are reported as unallocated expenses in the table above. We typically utilize our employee and infrastructure resources across our development programs. As a result, we generally do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

Platform and early research reported in the table above includes direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform used across programs, process and analytical development, early research and development for prospective product candidates and new technologies, and other costs in support of research and development activities.

General and Administrative Expense

Our general and administrative expenses consist primarily of salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for employees performing functions other than research and development. This includes certain personnel in executive, commercial, corporate development, finance, legal, human resources, information technology, facilities and administrative support functions. Additionally, general and administrative expenses include costs associated with accounting, legal, commercial and other corporate advisory services, obtaining and maintaining patents, insurance, information systems and other general corporate activities, as well as facility-related costs and other corporate overhead costs not otherwise allocated to research and development expense. We expect that our general and administrative expenses will increase as we continue to develop, and potentially commercialize, our product candidates. Specifically, we expect general and administrative costs associated with the potential commercialization of our product candidates to increase in future periods as we and/or our commercial partners prepare for and carry out product launch efforts, in particular for the potential commercialization of our RGX-202 and ABBV-RGX-314 product candidates.

Other Income (Expense)

Interest Income from Licensing

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees.

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents, marketable securities and non-marketable equity securities. Cash equivalents are comprised of money market mutual funds and highly liquid debt securities with original maturities of 90 days or less at acquisition. Marketable securities are comprised of available-for-sale debt securities.

Interest Expense

Interest expense is primarily associated with our royalty monetization liabilities, including our December 2020 Zolgensma royalty purchase agreement (2020 Royalty Purchase Agreement) and May 2025 royalty bond (2025 Royalty Bond) with HCR. For further information regarding our royalty monetization liabilities and associated interest expense, please refer to Note 7, "Royalty Monetization Liabilities" to the accompanying unaudited consolidated financial statements.

Critical Accounting Policies and Estimates

This Management's Discussion and Analysis of Financial Condition and Results of Operations is based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

Our significant accounting policies are fully described in Note 2 to the accompanying unaudited consolidated financial statements and in Note 2 to our audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2024. Other than the accounting policies described below, there have been no significant changes in our critical accounting policies and estimates since December 31, 2024.

Revenue Recognition

We recognize revenue in accordance with Accounting Standards Codification (ASC) 606, *Revenue from Contracts with Customers* (ASC 606). ASC 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of ASC 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of ASC 606 only when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, for contracts within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an interest rate which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of our product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the

option of the licensee. Consideration payable to us under our license and collaboration agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products, (v) fees for services related to the development and manufacturing of licensed products and (vi) other consideration payable upon optional goods and services purchased by licensees and collaborators.

We evaluate our agreements with collaboration partners to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (ASC 808). For collaboration arrangements within the scope of ASC 808 that contain multiple elements, we identify the various transactions with the counterparty and determine if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of ASC 606. For transactions that are accounted for pursuant to ASC 808, an appropriate method of recognition and presentation is determined and consistently applied. For transactions that are accounted for pursuant to ASC 606, we apply the five-step model as described in our revenue recognition policies.

Our license and collaboration agreements are accounted for as contracts with customers within the scope of ASC 606, with the exception of transactions for which the counterparty is determined not to be a customer. At the inception of each agreement, we determine the contract term for purposes of applying the requirements of ASC 606. Licenses are generally terminable at the option of the licensee with advance notice to us. For each license granted, we evaluate these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options.

Performance obligations under our license and collaboration agreements may include (i) the delivery of intellectual property licenses, (ii) development and manufacturing services to be performed by us related to licensed products and (iii) options granted to purchase additional goods and services, to the extent the options convey material rights. At the inception of each license agreement which contains performance obligations for development or other services, we evaluate whether the license is distinct from the services, which requires judgment. In making this determination, we consider, among other things, the stage of development of the licensed products and whether the services will significantly impact further development of the licensed products. If it is determined that the license is not distinct from the services, the license is combined with the services into a single performance obligation. Agreements may provide licensees and collaborators with options to purchase additional goods or other services, including options to purchase commercial supply of licensed products. Options are evaluated at the inception of the agreement to determine whether they provide material rights to the customer. In making this determination, we consider whether the options are priced at an incremental discount to the standalone selling price of the underlying goods or services, in which case the option is considered to be a material right. Material rights are accounted for as separate performance obligations under the current arrangement.

We evaluate the transaction price of our license and collaboration agreements at contract inception and at each reporting date. The transaction price includes the fixed consideration payable to us over the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the agreements may include up-front and annual fees payable to us over the contract term and fixed fees for development and other services. Variable consideration under the agreements may include development and sales-based milestone payments, payments for development and other services, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by the customer is excluded from the transaction price and not accounted for as part of the arrangement until the option is exercised.

The transaction price of our license and collaboration arrangements is allocated to the underlying performance obligations based on their relative standalone selling prices and recognized as revenue when (or as) the performance obligations are satisfied. Variable consideration payable based on services performed is allocated directly to the performance obligation for such services. Consideration allocated to performance obligations for the delivery of intellectual property licenses is recognized as license and royalty revenue in full upon the delivery of the license. Consideration allocated to performance obligations for development and manufacturing services is recognized as service revenue as we perform the services. Consideration allocated to performance obligations for material rights to purchase additional goods and services is recognized as revenue upon the satisfaction of the performance obligations underlying the optional goods and services purchased by the customer. Service revenue is recognized using a measure of progress that best reflects the pattern of satisfaction of the performance obligations. At each reporting date, we re-evaluate the measure of progress and adjust service revenue on a cumulative catch-up basis to reflect our best estimate of the services performed to date versus the total services to be performed under the arrangement.

Development milestone payments are payable to us upon the achievement of specified development milestones. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that

a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price. Milestone payments contingent on the achievement of development milestones that are not within our control or the control of the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of each outstanding development milestone and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. The increase to the transaction price as a result of any such adjustments is then allocated to the underlying performance obligations in a manner similar to the allocation of the initial transaction price and, to the extent the performance obligations are satisfied, recognized as revenue on a cumulative catch-up basis in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments, including milestones payable upon first commercial sales of licensed products, and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as license and royalty revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA. We recognize royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require us to estimate royalty revenue for periods of net sales which have not yet been reported to us. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

We receive payments from licensees and collaborators based on the billing schedules established in the associated agreements. Amounts recognized as revenue which have not yet been received from the customer are recorded as accounts receivable when our rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from customers are recorded as contract assets when our rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets if the consideration is expected to be realized within 12 months from the reporting date, or as other assets if the consideration is expected to be realized in periods beyond 12 months from the reporting date. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of revenue in the period of the termination. Amounts received by us prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Results of Operations

Our consolidated results of operations were as follows (in thousands):

	Three Months Ended June 30,		Change	Six Months Ended June 30,		Change
	2025	2024		2025	2024	
Revenues						
License and royalty revenue	\$ 18,465	\$ 21,846	\$ (3,381)	\$ 105,514	\$ 37,190	\$ 68,324
Service revenue	2,894	449	2,445	4,857	727	4,130
Total revenues	21,359	22,295	(936)	110,371	37,917	72,454
Operating Expenses						
Cost of license and royalty revenues	5,209	10,579	(5,370)	8,645	14,862	(6,217)
Research and development	59,500	48,869	10,631	112,587	103,713	8,874
General and administrative	19,883	18,855	1,028	40,230	37,146	3,084
Impairment of long-lived assets	—	—	—	—	2,101	(2,101)
Other operating expenses (income)	45	29	16	60	(5)	65
Total operating expenses	84,637	78,332	6,305	161,522	157,817	3,705
Loss from operations	(63,278)	(56,037)	(7,241)	(51,151)	(119,900)	68,749
Other Income (Expense)						
Interest income from licensing	21	29	(8)	46	66	(20)
Investment income	3,379	3,468	(89)	5,880	5,937	(57)
Interest expense	(10,993)	(449)	(10,544)	(19,563)	(2,422)	(17,141)
Total other income (expense)	(7,593)	3,048	(10,641)	(13,637)	3,581	(17,218)
Net loss	\$ (70,871)	\$ (52,989)	\$ (17,882)	\$ (64,788)	\$ (116,319)	\$ 51,531

Comparison of the Three Months Ended June 30, 2025 and 2024

License and Royalty Revenue. License and royalty revenue decreased by \$3.4 million, from \$21.8 million for the three months ended June 30, 2024 to \$18.5 million for the three months ended June 30, 2025. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased by \$3.3 million, from \$21.8 million for the second quarter of 2024 to \$18.4 million for the second quarter of 2025. Novartis reported Zolgensma sales of \$297 million for the second quarter of 2025, a decrease of 15% from the second quarter of 2024, driven by lower incidence of SMA during the period.

Service Revenue. Service revenue increased by \$2.4 million, from \$0.4 million for the three months ended June 30, 2024 to \$2.9 million for the three months ended June 30, 2025. The increase was primarily attributable to \$2.7 million of development service revenue recognized under our collaboration with Nippon Shinyaku in the second quarter of 2025.

Research and Development Expense. Research and development expenses increased by \$10.6 million, from \$48.9 million for the three months ended June 30, 2024 to \$59.5 million for the three months ended June 30, 2025. The increase was primarily attributable to the following:

- an increase of \$5.7 million in manufacturing-related expenses and other clinical supply costs for our lead product candidates, largely driven by ABBV-RGX-314 clinical supply and purchases of raw materials;
- an increase of \$3.2 million in costs associated with clinical trials and regulatory activities, largely driven by clinical trial expenses for ABBV-RGX-314 and RGX-202 pivotal trials; and
- an increase of \$1.4 million in personnel-related costs as a result of increased headcount of development personnel, net of a \$0.7 million decrease in stock-based compensation expense.

General and Administrative Expense. General and administrative expenses increased by \$1.0 million, from \$18.9 million for the three months ended June 30, 2024 to \$19.9 million for the three months ended June 30, 2025. The increase was primarily attributable to personnel-related costs for general and administrative personnel and professional services and consulting fees, including legal and other corporate advisory services.

Interest Expense. Interest expense increased by \$10.5 million, from \$0.4 million for the three months ended June 30, 2024 to \$11.0 million for the three months ended June 30, 2025. The increase was primarily attributable to interest expense under our royalty monetization liabilities, driven largely by an increase in forecasted Zolgensma royalties expected to be paid to HCR under the 2020 Royalty Purchase Agreement and interest expense incurred to date under the 2025 Royalty Bond issued in May 2025.

Comparison of the Six Months Ended June 30, 2025 and 2024

License and Royalty Revenue. License and royalty revenue increased by \$68.3 million, from \$37.2 million for the six months ended June 30, 2024 to \$105.5 million for the six months ended June 30, 2025. The increase was primarily attributable to \$70.0 million of upfront license revenue recognized under our collaboration with Nippon Shinyaku in the first quarter of 2025.

Service Revenue. Service revenue increased by \$4.1 million, from \$0.7 million for the six months ended June 30, 2024 to \$4.9 million for the six months ended June 30, 2025. The increase was primarily attributable to \$4.5 million of development service revenue recognized under our collaboration with Nippon Shinyaku in the first half of 2025.

Research and Development Expense. Research and development expenses increased by \$8.9 million, from \$103.7 million for the six months ended June 30, 2024 to \$112.6 million for the six months ended June 30, 2025. The increase was primarily attributable to the following:

- an increase of \$8.9 million in manufacturing-related expenses and other clinical supply costs for our lead product candidates, largely driven by ABBV-RGX-314 clinical supply and purchases of raw materials; and
- an increase of \$2.0 million in personnel-related costs as a result of increased headcount of development personnel, net of a \$1.7 million decrease in stock-based compensation expense.

The increase in research and development expenses was partially offset by a decrease of \$2.7 million in overall costs for clinical trials, preclinical activities and other early-stage development.

General and Administrative Expense. General and administrative expenses increased by \$3.1 million, from \$37.1 million for the six months ended June 30, 2024 to \$40.2 million for the six months ended June 30, 2025. The increase was primarily attributable to personnel-related costs for general and administrative personnel and professional services and consulting fees, including legal and other corporate advisory services.

Interest Expense. Interest expense increased by \$17.1 million, from \$2.4 million for the six months ended June 30, 2024 to \$19.6 million for the six months ended June 30, 2025. The increase was primarily attributable to interest expense under our royalty monetization liabilities, driven largely by an increase in forecasted Zolgensma royalties expected to be paid to HCR under the 2020 Royalty Purchase Agreement and interest expense incurred to date under the 2025 Royalty Bond issued in May 2025.

Liquidity and Capital Resources

Sources of Liquidity

As of June 30, 2025, we had cash, cash equivalents and marketable securities of \$363.6 million, which were primarily derived from the royalty monetization in May 2025 and the up-front payment received under the Nippon Shinyaku Collaboration Agreement in March 2025, each as described below, and the sale of our common stock and pre-funded warrants in March 2024. We expect that our cash, cash equivalents and marketable securities as of June 30, 2025 will enable us to fund our operating expenses and capital expenditure requirements, and are sufficient to meet our financial commitments and obligations, for at least the next 12 months from the date of this report based on our current business plan.

In May 2025, we entered into a loan agreement with HCR pursuant to which HCR will provide us with an aggregate limited recourse loan of up to \$250.0 million (the 2025 Royalty Bond). The 2025 Royalty Bond is disbursable to us in three tranches, with \$150.0 million funded on the closing date in May 2025, \$50.0 million available to be funded if sales of a specified product exceed a specified sales threshold prior to December 31, 2026, and \$50.0 million available to be funded if both parties exercise an option in 2027. Proceeds received from the initial funding tranche of the 2025 Royalty Bond in May 2025, net of discounts and transaction costs, were \$144.5 million. The 2025 Royalty Bond matures in 2035, subject to potential extension, and bears interest at a rate of 9.75% plus the 3-month secured overnight financing rate as administered by the Federal Reserve Bank of New York (SOFR), with a minimum interest rate of 14.0%. Prior to the maturity date, interest and principal under the 2025 Royalty Bond shall be paid quarterly to HCR solely from proceeds received, net of upstream obligations to licensors, from certain specified royalties, milestone payments, license fees and other consideration payable to us under the Zolgensma license with Novartis Gene Therapies, the Nippon Shinyaku Collaboration Agreement and certain other NAV Technology Platform license agreements.

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In January 2025, we entered into the Nippon Shinyaku Collaboration Agreement for the development and commercialization of RGX-121 and RGX-111 in the United States and certain countries in Asia. Pursuant to the Nippon Shinyaku Collaboration Agreement, we received an up-front payment of \$110.0 million following the effective date of the agreement in March 2025 and are eligible to receive up to \$700.0 million upon the achievement of specified development and sales-based milestones. We are also eligible to receive double-digit royalties on net sales of RGX-121 and RGX-111 by Nippon Shinyaku, subject to specified offsets and reductions.

We intend to devote the majority of our current capital to preclinical research, clinical development, seeking regulatory approval of our product candidates and, if approved, commercialization of our product candidates, as well as additional capital expenditures needed to support these activities. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the total amount of operating expenditures and capital outlays necessary to complete the development of our product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect which could accelerate our liquidity needs.

At-the-Market Offering Program

In December 2024, we entered into a Sales Agreement with Leerink Partners LLC (Leerink) pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to \$150.0 million from time to time through Leerink, acting as our sales agent (the Leerink ATM Program). As of June 30, 2025, no shares of common stock had been sold under the Leerink ATM Program. We intend to use proceeds obtained from the sale of shares under the Leerink ATM Program, if any, for general corporate purposes.

Cash Flows

Our consolidated cash flows were as follows (in thousands):

	Six Months Ended June 30,	
	2025	2024
Net cash used in operating activities	\$ (15,713)	\$ (100,952)
Net cash provided by (used in) investing activities	(95,693)	12,915
Net cash provided by financing activities	133,438	111,280
Net increase in cash and cash equivalents and restricted cash	\$ 22,032	\$ 23,243

Cash Flows from Operating Activities

Our net cash used in operating activities for the six months ended June 30, 2025 decreased by \$85.2 million from the six months ended June 30, 2024, largely as a result of the \$110.0 million up-front fee received from Nippon Shinyaku in March 2025. We expect to continue to incur regular net cash outflows from operations for the foreseeable future as we continue the development and advancement of our product candidates and other research programs.

For the six months ended June 30, 2025, our net cash used in operating activities of \$15.7 million consisted of a net loss of \$64.8 million, offset by favorable changes in operating assets and liabilities of \$24.0 million and adjustments for non-cash items of \$25.1 million. The changes in operating assets and liabilities include an increase in deferred revenue of \$37.7 million, which was driven primarily by the deferred portion of the \$110.0 million up-front payment received under our collaboration with Nippon Shinyaku in the first quarter of 2025. The favorable changes in operating assets and liabilities were partially offset by an increase in prepaid expenses and other current assets of \$7.3 million, which was driven primarily by an increase in net cost reimbursement due from AbbVie under our ABBV-RGX-314 collaboration and increases in prepaid clinical trial services and software licenses. Other changes in operating working capital occurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expense of \$17.2 million and depreciation and amortization expense of \$7.9 million.

For the six months ended June 30, 2024, our net cash used in operating activities of \$101.0 million consisted of a net loss of \$116.3 million and unfavorable changes in operating assets and liabilities of \$12.2 million, offset by adjustments for non-cash items of \$27.6 million. The changes in operating assets and liabilities include a decrease in total accounts payable and accrued expenses and other current liabilities of \$14.0 million, which was driven largely by decreases in accrued sublicense fees, royalties and personnel-related expenses. Other changes in operating working capital occurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expense of \$19.0 million and depreciation and amortization expense of \$8.2 million.

Cash Flows from Investing Activities

For the six months ended June 30, 2025, our net cash used in investing activities consisted of \$230.3 million used to purchase marketable debt securities and \$1.4 million used to purchase property and equipment, offset by \$136.0 million in maturities of marketable debt securities.

For the six months ended June 30, 2024, our net cash provided by investing activities consisted of \$151.7 million in maturities of marketable debt securities, offset by \$137.7 million used to purchase marketable debt securities and \$1.0 million used to purchase property and equipment.

Cash Flows from Financing Activities

For the six months ended June 30, 2025, our net cash provided by financing activities primarily consisted of \$144.5 million in proceeds received from the issuance of the 2025 Royalty Bond and warrants to HCR in May 2025, net of discounts and transaction costs paid during the period, and was partially offset by \$10.9 million of royalties paid, net of interest, under our royalty monetization liabilities.

For the six months ended June 30, 2024, our net cash provided by financing activities primarily consisted of \$131.4 million in proceeds received from the public offering of common stock and pre-funded warrants completed in March 2024, net of underwriting discounts and commissions and other offering expenses paid during the period, and \$1.5 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan. Our net cash provided by financing activities was partially offset by \$20.5 million of royalties paid, net of interest, under our royalty monetization liabilities.

Additional Capital Requirements

Our material capital requirements from known contractual and other obligations primarily relate to our vendor service contracts and purchase commitments, in-license agreements, operating lease agreements and royalty monetization liabilities. Our material commitments and obligations are further described in Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" of our Annual Report on Form 10-K for the year ended December 31, 2024, and in the notes to the audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2024. Other than the changes described in the notes to the unaudited consolidated financial statements accompanying this Quarterly Report on Form 10-Q, including Note 7, "Royalty Monetization Liabilities," and Note 8, "Commitments and Contingencies," there have been no material changes to our commitments and obligations since December 31, 2024.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of \$996.9 million as of June 30, 2025. Our transition to recurring profitability is dependent upon achieving a level of revenues adequate to support our cost structure, which depends heavily on the successful development, approval and commercialization of our product candidates. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect to continue to incur significant research and development and general and administrative expenses for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect to continue to incur capital expenditures associated with building out additional laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity;

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- the costs, timing and outcome of regulatory review of our product candidates;
- the impact of any government-imposed tariffs or other trade barriers on cost of goods and services, particularly related to partnered product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;
- revenue received from commercial sales of Zolgensma;
- revenue received from other commercial sales of our licensees' and collaborators' products, should any of the product candidates receive marketing approval, and other revenues received under our licensing agreements and collaborations;
- the timing and amount of Zolgensma royalties and other specified royalties, milestones and consideration under our license agreements and collaborations paid to HCR under our royalty monetization agreements;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration Agreement relating to ABBV-RGX-314 and the Nippon Shinyaku Collaboration Agreement relating to RGX-121 and RGX-111, and our ability to timely achieve any milestones set forth in such agreements or collaborations;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

The issuance of additional securities, whether equity or debt, by us, including through our at-the-market program, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or others that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Off-Balance Sheet Arrangements

We did not have any off-balance sheet arrangements during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

For information regarding market risk, refer to Item 7A, "Quantitative and Qualitative Disclosures About Market Risk," included in our Annual Report on Form 10-K for the year ended December 31, 2024. There have been no material changes to our exposure to market risk during the six months ended June 30, 2025.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2025. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of June 30, 2025, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended June 30, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

Item 1A. Risk Factors.

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2024. There have been no material changes from the risk factors previously disclosed in such filing.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

Rule 10b5-1 Trading Plans

During the three months ended June 30, 2025, none of our directors or Section 16 reporting officers adopted or terminated any Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement (as such terms are defined in Item 408 of the SEC's Regulation S-K).

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Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	6/7/21	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
4.1	REGENXBIO Inc. Warrant to Purchase Common Stock	S-3	4.5	6/13/25	
10.1*	REGENXBIO Inc. 2025 Equity Incentive Plan	S-8	99.1	6/13/25	
10.2*	Form of Restricted Stock Unit Award Agreement for the 2025 Equity Incentive Plan				X
10.3*	Form of Stock Option Award Agreement for the 2025 Equity Incentive Plan				X
10.4†	Loan Agreement dated May 16, 2025 between REGENXBIO RS LLC and affiliate of HealthCare Royalty Management, LLC				X
31.1	Certification of the Chief Executive Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2025 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Loss (iii) Consolidated Statements of Stockholders' Equity (iv) Consolidated Statements of Cash Flows (v) Notes to Consolidated Financial Statements				X
104	The cover page from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2025 formatted in Inline XBRL (included in Exhibit 101)				

* Management contract or compensatory plan or arrangement.

† Portions of this exhibit have been omitted.

The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

REGENXBIO Inc.

Dated: August 7, 2025

/s/ Curran Simpson
Curran Simpson
President and Chief Executive Officer
(Principal Executive Officer)

Dated: August 7, 2025

/s/ Mitchell Chan
Mitchell Chan
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

**REGENXBIO INC.
2025 EQUITY INCENTIVE PLAN
NOTICE OF RESTRICTED STOCK UNIT AWARD**

REGENXBIO Inc. (the “Company”) has granted you (the “Grantee”) Stock Units (this “Award”) with respect to shares of the common stock of the Company on the following terms:

Name of Grantee:	[Participant Name]
Total Number of Stock Units Granted:	[Number of Stock Units]
Date of Grant:	[Grant Date]
Vesting Schedule:	[Vesting Schedule]

You and the Company agree that these Stock Units are granted under and governed by the terms and conditions of the Company’s 2025 Equity Incentive Plan (the “Plan”) and the Restricted Stock Unit Award Agreement, both of which are incorporated herein by reference.

This Notice of Restricted Stock Unit Award may be executed and delivered electronically whether via the Company’s intranet or the Internet site of a third party or via email or any other means of electronic delivery specified by the Company. By your acceptance hereof (whether written, electronic or otherwise), you agree, to the fullest extent permitted by law, that in lieu of receiving documents in paper format, you accept the electronic delivery of any documents relating to the Plan or this Award (including, without limitation, the Plan, this Notice of Restricted Stock Unit Award, the Restricted Stock Unit Award Agreement, account statements, prospectuses required by the Securities and Exchange Commission and other legally required notices, and other communications and information) and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements). You agree that the Company may deliver these documents by any means of electronic delivery specified by the Company. The Company may also use an automated system for the documentation, granting or settlement of Awards.

If you do not actively accept this Award within three months of the Date of Grant listed above, you are deemed to have accepted the Award, subject to all of the terms and conditions in this Notice of Restricted Stock Unit Award, the Plan, and the Restricted Stock Unit Award Agreement, unless otherwise determined by the Administrator.

GRANTEE:	REGENXBIO INC.
[Participant Name]	By:
	Name: [Authorized Individual Name]
	Title: [Authorized Individual Title]

REGENXBIO INC.
2025 EQUITY INCENTIVE PLAN

RESTRICTED STOCK UNIT AWARD AGREEMENT

1. Grant of Stock Units

Subject to all of the terms and conditions set forth in the Notice of Restricted Stock Unit Award and this Restricted Stock Unit Award Agreement (collectively, this “Agreement”), and in the Plan, the Company has granted you the total number of Stock Units specified in the Notice of Restricted Stock Unit Award.

All capitalized terms used in this Agreement shall have the meanings assigned to them in this Agreement or the Plan. References to “you” and “your” in this Agreement refer to the Grantee or the Participant who holds this Award.

For all purposes applicable to this Award, “Service” means your continuous service as an Employee, Outside Director or Consultant.

2. Payment for Stock Units

No payment is required for the Stock Units that you are receiving.

3. Vesting

The Stock Units vest as shown in the Notice of Restricted Stock Unit Award, as otherwise agreed upon in an applicable employment agreement, or pursuant to and subject to the terms of Article 9 of the Plan with respect to a Change in Control. No additional Stock Units vest after your Service has terminated for any reason.

4. Forfeiture

If your Service terminates for any reason, then your Stock Units will be forfeited to the extent that they have not vested before your termination date and do not vest as a result of your termination of Service. This means that any Stock Units that have not vested under this Agreement will be cancelled immediately. You receive no payment for Stock Units that are forfeited.

The Administrator determines when your Service terminates for this purpose.

5. Leaves of Absence and Part-Time Work

For purposes of this Award, your Service does not terminate when you go on a military leave, a sick leave or another *bona fide* leave of absence, if the leave was approved by the Company in writing and if continued crediting of Service is required by applicable law, the Company’s leave of absence policy, or the terms of your leave. However, your Service terminates when the



approved leave ends, unless you immediately return to active work.

If you go on a leave of absence, then the vesting schedule specified in the Notice of Restricted Stock Unit Award may be adjusted in accordance with the Company's leave of absence policy or the terms of your leave. If you commence working on a part-time basis, the Company may adjust the vesting schedule so that the rate of vesting is commensurate with your reduced work schedule.

6. Settlement of Stock Units

Each Stock Unit will be settled promptly following the applicable date as of which such Stock Unit vests (each such date, a "Vesting Date"). However, each Stock Unit must be settled no later than the March 15th of the calendar year following the calendar year in which it vests.

At the time of settlement, you will receive one Common Share for each vested Stock Unit.

7. Limitations on Issuance

The Common Shares issuable pursuant to this Award may not be issued unless such issuance is in compliance with all applicable federal and state laws and regulations, as they are in effect on the date of issuance.

8. Nature of Stock Units

Your Stock Units are mere bookkeeping entries. They represent only the Company's unfunded and unsecured promise to issue Common Shares on a future date. As a holder of Stock Units, you have no rights other than the rights of a general creditor of the Company.

9. Withholding Taxes

The Company will, to the greatest extent permitted under the Plan and applicable law, satisfy the amount of withholding taxes that the Company determines is sufficient to satisfy the withholding tax requirements with respect to the vesting and settlement of the Stock Units ("Withholding Taxes") through the sale of a number of Common Shares subject to the Award and the remittance of the cash proceeds of such sale to the Company (a "sell-to-cover transaction"), subject to the following exceptions:

- (1) To the greatest extent permitted under the Plan and applicable law, the Company may elect, at least 45 calendar days prior to the applicable Vesting Date, in its sole discretion to instead satisfy the Withholding Taxes through the Company withholding the number of Common Shares subject to the Award with an aggregate

Fair Market Value as of the date they are withheld that is necessary to satisfy the Withholding Taxes (a “net settlement”).

- (2) Notwithstanding the Company’s discretion to elect to satisfy the Withholding Taxes through net settlement, you may elect, at least 30 calendar days prior to the applicable Vesting Date, to pay the Withholding Taxes through 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 an Affiliate; or (ii) tendering a cash payment to the Company (which may be in the form of a check, electronic wire transfer or other method permitted by the Company).

If the Company conducts a “sell-to-cover transaction,” you authorize the Company to make payment from the cash proceeds of this sale directly to the appropriate taxing authorities in an amount equal to the Withholding Taxes.

It is the Company’s intent that the mandatory sell-to-cover transaction cover Withholding Taxes imposed by the Company on the Participant in connection with the vesting and settlement of this Award comply with the requirements of Rule 10b5-1(c)(1)(i)(B) and Rule 10b5-1(c)(1)(ii)(D)(3) under the Exchange Act and be interpreted to comply with the requirements of Rule 10b5-1(c) under the Exchange Act with respect to the Participant.

Notwithstanding any other provision of this Agreement or the Plan, if you are subject to Section 16 of the Exchange Act (pursuant to Rule 16a-2 promulgated thereunder) at the time that all or any portion of the Award becomes subject to tax of any kind (including, but not limited to, federal, state, local, or non-U.S. income or employment tax), then the Company shall satisfy such withholding obligations using the “net settlement” method described above. For the avoidance of doubt, any such withholding shall, to the extent applicable, be carried out in accordance with Treas. Reg. § 1.409A-3(j)(4)(vi) or (xi).

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

You agree not to sell any Common Shares issued pursuant to your Stock Units at a time when applicable laws, Company policies or an agreement between the Company and its

10. Restrictions on Resale

underwriters prohibit a sale. This restriction will apply as long as your Service continues and for such period of time after the termination of your Service as the Company may specify.

11. Stock Units Are Nontransferable; Beneficiary Designations

You may not sell, transfer, assign, pledge or otherwise dispose of any Stock Units. For instance, you may not sell your Stock Units or use your Stock Units as security for a loan. If you attempt to do any of these things, this Award will immediately become invalid.

If the Service Provider dies after Stock Units have vested and before such Stock Units have been settled, then the Service Provider's estate will receive such vested Stock Units that the Service Provider holds at the time of the Service Provider's death.

Regardless of any marital property settlement agreement, the Company is not obligated to recognize your former spouse's interest in your Stock Units in any way.

12. Employment at Will

Neither your Award nor this Agreement gives you the right to be retained by the Company, a Parent, a Subsidiary, or an Affiliate in any capacity, nor the right to receive any future Awards. The Company and its Parents, Subsidiaries, and Affiliates reserve the right to terminate your Service at any time, with or without cause.

13. Stockholder Rights

Your Stock Units carry neither voting rights nor rights to receive dividends. Neither you, nor your estate or heirs, have rights as a stockholder of the Company unless and until your Stock Units are settled by the Company issuing Common Shares to you. No adjustments are made for dividends or other rights if the applicable record date occurs before the Stock Units are settled in Common Shares. You will not receive dividend equivalents pursuant to this Award.

14. Insider Trading Policies and Laws

You agree to comply with the Company's Insider Trading Policy and code of conduct (or related policies) as may be adopted or amended from time to time by the Board (or a duly authorized committee thereof). In addition, you shall comply with any applicable insider trading restrictions under securities laws, market abuse laws and/or other similar laws in the United States and in your country of residence (if different).

15. Recoupment or Clawback Policy

This Award, and the Common Shares acquired upon settlement of this Award, shall be subject to any recoupment or clawback policy that the Company or any Affiliate has adopted or may

adopt from time to time (the “Clawback Policy”). In addition, in consideration for this Award, you hereby agree that all outstanding incentive awards that have been made to you under the Plan or otherwise are also subject to the Clawback Policy. For the avoidance of doubt, the Clawback Policy may provide for the forfeiture of this Award or the recoupment of any Common Shares previously issued in connection with any Award granted under the Plan. This Agreement shall not affect the Company’s ability to pursue any other available rights and remedies under applicable law.

- 16. Adjustments** In the event of a stock split, a stock dividend or a similar change in Common Shares, the Administrator may, in its discretion, adjust the number and kind of shares covered by this Award.
- 17. Effect of Certain Corporate Transactions** If the Company is a party to a Change in Control, which may include a merger of the Company into another entity or certain other transactions described in the Plan, then this Award will be subject to the applicable provisions of Article 9 of the Plan.
- 18. Applicable Law** This Agreement will be interpreted and enforced under the laws of the State of Delaware (without regard to its choice-of-law provisions).
- 19. The Plan and Other Agreements** The text of the Plan is incorporated in this Agreement by reference, and this Agreement shall be governed by and construed in accordance with the Plan. In the event of any conflict between the provisions of the Plan and the provisions of this Agreement, or as to matters as to which this Agreement is silent, the provisions of the Plan shall be controlling and determinative.
- 20. Entire Agreement** The Plan and this Agreement constitute the entire understanding between you and the Company regarding this Award. Any prior agreements, commitments or negotiations concerning this Award are superseded.
- 21. Successors and Third-Party Beneficiaries** This Agreement shall be binding upon any successor of the Company, in accordance with the terms of this Agreement and the Plan. Each of the Company’s Affiliates shall be deemed to be a third-party beneficiary under this Agreement. The provisions of this Agreement extend to these third-party beneficiaries.
- 22. Unsecured and Unfunded Agreement** None of your rights hereunder shall be greater than the right of an unsecured general creditor of the Company. Any payments to be made hereunder shall be paid from the general funds of the Company, and no special or separate fund shall be established

and no segregation of assets shall be made to assure payment of such amounts.

23. Notices

Notices and communications under this Agreement must be in writing (and in the case of notices by the Company, any such notice must be made by an individual authorized by the Administrator to communicate regarding the subject of the notice) and, unless provided otherwise in this Agreement or by the Administrator, either personally delivered or sent by registered or certified United States mail, return receipt requested, postage prepaid. Notices to the Company must be addressed to its principal executive offices to the attention of the President or the General Counsel, or any other address designated by the Company in a written notice to you. Notices to you will be directed to your address then currently on file with the Company, or at any other address given by you in a written notice to the Company.

24. Waiver

Any waiver of any provision contained in this Agreement shall not be valid unless made in writing and signed by the person or persons sought to be bound by such waiver. The waiver by the Company of your breach of any provision of this Agreement shall not operate or be construed as a waiver of any subsequent breach by you.

25. Amendment

No amendment or modification hereof shall be valid unless it shall be in writing and signed by all parties hereto, except for any amendment or modification (i) made in connection with Articles 9 or 13.3 of the Plan, (ii) that the Administrator determines would not materially impair your rights under this Agreement, or (iii) that the Administrator determines is required to satisfy any law or regulation.

26. Severability and Reformation

If any one or more provisions of this Agreement are determined to be illegal or otherwise unenforceable, in whole or in part, the remaining provisions of this Agreement shall nevertheless be binding and enforceable. It is your intention and the Company's intention that if any of your restrictions, limitations, or obligations set forth in this Agreement are found by a court of competent jurisdiction to be overly broad, unreasonable, or otherwise unenforceable then these restrictions, limitations, or obligations shall be modified and enforced to the greatest extent that the court deems permissible.

27. Code Section 409A

The intent of the parties is that payments and benefits under this Agreement qualify under the short-term deferral exception to Code Section 409A, and accordingly, to the maximum extent

permitted, this Agreement shall be interpreted and administered in accordance with such intention. Nothing in this Agreement shall be interpreted or construed to transfer any liability for any tax (including a tax or penalty due as a result of a failure to comply with Code Section 409A) to the Company or to any other individual or entity, and the Company shall have no liability to you, or any other party, if this Award is not exempt from or compliant with Code Section 409A.

This paragraph applies only if the Company determines that you are a “specified employee,” as defined in the regulations under Code Section 409A, at the time of your “separation from service,” as defined in those regulations. If this paragraph applies, and any Stock Units subject to this Award constitute nonqualified deferred compensation within the meaning of Code Section 409A, then any Stock Units that otherwise would have been settled during the first six months following your separation from service will instead be settled during the seventh month following your separation from service.

28. Whistleblower Protections

Nothing in this Agreement, any other agreement, or any policy of the Company or its Affiliates is intended, or should be interpreted, to prohibit you from (1) reporting possible violations of federal law or regulation to any government agency or entity, (2) making any disclosures that are protected under the whistleblower provisions of federal law or regulation, or (3) otherwise cooperating with any government inquiry, in each case without advance approval by, or prior, contemporaneous, or subsequent notice to, anyone in the Company or its Affiliates.

29. Data Privacy

You acknowledge and agree that the Company and its Affiliates will process and retain certain personal data for the purposes of (1) calculating Awards, (2) monitoring Award terms and conditions, and (3) otherwise administering the Plan and Awards made under it. Such personal data may include, among other things, your name, address, email address, social security number, pay data, job title, and employment dates. You consent to such processing, and to the sharing of such personal data with the Company, its Affiliates, its agents, its advisers, its regulators, and tax authorities, wherever appropriate.

30. 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 Common Shares. You are hereby advised to consult with your own personal tax, legal, and financial advisors

regarding your participation in the Plan before taking any action related to this Award or the Plan.

31. Imposition of Other Requirements

The Company reserves the right to impose other requirements on your participation in the Plan, on this Award, and on any Common Shares acquired under the Plan, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require you (or any permitted transferee) to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

BY SIGNING OR OTHERWISE ACCEPTING THE NOTICE OF RESTRICTED STOCK UNIT AWARD, YOU AGREE TO ALL OF THE TERMS AND CONDITIONS DESCRIBED IN THIS AGREEMENT AND IN THE PLAN.

**REGENXBIO INC.
2025 EQUITY INCENTIVE PLAN
NOTICE OF STOCK OPTION GRANT**

REGENXBIO Inc. (the “Company”) has granted you (the “Optionee”) the following option (the “Option”) to purchase shares of the common stock of the Company:

Name of Optionee:	[Participant Name]
Total Number of Common Shares:	[Number of Options]
Type of Option:	[ISO or NSO]
Exercise Price per Common Share:	[Exercise Price]
Date of Grant:	[Grant Date]
Vesting Commencement Date:	[Vesting Commencement Date]
Vesting Schedule:	[Vesting Schedule]
Expiration Date:	[10th anniversary of Date of Grant] . This Option expires earlier if your Service terminates earlier, as described in the Stock Option Agreement, and may terminate earlier in connection with certain corporate transactions as described in Article 9 of the Plan.

You and the Company agree that this Option is granted under and governed by the terms and conditions of the Company’s 2025 Equity Incentive Plan (the “Plan”) and the Stock Option Agreement, both of which are incorporated herein by reference.

This Notice of Stock Option Grant may be executed and delivered electronically whether via the Company’s intranet or the Internet site of a third party or via email or any other means of electronic delivery specified by the Company. By your acceptance hereof (whether written, electronic or otherwise), you agree, to the fullest extent permitted by law, that in lieu of receiving documents in paper format, you accept the electronic delivery of any documents relating to the Plan or this Option (including, without limitation, the Plan, this Notice of Stock Option Grant, the Stock Option Agreement, account statements, prospectuses required by the Securities and Exchange Commission and other legally required notices, and other communications and information) and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements). You agree that the Company may deliver these documents by any means of electronic delivery specified by the Company. The Company may also use an automated system for the documentation, granting or exercise of Awards.

If you do not actively accept this Option within three months of the Date of Grant listed above, you are deemed to have accepted the Option, subject to all of the terms and conditions in this Notice of Stock Option Grant, the Plan, and the Stock Option Agreement, unless otherwise determined by the Administrator.

OPTIONEE

[Participant Name]

REGENXBIO INC.

By:

Name: **[Authorized Individual Name]**

Title: **[Authorized Individual Title]**

REGENXBIO INC.
2025 EQUITY INCENTIVE PLAN

STOCK OPTION AGREEMENT

1. Grant of Option

Subject to all of the terms and conditions set forth in the Notice of Stock Option Grant and this Stock Option Agreement (collectively, this “Agreement”), and in the Plan, the Company has granted you an Option to purchase up to the total number of Common Shares specified in the Notice of Stock Option Grant at the Exercise Price indicated in the Notice of Stock Option Grant.

All capitalized terms used in this Agreement shall have the meanings assigned to them in this Agreement or the Plan. References to “you” and “your” in this Agreement refer to the Optionee of the Option.

For all purposes applicable to this Option, “Service” means your continuous service as an Employee, Outside Director or Consultant.

2. Tax Treatment

This Option is intended to be an incentive stock option described in Code Section 422(b) (“ISO”) or a nonstatutory stock option, meaning a stock option not described in Code Sections 422 or 423 (“NSO”) as provided in the Notice of Stock Option Grant. However, even if this Option is designated as an ISO in the Notice of Stock Option Grant, it shall be deemed to be an NSO to the extent it does not qualify as an ISO under federal tax law, including under the \$100,000 annual limitation under Section 422(d) of the Code.

3. Vesting

This Option vests and becomes exercisable in accordance with the vesting schedule set forth in the Notice of Stock Option Grant, as otherwise agreed upon in an applicable employment agreement, or pursuant to and subject to the terms of Article 9 of the Plan with respect to a Change in Control.

In no event will this Option vest or become exercisable for additional Common Shares after your Service has terminated for any reason.

4. Term

This Option expires in any event at the close of business at Company headquarters on the day before the 10th anniversary of the Date of Grant, as shown in the Notice of Stock Option Grant. (This Option will expire earlier if your Service terminates, as

described below, and this Option may be terminated earlier as provided in Article 9 of the Plan.)

Upon its expiration or termination, this Option shall have no further force or effect and you shall have no further rights under this Option or to any Common Shares that have not been purchased pursuant to your prior exercise of this Option.

5.Termination of Service

If your Service terminates for any reason, this Option will expire immediately to the extent the Option is unvested as of your termination date and does not vest as a result of your termination of Service. The Administrator determines when your Service terminates for all purposes of this Option.

6.Regular Termination

If your Service terminates for any reason except death or Disability (including a termination by reason of your employer or other service recipient ceasing to be a Subsidiary of the Company, as applicable), then this Option, to the extent vested and exercisable as of your termination date, will expire at the close of business at Company headquarters on the date three months after your termination date.

7.Death

If you die before your Service terminates (or you die within the first three months after your Service terminates), then this Option, to the extent vested and exercisable as of your termination date, will expire at the close of business at Company headquarters on the date 12 months after the date of death (or the date of your termination of Service, if earlier).

8.Disability

If your Service terminates because of your Disability, then this Option, to the extent vested and exercisable as of your termination date, will expire at the close of business at Company headquarters on the date 12 months after your termination date.

For all purposes under this Agreement, “Disability” means that the Administrator determines in its sole discretion that you are unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or which has lasted, or can be expected to last, for a continuous period of not less than one year.

9.Leaves of Absence and Part-Time Work

For purposes of this Option, your Service does not terminate when you go on a military leave, a sick leave or another *bona fide* leave of absence, if the leave was approved by the Company in writing and if continued crediting of Service is required by applicable law, the Company’s leave of absence policy, or the

terms of your leave. However, your Service terminates when the approved leave ends, unless you immediately return to active work.

If you go on a leave of absence, then the vesting schedule specified in the Notice of Stock Option Grant may be adjusted in accordance with the Company's leave of absence policy or the terms of your leave. If you commence working on a part-time basis, the Company may adjust the vesting schedule so that the rate of vesting is commensurate with your reduced work schedule.

10. Notice Concerning Incentive Stock Option Treatment

Even if this Option is designated as an ISO in the Notice of Stock Option Grant, it ceases to qualify for favorable tax treatment as an ISO to the extent that it is exercised: (a) more than three months after the date when you cease to be an Employee for any reason other than death or permanent and total disability (as defined in Section 22(e)(3) of the Code), (b) more than 12 months after the date when you cease to be an Employee by reason of permanent and total disability (as defined in Section 22(e)(3) of the Code) or (c) on or after the first day following the end of the first three months of your leave of absence, unless your reemployment rights following such leave were guaranteed by statute or by contract.

11. Restrictions on Exercise

The Company will not permit you to exercise this Option unless the issuance of Common Shares is in compliance with all applicable federal and state laws and regulations, as they are in effect on the date of issuance.

12. Notice of Exercise

You may exercise only Options that are then exercisable in accordance with the vesting schedule set forth in the Notice of Stock Option Grant or pursuant to Article 9 of the Plan with respect to a Change in Control. When you wish to exercise this Option, you must notify the Company by filing the proper "Notice of Exercise" form at the address given on the form or, if the Company has designated a brokerage firm to administer the Plan, you must notify such brokerage firm in the manner such brokerage firm requires. Your Notice of Exercise must specify how many Common Shares you wish to purchase. The exercise of your Option will be effective only when (i) you have completed, signed, and delivered the Notice of Exercise in the form supplied by the Company (which may be electronic) and the Company receives it; (ii) you have made payment to the Company, pursuant to the terms of this Agreement, of an amount equal to the Exercise Price multiplied by the number of Common Shares being purchased as specified in the Notice of Exercise;

and (iii) you have satisfied, in a manner acceptable to the Company, any withholding liability under any state, federal, or other law arising in connection with the exercise of the Option.

However, if you wish to exercise this Option by executing a same-day sale (as described below), you must follow the instructions of the Company and the broker who will execute the sale.

If someone else wants to exercise this Option after your death, that person must prove to the Company's satisfaction that he or she is entitled to do so.

You may exercise your Option only for whole shares.

13. Form of Payment

When you submit your Notice of Exercise, you must include payment of the aggregate Exercise Price for the Common Shares that you are purchasing. To the extent permitted by applicable law, and if approved by the Administrator, payment may be made in one (or a combination of two or more) of the following forms:

By delivering to the Company your personal check, a cashier's check or a money order, or arranging for a wire transfer.

By delivering to the Company certificates for unrestricted Common Shares that you already own, along with any forms needed to effect a transfer of those Common Shares to the Company. The Fair Market Value of such Common Shares, determined as of the effective date of the option exercise, will be applied to the aggregate Exercise Price. Instead of surrendering Common Shares, you may attest to the ownership of those Common Shares on a form provided by the Company and have the same number of Common Shares subtracted from the Common Shares issued to you.

By giving to a securities broker approved by the Administrator irrevocable directions to sell all or part of the Common Shares being purchased as specified in the Notice of Exercise and to deliver to the Company, from the sale proceeds, an amount sufficient to pay the aggregate Exercise Price. (The balance of the sale proceeds, if any, will be delivered to you.) The directions must be given in accordance with the instructions of the Administrator and the broker. This exercise method is sometimes called a "same-day sale."

14. Withholding Taxes

You will not be allowed to exercise this Option unless you make arrangements acceptable to the Company to pay any withholding

taxes that may be due as a result of the exercise of the Option. These arrangements include payment in cash. With the Administrator's consent, these arrangements may also include (a) payment from the proceeds of the sale of Common Shares through a Company-approved broker, (b) withholding Common Shares that otherwise would be issued to you when you exercise this Option with a Fair Market Value no greater than the minimum amount required by law to be withheld, (c) surrendering Common Shares that you previously acquired with a Fair Market Value no greater than the minimum amount required by law to be withheld, or (d) withholding cash from other compensation. The Fair Market Value of withheld or surrendered Common Shares, determined as of the date they are withheld or surrendered, will be applied to the withholding taxes.

15.Restrictions on Resale

You agree not to sell any Common Shares issued pursuant to your exercise of this Option at a time when applicable laws, Company policies or an agreement between the Company and its underwriters prohibit a sale. This restriction will apply as long as your Service continues and for such period of time after the termination of your Service as the Company may specify.

**16.Option Is Nontransferable;
Beneficiary Designations**

Prior to the Service Provider's death, only the Service Provider (*i.e.*, an Employee, Outside Director or Consultant, subject to the terms of the Plan) may exercise this Option. You may not sell, transfer, assign, pledge or otherwise dispose of this Option. For instance, you may not sell this Option or use it as security for a loan. If you attempt to do any of these things, this Option will immediately become invalid.

The Service Provider may, however, dispose of this Option in the Service Provider's will or by means of a written beneficiary designation. A beneficiary designation must be filed with the Company on the proper form. It will be recognized only if it has been received at the Company's headquarters before the Service Provider's death. If the Service Provider files no beneficiary designation or if none of the Service Provider's designated beneficiaries survives the Service Provider, then the Service Provider's estate will receive the vested portion of the Option that the Service Provider holds at the time of the Service Provider's death. To receive the Service Provider's Option, the Service Provider's beneficiary or a representative of the Service Provider's estate must acknowledge and agree in writing, in a form acceptable to the Administrator, to be bound by the provisions of this Agreement and the Plan as if such beneficiary or representative of the estate were the Service Provider.

Regardless of any marital property settlement agreement, the Company is not obligated to honor a notice of exercise from your former spouse, nor is the Company obligated to recognize your former spouse's interest in your Option in any other way.

17. Employment at Will

Neither your Option nor this Agreement gives you the right to be retained by the Company, a Parent, a Subsidiary, or an Affiliate in any capacity, nor the right to receive any future Awards. The Company and its Parents, Subsidiaries, and Affiliates reserve the right to terminate your Service at any time, with or without cause.

18. Stockholder Rights

Your Option carries neither voting rights nor rights to receive dividends. Neither you, nor your estate or heirs, have rights as a stockholder of the Company unless and until you have exercised this Option by giving the required Notice of Exercise to the Company, paying the applicable aggregate Exercise Price, and satisfying any applicable withholding taxes. No adjustments are made for dividends or other rights if the applicable record date occurs before you exercise this Option.

19. Insider Trading Policies and Laws

You agree to comply with the Company's Insider Trading Policy and code of conduct (or related policies) as may be adopted or amended from time to time by the Board (or a duly authorized committee thereof). In addition, you shall comply with any applicable insider trading restrictions under securities laws, market abuse laws and/or other similar laws in the United States and in your country of residence (if different).

- 20. Recoupment or Clawback Policy** This Option, and the Common Shares acquired upon exercise of this Option, shall be subject to any recoupment or clawback policy that the Company or any Affiliate has adopted or may adopt from time to time (the “Clawback Policy”). In addition, in consideration for this Award, you hereby agree that all outstanding incentive awards that have been made to you under the Plan or otherwise are also subject to the Clawback Policy. For the avoidance of doubt, the Clawback Policy may provide for the forfeiture of this Option or the recoupment of any Common Shares previously issued in connection with any Award granted under the Plan. This Agreement shall not affect the Company’s ability to pursue any other available rights and remedies under applicable law.
- 21. Adjustments** In the event of a stock split, a stock dividend or a similar change in Common Shares, the Administrator may, in its discretion, adjust the number and kind of shares covered by this Option and the Exercise Price.
- 22. Effect of Certain Corporate Transactions** If the Company is a party to a Change in Control, which may include a merger of the Company into another entity or certain other transactions described in the Plan, then this Option will be subject to the applicable provisions of Article 9 of the Plan.
- 23. Applicable Law** This Agreement will be interpreted and enforced under the laws of the State of Delaware (without regard to its choice-of-law provisions).
- 24. The Plan and Other Agreements** The text of the Plan is incorporated in this Agreement by reference, and this Agreement shall be governed by and construed in accordance with the Plan. In the event of any conflict between the provisions of the Plan and the provisions of this Agreement, or as to matters as to which this Agreement is silent, the provisions of the Plan shall be controlling and determinative.
- 25. Entire Agreement** The Plan and this Agreement constitute the entire understanding between you and the Company regarding this Option. Any prior agreements, commitments or negotiations concerning this Option are superseded.
- 26. Successors and Third-Party Beneficiaries** This Agreement shall be binding upon any successor of the Company, in accordance with the terms of this Agreement and the Plan. Each of the Company’s Affiliates shall be deemed to be a third-party beneficiary under this Agreement. The provisions of this Agreement extend to these third-party beneficiaries.

27.Unsecured and Unfunded Agreement

None of your rights hereunder shall be greater than the right of an unsecured general creditor of the Company. Any payments to be made hereunder shall be paid from the general funds of the Company, and no special or separate fund shall be established and no segregation of assets shall be made to assure payment of such amounts.

28.Notices

Notices and communications under this Agreement must be in writing (and in the case of notices by the Company, any such notice must be made by an individual authorized by the Administrator to communicate regarding the subject of the notice) and, unless provided otherwise in this Agreement or by the Administrator, either personally delivered or sent by registered or certified United States mail, return receipt requested, postage prepaid. Notices to the Company must be addressed to its principal executive offices to the attention of the President or the General Counsel, or any other address designated by the Company in a written notice to you. Notices to you will be directed to your address then currently on file with the Company, or at any other address given by you in a written notice to the Company.

29.Waiver

Any waiver of any provision contained in this Agreement shall not be valid unless made in writing and signed by the person or persons sought to be bound by such waiver. The waiver by the Company of your breach of any provision of this Agreement shall not operate or be construed as a waiver of any subsequent breach by you.

30.Amendment

No amendment or modification hereof shall be valid unless it shall be in writing and signed by all parties hereto, except for any amendment or modification (i) made in connection with Articles 9 or 13.3 of the Plan, (ii) that the Administrator determines would not materially impair your rights under this Agreement, or (iii) that the Administrator determines is required to satisfy any law or regulation.

31.Severability and Reformation

If any one or more provisions of this Agreement are determined to be illegal or otherwise unenforceable, in whole or in part, the remaining provisions of this Agreement shall nevertheless be binding and enforceable. It is your intention and the Company's intention that if any of your restrictions, limitations, or obligations set forth in this Agreement are found by a court of competent jurisdiction to be overly broad, unreasonable, or otherwise unenforceable then these restrictions, limitations, or

obligations shall be modified and enforced to the greatest extent that the court deems permissible.

32.Code Section 409A

This Agreement and payments hereunder shall be interpreted to be exempt from the requirements of Code Section 409A pursuant to Treasury Regulations section 1.409A-1(b)(5)(i). Nothing in this Agreement shall be interpreted or construed to transfer any liability for any tax (including a tax or penalty due as a result of a failure to comply with Code Section 409A) to the Company or to any other individual or entity, and the Company shall have no liability to you, or any other party, if this Award is not exempt from or compliant with Code Section 409A.

33.Whistleblower Protections

Nothing in this Agreement, any other agreement, or any policy of the Company or its Affiliates is intended, or should be interpreted, to prohibit you from (1) reporting possible violations of federal law or regulation to any government agency or entity, (2) making any disclosures that are protected under the whistleblower provisions of federal law or regulation, or (3) otherwise cooperating with any government inquiry, in each case without advance approval by, or prior, contemporaneous, or subsequent notice to, anyone in the Company or its Affiliates.

34.Data Privacy

You acknowledge and agree that the Company and its Affiliates will process and retain certain personal data for the purposes of (1) calculating Awards, (2) monitoring Award terms and conditions, and (3) otherwise administering the Plan and Awards made under it. Such personal data may include, among other things, your name, address, email address, social security number, pay data, job title, and employment dates. You consent to such processing, and to the sharing of such personal data with the Company, its Affiliates, its agents, its advisers, its regulators, and tax authorities, wherever appropriate.

35.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 Common Shares. You are hereby advised to consult with your own personal tax, legal, and financial advisors regarding your participation in the Plan before taking any action related to the Option or the Plan.

36.Imposition of Other Requirements

The Company reserves the right to impose other requirements on your participation in the Plan, on the Option, and on any Common Shares acquired under the Plan, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require you (or any permitted

transferee) to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

BY SIGNING OR OTHERWISE ACCEPTING THE NOTICE OF STOCK OPTION GRANT, YOU AGREE TO ALL OF THE TERMS AND CONDITIONS DESCRIBED IN THIS AGREEMENT AND IN THE PLAN.

Certain identified information has been excluded from this exhibit because such information both (i) is not material and (ii) would likely cause competitive harm if publicly disclosed. Excluded information is indicated with brackets and asterisks.

LOAN AGREEMENT

Dated as of May 16, 2025

Between

REGENXBIO RS LLC,

as Borrower,

and

HCR RGNX ROYALTY SPV, LP,

as Lender

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Schedule 7.01(m)(iv)	Listed Patents – Validity and Enforceability
Schedule 7.01(m)(vii)	Listed Patents – No Infringement
Schedule 7.01(o)(iii)	Existing License Agreements – Amendment & Waiver Proposals
Schedule 7.01(o)(x)	Existing License Agreements – Audits
Schedule 7.02(f)	Company Proceedings
Schedule 7.02(j)	Company Broker's Fees
Schedule 7.02(m)	Material Contracts
Schedule 7.02(n)(i)	In-License Agreements; Reports; Notices
Schedule 7.02(n)(v)	In-License Agreements – Breaches
Schedule 7.02(n)(ix)	In-License Agreements – Notices
Schedule 7.02(t)	Insurance
Schedule 8.11(c)	Licensed Product Jurisdictions
Schedule 8.12(a)	Product-Specific Patents
Schedule 8.12(b)	Zolgensma IT Product-Specific Patents
Schedule 12.01	Assignment

This LOAN AGREEMENT (this “Agreement”) dated as of May 16, 2025, is entered into by and between REGENXBIO RS LLC, a Delaware limited liability company, as borrower (“Borrower”), and HCR RGNX ROYALTY SPV, LP, a Delaware limited partnership (“Lender”).

Capitalized terms not otherwise defined herein shall have the meanings set forth in, or by reference in, Article I below.

RECITALS

WHEREAS, Borrower has requested that Lender make the Initial Tranche Loan to Borrower on the Closing Date and Lender is willing to make the Initial Tranche Loan on the Closing Date, on the terms and subject to the conditions set forth herein;

WHEREAS, Borrower and Lender wish to set forth the terms for the Subsequent Milestone Tranche Loan, in the event that the Zolgensma IT Milestone Event is achieved and the parties effect the Subsequent Milestone Tranche Loan on the terms and subject to the conditions set forth herein;

WHEREAS, Borrower and Lender wish to set forth the terms for the Subsequent Additional Tranche Loan, in the event that Borrower and Lender desire to exercise their respective options to effect the Subsequent Additional Tranche Loan on the terms and subject to the conditions set forth herein; and

NOW, THEREFORE, in consideration of the mutual promises of the Parties, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, it is mutually agreed by the Parties as follows:

Article I. CERTAIN DEFINITIONS

Section 1.01 Definitions. As used herein:

“Account Bank” means Citizens Bank, N.A. or such other bank or financial institution approved by Lender and Borrower.

“Accreted Principal” has the meaning set forth in Section 3.01(c).

“Affiliate” means, with respect to any Person, any other Person that directly, or indirectly through one or more intermediaries, controls, or is controlled by, or is under common control with, such Person. For the purposes of this Agreement, “control” (including, with correlative meaning, the terms “controlling” and “controlled”) means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise.

“Agreement” has the meaning set forth in the preamble hereto.

“Amortization Payments” means the principal payments of the Loan due under Section 4.03(b) hereof.

“Applicable Law” means, with respect to any Person, all laws, rules, regulations and orders of Governmental Entities applicable to such Person or any of its properties or assets.

“Assignee” means any other Person to which Lender has assigned or is assigning its rights and obligations hereunder, whether or in whole or in part.

“Assignment and Acceptance” means a written instrument of assignment in the form set forth in Exhibit A, executed by and between the parties to an assignment under Section 12.01 hereof.

“Bankruptcy Law” means Title 11 of the United States Code entitled “Bankruptcy” and all other liquidation, conservatorship, bankruptcy, assignment for the benefit of creditors, moratorium, rearrangement, receivership, insolvency, reorganization, or similar debtor relief laws of the U.S. or other applicable jurisdictions (domestic or foreign) from time to time in effect and affecting the rights of creditors generally.

“Base Escrow Agreement” means that certain Master Escrow Agreement, dated as of December 24, 2020, by and between RPA Purchaser Rep and the Account Bank, attached as Exhibit B hereto.

“Bill of Sale” means the Bill of Sale and Assumption Agreement, dated as of the Closing Date, delivered by the Company to Borrower under the Contribution Agreement with respect to the “Transferred Assets” (as such term is defined in the Contribution Agreement).

“Borrower” shall have the meaning set forth in the preamble hereto.

“Borrower’s Organizational Documents” means the certificate of formation and operating agreement (or similar documents) of Borrower or the functional equivalent of the foregoing.

“Business Day” means any day other than a Saturday, Sunday or other day on which commercial banks are authorized to close under the Laws of, or are in fact closed in, the state where Lender’s Office is located; provided that, with respect to notices and determinations in connection with, and payments of or with respect to the Loan, such day is also a U.S. Government Securities Business Day.

“Calendar Quarter” means, for the first calendar quarter, the period beginning on the Closing Date and ending on the last day of the calendar quarter in which the Closing Date falls, and thereafter each successive period of three consecutive calendar months ending on March 31, June 30, September 30 or December 31.

“Capital Stock” of any Person means any and all shares, interests, memberships, ownership interest units, rights to purchase, warrants, options, participations or other equivalents of or interests in (however designated) equity of such Person, including any preferred stock, and

including, if such Person is a partnership, partnership interests (whether general or limited) and any other interest or participation that confers on a Person the right to receive a share of the profits and losses of, or distributions of property of, such partnership, and including, if such Person is a limited liability company, membership interests and any other interest or participation that confers on a Person the right to receive an interest in the profits and losses of, or distributions of property of, such limited liability company, in each case whether outstanding on the date hereof or issued after the date hereof, but excluding any Indebtedness convertible into or exchangeable for such equity.

“Change in Law” means the occurrence, after the Closing Date, of any of the following: (a) the adoption or taking effect of any law, rule, regulation or treaty, (b) any change in any law, rule, regulation or treaty or in the administration, interpretation, implementation or application thereof by any Governmental Entity or (c) the making or issuance of any request, rule, guideline or directive (whether or not having the force of law) by any Governmental Entity; provided that, notwithstanding anything herein to the contrary, (x) the Dodd-Frank Wall Street Reform and Consumer Protection Act and all requests, rules, guidelines or directives thereunder or issued in connection therewith and (y) all requests, rules, guidelines or directives promulgated by the Bank for International Settlements, the Basel Committee on Banking Supervision (or any successor or similar authority) or the U.S. or foreign regulatory authorities, in each case pursuant to Basel III, shall in each case be deemed to be a “Change in Law,” regardless of the date enacted, adopted or issued.

“Change of Control” means any “person” or “group” (as such terms are used in Sections 13(d) and 14(d) of the Exchange Act, but excluding any employee benefit plan of such person or its subsidiaries, and any person or entity acting in its capacity as trustee, agent or other fiduciary or administrator of any such plan) becomes the “beneficial owner” (as defined in Rules 13d-3 and 13d-5 under the Exchange Act) of more than fifty percent (50%) of the equity interests of the Company entitled to vote for members of its board of directors on a fully diluted basis (and taking into account all such securities that such person or group has the right to acquire pursuant to any option right).

“Closing Date” means May 16, 2025.

“CME” means CME Group Benchmark Administration Limited.

“Code” means the Internal Revenue Code of 1986, as amended.

“Collateral” has the meaning set forth in the Security Agreement.

“Collection Account” means the Escrow Account established and maintained at any Account Bank solely for the purpose of receiving remittance of receivables of Borrower pursuant to the Covered License Agreements and disbursement thereof as provided herein or in the RPA Transaction Documents, and any successor Collection Account entered into in accordance with Section 4.02 and the related Escrow Agreement.

“Commercialization” means, on a country-by-country basis, any and all activities with respect to the distribution, marketing, detailing, promotion, selling and securing of reimbursement of the Licensed Products in the Territory, which shall include, as applicable, post-marketing approval studies, post-launch marketing, promoting, detailing, marketing research, distributing, customer service, selling the Licensed Products, importing, exporting or transporting the Licensed Products for sale, and regulatory compliance with respect to the foregoing.

“Company” means REGENXBIO Inc., a Delaware corporation, which is the direct sole parent of Borrower.

“Competitive Infringement” is defined in Section 8.13(a).

“Confidential Information” means any and all technical and non-technical non-public information provided by either Party to the other (including, without limitation, any notices or other information provided pursuant to Section 8.08), either directly or indirectly, whether in graphic, written, electronic or oral form, and marked or identified at the time of disclosure as confidential, or which by its context would reasonably be deemed to be confidential, including without limitation information relating to a Party’s revenues, net sales, costs, technology, products and services, and any business, financial or customer information relating to a Party. Confidential Information shall not include any information that a Party can demonstrate was: (i) known to the general public at the time of its disclosure to such Party or its Affiliates, or thereafter became generally known to the general public, other than as a result of actions or omissions of the receiving Party, its Affiliates, or anyone to whom the receiving Party or its Affiliates disclosed such portion; (ii) known by the receiving Party or its Affiliates prior to the date of disclosure by the disclosing Party; (iii) disclosed to the receiving Party or its Affiliates on an unrestricted basis from a source unrelated to the disclosing Party and not known by the receiving Party or its Affiliates (after due inquiry) to be under a duty of confidentiality to the disclosing Party; or (iv) independently developed by the receiving Party or its Affiliates by personnel that did not use the Confidential Information of the disclosing Party in connection with such development. For clarity, this Agreement shall supersede the Confidentiality Agreement and the Confidentiality Agreement shall cease to be of any force and effect following the execution of this Agreement; provided, however, that all information falling within the definition of “Confidential Information” set forth in the Confidentiality Agreement shall also be deemed Confidential Information disclosed pursuant to this Agreement, and the use and disclosure of such Confidential Information following the date of this Agreement shall be subject to the provisions of Section 12.18.

“Confidentiality Agreement” means that certain Confidentiality Agreement, dated as of [****], by and between the Company and HealthCare Royalty Management LLC, as amended.

“Conforming Changes” means, with respect to the use, administration of or any conventions associated with SOFR or Three-Month Term SOFR, as applicable, any conforming changes to the definitions of “SOFR,” “Three-Month Term SOFR,” “Fixed Interest,” and “Calendar Quarter,” timing and frequency of determining rates and making payments of interest and other technical, administrative or operational matters (including, for the avoidance of doubt, the definitions of “Business Day” and “U.S. Government Securities Business Day”, timing of

borrowing requests or prepayment and length of lookback periods) that Lender, in consultation with Borrower, reasonably decides may be appropriate to reflect the adoption and implementation of such applicable rate(s) and to permit the administration thereof by Lender in a manner substantially consistent with market practice (or, if Lender reasonably decides that adoption of any portion of such market practice is not administratively feasible or that no market practice for the administration of such rate exists, in such other manner of administration as Lender, in consultation with Borrower reasonably decides is reasonably necessary in connection with the administration of this Agreement and any other Transaction Document).

“Contract” means any contract, agreement, indenture, lease, license, deed, commitment, obligation or instrument.

“Contribution” means the sale, transfer, assignment, contribution and conveyance of the Transferred Assets pursuant to the Contribution Agreement.

“Contribution Agreement” means the Contribution and Servicing Agreement, dated as of the Closing Date, between the Company and Borrower, in the form of Exhibit C hereto.

“Contributor Event of Default” has the meaning set forth in the Contribution Agreement.

“Covered License Agreements” means the Existing License Agreements and Future License Agreements.

“Covered Taxes” means all Taxes, other than Excluded Taxes, imposed on or with respect to any payment made by or on account of any obligation of Borrower under any Loan Documents.

“Default” means any condition or event which constitutes an Event of Default or which, with the giving of notice or the lapse of time or both (in each case to the extent described in the relevant sub-clauses of the definition of “Event of Default”) would, unless cured or waived, become an Event of Default.

“Default Rate” means, for any period for which an amount is overdue, a rate per annum equal for each day in such period to the lesser of (i) 2.00% plus the rate of interest otherwise applicable to the Loan as provided in Section 4.01 and the definition of “Fixed Interest” and (ii) the maximum rate of interest permitted under Applicable Law.

“Disqualified Capital Stock” of any Person means any class of Capital Stock of such Person that, by its terms, or by the terms of any related agreement or of any security into which it is convertible, puttable or exchangeable, is, or upon the happening of any event (other than a Change of Control) or the passage of time would be, required to be redeemed by such Person, whether or not at the option of the holder thereof, or matures or is mandatorily redeemable, pursuant to a sinking fund obligation or otherwise, in whole or in part, on or prior to the date which is [****] days after the Scheduled Maturity Date; provided, however, that any class of Capital Stock of such Person that, by its terms, authorizes such Person to satisfy in full its obligations with respect to the payment of dividends or upon maturity, redemption (pursuant to a sinking fund or

otherwise) or repurchase thereof or otherwise by the delivery of Capital Stock that is not Disqualified Capital Stock, and that is not convertible, puttable or exchangeable for Disqualified Capital Stock or Indebtedness, will not be deemed to be Disqualified Capital Stock so long as such Person satisfies its obligations with respect thereto solely by the delivery of Capital Stock that is not Disqualified Capital Stock.

“Dollars” or “\$” means lawful money of the U.S.

“Electronic Record” and “Electronic Signature” have the meanings assigned to them, respectively, by 15 USC §7006, as it may be amended from time to time.

“Emory” means Emory University, a Georgia nonprofit corporation.

“Emory Agreement” means the License Agreement, dated as of August [****], 2018, by and between the Company and Emory, and as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“Emory Consent” is defined in Section 6.01(g).

“Emory Patents Intercompany Out-License Agreement” means the Emory Patents License Agreement, dated as of the date hereof, by and between Borrower, as licensor, and the Company, as licensee, as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“ERISA” means the Employee Retirement Income Security Act of 1974, as amended, and the regulations promulgated thereunder.

“Escrow Account” means, collectively, any escrow account established and maintained at the Account Bank pursuant to the Escrow Agreement, the Security Agreement and this Agreement.

“Escrow Agreement” means the Base Escrow Agreement, as amended pursuant to Section 4.02(a).

“Event of Default” means the occurrence of any of the events set forth below:

(a) Borrower fails to pay any amount of principal of the Loan (i) on the Maturity Date or (ii) within [****] Business Days after the same otherwise becomes due and payable.

(b) Except as permitted by Section 4.01, Borrower fails to pay any interest on the Loan (including, without limitation, Fixed Interest) or make payment of any other amounts payable under this Agreement, in each case within [****] Business Days after the same becomes due and payable.

(c) Any representation or warranty of Borrower in any Loan Document to which it is party or in any certificate delivered by Borrower in connection with the Loan

Documents to Lender proves to have been incorrect in any material respect at the time it was made or deemed made; provided that if the consequences of the failure of such representation or warranty to be true and correct can be cured, such failure continues for a period of [****] days without such cure after the earlier of the date Borrower becomes aware of such failure or the date Lender provides Notice of such failure to Borrower.

(d) Borrower fails to perform or observe in any material respect any covenant or agreement contained in Section 9.01(a), Section 9.02, Section 9.04, Section 9.07 or Section 9.08 (or, for any such covenant qualified by “Material Adverse Effect”, Borrower fails to perform or observe such covenant in any respect).

(e) Borrower fails to perform or observe in any material respect any other covenant or agreement contained in the Loan Documents to which it is a party (other than those referred to in the preceding clauses of this definition) if such failure is not remedied on or before the [****] day after Notice thereof from Lender (or, for any such covenant qualified by “Material Adverse Effect”, Borrower fails to perform or observe such covenant in any respect).

(f) Borrower fails to perform or observe any covenant or agreement contained in the RPA Transaction Documents to which it is a party if such failure is not remedied on or before the [****] day after Notice thereof from the RPA Purchaser Rep.

(g) A Contributor Event of Default occurs and is continuing.

(h) Borrower (i) fails to pay when due (whether by scheduled maturity, required prepayment, acceleration, demand or otherwise) any Indebtedness (other than the Obligations hereunder) of [****] or more or (ii) fails to perform or observe any covenant or agreement to be performed or observed by it contained in any agreement or in any instrument evidencing any of its Indebtedness (other than the Obligations hereunder) of [****] or more and, as a result of such failure, any other party to that agreement or instrument is entitled to exercise the right to accelerate the maturity of any Indebtedness thereunder.

(i) Any uninsured judgment, decree or order in an amount in excess of [****] shall be rendered against Borrower and either (i) enforcement proceedings shall have been commenced upon such judgment, decree or order or (ii) such judgment, decree or order shall not have been stayed or bonded pending appeal, vacated or discharged, within [****] days from entry.

(j) An Insolvency Event shall occur.

(k) (i) Any of the Loan Documents shall cease to be in full force and effect, (ii) the validity or enforceability of any Loan Document is disaffirmed or challenged in writing by Borrower, the Company or any of their respective Affiliates, or (iii) this Agreement, the Security Agreement or the Pledge Agreement shall cease to give Lender the rights purported to be created hereby or thereby (including a first priority perfected Lien on

(subject to Permitted Liens and, solely with respect to the continuing first priority granted to Secured Party under the Loan Documents, Permitted Liens entitled to priority under Applicable Law) the assets of Borrower that constitute Collateral (except as otherwise expressly provided herein and therein)) other than as a direct result of any action by Lender or failure of Lender to perform an obligation of Lender hereunder.

(l) Borrower fails to perform or observe any covenant or agreement contained in any Material Contract to which it is a party or any of Borrower's Organizational Documents, and such failure is not cured or waived within any applicable grace period, and in the case of any provision in Borrower's Organizational Documents, if not cured, is not waived by Lender, or any Material Contract shall cease to be in full force and effect, and in the case of any provision in a Material Contract, such failure to perform or observe results in a termination of such Material Contract, and any such failure, cessation or termination could reasonably be expected to have a Material Adverse Effect.

(m) Any Specified Covered License Agreement is terminated or cancelled by the Licensee, in each case prior to the Scheduled Maturity Date and is not replaced in accordance with Section 8.17(b) hereof within [****] days after such termination or cancellation; provided that such failure to replace shall not constitute an Event of Default for so long as Borrower and the Company are engaged in discussions with a Third Party with respect to a New Arrangement, continue to exert commercially reasonable efforts, as measured at the time, to effect such New Arrangement and have a good faith reasonable belief that such discussions will result in the entry into a New Arrangement within a reasonable period of time thereafter.

(n) Any security interest purported to be created by the Security Agreement shall cease to be in full force and effect, or shall cease to give the rights, powers and privileges purported to be created and granted hereunder or thereunder (including a perfected first priority security interest in and Lien on (subject in each case to Permitted Liens and, solely with respect to the continuing first priority granted to Secured Party under the Loan Documents, Permitted Liens entitled to priority under Applicable Law) substantially all of the Collateral (except as otherwise expressly provided herein and therein)) in favor of Lender pursuant hereto or thereto (other than as a result of the failure by Lender of taking any action required to maintain the perfection of such security interests), or shall be asserted by Borrower not to be a valid, perfected, first priority (except as otherwise expressly provided in this Agreement or such Security Agreement) security interest in the Collateral and/or Borrower takes any action that could reasonably be expected to impair Lender's security interest in any of the Collateral (other than granting Permitted Liens or permitting such Permitted Liens to exist).

"Exchange Act" means the Securities Exchange Act of 1934, as amended, and the regulations promulgated thereunder.

"Excluded Payments" means all payments under the Covered License Agreements that do not constitute the Included Royalty Interest.

“Excluded Taxes” means any of the following Taxes imposed in each case on or with respect to a payment to any Lender (a) any Taxes imposed on or measured by net income (however denominated), franchise Taxes and branch profits Taxes, in each case (x) as a result of such Lender being organized under the laws of, or having its principal office or its applicable lending office located in, the jurisdiction imposing such Tax (or any political subdivision thereof) or (y) that are Other Connection Taxes, (b) any U.S. federal withholding Tax imposed on amounts payable to or for the account of such Lender with respect to an applicable interest in the Loan pursuant to a law in effect on the date on which (i) such Lender acquires the applicable interest in the Loan or (ii) such Lender changes its lending office, except, in each case, to the extent that (A) such Tax was imposed on Lender’s assignor (if any) immediately prior to the assignment to such Lender, or was imposed on such Lender immediately prior to the change of such Lender’s lending office, and (B) such Tax described in (A) was a Covered Tax, (c) any Tax that is attributable to such Lender’s failure to comply with Section 5.01(a) and (d) any Tax withheld pursuant to FATCA.

“Existing License Agreements” means the (a) Novartis License Agreement, (b) Nippon Shinyaku License Agreement, (c) Spacecraft License Agreement, (d) Ultragenyx License Agreement, and (e) Ultragenyx Option and License Agreement, in each case, together with such amendments or other modifications attached thereto, as applicable, in the forms attached to this Agreement as Exhibit M, respectively, and as assigned, transferred and contributed to Borrower pursuant to the Contribution Agreement.

“Exploit” means, with respect to the Licensed Product, the manufacture, use, sale, offer for sale (including marketing and promotion), importation, distribution or other Commercialization; and “Exploitation” shall have the correlative meaning.

“FATCA” means Sections 1471 through 1474 of the Code, as of the date of this Agreement (or any amended or successor version that is substantively comparable and not materially more onerous to comply with), any current or future regulations or official interpretations thereof, any agreements entered into pursuant to current Section 1471(b)(1) of the Code (or any amended or successor version described above) and any fiscal or regulatory legislation, or official administrative rules or other official administrative guidance adopted pursuant to any intergovernmental agreement, treaty or convention among Governmental Entities and implementing such Sections of the Code.

“FDA” means the United States Food and Drug Administration.

“Final Payment Premium” means, as of any date of determination, an amount equal to five percent of the Principal Amount as of such date of determination.

“Financial Statements” means, the consolidated balance sheets of the Company, audited at December 31, 2024, and December 31, 2023, and the related consolidated statements of operations and comprehensive loss, cash flows and changes in stockholders’ equity of the Company audited for the years ended December 31, 2024, and December 31, 2023, and the accompanying notes thereto, as filed within Forms 10-K and 10-Q with the SEC.

“Fixed Interest” means interest with respect to the Loan, accruing with respect to the outstanding principal balance thereof, at a rate per annum equal, for any Calendar Quarter, to the sum of (a) nine-and-three-quarters percent plus (b) Three-Month Term SOFR for such Calendar Quarter.

“Foreign Lender” means any Lender which is not a U.S. Person.

“Future License Agreements” means any and all future Out-Licenses for RGX-111 or RGX-121 in the territories not included in the Nippon Shinyaku License Agreement. For the avoidance of doubt, “Future License Agreements” does not include any future Out-Licenses other than Out-Licenses for RGX-111 or RGX-121 in the territories not included in the Nippon Shinyaku License Agreement.

“Future Licensees” means the licensee-parties to any Future License Agreement.

“GAAP” means the generally accepted accounting principles in the U.S. in effect from time to time; provided that in the event such principles change after the Closing Date in a manner which affects compliance with this Agreement by Borrower (including without limitation in the determination of payments in respect of the Included Royalty Interest), such change shall be ignored for the purpose of determining such compliance.

“Governmental Entity” means any government, regulatory or administrative agency or commission, or other governmental agency, authority, instrumentality or body (including a court), whether foreign, federal, state or local, including any applicable Patent Office, the FDA, the European Medicines Agency, the United States National Institutes of Health, or any other governmental authority in any country.

“GSK” means SmithKline Beecham Corporation, a Pennsylvania corporation doing business as GlaxoSmithKline.

“GSK Agreement” means the License Agreement, dated as of March 6, 2009, as amended on April 15, 2009, by and between the Company and GSK, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“GSK Patents Intercompany Out-License Agreement” means the GSK Patents License Agreement, dated as of the date hereof, by and between Borrower, as Licensor, and the Company, as Licensee, as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“Guarantee” means, as to any Person: (a) any obligation, contingent or otherwise, of such Person guaranteeing or having the economic effect of guaranteeing any Indebtedness or other obligation payable or performable by another Person (the “primary obligor”) in any manner, whether directly or indirectly, and including any obligation of such Person, direct or indirect (i) to purchase or pay (or advance or supply funds for the purchase or payment of) such Indebtedness or other obligation, (ii) to purchase or lease property, securities or services for the purpose of assuring the obligee in respect of such Indebtedness or other obligation of the payment or performance of such Indebtedness or other obligation, (iii) to maintain working capital, equity capital or any other

financial statement condition or liquidity or level of income or cash flow of the primary obligor so as to enable the primary obligor to pay such Indebtedness or other obligation, or (iv) entered into for the purpose of assuring in any other manner the obligee in respect of such Indebtedness or other obligation of the payment or performance thereof or to protect such obligee against loss in respect thereof (in whole or in part); or (b) any Lien on any assets of such Person securing any Indebtedness or other obligation of any other Person, whether or not such Indebtedness or other obligation is assumed by such Person.

“Included Royalty Interest” means, with respect to each Calendar Quarter, the payments received during such Calendar Quarter in respect of the Royalty Interest.

“Indebtedness” means, with respect to any Person, (i) indebtedness pursuant to an agreement or instrument involving or evidencing money borrowed, the advance of credit, a conditional sale or a transfer with recourse or with an obligation to repurchase (but excluding trade credit and accounts payable in the ordinary course of business), (ii) any capitalized lease, (iii) any obligations with respect to Disqualified Capital Stock, (iv) indebtedness of a third party secured by (or for which the holder of such indebtedness has an existing right, contingent or otherwise, to be secured by) any Lien on assets owned or acquired by such Person, whether or not the indebtedness secured thereby has been assumed (but only to the extent of such Lien), (v) net amounts owing pursuant to an interest rate protection agreement, foreign currency exchange agreement or other hedging arrangement, (vi) a reimbursement obligation under a letter of credit issued for the account of such Person, or (vii) all Guarantees with respect to Indebtedness of the types specified in clauses (i) through (vi) above of another Person. For the avoidance of doubt, the Indebtedness of any Person shall include the Indebtedness of any other entity to the extent such Person is directly liable therefor as a result of such Person’s ownership interest in or other relationship with such entity, except to the extent the terms of such Indebtedness provide that such Person is not liable therefor.

“Indemnified Liabilities” means, collectively, any and all liabilities, obligations, losses, damages, penalties, claims, costs, expenses and disbursements of any kind or nature whatsoever (including the reasonable fees and disbursements of counsel for Indemnites in connection with any investigative, administrative or judicial proceeding commenced or threatened by any Person whether or not any such Indemnitee shall be designated as a party or a potential party thereto, and whether or not such Indemnitee is required by Applicable Law to be involved therein, and any fees or expenses actually incurred by Indemnites in enforcing the indemnity provided herein), whether direct, indirect or consequential, whether based on any federal, state or foreign laws, statutes, rules or regulations (including securities and commercial laws, statutes, rules or regulations), on common law or equitable cause or on contract or otherwise, imposed on, incurred by, or asserted against any such Indemnitee, in any manner relating to or arising out of this Agreement or the other Loan Documents or the transactions contemplated hereby or thereby (including any enforcement of any of the Loan Documents (including any sale of, collection from, or other realization upon any of the Collateral)).

“Indemnitee” means Lender and its Affiliates and their respective officers, partners, directors, trustees, employees, agents and controlling Persons.

“Initial Search Period” has the meaning set forth in Section 8.17(b).

“Initial Tranche Loan” means, prior to its disbursement, the loan to be made by Lender to Borrower in accordance with Section 2.01(a), and from and after its disbursement, at any time the aggregate principal amount loaned to Borrower on the Closing Date pursuant to Section 2.01(a), plus any Accreted Principal thereunder outstanding at such time.

“Initial Tranche Loan Commitment” means the amount of \$150,000,000.

“Initial Tranche Note” means the note, in the form attached hereto as Exhibit D-1, issued by Borrower to Lender evidencing the Initial Tranche Loan made on the Closing Date to Borrower and any replacement(s) thereof issued in accordance with Section 12.09.

“In-License Agreements” means the (a) Emory Agreement, (b) GSK Agreement, (c) Penn Agreement and (d) UoM Agreement, in each case, together with such amendments or other modifications attached thereto, as applicable, and as assigned, transferred and contributed or licensed to Borrower pursuant to the Contribution Agreement or the Intercompany In-License Agreement, as applicable.

“Insolvency Event” means the occurrence of any of the following with respect to Borrower or the Company:

(i) (A) an involuntary proceeding shall be commenced or an involuntary petition shall be filed in a court of competent jurisdiction seeking (x) relief in respect of Borrower or the Company, or of a substantial part of the property of Borrower or the Company, under any Bankruptcy Law now or hereafter in effect, (y) the appointment of a receiver, trustee, custodian, sequestrator, conservator or similar official for Borrower or the Company for a substantial part of the property of Borrower or the Company or (z) the winding-up or liquidation of Borrower or the Company, which proceeding or petition shall continue undismissed for [****] calendar days or (B) an order of a court of competent jurisdiction approving or ordering any of the foregoing shall be entered;

(ii) Borrower or the Company shall (A) voluntarily commence any proceeding or file any petition seeking relief under any Bankruptcy Law now or hereafter in effect, (B) apply for the appointment of a receiver, trustee, custodian, sequestrator, conservator or similar official itself or for a substantial part of its property, (C) fail to contest in a timely and appropriate manner any proceeding or the filing of any petition described in clause (i) of this definition, (D) file an answer admitting the material allegations of a petition filed against it in any proceeding described in clause (i) of this definition, (E) make a general assignment for the benefit of creditors or (F) wind up or liquidate (except as permitted under this Agreement);

(iii) Borrower or the Company shall take any action in furtherance of or for the purpose of effecting, or indicating its consent to, approval of, or acquiescence in, any of the acts set forth in clause (i) or (ii) of this definition; or

(iv) Borrower or the Company shall become unable, admit in writing its inability, or fail generally, to pay its debts as they become due.

“Insurance Providers” means the insurance companies set forth in Schedule 7.02(t) or insurance companies rated at least as high as the ratings given, as of the Closing Date (according to A.M. Best Company, Inc.).

“Intellectual Property” means all intellectual property covering the sale, manufacture, use, importation or marketing of any Licensed Product in such Licensed Product’s Territory, including but not limited to patents, patent applications, trademarks, trademark applications and know-how, necessary for the sale, manufacture, use, importation or marketing of such Licensed Product that is owned, licensed in or controlled (and if controlled, only to the extent of control) by Borrower (after giving effect to the contribution under the Contribution Agreement) as of the Closing Date and during term of this Agreement.

“Intercompany In-License Agreement” means the Penn Patents Intercompany In-License Agreement and the NAV Intercompany In-License Agreement.

“Intercompany Out-License Agreement” means the Emory Patents Intercompany Out-License Agreement, the GSK Patents Intercompany Out-License Agreement and the UoM Patents Intercompany Out-License Agreement.

“Interest Payment Date” means, for each applicable Calendar Quarter, the date that is 60 days after the last day of such Calendar Quarter, or if any such day is not a Business Day, on the next succeeding Business Day.

“Knowledge” means, with respect to Borrower or the Company, the actual knowledge of [****].

“Law” means any law, constitution, statute, rule, regulation, code, ordinance, treaty order or decree, whether domestic or foreign, and all applicable requirements, official guidance, rules, consents, approvals and authorizations issued or promulgated by any Governmental Entity.

“Lender” means Lender (as defined in the preamble hereto) and any successors and assigns under Section 12.01(b).

“Lender Account” means such account of Lender maintained at such banking institution as Lender may specify in its discretion from time to time in writing to Borrower at least [****] Business Day prior to any Interest Payment Date or other date on which payments are to be made to Lender pursuant to the Loan Documents.

“Lender Expense Amount” means the reasonable and documented fees and out-of-pocket expenses of Lender incurred in connection with the making of the Loan, including legal fees and expenses and expenses incurred in connection with Lender’s due diligence investigation.

“Licensed Products” mean, collectively, (a) “Licensed Products” as defined in the Novartis License Agreement, (b) “Licensed Products” as defined in the Nippon Shinyaku

License Agreement, (c) “Licensed Products” as defined in the Spacecraft License Agreement, (d) “Licensed Products” as defined in the Ultragenyx License Agreement, and (e) “Licensed Product” as defined in the Ultragenyx Option and License Agreement.

“Licensees” means each of (a) Novartis, (b) Nippon Shinyaku, (c) Spacecraft, (d) Ultragenyx and (e) any Future Licensees, or any successor thereto, as applicable.

“Licensors” means each of (a) Emory, (b) GSK, (c) Penn and (d) UoM.

“Lien” means any mortgage, pledge, hypothecation, assignment, deposit arrangement, encumbrance, lien (statutory or other), charge, or preference, priority or other security interest or preferential arrangement in the nature of a security interest of any kind or nature whatsoever (including any conditional sale or other title retention agreement, any easement, right of way or other encumbrance on title to real property, and any financing lease having substantially the same economic effect as any of the foregoing).

“Listed Patents” has the meaning set forth in Section 7.01(m)(i).

“Loan” means, prior to their respective disbursement, the loan or loans to be made by Lender and borrowed by Borrower in accordance with and subject to Section 2.01 and Section 2.02, and after their respective disbursement to Borrower, at any time the sum of the Initial Tranche Loan, the Subsequent Milestone Tranche Loan (if any) and the Subsequent Additional Tranche Loan (if any).

“Loan Documents” means this Agreement, the Initial Tranche Note, the Subsequent Milestone Tranche Note (if any), the Subsequent Additional Tranche Note (if any), the Security Agreement, the Pledge Agreement, the Contribution Agreement, Intercompany In-License Agreement, Intercompany Out-License Agreement, the Bill of Sale, the Escrow Agreement, the Warrant, and all other documents delivered in connection therewith.

“Material Adverse Effect” means (a) an Insolvency Event, (b) a material adverse change in the business, operations, properties, results of operations or financial condition of Borrower, taken as a whole; (c) a material adverse effect on the validity or enforceability of the Loan Documents taken as a whole or any material provision hereof or thereof; (d) a material adverse effect on the ability of Borrower or the Company to consummate the transactions contemplated by the Loan Documents, or on the ability of Borrower or the Company to perform its obligations under the Loan Documents to which it is a party, in each case, taken as a whole; (e) a material adverse effect on the rights of Borrower under any Covered License Agreement or any In-License Agreement, (f) a material adverse effect on the value of the Included Royalty Interest (taking into account the timing, amount and duration thereof), or (g) a material adverse effect on the rights or remedies of Lender under the Loan Documents, taken as a whole.

“Material Contract” means any Contract to which Borrower, the Company, or a Subsidiary of the Company, as the case may be in the context in which used, is a party or any of the respective assets or properties of Borrower, the Company or such Subsidiary are bound or committed (other than the Transaction Documents) and for which any breach, violation,

nonperformance or early cancellation could reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect. The Material Contracts as of the date hereof are identified on Schedule 7.02(m).

“Material Contract Counterparty” means a counterparty to any Material Contract, other than RPA Purchaser Rep and the RPA Purchasers.

“Material Other Reports” means all material notices and reports (excluding Royalty Reports) delivered pursuant to any Covered License Agreement, including, without limitation, (a) development records pursuant to Section 3.12 of the Nippon Shinyaku License Agreement, agenda materials and meeting minutes pursuant to Section 7.2 of the Nippon Shinyaku License Agreement and milestone event notices pursuant to Section 8.2.3 of the Nippon Shinyaku License Agreement, (b) reports pursuant to Sections 3.7.3 and 3.7.4 of the Spacecraft License Agreement, development plans pursuant to Section 4.2 of the Spacecraft License Agreement, development progress reports pursuant to Section 4.3 of the Spacecraft License Agreement, and improvement reports pursuant to Section 4.5 of the Spacecraft License Agreement, (c) reports pursuant to Section 3.6.2 of the Ultragenyx Option and License Agreement, reports pursuant to Section 3.7.3 of the Ultragenyx Option and License Agreement, development plans pursuant to Section 4.3 of the Ultragenyx Option and License Agreement, development progress reports pursuant to Section 4.4 of the Ultragenyx Option and License Agreement, and improvement reports pursuant to Section 4.6 of the Ultragenyx Option and License Agreement, (d) reports pursuant to Section 3.4.2(d) of the Ultragenyx License Agreement, development plans pursuant to Section 4.3 of the Ultragenyx License Agreement, development progress reports pursuant to Section 4.4 of the Ultragenyx License Agreement, and improvement reports pursuant to Section 4.6 of the Ultragenyx License Agreement, (e) reports pursuant to Section 3.6.3 of the Novartis License Agreement, (f) the applicable section(s) of any Future License Agreement(s).

“Maturity Date” means the earlier of (i) the Scheduled Maturity Date and (ii) the date of satisfaction in full of the Loan.

“Maximum Lawful Rate” means the highest rate of interest permissible under Applicable Law.

“Modification” has the meaning set forth in Section 8.19(a).

“NAV Intercompany In-License Agreement” means the NAV License Agreement, dated as of the date hereof, by and between the Company, as licensor, and Borrower, as licensee, as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“New Arrangement” has the meaning set forth in Section 8.17(b).

“New Arrangement Expenses” has the meaning set forth in Section 8.17(b).

“Nippon Shinyaku” means Nippon Shinyaku Co., Ltd., a limited liability company organized under the laws of Japan.

“Nippon Shinyaku License Agreement” means the Collaboration and License Agreement, dated as of January 14, 2025, by and between the Company and Nippon Shinyaku, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“Nippon Shinyaku Notice” has the meaning set forth in Section 8.24.

“Note” means any or all of the Initial Tranche Note, the Subsequent Milestone Tranche Note and the Subsequent Additional Tranche Note.

“Notice of Prepayment” means the notice of prepayment, in the form of Exhibit F hereto.

“Notice of Subsequent Additional Tranche Borrowing” means an irrevocable notice, substantially in the form set forth in Exhibit E-2, to be given by Borrower to Lender in accordance with Section 2.02(b).

“Notice of Subsequent Milestone Tranche Borrowing” means an irrevocable notice, substantially in the form set forth in Exhibit E-1, to be given by Borrower to Lender in accordance with Section 2.02(a).

“Notices” means, collectively, notices, consents, approvals, reports, designations, requests, waivers, elections and other communications.

“Novartis” means Novartis Gene Therapies, Inc. f/k/a AveXis, Inc., a Delaware corporation.

“Novartis License Agreement” means the License Agreement, dated as of March 21, 2014, as amended on January 8, 2018 (the “First Novartis Amendment”), by and between the Company and Novartis, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“Obligations” means, without duplication, the Loan, Fixed Interest and all present and future Indebtedness, Taxes, liabilities, obligations, covenants, duties, and debts, owing by Borrower to Lender, arising under or pursuant to the Loan Documents, including all principal, interest, premium, charges, expenses, fees and any other sums chargeable to Borrower hereunder and under the other Loan Documents (and including any interest, fees and other charges that would accrue but for the filing of a bankruptcy action with respect to Borrower, whether or not such claim is allowed in such bankruptcy action).

“Office” means, with respect to Lender, its Stamford, Connecticut office, and with respect to any other Lender, the office of Lender designated as its “Office” in an Assignment and Acceptance, or such other office as may be otherwise designated in writing from time to time by Lender to Borrower.

“Organizational Document” means, with respect to any Person, (i) in the case of any corporation, the certificate of incorporation and by-laws (or similar documents) of such

Person, (ii) in the case of any limited liability company, the certificate of formation and operating agreement (or similar documents) of such Person, (iii) in the case of any limited partnership, the certificate of formation and limited partnership agreement (or similar documents) of such Person, (iv) in the case of any general partnership, the partnership agreement (or similar document) of such Person, and (v) in any other case, the functional equivalent of the foregoing.

“Other Connection Taxes” means, with respect to any Lender, Taxes imposed as a result of a present or former connection between such Lender and the jurisdiction imposing such Tax (other than any connections arising from such Lender having executed, delivered, become a party to, performed its obligations under, received payments under, received or perfected a security interest under, engaged in any other transaction pursuant to or enforced any Loan Document, or sold or assigned any interest in the Loan or Loan Document).

“Other Taxes” means all present or future stamp, court, documentary, intangible, recording, filing or similar Taxes that arise from any payment made under, from the execution, delivery, performance, enforcement or registration of, from the receipt or perfection of a security interest under, or otherwise with respect to, any Loan Document, except any such Taxes that are Other Connection Taxes imposed with respect to an assignment.

“Out-License” means each license, settlement agreement or other agreement or arrangement between the Company, Borrower or any of their Affiliates and any Third Party pursuant to which the Company, Borrower or any of their Affiliates grants a license, sublicense or similar grant of any Intellectual Property that is necessary for the Exploitation of a Licensed Product.

“Party” and “Parties” means Lender and Borrower, individually and collectively.

“Patent” means any and all issued patents and pending patent applications, including without limitation, all provisional applications, substitutions, continuations, continuations-in-part, divisions, and renewals, all letters patent granted thereon, and all patents-of-addition, reissues, reexaminations and extensions or restorations by existing or future extension or restoration mechanisms (including regulatory extensions), claiming or covering the Licensed Products, or composition of matter, formulation, or methods of manufacture or use thereof, that are issued or filed on or after the date of this Agreement, including those identified in Section 7.01(m), in each such case, which are owned, co-owned or controlled by, issued or licensed to, licensed by, or hereafter acquired or licensed by, Borrower or any Subsidiary.

“Patent Office” means the respective patent office (foreign or domestic) for any patent.

“Patriot Act” means the USA Patriot Act, Public Law No. 107-56.

“Payment in Full” means the payment in full in good funds of the Loan and other Obligations (other than contingent indemnification obligations for which such claims have been reserved).

“Payments” means due and owing payments of Amortization Payments and Fixed Interest (each under Section 4.04 hereof), including, in each case any default, additional interest or prepayment premium charged hereunder.

“Penn” means The Trustees of the University of Pennsylvania.

“Penn Agreement” means the License Agreement, effective on February 24, 2009, as amended on March 6, 2009, September 9, 2014, April 29, 2016, April 4, 2019, September 11, 2020, and March 21, 2022, by and between the Company and Penn, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“Penn Patents Intercompany In-License Agreement” means the Penn Patents License Agreement, dated as of the date hereof, by and between the Company, as licensor, and Borrower, as licensee, as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“Permitted Liens” means:

(a) Liens created pursuant to any Loan Document;

(b) Liens in favor of a banking or other financial institution arising as a matter of law or under customary contractual provisions encumbering deposits or other funds maintained with such banking or other financial institution (including the right of set off and grants of security interests in deposits and/or securities held by such banking or other financial institution) and that are within the general parameters customary in the banking industry;

(c) Liens securing the claims of materialmen, mechanics, carriers, landlords, warehousemen and similar Persons, and attachment, judgment and other similar Liens arising in connection with court proceedings so long as the judgment claims secured thereby do not otherwise constitute an Event of Default under clause (i) of the definition of “Event of Default”;

(d) any right, title or interest of a licensor under a license or sublicense to which Borrower is a party as licensee or sublicensee, and any restrictions under a license to which Borrower is a party as licensee or sublicensee;

(e) (i) leases, subleases, licenses, or sublicenses of the assets or properties of Borrower thereof, in each case entered into in the ordinary course of business and not interfering in any material respect with the business of Borrower and (ii) the Covered License Agreements and any New Arrangement or other license replacing such Covered License Agreement in accordance with Section 8.17(b);

(f) Liens for ad valorem property Taxes that are not yet due and payable (other than Taxes relating exclusively to the Included Royalty Interest), Liens in respect of Taxes to the extent such Taxes are being contested in good faith by appropriate proceedings and for which adequate reserves have been established in accordance with GAAP;

(g) Liens in respect of property of Borrower imposed by Applicable Law which were incurred in the ordinary course of business and do not secure Indebtedness for borrowed money, such as carriers', warehousemen's, distributors', wholesalers', materialmen's and mechanics' liens and other similar Liens arising in the ordinary course of business; and

(h) banker's liens for collection or rights of set off or similar rights and remedies as to deposit accounts or other funds maintained with depositary institutions; provided that such deposit accounts or funds are not established or deposited for the purpose of providing collateral for any Indebtedness and are not subject to restrictions on access by Borrower in excess of those required by applicable banking regulations.

"Person" means any natural person, firm, corporation, limited liability company, partnership, joint venture, association, joint-stock company, trust, unincorporated organization, Governmental Entity or any other legal entity, including public bodies, whether acting in an individual, fiduciary or other capacity.

"Plan Assets" means assets of any (i) employee benefit plan (as defined in Section 3(3) of ERISA) subject to the fiduciary responsibility provisions of Title I of ERISA, (ii) plan (as defined in Section 4975(e)(1) of the Code) subject to Section 4975 of the Code or (iii) entity whose underlying assets include assets of any such employee benefit plan or plan by reason of the investment by an employee benefit plan or plan in such entity.

"Pledge Agreement" means the Pledge and Security Agreement, dated as of the Closing Date, between the Company and Lender, in the form of Exhibit G hereto, pursuant to which the Capital Stock of Borrower is pledged to Lender.

"Prepayment Event Date" means the date of occurrence of a Prepayment Trigger.

"Prepayment Trigger" means the occurrence of both (i) an Event of Default and (ii) (unless prohibited by operation of Law) the acceleration of the maturity of the Loan.

"Prime Rate" means, with respect to any Calendar Quarter, the fluctuating rate per annum equal to the highest rate published in the "Money Rates" section of The Wall Street Journal as the "prime rate" then in effect (or, if such source is not available for any reason, such alternative source as determined by Lender) on the [****] Business Day of such Calendar Quarter.

"Principal Amount" means, as of any date of determination, and without duplication, the amount equal to the sum of: (i) the original amount of the Initial Tranche Loan Commitment, plus, (ii) the original amount of the Subsequent Milestone Tranche Loan, if any, plus, (iii) the original amount of the Subsequent Additional Tranche Loan, if any, plus, (iv) any Accreted Principal accrued as of such date, minus, (v) any payment in respect of principal as provided for in Section 3.01, 3.02, 4.03 or 4.04 or otherwise.

"Proceeding" means an action or proceeding brought against a Party as a defendant, for purposes of all legal proceedings arising out of or relating to this Agreement or the transactions contemplated hereby.

“Proceeds” means any amounts actually received by Borrower from a Person (other than Lender) as a result of any settlement or resolution of any actions, suits, proceedings, claims or disputes related to, and to the extent involving, the Included Royalty Interest, except for any such amounts (a) that are required to be paid to a Licensee under a Covered License Agreement or (b) that are otherwise used to reimburse or indemnify a Licensee for costs, expenses, legal fees or other fees relating to such actions, suits, proceedings, claims or disputes.

“Product-Specific Patents” has the meaning set forth in Section 8.12(a).

“Prosecute” means preparing, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences, and defending against any claims of invalidity or unenforceability; and “Prosecution” shall have the correlative meaning.

“Quarterly Interest Shortfall” has the meaning set forth in Section 3.01(c).

“Register” means a record of ownership in which Borrower registers by book entry the interests (including any rights to receive payment hereunder) of Lender in the Loans and any assignment of any such interest, obligation or right as described in Section 5.05.

“Regulatory Agency” means a Governmental Entity with responsibility for the regulation of the research, development, marketing or sale of drugs or pharmaceuticals in any jurisdiction, including the FDA and the European Medicines Agency.

“Representative” means, collectively, with respect to any Person, the trustees, directors, board members, members, partners, managers, officers, employees, agents, advisors or other representatives (including attorneys, accountants, consultants, scientists and financial advisors) of such Person.

“RGX-111” means the Company’s recombinant AAV9 capsid containing a human α -L-iduronidate (hIDUA) expression cassette for the treatment of MPS I.

“RGX-121” means the Company’s recombinant AAV9 capsid containing a human iduronate-2-sulfate (hIDS) expression cassette for the treatment of MPS II.

“Royalty Deductions” means in respect of any Royalty Interest, and subject to Schedule 1.01 (including the footnotes thereto), (a) any adjustments, modifications, offsets, credits, reductions or deductions to the Royalty Interest made pursuant to any Covered License Agreement, (b) any royalties, milestone and sublicense fees payable by Borrower in respect to royalty, milestone and sublicense fee payments to GSK in accordance with Sections 3.3 and 3.4 of the GSK Agreement, (c) to the extent not offset on account of amounts paid in respect of clause (b) or otherwise, any royalties payable by the Company in respect to royalty payments to Penn in accordance with Section 3.2(c) of the Penn Agreement, (d) any royalties, milestone and sublicense fees payable by Borrower in respect to royalty, milestone and sublicense fee payments to Emory in accordance with Sections 3.2, 3.3 and 3.4 of the Emory Agreement, and (e) any royalties, milestone and sublicense fees payable by Borrower in respect to royalty, milestone and sublicense

fee payments to UoM in accordance with Sections 11.3 and 11.4 of the UoM Agreement and Section 6.4 of Exhibit A of the UoM Agreement.

“Royalty Interest” means the royalties, milestones and sublicense fees (together with the right to receive such royalties, milestones and sublicense fees) payable to Borrower (including as assignee of the Company’s rights under the Existing License Agreements pursuant to the Contribution Agreement) under (a) Sections 3.3, 3.4 and 3.5 of the Novartis License Agreement (b) Sections 1(iv) and 1(v) of the First Novartis Amendment, (c) Sections 8.2.1 (solely to the extent such milestone payment is attributed to the 121 Licensed Products (as defined in the Nippon Shinyaku License Agreement) and up to an amount [****]), 8.2.2 and 8.3.1 of the Nippon Shinyaku License Agreement, (d) Sections 3.2, 3.3, 3.4, 3.5 and 3.6 of the Spacecraft License Agreement, (e) Sections 3.2 and 3.3 of the Ultragenyx License Agreement, (f) Sections 3.1, 3.2, 3.3, 3.4 and 3.5 and Section 4.2 of the Ultragenyx Option and License Agreement, and (g) the similar applicable section(s) of any Future License Agreement(s) (including in each case payments constituting royalties, milestone payments, sublicense fees, settlement payments, judgments, securities, consideration or any other remuneration of any kind payable or received in respect of, or in substitution or compensation for, or otherwise in lieu of, such royalties, milestone or sublicense fee payments under each of the Covered License Agreements and all “accounts” (as such term is defined in the UCC) in respect of the Royalty Interest evidencing or giving rise to any of the foregoing) relating to Exploitation of the Licensed Product as provided in each of the Covered License Agreement, and any collections, recoveries, payments or other compensation made in lieu thereof and any amounts paid or payable to Borrower and/or any of its Subsidiaries in respect of such royalties, milestone or sublicense fee payments pursuant to Section 365(n) of the United States Bankruptcy Code derived from payments under each of the Covered License Agreement since the Closing Date, in each case after giving effect to all Royalty Deductions applicable thereto. For the avoidance of doubt, “Royalty Interest” shall exclude (i) Excluded Payments, and (ii) any amounts payable by the Company, Borrower, or Novartis to the RPA Purchasers pursuant to the Royalty Purchase Agreement until all Obligations (as such term is defined in the Royalty Purchase Agreement) have been satisfied. An illustrative calculation of the Royalty Interest and Royalty Deductions under each of the Existing License Agreements is attached as Schedule 1.01.

“Royalty Purchase Agreement” means that certain Royalty Purchase Agreement, dated as of December 22, 2020, by and among the Company, the RPA Purchaser Rep and the RPA Purchasers.

“Royalty Reports” means, with respect to the relevant Calendar Quarter of Borrower, the quarterly reports provided for under (a) Section 3.6.1 of the Novartis License Agreement, (b) Section 8.3.6 of the Nippon Shinyaku License Agreement, (c) Section 3.7.1 of the Spacecraft License Agreement, (d) Section 3.5.1 of the Ultragenyx License Agreement, (e) Section 3.7.1 of the Ultragenyx Option and License Agreement and (f) the applicable section(s) of any Future License Agreement(s) for the period thereunder corresponding to such quarter, together with relevant supporting documentation.

“RPA Purchaser Rep” means HCR Zolgensma SPV, LLC, as assignee of HCR Collateral Management, LLC.

“RPA Purchasers” means the “Purchasers” as such term is defined in the Royalty Purchase Agreement.

“RPA Transaction Documents” means the Royalty Purchase Agreement and the “Transaction Documents” as such term is defined in the Royalty Purchase Agreement.

“Scheduled Maturity Date” means the tenth anniversary of the Closing Date; provided, however, that if U.S. Patent Number [****] receives a term extension for at least [****] additional years of patent life, “Scheduled Maturity Date” shall mean the 12th anniversary of the Closing Date.

“SEC” means the United States Securities and Exchange Commission.

“Security Agreement” means the Security Agreement, substantially in the form of Exhibit H hereto, between Lender and Borrower, securing the Obligations of Borrower hereunder and the other Loan Documents, as supplemented by any amendments or supplements thereto.

“Senior Officer” means (i) in the case of Borrower, the Chief Executive Officer, Chief Financial Officer or Secretary and (ii) in the case of the Company, the Chief Executive Officer, Chief Financial Officer or Chief Legal Officer.

“SOFR” means the Secured Overnight Financing Rate as administered by the Federal Reserve Bank of New York (or a successor administrator).

“SOFR Unavailability Event” has the meaning set forth in Section 4.05.

“Spacecraft” means Spacecraft Seven, LLC, a Delaware limited liability company, as assignee of Rocket Pharmaceuticals, Ltd., a Cayman Islands exempted company.

“Spacecraft License Agreement” means the License Agreement, dated as of [****], by and between the Company and Spacecraft, and as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“Specified Covered License Agreements” means the (a) Novartis License Agreement and (b) Nippon Shinyaku License Agreement.

“Subsequent Additional Funding Date” means the date upon which the conditions precedent under Section 6.03 have been satisfied, which (subject to such satisfaction) shall be the date that is within [****] Business Days following receipt by Lender of the Notice of Subsequent Additional Tranche Borrowing but which shall not be a date later than the Subsequent Additional Tranche Loan Availability Termination Date without the consent of Lender in its sole discretion.

“Subsequent Additional Tranche Loan” means, prior to its disbursement, the loan that may be made by Lender to Borrower in accordance with Section 2.01(c), and from and after its disbursement, at any time the aggregate principal amount loaned to Borrower on the Subsequent Additional Funding Date pursuant to Section 2.01(c), plus any Accreted Principal thereunder outstanding at such time.

“Subsequent Additional Tranche Loan Availability Termination Date” means [****]; provided that if such date is not a Business Day, the “Subsequent Additional Tranche Loan Availability Termination Date” will be the preceding Business Day.

“Subsequent Additional Tranche Loan Commitment” means \$50,000,000.

“Subsequent Additional Tranche Note” means the note, in the form attached hereto as Exhibit D-3, issued by Borrower to Lender evidencing the Subsequent Additional Tranche Loan, if made, on the Subsequent Additional Funding Date to Borrower and any replacement(s) thereof issued in accordance with Section 12.09.

“Subsequent Milestone Funding Date” means the date upon which the conditions precedent under Section 6.02 have been satisfied, which (subject to such satisfaction) shall be the date that is within [****] Business Days following receipt by Lender of the Notice of Subsequent Milestone Tranche Borrowing but which shall not be a date later than the Subsequent Milestone Tranche Loan Availability Termination Date without the consent of Lender in its sole discretion.

“Subsequent Milestone Tranche Loan” means, prior to its disbursement, the loan that may be made by Lender to Borrower in accordance with Section 2.01(b), and from and after its disbursement, at any time the aggregate principal amount loaned to Borrower on the Subsequent Milestone Funding Date pursuant to Section 2.01(b), plus any Accreted Principal thereunder outstanding at such time.

“Subsequent Milestone Tranche Loan Availability Termination Date” means April 30, 2027; provided that if such date is not a Business Day, the “Subsequent Milestone Tranche Loan Availability Termination Date” will be the preceding Business Day.

“Subsequent Milestone Tranche Loan Commitment” means \$50,000,000.

“Subsequent Milestone Tranche Note” means the note, in the form attached hereto as Exhibit D-2, issued by Borrower to Lender evidencing the Subsequent Milestone Tranche Loan, if made, on the Subsequent Milestone Funding Date to Borrower and any replacement(s) thereof issued in accordance with Section 12.09.

“Subsidiary” means, with respect to any Person, at any time, any entity of which more than fifty percent (50%) of the outstanding voting stock or other equity interest entitled ordinarily to vote in the election of the directors or other governing body (however designated) is at the time beneficially owned or controlled directly or indirectly by such Person, by one or more such entities or by such Person and one or more such entities.

“Surviving Person” means, with respect to any Person involved in or that makes any disposition, the Person formed by or surviving such disposition or the Person to which such disposition is made.

“Taxes” means all present and future taxes, levies, imposts, duties, deductions, withholdings (including backup withholding), assessments or similar fees or other charges

imposed by any Governmental Entity, including any related interest, additions to tax or penalties applicable thereto.

“Term SOFR Screen Rate” means the forward-looking SOFR term rate administered by CME (or any successor administrator reasonably satisfactory to Lender) and published on the applicable Reuters screen page (or such other commercially available source providing such quotations as may be reasonably designated by Lender from time to time).

“Territory” means [***].

“Third Party” means any Person other than Borrower or its Affiliates.

“Three-Month Term SOFR” means, with respect to any Calendar Quarter, a rate per annum equal to the greater of (a) four-and-one-quarter percent per annum and (b) the [***] U.S. Government Securities Business Days prior to the first day of such Calendar Quarter; provided that, if the rate is not published prior to 11:00 a.m. on such determination date then “Three-Month Term SOFR” for purposes of this clause (b) means the three month Term SOFR Screen Rate on the [***] U.S. Government Securities Business Day immediately prior thereto.

“Transaction Documents” means the Loan Documents and the Organizational Documents.

“Transferred Assets” has the meaning set forth in the Contribution Agreement.

“U.S.” means the United States of America.

“U.S. Government Securities Business Day” means any Business Day, except any Business Day on which any of the Securities Industry and Financial Markets Association, the Nasdaq Stock Market or the Federal Reserve Bank of New York is not open for business because such day is a legal holiday under the federal laws of the U.S. or the laws of the State of New York, as applicable.

“U.S. Person” means any Person that is a “United States Person” as defined in Section 7701(a)(30) of the Code.

“U.S. Tax Compliance Certificate” has the meaning specified in Section 5.01(c)(ii).

“UCC” means the Uniform Commercial Code as in effect from time to time in New York; provided that, if, with respect to any financing statement or by reason of any provisions of Applicable Law, the perfection or the effect of perfection or non-perfection of the security interest or any portion thereof granted pursuant to the Loan Documents is governed by the Uniform Commercial Code as in effect in a jurisdiction of the U.S. other than New York, then “UCC” means the Uniform Commercial Code as in effect from time to time in such other jurisdiction for purposes of the provisions of this Agreement and any financing statement relating to such perfection or effect of perfection or non-perfection.

“Ultragenyx” means Ultragenyx Pharmaceutical Inc. (successor to Dimension Therapeutics, Inc.), a Delaware corporation.

“Ultragenyx License Agreement” means the License Agreement, dated as of [****], by and between the Company and Ultragenyx, as amended by that certain First Amendment, dated as of [****], and as amended by that certain Second Amendment, dated as of [****], and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“Ultragenyx Option and License Agreement” means the Option and License Agreement, dated as of [****], by and between the Company and Ultragenyx, as amended on [****], and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“UoM” means the Regents of the University of Minnesota, a Minnesota constitutional corporation.

“UoM Agreement” means the Exclusive Patent License Agreement, dated as of [****], as amended on [****], by and between the Company and UoM, and as may be further amended, amended and restated, supplemented or otherwise modified from time to time.

“UoM Notice” has the meaning set forth in Section 8.24.

“UoM Patents Intercompany Out-License Agreement” means the Minnesota Patents License Agreement, dated as of the date hereof, by and between Borrower, as licensor, and the Company, as licensee, as may be amended, amended and restated, supplemented or otherwise modified from time to time.

“Warrant” means a Warrant, in the form attached as Exhibit I.

“Zolgensma IT” means Zolgensma (onasemnogene abeparvovec-xioi) used to treat patients with spinal muscular atrophy through intrathecal delivery.

“Zolgensma IT Milestone Event” means that cumulative royalty-bearing sales of Zolgensma IT pursuant to the Novartis Agreement during any [****] consecutive Calendar Quarters ending on or prior to [****] shall have exceeded [****].

Section 1.02 Certain Interpretations. Except where expressly stated otherwise in this Agreement, the following rules of interpretation apply to this Agreement:

- (a) An accounting term not otherwise defined has the meaning assigned to it in accordance with GAAP.
- (b) Words of the masculine, feminine or neuter gender shall mean and include the correlative words of other genders.

(c) The definitions of terms shall apply equally to the singular and plural forms of the terms defined.

(d) “include,” “includes,” and “including” shall be deemed to be followed by the words “without limitation;”

(e) Unless otherwise specified, references to an agreement or other document include references to such agreement or document as from time to time amended, restated, reformed, supplemented or otherwise modified in accordance with the terms thereof (subject to any restrictions on such amendments, restatements, reformations, supplements or modifications set forth herein or in any of the other Transaction Documents) and include any annexes, exhibits and schedules attached thereto.

(f) References to any Applicable Law shall include such Applicable Law as from time to time in effect, including any amendment, modification, codification, replacement or reenactment thereof or any substitution therefor.

(g) References to a Person shall be construed to include such Person’s successors and permitted assigns (subject to any restrictions on assignment, transfer or delegation set forth herein or in any of the other Transaction Documents), and any reference to a Person in a particular capacity excludes such Person in other capacities.

(h) The word “will” shall be construed to have the same meaning and effect as the word “shall”.

(i) The words “hereof,” “herein,” “hereunder” and similar terms when used in this Agreement shall refer to this Agreement as a whole and not to any particular provision hereof, and Article, Section and Exhibit references herein are references to Articles and Sections of, and Exhibits to, this Agreement unless otherwise specified.

(j) In the computation of a period of time from a specified date to a later specified date, the word “from” means “from and including” and each of the words “to” and “until” means “to but excluding”.

(k) Where any payment is to be made, any funds are to be applied or any calculation is to be made under this Agreement on a day that is not a Business Day, unless this Agreement otherwise provides, such payment shall be made, such funds shall be applied and such calculation shall be made on the succeeding Business Day, and payments shall be adjusted accordingly.

Article II.
THE LOAN; DISBURSEMENT; CERTAIN FEES

Section 2.01 Initial Tranche Loan; Subsequent Milestone Tranche Loan; Subsequent Additional Tranche Loan.

(a) On the terms and subject to the conditions set forth herein, including the conditions set forth in Section 6.01 hereof, on the Closing Date, Lender shall make a loan hereunder to Borrower, and Borrower shall accept and borrow such loan from Lender, in a principal amount equal to the Initial Tranche Loan Commitment.

(b) On the terms and subject to the conditions set forth herein, including the conditions set forth in Section 6.02 hereof, in the event that Borrower delivers a Notice of Subsequent Milestone Tranche Borrowing prior to the Subsequent Milestone Tranche Loan Availability Termination Date, then on the Subsequent Milestone Funding Date, Lender shall make a loan hereunder to Borrower, and Borrower shall accept and borrow such loan from Lender, in a principal amount equal to the Subsequent Milestone Tranche Loan Commitment.

(c) On the terms and subject to the conditions set forth herein, including the conditions set forth in Section 6.03 hereof, in the event that Borrower delivers a Notice of Subsequent Additional Tranche Borrowing prior to the Subsequent Additional Tranche Loan Availability Termination Date, then on the Subsequent Additional Funding Date, Lender shall make a loan hereunder to Borrower, and Borrower shall accept and borrow such loan from Lender, in a principal amount equal to the Subsequent Additional Tranche Loan Commitment.

Section 2.02 Notices of Subsequent Tranche Borrowing.

(a) Notice of Subsequent Milestone Tranche Borrowing. Subject to Section 2.01(b), Borrower shall, not later than 5:00 p.m. (New York time) on or before the date that is [****] Business Days prior to the Subsequent Milestone Tranche Loan Availability Termination Date, and less than [****] Business Days prior to the proposed Subsequent Milestone Funding Date, deliver to Lender a Notice of Subsequent Milestone Tranche Borrowing, which shall (i) attach a Royalty Report demonstrating that the Zolgensma IT Milestone Event has been achieved, and (ii) state that Borrower requests to borrow the Subsequent Milestone Tranche Loan Commitment on the Subsequent Milestone Funding Date. Only one Notice of Subsequent Milestone Tranche Borrowing may be given by Borrower. Lender shall, no later than [****] days following its receipt of the Notice of Subsequent Milestone Tranche Borrowing, notify Borrower in writing whether or not it will, subject to the satisfaction of the conditions set forth in Section 6.02 hereof, make the Subsequent Milestone Tranche Loan to Borrower. The availability of the Subsequent Milestone Tranche Loan shall automatically terminate upon the earlier of (A) funding of the Subsequent Milestone Tranche Loan on the Subsequent Funding Date, and (B) the occurrence of the Subsequent Milestone Tranche Loan Availability Termination Date without Lender having received reasonable evidence that the Zolgensma IT Milestone Event was achieved.

(b) Notice of Subsequent Additional Tranche Borrowing. Subject to Section 2.01(c), Borrower shall, not later than 5:00 p.m. (New York time) on or before the date that is

[****] Business Days prior to the Subsequent Additional Tranche Loan Availability Termination Date, and less than [****] Business Days prior to the proposed Subsequent Additional Funding Date, deliver to Lender a Notice of Subsequent Additional Tranche Borrowing, which shall state that Borrower requests to borrow the Subsequent Additional Tranche Loan Commitment on the Subsequent Additional Funding Date. Only one Notice of Subsequent Additional Tranche Borrowing may be given by Borrower. Lender shall, no later than [****] days following its receipt of the Notice of Subsequent Additional Tranche Borrowing, notify Borrower in writing whether or not it will, subject to the satisfaction of the conditions set forth in Section 6.03 hereof, make the Subsequent Additional Tranche Loan to Borrower. The availability of the Subsequent Additional Tranche Loan shall automatically terminate upon the earlier of (i) funding of the Subsequent Additional Tranche Loan on the Subsequent Additional Funding Date, and (ii) the occurrence of the Subsequent Additional Tranche Loan Availability Termination Date without Lender having received a Notice of Subsequent Additional Tranche Borrowing.

Section 2.03 Disbursement and Borrowing. On the terms and subject to the conditions set forth herein:

(a) on the Closing Date, Lender shall wire transfer to the account of Borrower that Borrower has designated for such purpose or to Borrower's order an amount equal to (i) the Initial Tranche Loan Commitment less (ii) each of (A) \$1,500,000, which shall be retained by Lender to pay the Lender Expense Amount as of the Closing Date and (B) \$3,375,000, which shall be retained by Lender as an original issue discount (i.e., the Loan will be funded on a net basis); and

(b) on the Subsequent Milestone Funding Date, if any, Lender shall wire transfer to the account of Borrower that Borrower has designated for such purpose or to Borrower's order an amount equal to (i) the Subsequent Milestone Tranche Loan Commitment less (ii) each of (A) the Lender Expense Amount (as of such date and to the extent not previously paid) and (B) [****], which shall be retained by Lender as an original issue discount (i.e., the Loan will be funded on a net basis).

(c) on the Subsequent Additional Funding Date, if any, Lender shall wire transfer to the account of Borrower that Borrower has designated for such purpose or to Borrower's order an amount equal to (i) the Subsequent Additional Tranche Loan Commitment less (ii) each of (A) the Lender Expense Amount (as of such date and to the extent not previously paid) and (B) [****], which shall be retained by Lender as an original issue discount (i.e., the Loan will be funded on a net basis).

Section 2.04 Loan Not Revolving. The Loan is not revolving in nature, and any amount of the Loan repaid or prepaid may not be reborrowed.

**Article III.
REPAYMENT**

Section 3.01 Amortization; Maturity Date.

(a) If not earlier repaid in full, the unpaid balance of the outstanding Principal Amount of the Loan, together with any accrued and unpaid interest, the Final Payment Premium, and all other Obligations then outstanding, shall be due and payable in cash to the Lender Account on the Scheduled Maturity Date.

(b) The outstanding principal balance of the Loan and any interest or premium due with respect thereto shall be repayable solely from Included Royalty Interest except (i) in connection with prepayments required pursuant to Section 3.02, and (ii) if not earlier repaid in full, in repayment of the unpaid balance of the outstanding Principal Amount of the Loan, together with any accrued and unpaid interest, the Final Payment Premium, and all other Obligations then outstanding on the Scheduled Maturity Date.

(c) If the amount of the Included Royalty Interest for any Interest Payment Date is insufficient to pay all amounts of interest due on the Loan for such period (the amount of such shortfall, the "Quarterly Interest Shortfall"), then any such Quarterly Interest Shortfall shall increase the outstanding Principal Amount of the Loan by an amount equal to the Quarterly Interest Shortfall for the applicable Interest Payment Date (rounded up to the nearest whole dollar) and Lender shall be deemed to have made an additional term loan in a Principal Amount equal to the aggregate amount of such Quarterly Interest Shortfall (such additional term loan, "Accreted Principal"). Accreted Principal (and any adjustments to outstanding Principal Amount under Article V) shall be deemed to be part of the Loan made to Borrower for all purposes under this Agreement, and the Loan shall bear interest on such increased Principal Amount from and after the applicable Interest Payment Date in accordance with Section 4.01. In the event of any repayment or prepayment of the Loan (including, without limitation, principal payments due under Section 4.03(b)), accrued and unpaid Fixed Interest on the Principal Amount repaid or prepaid shall be payable on the date of such repayment or prepayment.

Section 3.02 Mandatory Prepayment.

(a) If any Event of Default has occurred and is continuing, then Lender may declare the outstanding Principal Amount of the Loan as of the date of the Prepayment Event Date plus any accrued and unpaid interest thereon to be immediately due and payable hereunder, in whole but not in part, to the extent permitted by law, together, if applicable, with the (A) Final Payment Premium, (B) any additional amounts due in respect thereof pursuant to Section 3.02(b), and (C) all other Obligations then outstanding together with all other amounts in respect thereof to the Lender Account, and the provisions of this Section 3.02 shall apply.

(b) In connection with the prepayment in full of the Loan outstanding, any unpaid amounts in respect of such prepaid Loan not consisting of principal or Fixed Interest (i.e., any unpaid amounts for indemnification, default interest, expense reimbursement and other amounts not consisting of principal or interest) shall be immediately due and payable.

(c) The date of prepayment of the Loan and any other amounts due to Lender under this Section 3.02, shall be [****] Business Day not more than [****] Business Days following the date the Prepayment Trigger has occurred. Not less than [****] Business Days prior to such prepayment date, Borrower shall provide to Lender a Notice of Prepayment showing the calculation of the amount to be prepaid and all other amounts payable in connection therewith under this Section 3.02. Such Notice of Prepayment shall constitute Borrower's irrevocable commitment to prepay the Loan outstanding and all such other amounts on such prepayment date.

Section 3.03 Increased Cost.

(a) Increased Costs Generally. If any Change in Law shall:

(i) impose, modify or deem applicable any reserve, special deposit, compulsory loan, insurance charge or similar requirement against assets of, deposits with or for the account of, or credit extended or participated in by, Lender;

(ii) subject any Lender to any Taxes (other than (A) Covered Taxes and Other Taxes and (B) Excluded Taxes) with respect to its loans, commitments, or other obligations, or its deposits, reserves, other liabilities or capital attributable thereto; or

(iii) impose on Lender or the applicable interbank market any other condition, cost or expense (other than Taxes) affecting this Agreement,

and the result of any of the foregoing shall be to increase the cost to Lender of making or maintaining the Loan (or of maintaining its obligation to make the Loan), then, upon written demand of Lender, the Company will pay to Lender such additional amount or amounts as will compensate Lender for such additional costs incurred or reduction suffered.

(b) Capital Requirements. If Lender determines that any Change in Law affecting Lender or the Office of Lender or Lender's holding company, if any, regarding capital or liquidity requirements has or would have the effect of reducing the rate of return on Lender's capital or on the capital of Lender's holding company, if any, as a consequence of this Agreement, the Loan made by Lender to a level below that which Lender or Lender's holding company could have achieved but for such Change in Law (taking into consideration Lender's policies and the policies of Lender's holding company with respect to capital adequacy), then from time to time Borrower will pay to Lender, as the case may be, such additional amount or amounts as will compensate Lender or Lender's holding company for any such reduction suffered.

(c) Certificates for Reimbursement. A certificate of Lender setting forth the amount or amounts necessary to compensate Lender or its holding company, as the case may be, as specified in clause (a) or (b) of this Section 3.03 and delivered to the Company shall be

conclusive absent manifest error. The Company shall pay Lender the amount shown as due on any such certificate within [****] days after receipt thereof.

(d) Delay in Requests. Failure or delay on the part of Lender to demand compensation pursuant to the foregoing provisions of this Section 3.03 shall not constitute a waiver of Lender's right to demand such compensation; provided that the Company shall not be required to compensate Lender pursuant to the foregoing provisions of this Section 3.03 for any increased costs incurred or reductions suffered more than nine months prior to the date that Lender notifies the Company of the Change in Law giving rise to such increased costs or reductions and of Lender's intention to claim compensation therefor (except that, if the Change in Law giving rise to such increased costs or reductions is retroactive, then the nine-month period referred to above shall be extended to include the period of retroactive effect thereof).

Section 3.04 Illegality. If Lender determines that any Law has made it unlawful, or that any Governmental Entity has asserted that it is unlawful, for Lender or its Office to perform any of its obligations hereunder or to make, maintain or fund or charge interest with respect to the Loan, or any Governmental Entity has imposed material restrictions on the authority of Lender to purchase or sell, or to take deposits of, Dollars in the applicable interbank market, then, on notice thereof by Lender to Borrower, any obligation of Lender to issue, make, maintain, fund or charge interest with respect to the Loan or to make the Loans shall be suspended until Lender notifies Borrower that the circumstances giving rise to such determination no longer exist. Upon receipt of such notice, Borrower shall, upon demand from Lender, prepay the Loan immediately.

Article IV.
INTEREST; EXPENSES; MAKING OF PAYMENTS

Section 4.01 Interest Rate.

(a) Interest Rate. The outstanding Principal Amount of the Loan shall bear interest consisting of the Fixed Interest, which shall be paid in cash as provided in this Section 4.01. All interest hereunder in respect of Fixed Interest shall be computed on the basis of a 360 day year of twelve 30-day months.

(b) Interest Generally. Interest on the Loan shall be due and payable in arrears on each Interest Payment Date. Subject to Section 3.02 above, all interest shall be due and payable solely from the Included Royalty Interest, except (i) following the occurrence of a Prepayment Trigger or (ii) in connection with repayment of all Obligations in connection with the Loan Agreement on the Scheduled Maturity Date. Interest hereunder shall be due and payable in accordance with the terms hereof before and after judgment, and before and after the commencement of any proceeding under any Debtor Relief Law.

Section 4.02 Collection Account.

(a) Establishment of Collection Account. No later than [****] days following the Closing Date, (i) Borrower and Lender shall negotiate in good faith with the RPA Purchaser Rep and the Account Bank to amend the Base Escrow Agreement to add Lender as a Principal (as

defined in the Base Escrow Agreement) and provide for the receipt and disbursement of all amounts paid to the Escrow Account (including the Royalty Interest, any Royalty Deductions and any Excluded Payments) under the applicable Covered License Agreement (including, as applicable, all payments that are due to an RPA Purchaser from the Novartis License Agreement, until all Obligations (as defined in the Royalty Purchase Agreement) have been satisfied) in accordance with the principles set forth on Schedule 4.02(a).

(b) Fees and Expenses. All fees, expenses and charges of the Account Bank shall be borne and paid pursuant to the terms of the Escrow Agreement.

(c) Replacement Collection Account. Prior to the Payment in Full, Borrower shall have no right to terminate the Collection Account without Lender's prior written consent; provided that, without Lender's consent to the change of location of such accounts (provided such location is in the U.S.), Borrower shall have the right from time to time to establish a replacement Collection Account with a replacement Account Bank; provided further that such replacement Account Bank entered into an Escrow Agreement with respect to such replacement accounts effective no later than the date of replacement, and Borrower instructs each Licensee to make payments to such new accounts.

For purposes of this Agreement, any reference to the "Escrow Agreement," or "Collection Account" shall refer to such replacement Escrow Agreement, Collection Account or Account Bank, as the context requires.

(d) Licensee Instruction Letter. No later than [****] days following the Closing Date (and in any case promptly following the satisfaction of the Parties' obligations under Section 4.02(a)), Borrower shall deliver a written notice to each Licensee specifying the assignment, or sublicense, as applicable, of the applicable Existing License Agreement to Borrower and instructions for payment thereafter with respect to all payments that are due and payable to Borrower in respect of or derived from such Existing License Agreement (which notice and instructions shall be in the form attached to the Contribution Agreement or otherwise reasonably satisfactory to Lender) and shall provide that Licensee is to remit any Royalty Interest and other payments (without giving effect to any Royalty Deductions) to the Collection Account (each such notice and instruction, a "Licensee Instruction Letter").

(e) Payments to Lender. If Borrower shall, notwithstanding the provisions of the applicable Covered License Agreement, Escrow Agreement or Licensee Instruction Letter, receive from a Licensee, the Account Bank or any other Person any Included Royalty Interest, Borrower shall promptly (and in any event no later than [****] Business Days) following the date on which Borrower has knowledge becomes aware of the receipt by Borrower or the Company of such Included Royalty Interest, remit to Lender such Included Royalty Interest.

(f) Payments to Borrower. If Lender shall, notwithstanding the provisions of the applicable Covered License Agreement, Escrow Agreement or Licensee Instruction Letter, receive from a Licensee, the Account Bank or any other Person (i) any payment that does not consist entirely of Included Royalty Interest, Lender shall promptly (and in any event no later than [****] Business Days) following the date any amount previously received by Lender is so

identified or Lender otherwise becomes aware of its receipt thereof, remit to Borrower such payment, or portion thereof, that does not constitute Included Royalty Interest.

(g) Offsets by a Licensee. If a Licensee sets off against the Included Royalty Interest any amount owing from the Company or Borrower to such Licensee in respect of any right of such Licensee against the Company or Borrower, as applicable, arising from or in connection with any matter other than the Included Royalty Interest (such amount owing from Borrower to such Licensee, the "Borrower Obligation"), then Borrower shall promptly (and in any event no later than [****] Business Days) following the date on which Borrower has Knowledge of such set-off (including the amount thereof and the nature and extent of the Borrower Obligation), pay to Lender the amount of such set-off. After Borrower makes the payment referred to in the first sentence of this Section 4.02(g), Borrower shall be entitled to, and Lender shall not be entitled to, any amounts recovered from such Licensee in respect of such set-off.

(h) Remittances. All remittances pursuant to this Section 4.02 shall be made (i) without set-off or deduction of any kind (except as required by applicable Law) and (ii) by wire transfer of immediately available funds to the account set forth in Exhibit J (if the payee is Borrower) or Exhibit K (if the payee is Lender) or to such other account as the relevant payee may designate in writing (such designation to be made at least [****] Business Days prior to any such payment).

(i) Payments Held In Trust. Each Party agrees that it shall hold any amounts received by it to which the other party hereto is entitled under Section 4.02(d) or Section 4.02(e) in trust and agrees that it shall have no right, title or interest whatsoever in such amounts.

Section 4.03 Application of Payments. On each Interest Payment Date, the Included Royalty Interest shall be applied by payment in cash to Lender, at the Lender Account in the following order of priority:

(a) Accrued and unpaid interest on the Loan for the period from and including the prior Interest Payment Date to and including the day before the current Interest Payment Date shall be payable in arrears on each Interest Payment Date.

(b) To the extent the Included Royalty Interest for the immediately preceding Calendar Quarter for such Interest Payment Date exceeds Fixed Interest accrued and payable on the Loan on such Interest Payment Date (such amount, the "Amortization Payment"), the Amortization Payment shall be applied to repay principal on the Loan outstanding at par.

Section 4.04 Interest on Late Payments. If any amount payable by Borrower to Lender hereunder is not paid when due (whether at stated maturity, by acceleration or otherwise), interest shall accrue on any such unpaid amounts, both before and after judgment during the period from and including the applicable due date, to but excluding the day the overdue amount is paid in full, at a rate per annum equal to the Default Rate. Interest accruing under this Section 4.04 shall be payable on demand of Lender. For the avoidance of doubt, Fixed Interest that is not paid in cash on the date due but that is added to the Principal Amount of the Loan as Accreted Principal in accordance with Section 3.01(c) shall accrue interest at the Fixed Interest from the date at which

it is incorporated as Accreted Principal and shall thereafter accrue interest at the Default Rate in the event that the Principal Amount of the Loan generally bears interest at the Default Rate.

Section 4.05 Inability to Determine Rate. Notwithstanding anything to the contrary in this Agreement or any other Transaction Document, if Lender determines (which determination shall be conclusive absent manifest error) that (a) adequate and reasonable means do not exist for ascertaining clause (b) of the definition of Three-Month Term SOFR, including, without limitation, because the Term SOFR Screen Rate is not available or published on a current basis and such circumstances are unlikely to be temporary, (b) the CME (or any successor administrator reasonably satisfactory to Lender) has made a public statement identifying a specific date after which SOFR shall or will no longer be made available, or permitted to be used for determining the interest rate of syndicated loans denominated in Dollars, or shall or will otherwise cease; provided that, in each case, at the time of such statement, there is no successor administrator that is reasonably satisfactory to Lender that will continue to provide SOFR, or (c) Lender determines for any reason that the Three-Month Term SOFR for the relevant Calendar Quarter does not adequately and fairly reflect the cost of funds to Lender (each a “SOFR Unavailability Event”), then (i) Lender will promptly so notify Borrower, and (ii) thereafter, (A) Lender and Borrower shall negotiate in good faith to amend this Agreement to replace Three-Month Term SOFR with an alternate benchmark rate, giving due consideration to any evolving or then-prevailing market convention, including any applicable recommendations made by the Board of Governors of the Federal Reserve System or the Federal Reserve Bank of New York, or a committee officially endorsed or convened by the Board of Governors of the Federal Reserve System or the Federal Reserve Bank of New York, or any successor thereto, for Dollar-denominated credit facilities for such alternative benchmarks, together with any proposed Conforming Changes, and (B) until such time as Borrower and Lender amend this Agreement as contemplated by the foregoing clause (A), clause (b) of the definition of Three-Month Term SOFR for the Calendar Quarter during which such SOFR Unavailability Event occurs will be a rate per annum equal to the Prime Rate as in effect on the date such SOFR Unavailability Event occurs and thereafter will be re-set on the first Business Day of each Calendar Quarter occurring thereafter.

Section 4.06 Administration and Enforcement Expenses. Borrower shall promptly reimburse Lender on demand for all reasonable costs and expenses incurred by Lender (including the reasonable fees and expenses of one outside counsel to Lender) as a consequence of or in connection with any Default, Event of Default, Prepayment Trigger or mandatory prepayment of the Loan.

Section 4.07 Making of Payments. Notwithstanding anything to the contrary contained herein, any Payment stated to be due hereunder or under any Note on a given day in a specified month shall be made or shall end (as the case may be), (i) if there is no such given day or corresponding day, on the last Business Day of such month or (ii) if such given day or corresponding day is not a Business Day, on the next succeeding Business Day.

Section 4.08 Setoff or Counterclaim. Each payment by Borrower under this Agreement or under any Note shall be made without setoff or counterclaim. Lender shall have the right to set off any and all amounts owed by Borrower and/or any of its Subsidiaries under this Agreement as provided in Section 10.03.

Article V.
TAXES

Section 5.01 Taxes.

(a) Any and all payments by or on account of any obligation of Borrower under any Loan Document shall be made without deduction or withholding for any Taxes, except as required by Applicable Law. If any applicable withholding agent is required by Applicable Law to make any withholding or deduction of Taxes in respect of any payment by or on account of any obligation of Borrower under any Loan Document, then the applicable withholding agent shall be entitled to make such withholding or deduction and shall timely pay directly to the relevant Governmental Entity the full amount required to be so withheld or deducted. If any such Tax withheld is a Covered Tax, then the outstanding Principal Amount with respect to the corresponding Loan at such time shall be increased by an amount equal to such Covered Tax.

(b) If any Covered Taxes are payable or paid by such any Lender, whether or not such Covered Taxes were correctly or legally imposed or asserted by the relevant Governmental Entity, then the outstanding Principal Amount at such time shall be increased by (i) the amount of such Covered Taxes and (ii) the amount of all reasonable expenses arising therefrom or with respect thereto. Lender shall promptly deliver to Borrower a certificate informing Borrower of any such amounts.

(c) Status of Lenders

(i) Any Lender that is eligible for an exemption from or reduction of withholding Tax with respect to any payments made under any Loan Document shall deliver to Borrower, at the time or times reasonably requested by Borrower, such properly completed and executed documentation reasonably requested by Borrower as will permit such payments to be made without withholding or at a reduced rate of withholding. In addition, any Lender, if reasonably requested by Borrower, shall deliver such other documentation prescribed by Applicable Law or reasonably requested by Borrower as will enable Borrower to determine whether or not such Lender is subject to backup withholding or information reporting requirements. Notwithstanding anything to the contrary in the preceding two sentences, the completion, execution and submission of such documentation (other than such documentation set forth in clauses (A), (B) and (D) of Section 5.01(c)(ii)) shall not be required if in Lender's reasonable judgment such completion, execution or submission would subject such Lender to any unreimbursed cost or expense or would materially prejudice the legal or commercial position of such Lender.

(ii) Without limiting the generality of the foregoing:

(A) any Lender that is a U.S. Person shall deliver to Borrower on or prior to the date on which such Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of Borrower), two duly executed copies of IRS Form W-9 certifying that such Lender is exempt from U.S. federal backup withholding tax;

(B) any Foreign Lender shall, to the extent it is legally eligible to do so, deliver to Borrower on or about the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of Borrower), whichever of the following is applicable:

(1) in the case of a Foreign Lender claiming the benefits of an income tax treaty to which the U.S. is a party, two duly executed copies of IRS Form W-8BEN or IRS Form W-8BEN-E establishing an exemption from, or reduction of, U.S. federal withholding Tax;

(2) two duly executed copies of IRS Form W-8ECI;

(3) in the case of a Foreign Lender claiming the benefits of the exemption for portfolio interest under Section 881(c) of the Code, (x) a certificate substantially in the form of Exhibit L-1 to the effect that such Foreign Lender is not a “bank” within the meaning of Section 881(c)(3)(A) of the Code, a “10 percent shareholder” of Borrower within the meaning of Section 871(h)(3)(B) of the Code, a “controlled foreign corporation” related to Borrower as described in Section 881(c)(3)(C) of the Code or effectively connected with the conduct of a trade or business in the U.S. (a “U.S. Tax Compliance Certificate”) and (y) two duly executed copies of IRS Form W-8BEN or IRS Form W-8BEN-E; or

(4) to the extent a Foreign Lender is not the beneficial owner, two duly executed copies of IRS Form W-8IMY, accompanied by IRS Form W-8ECI, IRS Form W-8BEN, IRS Form W-8BEN-E, a U.S. Tax Compliance Certificate substantially in the form of Exhibit L-2 or Exhibit L-3, IRS Form W-9, or other certification documents from each beneficial owner, as applicable; provided that if the Foreign Lender is a partnership (and not a participating Lender) and one or more direct or indirect partners of such Foreign Lender are claiming the portfolio interest exemption, such Foreign Lender may provide a U.S. Tax Compliance Certificate substantially in the form of Exhibit L-4 on behalf of such direct and/or indirect partner(s);

(C) any Foreign Lender shall, to the extent it is legally eligible to do so, deliver to Borrower on or prior to the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of Borrower), two duly executed copies of any other form prescribed by Applicable Law as a basis for claiming exemption from or a reduction in U.S. federal withholding Tax, together with such supplementary documentation as may be prescribed by Applicable Law or reasonably requested by Borrower to permit Borrower to determine the withholding or deduction required to be made; and

(D) if a payment made to a Lender under any Loan Document would be subject to U.S. federal withholding Tax imposed by FATCA if such Lender were to fail to comply with the applicable reporting requirements of FATCA (including those contained in Section 1471(b) or 1472(b) of the Code, as applicable), such Lender shall deliver to Borrower at the time or times reasonably requested by Borrower such documentation prescribed by Applicable Law (including as prescribed by Section 1471(b)(3)(C)(i) of the Code) and such additional documentation reasonably requested by Borrower as may be necessary for Borrower to comply with its obligations under FATCA and to determine whether such Lender has complied with such Lender's obligations under FATCA or to determine the amount, if any, to deduct and withhold from such payment. Solely for purposes of this clause (D), "FATCA" shall include any amendments made to FATCA after the date of this Agreement.

(iii) If any form, certification or other documentation previously delivered by any Lender expires or becomes obsolete or inaccurate in any respect, such Lender shall promptly update such form or certification or promptly notify Borrower in writing of its legal ineligibility to do so. Notwithstanding anything to the contrary, nothing in this Section 5.01(c) shall require any Lender to deliver any documentation that any such Lender is legally ineligible to provide.

Section 5.02 Receipt of Payment. Promptly after the date of any payment of Taxes by Borrower pursuant to this Article V, Borrower shall furnish to Lender the original or a certified copy of a receipt evidencing payment thereof or other evidence reasonably satisfactory to Lender.

Section 5.03 Other Taxes. Borrower shall timely pay to the relevant Governmental Entity in accordance with Applicable Law any Other Taxes.

Section 5.04 Refunds. If any Lender determines, in its sole discretion exercised in good faith, that it has received a refund of any Covered Taxes, it shall promptly notify Borrower of the amount of any such refund (including refunds of any related penalties, interest or other charges imposed by the relevant Governmental Entity and any additional interest paid by the relevant Governmental Entity) calculated net of all out-of-pocket expenses (including Taxes) (such net amount, a "Refund Amount"). If a Refund Amount is received, the outstanding Principal Amount at such time shall be reduced by any such Refund Amount. If Lender is required to repay any previously refunded amount to a Governmental Entity, any such payment along with the amount of any related interest, penalties, additions thereto and related reasonable out-of-pocket expenses will increase the outstanding Principal Amount at such time. Notwithstanding anything to the contrary in this Section 5.04, in no event will any Refund Amount reduce the outstanding Principal Amount to the extent it would place Lender in a less favorable net after-tax position than Lender would have been in if the Covered Taxes giving rise to such refund had not been deducted, withheld or otherwise imposed and added to the outstanding Principal Amount. This Section 5.04

shall not be construed to require any Lender to make available its tax returns (or any other information relating to its taxes that it deems confidential) to Borrower or any other Person.

Section 5.05 Registered Obligation.

(a) Borrower shall establish and maintain, at its address referred to in Section 12.03, (i) a Register in which Borrower agrees to register by book entry the interests (including any rights to receive payment hereunder) of Lender in the Loan, each of its obligations under this Agreement to participate in the Loan, and any assignment of any such interest, obligation or right, and (ii) accounts in the Register in accordance with its usual practice in which it shall record (1) the names and addresses of Lender(s) (and each change thereto pursuant to Sections 12.01 and 12.02), (2) the amount of the Loan described in clause (i) above, (3) the amount of any principal or interest due and payable or paid, and (4) any other payment received and its application to the Loan. The entries in the Register shall be conclusive, in the absence of manifest error, and Borrower and each Lender shall treat each person whose name is recorded in the Register as the owner of the Loans for all purposes of this Agreement, notwithstanding notice to the contrary. No error in the Register shall diminish any of Borrower's obligations to any Lender under any Loan Document.

(b) Notwithstanding anything to the contrary contained in any Loan Document or elsewhere, the Loan (including any Note evidencing such Loan) are registered obligations, the right, title and interest of Lender and its assignees in and to the Loan shall be transferable only upon notation of such transfer in the Register and no assignment thereof shall be effective until recorded therein. The parties hereto intend that the Loan will be at all times maintained in "registered form" within the meaning of Section 5f.103-1(c) of the U.S. Treasury Regulations, Sections 163(f), 871(h)(2) and 881(c)(2) of the Code and any related regulations (and any successor provisions).

Section 5.06 Tax Treatment.

(a) For U.S. federal income and applicable state and local income tax purposes, the Parties shall treat the Loan as indebtedness. Each Party agrees not to take any position that is inconsistent with the foregoing sentence on any Tax return or in any audit or other administrative or judicial proceeding or for any other tax purpose (including determination of any withholding responsibilities in respect of any amounts paid under any Loan Document), unless in each case, (i) otherwise required by a change in applicable Law after the date hereof, or (ii) a good faith resolution of a tax audit or other administrative or judicial tax proceeding.

(b) This Agreement is not intended to create a partnership, association or joint venture between or among Lender and/or Borrower or any Subsidiary. Each Party agrees not to refer to the other as a "partner" or the relationship as a "partnership" or "joint venture."

Section 5.07 Investment Units. Lender and Borrower agree that the Loan is part of an investment unit within the meaning of Section 1273(c)(2) of the Code, which also includes any Warrant issued to Lender in connection with the Loan. For all applicable U.S. federal income tax purposes, the issue price of the investment unit and the fair market value of the Warrant issued

in connection with the Loan shall be determined collectively by Borrower and Lender, acting in good faith, at the time such Loan is made to Borrower. The “issue price” of the Loan made by Lender pursuant to this Agreement (and any Note issued in connection therewith) shall equal (i) the issue price of the investment unit, minus (ii) the fair market value of the Warrant issued in connection with Lender’s Loan. Lender and Borrower agree that the allocation determined pursuant to this Section 5.07 will be used for purposes of Section 1273(c)(2) of the Code. Borrower and Lender agree to make any determinations under Treasury Regulations §1.1273-2(h)(2) consistently with the foregoing and to file all required tax returns consistently with the foregoing, as applicable, except as otherwise required by a change in Applicable Law after the date of this Agreement or a good faith resolution of a tax audit or administrative or judicial tax Proceeding.

Section 5.08 OID. The Loan is deemed to be made with original issue discount for U.S. federal income tax purposes. Requests for information regarding the issue price, amount of original issue discount, issue date and yield to maturity on the Loans shall be directed to Borrower care of Borrower’s Secretary at: 9804 Medical Center Drive, Rockville, MD 20850.

Section 5.09 Survival. Each party’s obligations under this Article V shall survive the any assignment of rights by, or the replacement of, a Lender, and the repayment, satisfaction or discharge of all obligations under any Loan Document.

Article VI. CLOSING CONDITIONS

Section 6.01 Conditions Precedent to the Initial Tranche Loan. The obligation of Lender to advance the Initial Tranche Loan on the Closing Date shall be subject to the fulfillment, to the sole satisfaction of Lender, of all of the following conditions precedent in addition to the conditions specified in Section 2.01(a) and Section 2.03(a):

(a) Borrower shall have executed and delivered to Lender the Initial Tranche Note, dated the Closing Date.

(b) Lender shall have received on or before the Closing Date an executed copy of an opinion of Covington & Burling LLP, counsel to Borrower and the Company, dated the Closing Date in form and substance reasonably satisfactory to Lender.

(c) Borrower and the Company shall each have delivered to Lender a certificate, dated the Closing Date, of a Senior Officer (the statements in which shall be true and correct on and as of the Closing Date): (i) attaching copies, certified by such officer as true and complete, of such party’s certificate of incorporation or other organizational documents (together with any and all amendments thereto); (ii) attaching copies, certified by such officer as true and complete, of resolutions of the Board of Directors (or similar governing body) of such party authorizing and approving the execution, delivery and performance by such party of the Loan Documents to which it is a party and the transactions contemplated herein and therein; (iii) setting forth the incumbency of the officer of such party who executed and delivered such Loan Documents, including therein a signature specimen of each such officer; and (iv) attaching copies,

certified by such officer as true and complete, of certificates of the appropriate Governmental Entity of the jurisdiction of formation, stating that such party was in good standing under the laws of such jurisdiction as of the Closing Date (or a date immediately prior thereto acceptable to Lender).

(d) This Agreement and the other Loan Documents shall have been executed and delivered to Lender by each party thereto (other than Lender), and Borrower shall have delivered, or caused to be delivered, such other documents as Lender reasonably requested, in each case, in form and substance satisfactory to Lender.

(e) The Transaction Documents shall be in full force and effect.

(f) No event shall have occurred and be continuing that (i) constitutes a Default or an Event of Default or a Prepayment Trigger or (ii) could reasonably be expected to constitute a Material Adverse Effect (without giving effect to the cure period applicable to a Prepayment Trigger based thereon), in each case both at the time of, and immediately after giving effect to, the making of the Loan on the Closing Date.

(g) All necessary governmental and third-party approvals, notices, consents and filings, including in connection with the Initial Tranche Loan, the Security Agreement, the Contribution Agreement and the other Loan Documents (other than the Subsequent Milestone Tranche Note and the Subsequent Additional Tranche Note) shall have been obtained or made and shall remain in full force and effect, including, without limitation, the consent required to be obtained from Emory under the Emory Agreement (the "Emory Consent").

(h) Borrower shall have delivered to Lender certified copies of UCC, United States Patent and Trademark Office and United States Copyright Office, tax and judgment lien searches, or equivalent reports or searches, each of a recent date listing all effective financing statements, lien notices or comparable documents that name Borrower as debtor and that are filed in those state and county jurisdictions in which Borrower is organized or maintains its principal place of business and such other searches that Lender deems necessary or appropriate, none of which encumber the Collateral covered or intended to be covered by the Loan Documents (other than any Permitted Liens and other Liens acceptable to Lender).

(i) Lender shall have received all UCC financing statements in appropriate form for filing under the UCC, and all other certificates, agreements, instruments, filings, recordings and other actions, including recordings in the United States Patent and Trademark Office and the United States Copyright Office that are necessary or reasonably requested by Lender in order to establish, protect, preserve and perfect the security interest in the assets of Borrower constituting Collateral as provided in the Security Agreement as a valid and perfected first priority security interest (subject to Permitted Liens and, solely with respect to the continuing first priority granted to Secured Party under the Loan Documents, Permitted Liens entitled to priority under Applicable Law) with respect to such assets shall have been duly effected (or arrangements therefor satisfactory to Lender shall have been made).

(j) Lender shall have received all documentation and other information required by bank regulatory authorities under applicable “know your customer” and anti-money laundering rules and regulations, including without limitation, the Patriot Act, including and the information described in Section 12.18.

(k) Lender shall have received such other approvals, opinions, documents or materials as Lender may reasonably request.

Section 6.02 Conditions Precedent to the Subsequent Milestone Tranche Loan. The obligation of Lender to advance the Subsequent Milestone Tranche Loan on the Subsequent Milestone Funding Date shall be subject to the fulfillment of all of the following conditions precedent in addition to the conditions specified in Section 2.01(b), Section 2.02(a) and Section 2.03(b):

(a) Borrower shall have executed and delivered to Lender a Notice of Subsequent Milestone Tranche Borrowing together with evidence of the satisfaction of the Zolgensma IT Milestone Event in accordance with Section 2.02(a).

(b) Borrower shall have executed and delivered to Lender the Subsequent Milestone Tranche Note evidencing the Subsequent Milestone Tranche Loan, dated the Subsequent Milestone Funding Date.

(c) No event shall have occurred and be continuing that constitutes a Default or an Event of Default or a Prepayment Trigger, in each case at the time of, and immediately after giving effect to, the making of the Subsequent Milestone Tranche Loan on the Subsequent Milestone Funding Date.

(d) Each Specified Covered License Agreement shall be in full force and effect, no material default in the payment of any amount in respect of the Royalty Interest under such Specified Covered License Agreement shall have occurred and no notice shall have been given or received with respect to effecting a termination or cancellation thereof.

(e) Borrower shall provide a certificate signed by a Senior Officer of Borrower certifying that the conditions in clauses (a)–(d) above have been satisfied.

Section 6.03 Conditions Precedent to the Subsequent Additional Tranche Loan. The obligation of Lender to advance the Subsequent Additional Tranche Loan on the Subsequent Additional Funding Date shall be subject to the fulfillment of all of the following conditions precedent in addition to the conditions specified in Section 2.01(c), Section 2.02(b) and Section 2.03(c):

(a) Borrower shall have executed and delivered to Lender a Notice of Subsequent Additional Tranche Borrowing in accordance with Section 2.02(b).

(b) Borrower shall have executed and delivered to Lender the Subsequent Additional Tranche Note evidencing the Subsequent Additional Tranche Loan, dated the Subsequent Additional Funding Date.

(c) No event shall have occurred and be continuing that constitutes a Default or an Event of Default or a Prepayment Trigger, in each case at the time of, and immediately after giving effect to, the making of the Subsequent Additional Tranche Loan on the Subsequent Additional Funding Date.

(d) Each Specified Covered License Agreement shall be in full force and effect, no material default in the payment of any amount in respect of the Royalty Interest shall have occurred and no notice shall have been given or received with respect to effecting a termination or cancellation thereof.

(e) Borrower shall provide a certificate signed by a Senior Officer of Borrower certifying that the conditions in clauses (a)-(d) above have been satisfied.

Article VII. REPRESENTATIONS AND WARRANTIES

Section 7.01 Borrower's Representations and Warranties. Borrower hereby represents and warrants to Lender as of the date of this Agreement (except for any representations and warranties which speak as to a specific date, which representations and warranties shall be made as of the date specified) as follows:

(a) Existence. Borrower is a limited liability company duly organized, validly existing and in good standing under the laws of the State of Delaware. Borrower has all limited liability company power, licenses, authorizations, consents and approvals required to carry on its business as now conducted and as proposed to be conducted in connection with the transactions contemplated by the Transaction Documents and the Existing License Agreements, except, in each case, as would not reasonably be expected to have resulted in (x) a Material Adverse Effect or (y) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest. Borrower is duly qualified to transact business and is in good standing in every jurisdiction in which such qualification or good standing is required by Applicable Law (except where the failure to be so qualified or in good standing would not result in, and could not reasonably be expected to have resulted in (a) a Material Adverse Effect, or (b) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest).

(b) No Conflicts. None of the execution and delivery by Borrower of any of the Loan Documents to which Borrower is party, the performance by Borrower of the obligations contemplated hereby or thereby or the consummation of the transactions contemplated hereby or thereby will: (i) contravene, conflict with, result in a breach, violation, cancellation or termination of, constitute a default (with or without notice or lapse of time, or both) under, require prepayment under, give any Person the right to exercise any remedy (including termination, cancellation or acceleration) or obtain any additional rights under, or accelerate the maturity or performance of or payment under, in any material respect, (A) any Applicable Law or any judgment, order, writ, decree, permit or license of any Governmental Entity to which Borrower or any of its Subsidiaries or any of their respective assets or properties may be subject or bound, (B) any term or provision

of any Contract (other than the Existing License Agreements or the In-License Agreements) to which Borrower or any of its Subsidiaries is a party or by which Borrower or any of its Subsidiaries or any of their respective assets or properties is bound or committed, (C) any term or provision of any of Borrower's Organizational Documents or (D) the Existing License Agreements or the In-License Agreements, except in the case of clause (A) or (B) above where any such event would not result in (1) a Material Adverse Effect, or (2) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive payments based on the Included Royalty Interest; or (ii), except as provided in or contemplated by any of the Transaction Documents, result in or require the creation or imposition of any Lien on the Patents, the Licensed Product or the Included Royalty Interest.

(c) Liens. Other than Liens granted pursuant to the Loan Documents, Borrower has not granted, nor does there exist, any Lien on the Collateral, the Patents or the Included Royalty Interest (other than Permitted Liens).

(d) Authorization. Borrower has all powers and authority to execute and deliver, and perform its obligations under, the Loan Documents to which it is party and to consummate the transactions contemplated hereby and thereby. The execution and delivery of each of the Loan Documents to which Borrower is party and the performance by Borrower of its obligations hereunder and thereunder have been duly authorized by Borrower. Each of the Loan Documents to which Borrower is party has been duly executed and delivered by Borrower. Each of the Loan Documents to which Borrower is party constitutes the legal, valid and binding obligation of Borrower, enforceable against Borrower in accordance with its respective terms, subject to applicable bankruptcy, insolvency, reorganization, moratorium or similar Applicable Laws affecting creditors' rights generally, general equitable principles and principles of public policy.

(e) Security Interests. Upon giving effect to the Contribution (and subject to the terms and conditions thereof) and the Intercompany In-License Agreements, (i) Borrower shall be the exclusive owner of the entire right, title (legal and equitable) and interest in, to and under the Transferred Assets, free and clear of all Liens, other than Permitted Liens; (ii) Borrower shall be entitled to be the sole recipient of all payments in respect of the Included Royalty Interest; and (iii) Borrower shall own (or have a license to) all assets that were previously owned (or licensed to) the Company necessary to perform its obligations under the Covered License Agreements. The Included Royalty Interest constituting Collateral granted to Lender on the Closing Date has not been pledged, sold, assigned, transferred, conveyed or granted by Borrower to any other Person, in each case, other than Permitted Liens. Upon granting by Borrower of the security interests in the Included Royalty Interest to Lender pursuant to the Security Agreement and the Pledge Agreement, and the completion of all actions necessary to perfect such security interests, Lender shall acquire a first priority security interest (subject to Permitted Liens and, solely with respect to the continuing first priority granted to Secured Party under the Loan Documents, Permitted Liens entitled to priority under Applicable Law) in the Included Royalty Interest free and clear of all Liens, other than Permitted Liens. Borrower has not caused, and to the Knowledge of Borrower no other Person has caused, the claims and rights of Lender created by any Loan Document in and to the Included Royalty Interest, to be subordinated to any creditor or any other Person.

(f) Consents. The execution and delivery by Borrower of the Loan Documents to which Borrower is party, the performance by Borrower of its obligations hereunder and thereunder and the consummation of any of the transactions contemplated hereunder and thereunder (including the granting of security interests in the Included Royalty Interest to Lender) do not require any consent, approval, license, order, authorization or declaration from, notice to, action or registration by or filing with any Governmental Entity or any other Person, except for (i) the filing of any applicable notices under securities laws, (ii) the filings necessary to perfect Liens created by the Loan Documents, (iii) those previously obtained and in full force and effect, (iv) consent, filings and registrations in connection with the Contribution as contemplated by the Contribution Agreement, (v) the Licensee Instruction Letters, (vi) the Emory Consent, (vii) the Nippon Shinyaku Notice, and (viii) the UoM Notice.

(g) Proceedings. Except as set forth on Schedule 7.01(g), to the Knowledge of Borrower there is no action, suit, arbitration proceeding, claim, citation, summons, subpoena, investigation or other proceeding (whether civil, criminal, administrative, regulatory, investigative or informal, and including by or before a Governmental Entity) pending or, to the Knowledge of Borrower, threatened in writing by or against Borrower or any of its Subsidiaries, at law or in equity, that (i) if adversely determined, would result in a Material Adverse Effect or (ii) challenges or seeks to prevent or delay the consummation of any of the transactions contemplated by any of the Loan Documents to which Borrower is party.

(h) Solvency. Upon consummation of the transactions contemplated by the Loan Documents and the application of the proceeds from the Initial Tranche Loan (a) the present fair saleable value of the properties and assets of Borrower and its Subsidiaries, taken as a consolidated group, on a going concern basis will be greater than the sum of its debts, liabilities and other obligations, including contingent liabilities, (b) the present fair saleable value of the properties and assets of Borrower on a going concern basis will not be less than the amount that would be required to pay its probable liabilities on its existing debts, liabilities and other obligations, including contingent liabilities, as they become absolute and matured, (c) Borrower will generally be able to realize upon its assets and pay its debts, liabilities and other obligations, including contingent obligations, as they become absolute and matured, (d) Borrower will not have unreasonably small capital with which to engage in its business as now conducted, (e) Borrower has not incurred, will not incur and does not have any present plans or intentions to incur debts or other obligations or liabilities beyond its ability to pay such debts or other obligations or liabilities as they become absolute and matured, (f) Borrower will not have become subject to any Insolvency Event and (g) Borrower will not have been rendered insolvent within the meaning of any Applicable Law. No step has been taken by Borrower or, to its Knowledge, any other Person to make Borrower subject to an Insolvency Event.

(i) No Default. No Default, Event of Default or Prepayment Trigger has occurred and is continuing, and no such event will occur upon the making of the Loan.

(j) Taxes. Borrower has filed (or caused to be filed) all Tax returns and reports required by Applicable Law to have been filed by it and has paid all Taxes required to be paid by it (including in its capacity as a withholding agent), except any such Taxes that are being contested

in good faith by appropriate proceedings, diligently conducted, and for which adequate reserves in accordance with GAAP.

(k) Broker's Fees. Except as set forth on Schedule 7.01(k), Borrower has not taken any action that would entitle any person or entity to any commission or broker's fee in connection with the transactions contemplated by this Agreement.

(l) Investigations. Borrower (a) has not violated and is not in violation of, and to its Knowledge, is not under investigation with respect to, and has not been threatened to be charged with or been given notice of any violation of, any Applicable Law or any judgment, order, writ, decree, injunction, stipulation, consent order, permit or license granted, issued or entered by any Governmental Entity and (b) is not subject to any judgment, order, writ, decree, injunction, stipulation, consent order, permit or license granted, issued or entered by any Governmental Entity, in each case, that would result in a Material Adverse Effect. Borrower is in compliance with the requirements of all Applicable Laws, except for any breach, violation or noncompliance thereof which would not result in a Material Adverse Effect.

(m) Intellectual Property.

(i) Patents. Schedule 7.01(m)(i) sets forth an accurate and complete list of all unexpired issued Patents and pending Patent applications owned or controlled by Borrower or the Company and, to the Knowledge of Borrower, covering the manufacture, use and sale of the Licensed Products in the applicable Territory (collectively, the "Listed Patents"). Schedule 7.01(m)(i) specifies with respect to each Listed Patent (i) the jurisdictions in which such Listed Patent is pending, allowed, granted or issued, (ii) the patent number or patent application number, as applicable, (iii) the registered owner thereof and (iv) if known by Borrower, the Licensed Product to which such Listed Patent or Listed Patent application relates.

(ii) *No Litigation*.

Neither Borrower nor the Company:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has received any written notice from any other Person, and
- (3) otherwise has any Knowledge,

that there are any pending or threatened litigations, interferences, reexaminations, oppositions or like Patent Office proceedings involving any of the Patents on Schedule 7.01(m)(i).

(iii) *Ownership of the Patents*.

Neither Borrower nor the Company:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has received any written notice from any other Person, and
- (3) otherwise has any Knowledge,

that (I) the applicable Licensor is not the sole owner of the entire right, title and interest in any of the Patents scheduled beneath such Licensor's name on Schedule 7.01(m)(i), free and clear of any encumbrances in the applicable Field (as defined in the applicable Covered License Agreement) (other than (w) any interest of the other Licensors, (x) any interest of the Company, (y) the applicable Covered License Agreement (and any encumbrances referred to therein or contemplated thereby) and (z) any encumbrances arising by operation of Law) or (II) there are any facts that would preclude the applicable Licensor from having clear title as the sole owner to any of the Listed Patents on Schedule 7.01(m)(i) in the applicable Field and Territory (other than as described in clauses (w), (x), (y) and (z) above).

(iv) *Validity and Enforceability.*

Except, for the avoidance of doubt, as set forth on Schedule 7.01(m)(iv), neither the Company nor Borrower:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has received any written notice from any other Person, and
- (3) otherwise has any Knowledge,

that any of the issued Patents on Schedule 7.01(m)(i) are unenforceable or invalid.

To the Knowledge of Borrower [***].

(v) *Inventorship.*

Neither Borrower nor the Company:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has received any written notice from any other Person, and
- (3) otherwise has any Knowledge,

that there is a Person who is or claims to be an inventor under any of the Listed Patents on Schedule 7.01(m)(i) who is not a named inventor thereof.

(vi) *No Challenges.*

Neither Borrower nor the Company:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has received any written notice from any other Person, and
- (3) otherwise has any Knowledge,

of any claim by any Person asserting that the manufacture, importation, sale, offer for sale or use of any of the Licensed Products infringes any Person's patents or other intellectual property rights. Neither Borrower nor the Company has obtained any written non-infringement, freedom to operate, clearance or invalidity opinions from outside counsel regarding the infringement or non-infringement of any Person's unexpired patent rights by any of the Licensed Products.

(vii) *No Infringement.*

Neither Borrower nor the Company:

- (1) has received any written notice from any Licensee or its Affiliates to the effect that (A) such Licensee believes or (B) any other Person has asserted,
- (2) has not received any written notice from any other Person, and
- (3) otherwise has no Knowledge,

that there is a Person who is engaging in or has engaged in any activity that infringes upon any of the Listed Patents for a competing product in the Field (as defined in the applicable Covered License Agreement) in the Territory. Except as set forth on Schedule 7.01(m)(vii), neither Borrower nor the Company has obtained any written non-infringement, freedom to operate, clearance or invalidity opinions from outside counsel regarding the Patents listed on Schedule 7.01(m)(iv) or any of the Licensed Products.

(viii) *Maintenance, etc.* Neither Borrower nor the Company has received any written notice from any Licensee or any other Person to the effect that, and neither Borrower nor the Company otherwise has any Knowledge that, such Licensee has not paid, or caused to be paid, all required maintenance fees and like payments with respect to the issued Listed Patents on Schedule 7.01(m)(i). Neither Borrower nor the Company has received any written notice from any Licensee or its Affiliates to the effect that such Licensee believes, or that any other Person has asserted, that any of the Listed Patents on Schedule 7.01(m)(i) have lapsed, expired or otherwise been terminated.

(n) Lending. Borrower is not engaged in the business of extending credit for the purpose of buying or carrying margin stock, and no portion of the Loan shall be used by Borrower for a purpose that violates Regulation T, U or X promulgated by the Board of Governors of the Federal Reserve System from time to time.

(o) Existing License Agreements.

(i) Borrower has provided to Lender the following: (i) a true, correct and complete copy of each Existing License Agreement; (ii) the Royalty Reports in respect of each Calendar Quarter ended on or prior to the date hereof that have been received by Borrower or the Company prior to the date hereof; (iii) the development progress reports that have been received by Borrower or the Company prior to the date hereof and (iv) all written notices delivered to the Licensees or their Affiliates by Borrower or the Company, or by the Licensees or their Affiliates to Borrower or the Company, pursuant to the Existing License Agreements that could reasonably be expected to have an effect on the value of the Royalty Interest in any material respect, in each case, since [****].

(ii) Each Existing License Agreement is a valid and binding obligation of Borrower and, to the Knowledge of Borrower, of the applicable Licensee, enforceable against each of Borrower and, to the Knowledge of Borrower, the applicable Licensee in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Neither Borrower nor the Company has received any written notice or, to the Knowledge of Borrower, other written notice from any Licensee or any Licensee's Affiliates challenging the validity or enforceability of the Existing License Agreements or any obligation of the Licensee to pay the Royalty Interest thereunder.

(iii) Neither Borrower nor the Company (i) has granted any material written waiver or, to the Knowledge of Borrower, any other material waiver, under any Existing License Agreement, (ii) has granted any waiver under any Existing License Agreement related to, or involving, any Royalty Interest or (iii) has released any Licensee, in whole or in part, from any of its material obligations under any Existing License Agreement, except, in each case of the immediately foregoing clauses (i), (ii) and (iii), to the extent set forth in the applicable Existing License Agreement. Except as set forth on Schedule 7.01(o)(iii), neither Borrower nor the Company has received from any Licensee or any Licensee's Affiliates any written proposal, and has not made any proposal to any Licensee or any Licensee's Affiliates, to amend or waive any provision of an Existing License Agreement.

(iv) Neither Borrower nor the Company has (i) given any Licensee or any Licensee's Affiliates any notice of termination of an Existing License Agreement pursuant to the applicable section of such Existing License Agreement (or otherwise) or (ii) received from any Licensee or any Licensee's Affiliates any written notice of termination of an Existing License Agreement. To the Knowledge of Borrower, no event has occurred that would give Borrower a right to terminate any Existing License Agreement pursuant to the applicable section of such Existing License Agreement. Neither Borrower nor the Company has received any written notice or, to Borrower's Knowledge, other written notice from any Licensee or any Licensee's Affiliates expressing any intention or desire to terminate any Existing License Agreement.

(v) Neither Borrower nor the Company has breached any provision of any Existing License Agreement in any material respect, and, to the Knowledge of Borrower, no Licensee has breached any provision of any Existing License Agreement in any material respect.

(vi) Either Borrower or the Company, as applicable, has received from each Licensee all of the Royalty Interests specified in the applicable reports delivered by any Licensee in respect of each calendar quarter ended on or prior to the date hereof that have been received by Borrower or the Company prior to the date hereof.

(vii) Except for Royalty Deductions pursuant to the applicable section of any Existing License Agreement and as illustrated on Schedule 1.01, no Royalty Interest has been and, to the Knowledge of Borrower, is, subject to any Royalty Deduction. To the Knowledge of Borrower, no event or condition exists that would permit any Licensee to claim any Royalty Deduction against payment of any Royalty Interest pursuant to clause (a) of the definition thereof, other than Royalty Deductions pursuant to the applicable section of any Existing License Agreement. Except for Royalty Deductions pursuant to the applicable section of any Existing License Agreement, neither Borrower nor the Company has received any written notice or, to its Knowledge, any other notice from any Licensee or any Licensee's Affiliates expressing an intention by such Licensee to take any Royalty Deductions or otherwise offset, credit against, reduce or deduct from the Included Royalty Interest because of any amount owed or claimed owed from Borrower or the Company to such Licensee. To the Knowledge of Borrower, except as specified in the section captioned "Anti-Stacking Calculations" in the Royalty Reports referenced in clause (a) of the definition thereof, there are no third-party patents triggering a setoff right against any Royalty Interest.

(viii) Neither Borrower nor the Company has consented to any assignment by any Licensee of, and, to the Knowledge of Borrower, no Licensee has assigned, any Existing License Agreement or any part thereof. Except as contemplated by the Transaction Documents and the RPA Transaction Documents, neither Borrower nor the Company has assigned, in whole or in part, and has not granted any liens upon or security interests with respect to, any Existing License Agreement or the Royalty Interest.

(ix) Neither Borrower nor the Company has given any notice to any Licensee or such Licensees' Affiliates regarding any claims for indemnification under the applicable sections of any Existing License Agreement.

(x) Except as set forth on Schedule 7.01(o)(x), Neither Borrower nor the Company has initiated any review or audit pursuant to the applicable section of any Existing License Agreement.

(xi) Other than the Existing License Agreements, there are no Contracts between Borrower or the Company or any of their Affiliates, on the one hand, and any Licensee or any Licensee's Affiliates, on the other hand, that (i) relate to any Licensed Product or (ii) that would reasonably be expected to result in a Material Adverse Effect.

(xii) Neither Borrower nor the Company has (i) received any written notice of any dispute from any Licensee or any Licensee's Affiliates for resolution pursuant to the applicable section of any Existing License Agreement or (ii) given any notice of any dispute to a Licensee for resolution pursuant to the applicable section of any Existing License Agreement.

(xiii) Neither Borrower nor the Company has received from any Licensee or any Licensee's Affiliates any written notice of any sub-license granted by a Licensee under any Existing License Agreement, and, to the Knowledge of Borrower, no executed sublicenses or other agreements have been entered into by any Licensee or any Licensee's Affiliates.

(p) Material Contracts.

(i) As of the Closing Date, Borrower is not a party to any Material Contract (other than, after giving effect to the Contribution thereof under the Contribution Agreement, the Material Contracts specified in Section 2.01(a) to the Contribution Agreement).

(ii) Except as separately disclosed in writing to Lender referencing this Section 7.01(p)(ii), neither Borrower nor, to Borrower's Knowledge, any Material Contract Counterparty is in breach in any material respect or default of any Material Contract and no circumstances or grounds exist that would, upon the giving of notice, the passage of time or both, give rise (A) to a claim by Borrower or any Material Contract Counterparty of a breach in any material respect or default of any Material Contract, or (B) to a right of rescission, termination, revision, setoff, or any other rights, by any Person, in, to or under any Material Contract. Borrower has not received from, or delivered to, any Material Contract Counterparty, any written or electronic notice alleging a breach in any material respect or default under any Material Contract, which breach or default has not been cured or waived as of the date hereof.

(iii) Upon the Contribution thereof to, and assumption thereof by, Borrower, each Material Contract shall be a valid and binding obligation of Borrower and, to the Knowledge of Borrower, of the applicable Material Contract Counterparty, enforceable against each of Borrower and, to the Knowledge of Borrower, each applicable Material Contract Counterparty in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar laws of general application relating to or affecting creditors' rights generally. Borrower has not received any written or electronic notice from any Material Contract Counterparty or any other Person challenging the validity or enforceability of any Material Contract. Neither Borrower, nor to the Knowledge of Borrower, any other Person, has delivered or intends to deliver any written or electronic notice to Borrower or a Material Contract Counterparty challenging the validity or enforceability of any Material Contract.

(iv) Neither Borrower nor to the Knowledge of Borrower, any Material Contract Counterparty, is contemplating to commence any case, proceeding or other action relating

to a Material Contract Counterparty's bankruptcy, insolvency, liquidation or dissolution or reorganization by any of the foregoing means.

(q) Capital Stock. No Capital Stock has been issued by Borrower other than the Capital Stock issued to the Company that is subject to the pledge to Lender under the Pledge Agreement.

(r) Office of Borrower. The chief place of business, the chief executive office and each office where Borrower keeps its records regarding the Included Royalty Interest are, as of the date hereof, each located at 9804 Medical Center Drive, Rockville, MD 20850.

(s) UCC Representations. Borrower (or any predecessor by merger or otherwise) has not, within the five-year period preceding the date hereof, had a name that differs from its name as of the date hereof.

Section 7.02 Borrower's Representations and Warranties as to the Company, Etc. Borrower hereby represents and warrants to Lender as of the date of this Agreement (except for any representations and warranties which speak as to a specific date, which representations and warranties shall be made as of the date specified), with respect to the Company and other matters, as follows:

(a) Existence. The Company is a corporation duly incorporated, validly existing and in good standing under the laws of Delaware and has all powers and authority, and all licenses, permits, franchises, authorizations, consents and approvals of all Governmental Entities, required to own its property and conduct its business as now conducted, except, in each case, as would not result in a Material Adverse Effect. The Company is duly qualified to transact business and is in good standing in every jurisdiction in which such qualification or good standing is required by Applicable Law (except where the failure to be so qualified or in good standing would not result in, and could not reasonably be expected to have resulted in (a) a Material Adverse Effect, or (b) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest).

(b) No Conflicts. None of the execution and delivery by the Company of any of the Transaction Documents to which the Company is party, the performance by the Company of the obligations contemplated hereby or thereby or the consummation of the transactions contemplated hereby or thereby will: (i) contravene, conflict with, result in a breach, violation, cancellation or termination of, constitute a default (with or without notice or lapse of time, or both) under, require prepayment under, give any Person the right to exercise any remedy (including termination, cancellation or acceleration) or obtain any additional rights under, or accelerate the maturity or performance of or payment under, in any respect, (A) any Applicable Law or any judgment, order, writ, decree, permit or license of any Governmental Entity to which the Company or any of its Subsidiaries or any of their respective assets or properties may be subject or bound, (B) any term or provision of any Contract to which the Company or any of its Subsidiaries is a party or by which the Company or any of its Subsidiaries or any of their respective assets or properties is bound or committed or (C) any term or provision of any of the organizational documents of the Company or any of its Subsidiaries, except in the case of clause (A) or (B) above

where any such event would not result in (1) a Material Adverse Effect, or (2) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the payments based on Included Royalty Interest; or (ii) except as provided in or contemplated by any of the Transaction Documents, result in or require the creation or imposition of any Lien on the Patents, the Licensed Product or the Included Royalty Interest.

(c) Liens. Except pursuant to, or as contemplated by, the Transaction Documents, the Company has not granted, nor does there exist, any Lien (other than Permitted Liens) on the Transaction Documents, the Patents or the Included Royalty Interest.

(d) Authorization. The Company has all powers and authority to execute and deliver, and perform its obligations under, the Transaction Documents to which it is party and to consummate the transactions contemplated hereby and thereby. The execution and delivery of each of the Transaction Documents to which the Company is party and the performance by the Company of its obligations hereunder and thereunder have been duly authorized by the Company. Each of the Transaction Documents to which the Company is party has been duly executed and delivered by the Company. Each of the Transaction Documents to which the Company is party constitutes the legal, valid and binding obligation of the Company, enforceable against the Company in accordance with its respective terms, subject to applicable bankruptcy, insolvency, reorganization, moratorium or similar Applicable Laws affecting creditors' rights generally, general equitable principles and principles of public policy.

(e) Consents. The execution and delivery by the Company of the Transaction Documents to which the Company is party, the performance by the Company of its obligations hereunder and thereunder and the consummation of any of the transactions contemplated hereunder and thereunder (including granting of security interests in the Included Royalty Interest to Lender) do not require any consent, approval, license, order, authorization or declaration from, notice to, action or registration by or filing with any Governmental Entity or any other Person, except for (i) the filing of any applicable notices under securities laws, (ii) the filings necessary to perfect Liens created by the Loan Documents, (iii) those previously obtained and in full force and effect, (iv) consents, filings and registrations in connection with the Contribution as contemplated by the Contribution Agreement, (v) the Emory Consent, (vi) the Nippon Shinyaku Notice, and (vii) the UoM Notice.

(f) Proceedings. Except as set forth on Schedule 7.02(f), there is no action, suit, arbitration proceeding, claim, citation, summons, subpoena, investigation or other proceeding (whether civil, criminal, administrative, regulatory, investigative or informal, and including by or before a Governmental Entity) pending or, to the Knowledge of the Company, threatened in writing (or, in the case of a threat by a Governmental Entity, threatened orally or in writing) by or against the Company or any of its Subsidiaries, at law or in equity, that (i) if adversely determined, would result in (A) a Material Adverse Effect, or (B) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest, or (ii) challenges or seeks to prevent or delay the consummation of any of the transactions contemplated by any of the Transaction Documents to which the Company is party.

(g) Solvency. Upon consummation of the transactions contemplated by the Transaction Documents and the application of the proceeds from the Initial Tranche Loan (a) the present fair saleable value of the Company's assets will be greater than the sum of its debts, liabilities and other obligations, including contingent liabilities, (b) the present fair saleable value of the properties and assets of the Company and its Subsidiaries, taken as a whole, will not be less than the amount that would be required to pay its probable liabilities on its existing debts, liabilities and other obligations, including contingent liabilities, as they become absolute and matured, (c) the Company will be generally able to realize upon its assets and pay its debts, liabilities and other obligations, including contingent obligations, as they become absolute and matured, (d) the Company will not have unreasonably small capital with which to engage in its business as now conducted, (e) the Company has not incurred, will not incur and does not have any present plans or intentions to incur debts or other obligations or liabilities beyond its ability to pay such debts or other obligations or liabilities as they become absolute and matured, (f) the Company will not have become subject to any Insolvency Event and (g) the Company will not have been rendered insolvent within the meaning of any Applicable Law. No step has been taken by the Company or, to its Knowledge, any other Person to make the Company subject to an Insolvency Event.

(h) No Default. No Default, Event of Default or Prepayment Trigger has occurred and is continuing, and no such event will occur upon the making of the Loan.

(i) Taxes. The Company has timely filed (or caused to be filed) all tax returns and reports required by Applicable Law to have been filed by it and has paid all Taxes required to be paid by it (including in its capacity as a withholding agent), except any such Taxes that are being contested in good faith by appropriate proceedings, diligently conducted, and for which adequate reserves in accordance with GAAP or where any such failure to file or pay would not result, individually or in the aggregate, in (a) a Material Adverse Effect, or (b) an adverse effect, in any respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest. None of the payments received (or to be received) by the Company or Borrower in respect of the Royalty Interest has been, or under current Law will be, subject to any withholding Tax or Other Tax, and neither the Company nor Borrower was ever required or requested to establish any entitlement to treaty benefits in order to avoid or minimize any such withholdings or deductions.

(j) Broker's Fees. Except as disclosed on Schedule 7.02(j), the Company has not taken any action that would entitle any person or entity to any commission or broker's fee in connection with the transactions contemplated by this Agreement.

(k) Investigations. None of the Company or any of its Subsidiaries (a) is in violation of, is under investigation with respect to or has been threatened in writing to be charged with or been given written notice of any violation of, any Applicable Law or any judgment, order, writ, decree, injunction, stipulation, consent order, permit or license granted, issued or entered by any Governmental Entity or (b) is subject to any judgment, order, writ, decree, injunction, stipulation, consent order, permit or license granted, issued or entered by any Governmental Entity, in each case of (a) and (b), that would reasonably be expected to result in (i) a Material Adverse Effect, or (ii) an adverse effect, in any material respect, including on the timing, amount or duration of, the Included Royalty Interest or the right of Lender to receive the payments based on Included

Royalty Interest. Each of the Company and any Subsidiary of the Company is in compliance with the requirements of all Applicable Laws, a breach of any of which would result in a Material Adverse Effect.

(l) Lending. The Company is not engaged in the business of extending credit for the purpose of buying or carrying margin stock, and no portion of the Loan shall be used by the Company for a purpose that violates Regulation T, U or X promulgated by the Board of Governors of the Federal Reserve System from time to time.

(m) Material Contracts.

(i) Schedule 7.02(m) hereto contains a list of each Material Contract to which the Company is a party. As of the Closing Date, there has been provided a true and complete copy of each of the Material Contracts to Lender in the electronic data room.

(ii) To the Company's Knowledge, except as separately disclosed in writing to Lender referencing this Section 7.02, neither the Company nor any Material Contract Counterparty is in breach in any material respect or default of any Material Contract and no circumstances or grounds exist that would, upon the giving of notice, the passage of time or both, give rise (A) to a claim by the Company or any Material Contract Counterparty of a breach in any material respect or default of any Material Contract, or (B) to a right of rescission, termination, revision, setoff, or any other rights, by any Person, in, to or under any Material Contract. The Company has not received from, or delivered to, any Material Contract Counterparty, any written or electronic notice alleging a breach in any material respect or default under any Material Contract, which breach or default has not been cured or waived as of the date hereof.

(iii) Each Material Contract is a valid and binding obligation of the Company and, to the Knowledge of the Company, of the applicable Material Contract Counterparty, enforceable against each of the Company and, to the Knowledge of the Company, each applicable Material Contract Counterparty in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar laws of general application relating to or affecting creditors' rights generally. The Company has not received any written or electronic notice from any Material Contract Counterparty or any other Person challenging the validity or enforceability of any Material Contract.

(iv) The Company has not received any notice from any Material Contract Counterparty or any other Person threatening or commencing any case, proceeding or other action relating to Material Contract Counterparty's bankruptcy, insolvency, liquidation or dissolution or reorganization by any of the foregoing means.

(n) In-License Agreements.

(i) Attached hereto as Schedule 7.02(n)(i) are the following: (A) a true, correct and complete copy of each In-License Agreement; (B) the reports delivered by the Company to each Licensor under each In-License Agreement, in each case, in respect of each Calendar Quarter ended on or prior to the date hereof (it being understood and agreed that all information in such reports that does not relate to or involve the Royalty Interest has been redacted); and (C) all material written notices delivered to each Licensor by the Company, or by any Licensor to the Company, pursuant to each In-License Agreement relating to or involving the applicable Licensed Product, in each case (A), (B) and (C), since [****].

(ii) Each In-License Agreement is a valid and binding obligation of the Company and, to the Knowledge of the Company, of each Licensor, enforceable against each of the Company and, to the Knowledge of the Company, each Licensor in accordance with its terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar laws of general application relating to or affecting creditors' rights generally. The Company has not received any written notice from any Licensor challenging the validity or enforceability of any In-License Agreement, to the extent related to or involving any Licensed Product.

(iii) In each case, to the extent related to or involving any Licensed Product, the Company (A) has not granted any material written waiver or, to the Knowledge of the Company, any other material waiver, under any In-License Agreement, and (B) has not released any Licensor, in whole or in part, from any of its material obligations under any In-License Agreement, except, in each case of the immediately foregoing clauses (A) and (B), to the extent set forth in such In-License Agreement. Since [****], the Company has not received from any Licensor any written proposal, and has not made any proposal to any Licensor, to amend or waive any provision of any In-License Agreement, to the extent related to or involving any Licensed Product.

(iv) The Company has not (A) given any Licensor any notice of termination of any In-License Agreement or (B) received from any Licensor any written notice of termination of any In-License Agreement. To the Knowledge of the Company, no event has occurred that would give any Licensor a right to terminate any In-License Agreement. The Company has not received any written notice from any Licensor expressing any intention or desire to terminate the any In-License Agreement.

(v) Except, for the avoidance of doubt, as set forth on Schedule 7.02(n)(v), the Company has not breached any provision of any In-License Agreement in any material respect, and, to the Knowledge of the Company, no Licensor has not breached any provision of their In-License Agreement in any material respect, in each case, related to or involving any Licensed Product.

(vi) The Company has paid to each Licensor any amounts required pursuant to each In-License Agreement related to or involving any Licensed Product prior to the date hereof. As of the Closing Date, no payments are owed by the Company to any Licensor pursuant to any In-License Agreement related to or involving any Licensed Product.

(vii) The Company has not consented to any assignment by any Licensor of, and, to the Knowledge of the Company, each Licensor has not assigned (other than as contemplated by the Transaction Documents), their respective In-License Agreement or any part thereof. Except as contemplated by the Transaction Documents, the Company has not assigned, in whole or in part, and has not granted any liens upon or security interests with respect to, any In-License Agreement, to the extent related to or involving any Licensed Product.

(viii) No Licensor has not initiated any review or audit pursuant to their respective In-License Agreement.

(ix) Except, for the avoidance of doubt, as set forth on Schedule 7.02(n)(ix), the Company has not (A) received any written notice of any dispute from any Licensor for resolution pursuant to any In-License Agreement or (B) given any notice of any dispute to any Licensor for resolution pursuant to any In-License Agreement, in each case related to or involving any Licensed Product, and in each case since [****].

(o) UCC Representations. The Company's exact legal name is, and for the prior five years has been, "REGENXBIO Inc." The Company is incorporated, and for the past five years has been incorporated, in the State of Delaware.

(p) Financial Statements. The Financial Statements of the Company are complete and accurate in all material respects, were prepared in conformity with GAAP applied on a consistent basis during the periods involved (except as may be indicated in the notes thereto) and present fairly in all material respects, in accordance with applicable requirements of GAAP, the consolidated financial position and the consolidated financial results of the operations of the Company and its Subsidiaries as of the dates and for the periods covered thereby and the consolidated statements of cash flows of the Company and its Subsidiaries for the periods presented therein. Since [****], there has been no Material Adverse Effect.

(q) Pledge Agreement; Enforceability. The Pledge Agreement, when executed and delivered by the parties thereto, is effective to create in favor of Lender legal, valid and enforceable (subject to bankruptcy, insolvency, reorganization, moratorium or similar Laws affecting creditors' rights generally or general equitable principles (regardless of whether enforcement is sought in equity or at law)) Liens on, and security interests in, the Capital Stock of Borrower, and, when (x) financing statements and other filings in appropriate form are filed in the offices of the Secretary of State of the State of Delaware and (y) upon the taking of possession or control by Lender of the Capital Stock certificates (if certificated) with duly executed instruments of transfer in blank, the Liens created by the Pledge Agreement shall constitute fully perfected Liens on, and security interests in, all right, title and interest of the Company in the Capital Stock of Borrower, subject to no Liens other than Permitted Liens.

(r) No Investment Company. The Company is not an “investment company,” or a company “controlled” by an “investment company”, within the meaning of the Investment Company Act of 1940.

(s) Regulation of Licensed Products. To the Knowledge of the Company, neither the FDA nor any other Regulatory Agency has stated that it is not likely to approve or is likely to withdraw approval of the Licensed Product.

(t) Insurance. The Company has the products-completed operations liability, property and casualty, commercial general liability, and umbrella liability insurance policies with the coverages and limits set forth on Schedule 7.02(t) carried with the Insurance Providers also set forth therein, and Borrower is covered under such policies.

(u) Beneficial Ownership. The Company does not beneficially own or control more than 10% of the outstanding voting stock or any other equity interest entitled ordinarily to vote in the election of the directors or other governing body (however designated) of any Person that is not a wholly owned Subsidiary.

Article VIII. AFFIRMATIVE COVENANTS

Borrower covenants and agrees with Lender that, until Payment in Full:

Section 8.01 Maintenance of Existence. Borrower shall at all times (a) preserve, renew and maintain in full force and effect its legal existence (except as otherwise permitted pursuant to Section 9.02(a) hereof) and good standing as a corporation under the Laws of the jurisdiction of its organization; (b) not change its name or its chief executive office as set forth herein without having given Lender the notice thereof required under Section 8.16; and (c) take all reasonable action to maintain all rights, privileges, permits, licenses and franchises necessary or desirable in the normal conduct of its business, except to the extent that failure to do so could not reasonably be expected to have a Material Adverse Effect.

Section 8.02 Use of Proceeds. Borrower shall use the net proceeds of the Loan received by it to acquire assets from the Company pursuant to the Contribution Agreement, to pay dividends and other distributions to the Company and for general corporate purposes.

Section 8.03 Financial Statements and Information.

(a) On or before the [****] day after the close of each quarter of each fiscal year, Borrower shall furnish to Lender a certificate of a Senior Officer of Borrower, which certificate shall include a statement that such officer has no knowledge, except as specifically stated, of any condition, event or act which constitutes a Default, Event of Default or Prepayment Trigger.

(b) For each fiscal quarter ending after the Closing Date, Borrower shall for each Covered License Agreement, within [****] Business Days following the end of such fiscal quarter, deliver or cause to be delivered to Lender a true copy of all Royalty Reports received

during such quarter and all Material Other Reports received during such quarter. In addition, Borrower shall, promptly upon receipt thereof, forward or cause to be forwarded to Lender copies of all other Notices, reports, updates and other data or information (i) pertaining to the Included Royalty Interest and other Collateral (ii) received from the Licensee or any Third Party which relate to events or circumstances that could reasonably be expected to have a Material Adverse Effect, or (iii) received from any Person that relate to the Intellectual Property and that could reasonably be expected to have a Material Adverse Effect, or that Lender reasonably requests.

(c) Borrower and Licensee shall consult with each other regarding the timing, manner and conduct of any review or audit of a Licensee's records and books of account with respect to the Included Royalty Interest. Borrower may, and if requested in writing by Lender, shall, exercise the audit rights under the applicable sections of the Covered License Agreements (subject to all restrictions and limitations thereon contained in the applicable Covered License Agreements); provided that Lender shall not be entitled to request such an audit (i) more frequently than once every calendar year or (ii) if such an audit would contravene the applicable sections of the Covered License Agreements. Lender shall pay the costs of the respective audit and shall be entitled to any reimbursement of the costs thereof by the Licensee as provided under the applicable sections of the Covered License Agreements. Any additional payments of the Royalty Interest due from a Licensee, together with interest thereon as provided under the Covered License Agreements, shall be paid by a Licensee to the Collection Account, and any refund due to a Licensee from any overpayment in respect of the Royalty Interest determined in any such audit shall be paid by Borrower in accordance with the applicable Covered License Agreements. Borrower and Lender will reasonably cooperate in the exercise of such audit rights in order to avoid unnecessary limitations on the timing, scope and conduct of such audits within the parameters specified in the applicable Covered License Agreement.

(d) Lender and its Representatives shall have the right, from time to time, not more than [****] per calendar quarter, during normal business hours and upon at least [****] Business Days' prior written notice to Borrower (provided that, after the occurrence and during the continuance of an Event of Default, Lender shall have the right, as often, at such times and with such prior notice, as Lender determines in its reasonable discretion), to visit the offices and properties of Borrower and the Company where books and records relating or pertaining to the Included Royalty Interest and the Collateral are kept and maintained (or, at Lender's option, to conduct a meeting by telecommunications), to discuss, with officers of Borrower and the Company, the business, operations, properties and financial and other condition of Borrower and the Company, to discuss the Covered License Agreements and the Licensed Products, to discuss the Royalty Reports and Material Other Reports, to verify compliance with the provisions of the Loan Documents regarding receipt and application of the Included Royalty Interest and, upon physical visits, to inspect and make extracts from and copies of the books and records of Borrower and the Company relating or pertaining to the Included Royalty Interest and the Collateral.

(e) All written information supplied by or on behalf of Borrower to Lender pursuant to this Section 8.03 (other than Section 8.03(a)) shall be accurate and complete in all material respects as of its date or the date so supplied. For the avoidance of doubt, Borrower makes no representations or warranties regarding the accuracy or completeness of any information it receives from a Third Party that it is required to furnish to Lender pursuant to this Section 8.03,

unless to the Knowledge of Borrower or the Company such information is inaccurate or incomplete in any material respect, in which case Borrower or the Company shall specify such inaccuracy or incompleteness.

Section 8.04 Books and Records. Borrower shall keep proper books, records and accounts in which entries in conformity with sound business practices and all requirements of Law applicable to it shall be made of all dealings and transactions in relation to its business, assets and activities and as shall permit the preparation of the consolidated financial statements of Borrower in accordance with GAAP.

Section 8.05 Governmental Authorizations. Borrower shall obtain, make and keep in full force and effect all authorizations from and registrations with Governmental Entities that may be required for the validity or enforceability against Borrower of this Agreement and the other Loan Documents to which it is a party.

Section 8.06 Compliance with Laws and Contracts.

(a) Borrower shall comply with all Applicable Laws and perform its obligations under all Material Contracts, if any, entered into after the Closing Date relative to the conduct of its business, except where the failure to comply could not reasonably be expected to result in a Material Adverse Effect. Borrower shall use commercially reasonable efforts to take all actions necessary to enforce its rights under each Material Contract, and perform all of its material obligations under each Material Contract, except to the extent that failure to do so could not reasonably be expected to have a Material Adverse Effect (subject to Section 9.01(a)).

(b) Borrower shall at all times comply with the margin requirements set forth in Section 7 of the Exchange Act and any regulations issued pursuant thereto, including, without limitation, Regulations T, U and X of the Board of Governors of the Federal Reserve System, 12 C.F.R., Chapter II.

Section 8.07 Plan Assets. Borrower shall not take any action that causes its assets to be deemed to be Plan Assets at any time.

Section 8.08 Notices.

(a) Borrower shall, promptly after an officer becomes aware thereof, give written Notice to Lender of each Default, Event of Default or Prepayment Trigger and each other event that has or could reasonably be expected to have a Material Adverse Effect; provided that in any of the foregoing situations where Borrower knows a press release or other public disclosure is to be made, Borrower shall use all commercially reasonable efforts to provide such information to Lender as early as possible but in no event later than simultaneously with such release or other public disclosure.

(b) Borrower shall promptly give written Notice to Lender upon receiving notice, or an officer otherwise becomes aware, of any default or event of default under any Material Contracts.

(c) Borrower shall, promptly (and in any event within [****] Business Days) after an officer becomes aware thereof, give written Notice to Lender of any litigation or proceedings to which Borrower is a party or which could reasonably be expected to have a Material Adverse Effect.

(d) Borrower shall, promptly after an officer becomes aware thereof, give written Notice to Lender of any litigation or proceedings challenging the validity of any Covered License Agreement or otherwise required under any Covered License Agreement, the Transaction Documents or any of the transactions contemplated therein.

(e) Borrower shall, promptly after an officer becomes aware thereof, give written Notice to Lender of the occurrence of any Material Adverse Effect.

(f) Borrower shall, promptly after receipt of any written notice from a Licensee pursuant to any Covered License Agreement of an event which has had, or would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect, provide a copy of such notice to Lender together with a summary of Borrower's intended response to Licensee.

Section 8.09 Payment of Taxes. Borrower (or the Company on behalf of Borrower, as applicable), shall timely file all tax returns and timely pay all material Taxes imposed on or in respect of Borrower's income or assets that are due and payable (including in its capacity as withholding agent) except for Taxes contested in good faith by appropriate proceedings, diligently conducted, and for which adequate reserves are maintained in accordance with GAAP.

Section 8.10 Waiver of Stay, Extension or Usury Laws. Notwithstanding any other provision of this Agreement or the other Loan Documents, if at any time the rate of interest payable by any Person under the Loan Documents exceeds the Maximum Lawful Rate, then, so long as the Maximum Lawful Rate would be exceeded, such rate of interest shall be equal to the Maximum Lawful Rate. If at any time thereafter the rate of interest so payable is less than the Maximum Lawful Rate, such Person shall continue to pay interest at the Maximum Lawful Rate until such time as the total interest received from such Person is equal to the total interest that would have been received had applicable law not limited the interest rate so payable. In no event shall the total interest received by Lender under this Agreement and the other Loan Documents exceed the amount which Lender could lawfully have received, had the interest due been calculated from the Closing Date at the Maximum Lawful Rate. Without limiting the foregoing, Borrower will not at any time, to the extent that it may lawfully not do so, insist upon, or plead, or in any manner whatsoever claim or take the benefit or advantage of, any stay or extension law or other law that would prohibit or forgive Borrower from paying all or any portion of the principal of or premium, if any, or interest on the Loan as contemplated herein, wherever enacted, now or at any time hereafter in force, or that may affect the covenants or the performance of this Agreement; and, to the extent that it may lawfully do so, Borrower hereby expressly waives all benefit or advantage of any such law and expressly agrees that it will not hinder, delay or impede the

execution of any power herein granted to Lender, but will suffer and permit the execution of every such power as though no such law had been enacted.

Section 8.11 Intellectual Property.

(a) To the extent permitted under any Covered License Agreement, Borrower shall not, and shall not permit or suffer the Company or any of its Affiliates to, consent to any judgment or settlement in any action, suit or proceeding referred to in the applicable section of any Covered License Agreement if such consent could reasonably be expected to have a Material Adverse Effect, without the prior written consent of Lender, which consent shall not be withheld, delayed or conditioned by Lender if doing so would result in Borrower breaching its obligation to not unreasonably withhold, delay or condition its consent under the applicable section of such Covered License Agreement.

(b) Borrower shall furnish Lender from time to time upon Lender's reasonable written request therefor, but in any event not more than [****] in per calendar year so long as no Event of Default is continuing, reasonably detailed statements and schedules further identifying and describing the Listed Patents and such other materials evidencing or reports pertaining to any Intellectual Property as Lender may reasonably request.

(c) Borrower shall, promptly upon obtaining Knowledge thereof, give written notice to Lender of any commercially significant infringement or interference by any Person, any claims of invalidity or unenforceability in any prosecution or litigation action relating to the Patents or patents relating to the Licensed Product granted in the U.S. or any of the jurisdictions specified on Schedule 8.11(c), in each case, if it could reasonably be expected to have a Material Adverse Effect.

Section 8.12 Prosecution and Maintenance of Product-Specific Patents.

(a) As between Lender and Borrower, Borrower shall have the sole right to Prosecute patent applications and issue patents within the Listed Patents set forth on Schedule 8.12(a) (collectively, the "Product-Specific Patents").

(b) Borrower shall use commercially reasonable efforts (it being understood and agreed that such efforts (i) shall be consistent with standards in the biotechnology industry and determined without taking into account any amounts owed to Lender and (ii) [****] shall not require Borrower to defend against any claims (including of invalidity or unenforceability) in any oppositions, inter partes review, interference, or other proceeding) to, and if required by Lender, Borrower shall, continue to Prosecute, or to cause the applicable Licensor(s) to Prosecute, any patent applications, patent term extensions (including supplementary protection certificates), or issued patents, with respect to the Product-Specific Patents; provided that such Prosecution shall be at Borrower's cost unless Lender requires Borrower to continue to Prosecute, or to cause the applicable Licensor(s) to Prosecute, in which case all costs and expenses (including attorneys' fees and expenses) incurred by Borrower or the Company in connection therewith shall be borne solely by Lender. Lender shall fund any retainers or advances required by the counsel employed by

Borrower or Company for such Prosecution required by Lender (such amounts to be credited or deducted from the actual amounts owed by Lender under the immediately preceding sentence).

(c) Lender acknowledges and agrees that (i) the rights and obligations under this Section 8.12 are subject to the rights of the Licensors under the In-License Agreements and (ii) Borrower's obligations only apply to the extent of Borrower's (or the Company's) rights with respect to participation in prosecuting the Product-Specific Patents under the In-License Agreements.

Section 8.13 Enforcement of Product-Specific Patents.

(a) Notice and Consultation. In the event that Lender or a Senior Officer of Borrower or the Company becomes aware of any potential infringement of any Product-Specific Patent by a third party and such Product-Specific Patent is being infringed by another product in the applicable Field (as defined in the applicable Covered License Agreement) (such infringement, a "Competitive Infringement"), then promptly (and in any event within [****] Business Days) following Lender or such Senior Officer of Borrower or the Company, respectively, becoming aware of such Competitive Infringement, Lender or Borrower, respectively, shall inform the other party hereto of such Competitive Infringement. Borrower shall provide to Lender a copy of any written notice of any Competitive Infringement of any Product-Specific Patents delivered or received by Borrower (other than any such notice provided by Lender) as soon as practicable (and in any event within [****] Business Days) following such delivery or receipt by Borrower. Promptly following a Senior Officer of Borrower or the Company becoming aware of any Competitive Infringement of any Product-Specific Patents (whether as a result of being notified by a Licensee pursuant to the applicable section(s) the applicable Covered License Agreement(s), by Lender pursuant to this Section 8.13(a), or otherwise), Borrower and Lender shall consult with each other with a view to determining the appropriate course of action to take with respect to such Competitive Infringement (in each case, subject to the terms and conditions of the applicable Covered License Agreement(s)).

(b) Enforcement. If Borrower has the right pursuant to any Covered License Agreement or Applicable Law to institute suit or other legal proceedings to enforce any of the Product-Specific Patents in respect of any Competitive Infringement, then promptly (and in any event within [****] Business Days) following a Senior Officer of Borrower or the Company becoming aware of such right of Borrower, Borrower shall provide notice of such right to Lender. In the event that Borrower declines to exercise such right, Borrower shall promptly give notice of such declination to Lender, and Lender shall have [****] Business Days to require that Borrower proceed, in consultation with Lender, to institute such a suit or other legal proceeding and to use commercially reasonable efforts to enforce the Product-Specific Patents in respect of such Competitive Infringement, and to exercise such rights and remedies relating to such Competitive Infringement as shall be available to Borrower under Applicable Law, but, in each case, subject to the terms and conditions of the applicable Covered License Agreement(s) and this Agreement. In connection with any such enforcement of the Product-Specific Patents, Borrower may employ any counsel, so long as such counsel is acceptable to Lender (such acceptance not to be unreasonably withheld or delayed).

(c) Allocation of Proceeds of Enforcement. To the extent in respect of any Competitive Infringement, the Proceeds of any enforcement of any of the Product-Specific Patents (i) by Borrower pursuant to this Section 8.13 and the applicable sections of the Covered License Agreements or (ii) by Borrower together with a Licensee pursuant to the applicable section of a Covered License Agreement, in each case of the immediately foregoing clauses (i) and (ii), after deduction (and reimbursement to Borrower and Lender) of all costs and expenses (including attorneys' fees and expenses) incurred by Borrower and Lender in connection with such enforcement pursuant to the first two sentences of Section 8.13(d) below, shall be allocated between, and paid to, Borrower and Lender in proportion to their respective interests in the applicable Royalty Interest (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be deemed to be in Lender's interest). To the extent not on account of Included Royalty Interest, the Proceeds of any enforcement of any of the Product-Specific Patents after deduction (and reimbursement to Borrower and Lender) of all costs and expenses (including attorneys' fees and expenses) incurred by Borrower and Lender in connection with such enforcement pursuant to the first two sentences of Section 8.13(d) below, shall be paid to Borrower.

(d) Allocation of Costs of Enforcement. All costs and expenses (including attorneys' fees and expenses) incurred by Borrower in connection with any enforcement of any of the Product-Specific Patents in respect of any Competitive Infringement shall be borne by Borrower and Lender in proportion to their respective interests in the applicable Royalty Interest (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be deemed to be in Lender's interest). Lender shall fund any retainers or advances required by the counsel employed by Borrower for such enforcement (such amounts to be credited or deducted from the actual amounts owed by Borrower and Lender under the immediately preceding sentence). Nothing contained herein shall limit Lender from retaining, at its sole cost, separate outside counsel who shall be permitted, where reasonably practicable, to consult with the lead counsel selected pursuant to Section 8.13(b) for such enforcement.

(e) Lender acknowledges and agrees that (i) the rights and obligations under this Section 8.13 are subject to the rights of the Licensors under the In-License Agreements (including any consent or approval rights or rights to control or participate in any enforcement actions) and (ii) Borrower's obligations only apply to the extent of Borrower's (or the Company's) rights with respect to enforcing the Product-Specific Patents under the In-License Agreements.

Section 8.14 Defense of Third-Party Infringement Claims.

(a) Third-Party Infringement. In the event that Lender or a Senior Officer of Borrower or the Company becomes aware of any claim or suit by any third party for infringement of a patent or other intellectual property of such third party as further described in the applicable sections of the Covered License Agreements ("Third-Party Infringement Claim") asserting infringement relating to the manufacture, importation, sale, offer for sale or use of the Licensed

Products, then promptly (and in any event within [****] Business Days) following Lender or such Senior Officer of Borrower or the Company, respectively, becoming aware of such Third-Party Infringement Claim, Lender or Borrower, respectively, shall inform the other party hereto of such Third-Party Infringement Claim. Borrower shall provide to Lender a copy of any written notice of any Third-Party Infringement Claim delivered or received by Borrower or the Company (other than any such notice provided by Lender) as soon as practicable (and in any event within [****] Business Days) following such delivery by Borrower or the Company or receipt by Borrower. Promptly following a Senior Officer of Borrower or the Company becoming aware of any Third-Party Infringement Claim (whether as a result of being notified by the Licensee pursuant to the applicable sections of the Covered License Agreements, by Lender pursuant to this Section 8.14, or otherwise), Borrower and Lender shall consult with each other with a view to determining the appropriate course of action to take with respect to such Third-Party Infringement Claim (in each case, subject to the terms and conditions of the applicable Covered License Agreement(s)).

(b) Enforcement. If Borrower has the right pursuant to any Covered License Agreement or Applicable Law to institute suit or other legal proceedings to enforce or defend any of the Product-Specific Patents in respect of any Third-Party Infringement Claim, then promptly (and in any event within [****] Business Days) following a Senior Officer of Borrower or the Company becoming aware of such right of Borrower, Borrower shall provide notice of such right to Lender. In the event that Borrower declines to exercise such right, Borrower shall promptly give notice of such declination to Lender, and Lender shall have [****] Business Days to require that Borrower proceed, in consultation with Lender, to institute such a suit or other legal proceeding and to use commercially reasonable efforts to enforce or defend the Product-Specific Patents in respect of such Third-Party Infringement Claim, and to exercise such rights and remedies relating to such Third-Party Infringement Claim as shall be available to Borrower under Applicable Law, but, in each case, subject to the terms and conditions of the applicable Covered License Agreement(s) and this Agreement. In connection with any such enforcement or defense of the Product-Specific Patents, Borrower may employ any counsel, so long as such counsel is acceptable to Lender (such acceptance not to be unreasonably withheld or delayed).

(c) Allocation of Proceeds of Enforcement. To the extent in respect of any Third-Party Infringement Claim, the Proceeds of any enforcement or defense of any of the Product-Specific Patents (i) by Borrower pursuant to this Section 8.14 and the applicable sections of the Covered License Agreements or (ii) by Borrower together with a Licensee pursuant to the applicable section of a Covered License Agreement, in each case of the immediately foregoing clauses (i) and (ii), after deduction (and reimbursement to Borrower and Lender) of all costs and expenses (including attorneys' fees and expenses) incurred by Borrower and Lender in connection with such enforcement or defense pursuant to the first two sentences of Section 8.14(d) below, shall be allocated between, and paid to, Borrower and Lender in proportion to their respective interests in the applicable Royalty Interest (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be deemed to be in Lender's interest). To the extent not on account of Included Royalty Interest, the Proceeds of any enforcement or defense of any of the Product-Specific Patents after deduction (and reimbursement to Borrower and Lender) of all costs and expenses (including attorneys' fees and expenses)

incurred by Borrower and Lender in connection with such enforcement or defense pursuant to the first two sentences of Section 8.14(d) below, shall be paid to Borrower.

(d) Allocation of Costs of Enforcement. All costs and expenses (including attorneys' fees and expenses) incurred by Borrower in connection with any enforcement or defense of any of the Product-Specific Patents in respect of any Third-Party Infringement Claim shall be borne by Borrower and Lender in proportion to their respective interests in the applicable Royalty Interest (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be deemed to be in Lender's interest). Lender shall fund any retainers or advances required by the counsel employed by Borrower for such enforcement or defense (such amounts to be credited or deducted from the actual amounts owed by Borrower and Lender under the immediately preceding sentence). Nothing contained herein shall limit Lender from retaining, at its sole cost, separate outside counsel who shall be permitted, where reasonably practicable, to consult with the lead counsel selected pursuant to Section 8.14(b) for such enforcement or defense.

Section 8.15 Security Documents; Further Assurances.

(a) Subject to Section 8.15(b), Borrower shall promptly, upon the reasonable request of Lender, at Borrower's expense, (a) execute, acknowledge and deliver, or cause the execution, acknowledgment and delivery of, and thereafter register, file or record, or cause to be registered, filed or recorded, in an appropriate governmental office, any document or instrument supplemental to or confirmatory of the Loan Documents or otherwise deemed by Lender reasonably necessary or desirable for the continued validity, perfection and priority of the Liens on the Collateral covered thereby subject to no other Liens except as permitted by the applicable Loan Document, or obtain any consents or waivers as may be necessary or appropriate in connection therewith; (b) deliver or cause to be delivered to Lender from time to time such other documentation, consents, authorizations, approvals and orders in form and substance reasonably satisfactory to Lender and Lender shall reasonably deem necessary to perfect or maintain the Liens on the Collateral pursuant to the Loan Documents; and (c) upon the exercise by Lender of any power, right, privilege or remedy pursuant to any Loan Document which requires any consent, approval, registration, qualification or authorization of any Governmental Entity execute and deliver all applications, certifications, instruments and other documents and papers that Lender may require. In addition, subject to Section 8.15(b), Borrower shall promptly, at its sole cost and expense, execute and deliver to Lender such further instruments and documents, and take such further action, as Lender may, at any time and from time to time, reasonably request in order to carry out the intent and purpose of this Agreement and the other Loan Documents to which it is a party and to establish and protect the rights, interests and remedies created, or intended to be created, in favor of Lender hereby and thereby.

(b) Notwithstanding anything to the contrary herein or in any other Loan Document, Borrower shall not have any obligation to (i) perfect or record any security interest or lien in any intellectual property included in the Collateral in any jurisdiction other than in the U.S. (or to enter into any foreign law governed charges, debentures, pledges or other security

agreements in respect thereof), (ii) obtain any landlord waivers, estoppels or collateral access letters, or (iii) obtain any consent of the Licensee to the assignment and pledge to Lender of the rights under the Covered License Agreements that are included in the Collateral.

Section 8.16 Information Regarding Collateral. Borrower shall not effect any change (i) in its legal name, (ii) in the location of its chief executive office, (iii) in its identity or organizational structure, (iv) in its federal Taxpayer Identification Number or organizational identification number, if any, or (v) in its jurisdiction of organization (in each case, including by merging with or into any other entity, reorganizing, dissolving, liquidating, reorganizing or organizing in any other jurisdiction), until (A) it shall have given Lender not less than [****] days prior written notice (in the form of an certificate of a duly authorized officer of Borrower), or such lesser notice period agreed to by Lender, of its intention so to do, clearly describing such change and providing such other information in connection therewith as Lender may reasonably request and (B) it shall have taken all action reasonably satisfactory to Lender to maintain the perfection and priority of the security interest of Lender in the Collateral, if applicable (subject to the limitations set forth in Section 8.15(b)). Borrower agrees to provide promptly Lender with certified Borrower's Organizational Documents reflecting any of the changes described in the preceding sentence. Borrower also agrees to notify promptly Lender of any change in the location of any office in which it maintains books or records relating to Collateral owned by it or any office or facility at which any portion of Collateral is located (including the establishment of any such new office or facility), other than (a) changes in location to a mortgaged property, (b) Collateral which is in-transit or in the possession of employees, and (c) Collateral which is out for repair or processing.

Section 8.17 Additional Collateral; New License Arrangement.

(a) With respect to any Collateral acquired after the Closing Date by Borrower that is not already subject to the Lien created by any of the Loan Documents or specifically excluded from the requirement to be subject to such Lien in the Loan Documents, Borrower shall promptly (and in any event within [****] days after the acquisition thereof) (i) execute and deliver to Lender such amendments or supplements to the relevant Loan Documents or such other documents as Lender shall deem necessary or advisable to grant for its benefit, a Lien on such property subject to no Liens other than Permitted Liens, and (ii) take all actions necessary to cause such Lien to be duly perfected in accordance with all applicable requirements of Law, including the filing of financing statements in such jurisdictions as may be reasonably requested by Lender. Subject to Section 8.15(b), Borrower shall otherwise take such actions and execute and/or deliver to Lender such documents as Lender shall reasonably require to confirm the validity, perfection and priority of the Lien of the Security Agreement on such after-acquired properties.

(b) Without limiting any other rights or remedies Lender may have under this Agreement, the Security Agreement or the Pledge Agreement, if any Licensee or Borrower terminates or provides written notice of termination of a Specified Covered License Agreement in its entirety, or a Specified Covered License Agreement otherwise terminates in its entirety, then, to the extent permitted by the survival provisions of such Specified Covered License Agreement and any provisions of the applicable In-License Agreement(s), Borrower shall use commercially reasonable efforts, at Lender's reasonable request and Lender's sole cost and expense (including

Lender's payment of Borrower's reasonable attorney's fees, if any, in connection therewith), in consultation and cooperation with Lender, for a period of [****] days (or such shorter period as set forth in this Section 8.17) (the "Initial Search Period"), to locate, negotiate and secure a license of the Intellectual Property with respect to the applicable Licensed Product (any such license, a "New Arrangement"); provided that the counterparty to such New Arrangement shall be reasonably acceptable to Borrower; provided further that Lender shall have the right to consent in writing to any New Arrangement, which approval shall not be unreasonably withheld or delayed. Borrower agrees to undertake in connection with any New Arrangement such obligations and liabilities, if any, as are comparable to the obligations and liabilities it currently has under the applicable Specified Covered License Agreement(s); provided that (i) in no event shall Borrower have any obligation in connection with the New Arrangement to renegotiate the applicable In-License Agreement(s) and (ii) such license shall include terms not materially more onerous to Borrower than those contained in the applicable Specified Covered License Agreement(s) with respect to the obligations and costs imposed on Borrower and not materially less favorable with respect to the rights and remedies of Borrower. Borrower shall not pay (or enter into any agreement to pay) any upfront costs, fees or expenses to a third party in connection with Borrower's efforts to locate, negotiate and secure a New Arrangement ("New Arrangement Expenses") without the prior written consent of Lender. In no event shall Borrower be required to incur any obligation of any kind with respect to, and any directions provided by Lender under this Section 8.17 shall not include any direction regarding, the prosecution, maintenance, enforcement or defense of the Intellectual Property. If Lender does not consent to such New Arrangement Expenses, Lender may, upon written notice to Borrower, terminate the Initial Search Period. Following the expiration or termination of the Initial Search Period, Lender may, at Lender's option and sole cost and expense, continue efforts to locate, negotiate and secure a New Arrangement; provided that Borrower shall have the right to consent in writing to any New Arrangement, which approval shall not be unreasonably withheld or delayed. Borrower shall use commercially reasonable efforts, at Lender's request and sole cost and expense (including Lender's payment of Borrower's reasonable attorney's fees, if any, in connection therewith) to provide cooperation and assistance to Lender in connection with Lender's efforts pursuant to the foregoing sentence. In the event Borrower enters into a New Arrangement, references in this Agreement to the Included Royalty Interest and the applicable Specified Covered License Agreement(s) shall be deemed to be references to any new royalty or other payments and the new license agreement constructed under the New Arrangement, and references to a Licensee shall be deemed to be references to the other party to such New Arrangement. Such New Arrangement shall also provide, for no additional consideration from Lender (other than, for clarity, the costs and expenses described in this Section 8.17(b)), that (i) Lender shall have the same rights as those acquired under the applicable Specified Covered License Agreement(s) pursuant to this Agreement and (ii) all payments and other consideration (including any upfront fees) thereunder (to the extent that such payments or other consideration would have constituted a Royalty Interest under the applicable Specified Covered License Agreement(s)) be made by the other party to such New Arrangement directly to Lender; provided that all such payments and other consideration (including any upfront fees) made by the other party to such New Arrangement shall be deemed to be a Royalty Interest hereunder for purposes of determining the Included Royalty Interest for the applicable Calendar Quarter(s). All out-of-pocket third-party expenses of Borrower (including

reasonable attorney's fees) incurred pursuant to this Section 8.17(b), shall be promptly reimbursed by Lender.

Section 8.18 Performance of Covered License Agreements and In-License Agreements. Borrower agrees that it shall (i) not breach any of the Covered License Agreements or In-License Agreements, in each case, in any respect material to the interests of Lender hereunder and (ii) use commercially reasonable efforts to cure any such breach by Borrower of any Covered License Agreement or In-License Agreement.

Section 8.19 Amendment of Covered License Agreements; Amendment of In-License Agreements.

(a) Amendment of a Covered License Agreement. Borrower shall provide Lender a copy of any proposed amendment, supplement, modification or waiver (each, a "Modification") of any provision of a Covered License Agreement as soon as practicable (and in any event not less than [****] Business Days) prior to the date Borrower proposes to execute such Modification. Borrower shall not, without the prior written consent of Lender (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification of any Covered License Agreement if such Modification would reasonably be expected to adversely affect in any material respect the value of the Included Royalty Interest (it being understood and agreed that any proposed Modification to the provisions of any Covered License Agreement governing the amount or calculation of the Included Royalty Interest or the procedures for payment of the Included Royalty Interest shall be deemed, for purposes of this Section 8.19, to have such an effect). Promptly (and in any event within [****] Business Days) following receipt by Borrower of a fully executed Modification of any Covered License Agreement, Borrower shall furnish a copy of such Modification to Lender.

(b) Amendment of an In-License Agreement. Borrower shall not, without the prior written consent of Lender (such consent not to be unreasonably withheld or delayed), execute or agree to execute any proposed Modification to an In-License Agreement, to the extent such Modification would reasonably be expected to adversely affect in any material respect the value of the Included Royalty Interest (it being understood and agreed that any proposed Modification to the provisions of an In-License Agreement governing the amount or calculation of the payments of any Royalty Interest or the procedures for payment of such payments shall be deemed, for purposes of this Section 8.19, to have such an effect, and notwithstanding anything to the contrary in this Section 8.19, it shall be deemed reasonable for Lender to withhold its consent to any such proposed Modification to the extent adverse to Lender (as determined by Lender in its sole discretion) unless Borrower has concurrently offered to Lender to amend this Agreement to eliminate such adverse effect). For clarity, Modifications to In-License Agreements relating solely to patents and patent applications that do not relate to the Licensed Products in the Territory shall be deemed not to have an adverse effect and shall not require prior written consent of Lender.

Section 8.20 Enforcement of Covered License Agreements.

(a) Notice of Licensee's Breaches. Promptly (and in any event within [****] Business Days) following a Senior Officer of Borrower or the Company becoming aware of a

breach of a Covered License Agreement by the applicable Licensee that would reasonably be expected to adversely affect in any material respect the value of the Included Royalty Interest, Borrower shall provide notice of such breach to Lender.

(b) Enforcement of Covered License Agreements. Borrower and Lender shall consult with each other regarding any breach referred to in Section 8.20(a) and as to the timing, manner and conduct of any enforcement of the applicable Licensee's obligations under the Covered License Agreement relating thereto.

(i) Enforcement. Borrower may, and if requested in writing by Lender within [****] Business Days after receipt of notice of such breach pursuant to Section 8.20(a), shall, proceed in consultation with Lender, to use commercially reasonable efforts to enforce compliance by any Licensee with the relevant provisions of the applicable Covered License Agreement and to use commercially reasonable efforts to exercise such rights and remedies relating to such breach as shall be available to Borrower, whether under the applicable Covered License Agreement or by operation of Applicable Law.

(ii) Lead Counsel. In connection with any enforcement of a Licensee's obligations under the applicable Covered License Agreement in respect of any breach referred to in Section 8.20(a) (regardless of whether such enforcement is initiated by Borrower as a result of a written request from Lender or initiated by Borrower in the absence of any such request), the lead counsel selected by Borrower shall be such counsel as Lender shall recommend for such purpose (as long as such counsel is reasonably acceptable to Borrower).

(c) Allocation of Proceeds of Enforcement. The Proceeds of any enforcement of a Licensee's obligations under the applicable Covered License Agreement in respect of any breach referred to in Section 8.20(a) (regardless of whether such enforcement is initiated by Borrower as a result of a written request from Lender or initiated by Borrower in the absence of any such request), after deduction (and reimbursement to Borrower and Lender) of all costs and expenses (including attorneys' fees and expenses) incurred by Borrower and Lender in connection with such enforcement pursuant to the first two sentences of Section 8.20(d) Section 8.20(b)(ii) below, shall be paid to Borrower and Lender in proportion to their respective interests in the Proceeds (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be deemed to be in Lender's interest).

(d) Allocation of Costs of Enforcement. All costs and expenses (including attorneys' fees and expenses) incurred by Borrower in connection with any enforcement of a Licensee's obligations under the applicable Covered License Agreement in respect of any breach referred to in Section 8.20(a) shall be borne by Borrower and Lender (as such costs and expenses are incurred) in proportion to their respective interests in the Proceeds (taking into account any variation in such interests over different time periods, if applicable, and it being understood and agreed that in the event any Proceeds are reasonably likely to be used to pay principal, interest or any other fees or expenses due from time to time in respect of the Loan, such Proceeds shall be

deemed to be in Lender's interest), including any retainers or advances required by the lead counsel selected pursuant to Section 8.20(b)(ii) for such enforcement (and that are incurred by Borrower). Nothing contained herein shall limit Lender from retaining, at its sole cost, separate outside counsel who shall be permitted, where reasonably practicable, to consult with the lead counsel selected pursuant to Section 8.20(b)(ii) for such enforcement.

Section 8.21 Approval of Assignments of License Agreement.

(a) Assignments by the Licensee. Promptly (and in any event within [****] Business Days) following receipt by Borrower of a written request from a Licensee for consent to assign the applicable Covered License Agreement (in whole or in part) pursuant to such Covered License Agreement, if applicable, Borrower shall provide notice thereof to Lender. Borrower and Lender shall consult with each other regarding whether to grant such consent, and Borrower shall not grant or withhold such consent without the prior written consent of Lender (such consent of Lender not to be unreasonably withheld or delayed). Notwithstanding anything to the contrary contained in this Section 8.21, and for the avoidance of doubt, no consent of Lender shall be required in connection with any assignment by a Licensee to which Borrower does not have a consent under the applicable Covered License Agreement, including an assignment by such Licensee to a wholly owned Affiliate or in connection with a Change of Control (subject to meeting the requirements of the applicable Covered License Agreement).

(b) Assignments by Borrower. Borrower may not assign any Covered License Agreement (in whole or in part) without the prior written consent of Lender (such consent not to be unreasonably withheld or delayed); provided that no such consent shall be required in connection with (i) any assignment, sale or transfer (in whole or in part) of Borrower's right, title and interest in and to the Excluded Payments or the delegation of any of Borrower's duties with respect to the Excluded Payments, (ii) any assignment, sale or transfer of Borrower's right, title and interest in and to substantially all of the assets of Borrower related to, or necessary to perform Borrower's obligations in respect of, any Covered License Agreement and (iii) any assignment to an Affiliate, in which case Borrower shall remain responsible for the performance of this Agreement by such Affiliate.

(c) Copies of Assignments. Promptly (and in any event no later than [****] Business Days) following Borrower's, or the Company's, as applicable, receipt of any fully executed assignment of a Covered License Agreement by the applicable Licensee or Borrower, Borrower shall furnish a copy of such assignment to Lender.

Section 8.22 Rights Under Royalty Purchase Agreement. All rights of Lender under Section 8.17 through Section 8.21 are subject in all respects to the rights of the RPA Purchaser Rep and the RPA Purchasers under the RPA Transaction Documents.

Section 8.23 Acknowledgment and Agreement by Lender; Limitation of Borrower's and the Company's Duties and Obligations.

(a) Notwithstanding any provision of this Agreement (including other provisions of this Article VIII) to the contrary, nothing contained in this Agreement shall obligate

Borrower or the Company to take any action, or omit to take any action, that (i) would conflict with, violate or cause a violation of, contravene or cause a default under, any Covered License Agreement, any In-License Agreement or any Applicable Law or any judgment binding upon, or any guidelines or policies of, Borrower or the Company, (ii) would otherwise, in the judgment of Borrower or the Company (exercised reasonably), adversely affect in any material respect Borrower or the Company, including by means of exposing Borrower or the Company to material liability (whether in relation to the transactions contemplated by any Covered License Agreement, any In-License Agreement or otherwise), or (iii) would, or would involve any disclosure that would, result in the loss or waiver of any attorney-client privilege available to Borrower or the Company; provided that Borrower and the Company shall use their commercially reasonable efforts to implement arrangements that would permit such action, omission or disclosure while preserving such privilege.

(b) Notwithstanding any provision of this Agreement (including other provisions of this Article VIII) to the contrary, nothing contained in this Agreement shall be construed to prevent, limit or restrict Borrower or the Company from exercising, pursuing and enforcing its rights in respect of any Excluded Payments in a commercially reasonable manner; provided, however, that (i) all costs and expenses (including attorneys' fees and expenses) incurred by Borrower or the Company in connection therewith shall be borne solely by Borrower and the Company, (ii) neither Borrower nor the Company shall, in connection therewith, terminate any Covered License Agreement without the prior written consent of Lender (such consent not to be unreasonably withheld or delayed), and (iii) in connection therewith neither Borrower nor the Company shall execute or agree to execute any proposed Modification of any Covered License Agreement or any In-License Agreement other than pursuant to Section 8.19(a) and Section 8.19(b), as applicable.

Section 8.24 Nippon Shinyaku Notice and UoM Notice. No later than [****] days following the Closing Date, Borrower shall have delivered or shall have caused the Company to have delivered (a) the notice required to be delivered to Nippon Shinyaku under the Nippon Shinyaku License Agreement in the form attached hereto as Exhibit N (the "Nippon Shinyaku Notice") and (b) the notice required to be delivered to UoM under the UoM Agreement in the form attached hereto as Exhibit O (the "UoM Notice").

Article IX. NEGATIVE COVENANTS

Borrower covenants and agrees with Lender that, until Payment in Full:

Section 9.01 Activities of Borrower.

(a) Borrower shall not amend, modify, waive or terminate (other than expiration in accordance with its terms) any provision of, or permit or agree to the amendment, modification, waiver or termination (other than expiration in accordance with its terms) of any provision of, any of the Transaction Documents or Material Contracts, without the consent of Lender (x) in its sole and absolute discretion, if the related amendment, modification, waiver or termination could reasonably be expected to have an adverse effect, in any respect, on the timing,

amount or duration of, the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest and (y) with respect to any other matters, such consent not to be unreasonably withheld or delayed. Borrower shall not establish or acquire any Subsidiaries.

(b) Borrower shall not:

(i) fail to hold itself out to the public and all other persons as a legal entity separate from the owners of its Capital Stock and from any other person;

(ii) commingle its assets with assets of any other Person except in connection with, and for the limited purposes of, operation of the Escrow Account;

(iii) fail to conduct its business only in its own name, nor fail to comply with all organizational formalities necessary to maintain its separate existence;

(iv) fail to maintain separate financial statements, showing its assets and liabilities separate and apart from those of any other person nor have its assets listed on any financial statement of any other person; provided, however, that Borrower's assets may be included in a consolidated financial statement of its Affiliates in conformity with applicable provisions of GAAP (provided that such assets shall also be listed on Borrower's own separate balance sheet);

(v) fail to pay its own liabilities and expenses only out of its own funds; provided that the foregoing shall not prohibit the payment of any liabilities and expenses by the Company on behalf of Borrower so long as such payments are subject to reimbursement or are otherwise recorded as capital contributions or intercompany loans;

(vi) enter into any transaction with an Affiliate except transactions that are at prices and on terms and conditions that could be obtained on an arm's-length basis from unrelated Third Parties (it being agreed by the Parties that the Transaction Documents and the transactions contemplated or required thereby, including the use of proceeds thereof, comply with this requirement);

(vii) issue any securities of any kind except as contemplated by this Agreement and the other Transaction Documents;

(viii) fail to correct any known misunderstanding regarding its separate identity and not identify itself as a department or division of any other Person;

(ix) fail to maintain adequate capital in light of its contemplated business purpose, transactions and liabilities; provided, however, that the foregoing shall not require the holders of its Capital Stock to make additional capital contributions to Borrower;

(x) fail to cause the representatives of Borrower to act at all times with respect to Borrower consistently and in furtherance of the foregoing and in the best interests of Borrower;

(xi) make any payment or distribution of assets with respect to any obligation of any other person other than as required or permitted by the Transaction Documents or under trade or commercial agreements entered into in the ordinary course of business;

(xii) engage in any business activity other than activities required or permitted by the Transaction Documents and other activities related to the Covered License Agreements and any New Arrangement that is implemented hereunder and the borrowing, payment and repayment of amounts provided for hereunder and under the other Loan Documents and any activities ancillary or related thereto; or

(xiii) fail to timely file any tax returns or timely pay any Taxes as may be required under Law (including in its capacity as withholding agent) except for taxes contested in good faith by appropriate proceedings, diligently conducted, and for which adequate reserves are maintained in accordance with GAAP.

(c) Borrower shall not issue any Capital Stock in certificated form.

Section 9.02 Merger; Sale of Assets; Future License Agreements.

(a) Borrower shall not merge or consolidate with or into (whether or not Borrower is the Surviving Person) any other Person and Borrower will not sell, convey, assign, transfer, lease, sublease, license, sublicense or otherwise dispose of all or substantially all of Borrower's assets to any Person in a single transaction or series of related transactions; provided that nothing in this Section 9.02(a) shall prohibit a Change of Control of the Company; and provided further that, to the extent the Company is party to a Change of Control and is not the surviving Person, the acquiror or surviving Person shall expressly assume all obligations of the Company under the Transaction Documents to which the Company is a party, if such assumption does not occur by operation of law, in which case such surviving Person shall succeed to, and be substituted for, the Company under the Transaction Documents to which the Company is a party.

(b) Borrower shall not sell, assign, convey, transfer, lease, sublease, license, sublicense or otherwise dispose of (including by way of merger or consolidation) any right, title or interest in or to, the Material Contracts or the Included Royalty Interest, other than pursuant to Permitted Liens;

(c) Borrower shall not, and shall cause the Company not to, sell, assign, convey, transfer, lease, sublease, license, sublicense or otherwise dispose of any right, title or interest in or to the Penn Agreement, the Penn Patents Intercompany In-License Agreement and the Patents licensed to Borrower pursuant thereto, and the NAV Intercompany In-License Agreement and the Patents licensed to Borrower pursuant thereto, except as could not reasonably be expected to have an adverse effect, in any respect, on the timing, amount or duration of the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest;

(d) Notwithstanding anything to the contrary in Section 9.02(a)-(b), Lender's consent shall not be required for (i) the Company to enter into Out-Licenses that are excluded from the definition of "Future License Agreements" or (ii) Borrower to enter into any Future License

Agreement unless such Future License Agreement would be reasonably expected to impair in any material respect or have a material adverse effect on an Existing License Agreement; provided, that Borrower shall deliver a Licensee Instruction Letter to each Future Licensee directing that all amounts payable to Borrower under such Future License Agreement be paid to the Collection Account, and provided, further, that Borrower agrees to execute, or cause the Company to execute, any amendments to the Intercompany In-License Agreements or the Intercompany Out-License Agreements that are necessary for Borrower to enter into such Future License Agreement.

Section 9.03 Liens. Borrower shall not, and shall cause the Company not to, create or suffer to exist any Lien on or with respect to Collateral, except for Permitted Liens. Borrower shall not, and shall cause the Company not to, create or suffer to exist any Lien on or with respect to the Penn Agreement, the Penn Patents Intercompany In-License Agreement and the Patents licensed to Borrower pursuant thereto, or the NAV Intercompany In-License Agreement and the Patents licensed to Borrower pursuant thereto, except for Permitted Liens or Liens that could not reasonably be expected to have an adverse effect, in any respect, on the timing, amount or duration of the Included Royalty Interest or the right of Lender to receive the Included Royalty Interest.

Section 9.04 Investment Company Act. Neither Borrower nor any of its Subsidiaries shall be or become an investment company subject to registration under the Investment Company Act of 1940.

Section 9.05 Limitation on Additional Indebtedness. Borrower shall not, directly or indirectly, incur or suffer to exist any Indebtedness; provided that Borrower may incur:

(a) Indebtedness under this Agreement, the other Transaction Documents, the Covered License Agreements (including any New Arrangement related thereto) and the In-License Agreements, as applicable;

(b) Indebtedness representing obligations for the payment of money incurred in the ordinary course of business for goods or services rendered, unsecured, not overdue (unless subject to a good faith dispute);

(c) Indebtedness secured by Liens of any of the types described under clause (d) of the definition of Permitted Liens; and

(d) Indebtedness consisting of the financing of insurance premiums with the providers of such insurance or their affiliates in the ordinary course of business.

Section 9.06 Limitation on Transactions with Affiliates. Borrower shall not, directly or indirectly, enter into any transaction or series of related transactions or participate in any arrangement (including any purchase, sale, lease or exchange of assets or the rendering of any service) with any Affiliate other than the Transaction Documents or in the ordinary course of

business of Borrower upon fair and reasonable terms no less favorable to Borrower than it would obtain in a comparable arm's-length transaction with a Third Party.

Section 9.07 ERISA.

(a) Borrower shall not sponsor, maintain or contribute to, or agree to sponsor, maintain or contribute to, any employee benefit plan (as defined in Section 3(3) of ERISA) whether or not subject to ERISA, that could, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(b) Borrower shall not engage in a non-exempt prohibited transaction under Section 406 of ERISA or Section 4975 of the Code or in any transaction that, assuming that no assets of Lender are or are deemed to be Plan Assets, would cause any obligation or action taken or to be taken hereunder (or the exercise by Lender of any of its rights under the Note, this Agreement or the other Loan Documents) to be a non-exempt prohibited transaction under such provisions.

(c) Borrower shall not incur any liability with respect to any obligation to provide medical benefits with respect to any person beyond their retirement or other termination of service, other than coverage mandated by law, that could, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

Section 9.08 Dividends and Distributions. Borrower will not, directly or indirectly, make any dividends or other distributions to holders of its Capital Stock (i) except as permitted under Borrower's Organizational Documents and the Pledge Agreement or (ii) while an Event of Default or Prepayment Trigger has occurred and is continuing.

Section 9.09 Rights Under the Royalty Purchase Agreement. Nothing in this Article IX shall prevent Borrower from taking all such actions as are required by the RPA Transaction Documents.

**Article X.
EVENTS OF DEFAULT**

Section 10.01 Events of Default. If one or more of Events of Default occurs and is continuing, Lender shall be entitled to the remedies set forth in Section 10.02.

Section 10.02 Default Remedies. If any Event of Default shall occur and be continuing, Lender may, by Notice to Borrower, (a) exercise all rights and remedies available to Lender hereunder and under the other Loan Documents and applicable law (which exercise may be determined in its sole discretion and which such exercise shall not constitute an election of remedies), including enforcement of the security interests created thereby, (b) declare the Loan, all interest thereon and all other Obligations to be immediately due and payable, whereupon all such amounts shall become immediately due and payable, all without diligence, presentment, demand of payment, protest or further notice of any kind, which are expressly waived by Borrower

and (c) declare the obligations of Lender hereunder to be terminated, whereupon such obligations shall terminate; provided, however, that if any event of any kind referred to in clause (j) of the definition of “Event of Default” herein occurs as a result of an Insolvency Event of Borrower, all amounts payable hereunder by Borrower shall become immediately due and payable and Lender shall be entitled to exercise rights and remedies under the Loan Documents and applicable law without diligence, presentment, demand of payment, protest or notice of any kind (including any notice by Lender of a declaration requiring prepayment of the Loan under Section 3.02, should Lender so elect), all of which are hereby expressly waived by Borrower. Each Notice delivered pursuant to this Section 10.02 shall be effective when sent.

Section 10.03 Right of Set-off; Sharing of Set-off.

(a) If any amount payable hereunder is not paid as and when due, Borrower irrevocably authorizes Lender (i) to proceed, to the fullest extent permitted by Applicable Law, without prior notice, by right of set-off, bankers’ lien, counterclaim or otherwise, against any assets of Borrower in any currency that may at any time be in the possession of Lender or any of its Affiliates, to the full extent of all amounts payable to Lender hereunder or (ii) to charge to Borrower’s account with Lender or any of its Affiliates the full extent of all amounts payable by Borrower to Lender hereunder; provided, however, that Lender shall notify Borrower of the exercise of such right promptly following such exercise.

(b) If any Lender shall, by exercising any right of setoff or counterclaim or otherwise, obtain payment in respect of any principal of or interest on the Loan or other obligations owed to such Lender resulting in such Lender’s receiving payment of a proportion of the aggregate amount of the Loan and accrued interest thereon or other obligations owed to such Lender greater than its pro rata share thereof as provided herein, then Lender receiving such greater proportion shall (a) notify the other Lenders of such fact, and (b) purchase (for cash at face value) participations in the Loan and such other obligations of the other Lenders, or make such other adjustments as shall be equitable, so that the benefit of all such payments shall be shared by Lenders ratably in accordance with the aggregate amount of principal of and accrued interest on their respective Loan and other amounts owing them; provided that the provisions of this Section 10.03(b) shall (x) not be construed to apply to (A) any payment made by Borrower pursuant to and in accordance with the express terms of this Agreement or (B) any payment obtained by a Lender as consideration for the assignment of or sale of a participation in the Loan to any assignee and (y) only be applicable if there is more than one Lender.

Section 10.04 Rights Not Exclusive. The rights provided for herein are cumulative and are not exclusive of any other rights, powers, privileges or remedies provided by Law.

**Article XI.
INDEMNIFICATION**

Section 11.01 Losses.

(a) Borrower agrees to defend (subject to Indemnitees' selection of counsel), indemnify, pay and hold harmless, each Indemnitee from and against any and all Indemnified Liabilities, in all cases, arising, in whole or in part, out of or relating to any claim, notice, suit or proceeding commenced or threatened in writing (including, without limitation, by electronic means) by any Person (including any Governmental Entity) other than Borrower, the Company or any of Lender's Affiliates; provided that Borrower shall not have any obligation to any Indemnitee hereunder with respect to any Indemnified Liabilities to the extent such Indemnified Liabilities arise from (i) the gross negligence or willful misconduct of an Indemnitee, (ii) a breach by an Indemnitee of any Transaction Document or (iii) arise from any claim, action, suit, inquiry, litigation, investigation or proceeding that does not involve an act or omission of Borrower and that is brought by an Indemnitee against any other Indemnitee. To the extent that the undertakings to defend, indemnify, pay and hold harmless set forth in this Section 11.01 may be unenforceable in whole or in part because they violate of any law or public policy, Borrower shall contribute the maximum portion that it is permitted to pay and satisfy under applicable law to the payment and satisfaction of all Indemnified Liabilities incurred by Indemnitees or any of them. This Section 11.01 shall not apply with respect to Taxes other than any Taxes that represent losses, claims, damages, etc. arising from any non-Tax claim.

(b) To the extent permitted by applicable law, no Party shall assert, and each Party hereby waives, any claim against each other Party and such Party's Affiliates, directors, employees, attorneys or agents, on any theory of liability, for special, indirect, consequential or punitive damages (as opposed to direct or actual damages) (whether or not the claim therefor is based on contract, tort or duty imposed by any applicable legal requirement) arising out of, in connection with, as a result of, or in any way related to, this Agreement or any Loan Document or any agreement or instrument contemplated hereby or thereby or referred to herein or therein, the transactions contemplated hereby or thereby, the Loan or the use of the proceeds thereof or any act or omission or event occurring in connection therewith, and each Party hereby waives, releases and agrees not to sue upon any such claim or any such damages, whether or not accrued and whether or not known or suspected to exist in its favor.

Section 11.02 Assumption of Defense; Settlements. If Lender is entitled to indemnification under this Article XI with respect to any action or proceeding brought by a third party that is also brought against Borrower, Borrower shall be entitled to assume the defense of any such action or proceeding with counsel reasonably satisfactory to Lender. Upon assumption by Borrower of the defense of any such action or proceeding, Lender shall have the right to participate in such action or proceeding and to retain its own counsel but Borrower shall not be liable for any legal expenses of other counsel subsequently incurred by Lender in connection with

the defense thereof unless (i) Borrower has otherwise agreed to pay such fees and expenses, (ii) Borrower shall have failed to employ counsel reasonably satisfactory to Lender in a timely manner or (iii) Lender shall have been advised by counsel that there are actual or potential conflicting interests between Borrower and Lender, including situations in which there are one or more legal defenses available to Lender that are different from or additional to those available to Borrower; **provided, however,** that Borrower shall not, in connection with any one such action or proceeding or separate but substantially similar actions or proceedings arising out of the same general allegations, be liable for the fees and expenses of more than one separate firm of attorneys at any time for Lender, except to the extent that local counsel, in addition to its regular counsel, is required in order to effectively defend against such action or proceeding. Borrower shall not consent to the terms of any compromise or settlement of any action defended by Borrower in accordance with the foregoing without the prior written consent of Lender unless such compromise or settlement (x) includes an unconditional release of Lender from all liability arising out of such action and (y) does not include a statement as to or an admission of fault, culpability or a failure to act, by or on behalf of Lender. Borrower shall not be required to indemnify Lender for any amount paid or payable by Lender in the settlement of any action, proceeding or investigation without the written consent of Borrower, which consent shall not be unreasonably withheld, conditioned or delayed.

**Article XII.
MISCELLANEOUS**

Section 12.01 Assignments.

(a) Borrower shall not be permitted to assign this Agreement without the prior written consent of Lender and any purported assignment in violation of this Section 12.01 shall be null and void.

(b) Lender may at any time assign its rights and obligations hereunder, in whole or in part, to an Assignee and Lender may at any time pledge its rights and obligations hereunder to an Assignee, in each case as set forth on Schedule 12.01.

(c) The parties to each assignment shall execute and deliver to Borrower an Assignment and Acceptance. Upon an assignment pursuant to Section 12.01(b) hereunder, (i) each reference in this Agreement to a "Lender" shall be deemed to be a reference to the assignor and the assignee to the extent of their respective interests, (ii) such assignee shall be a Lender party to this Agreement and shall have all the rights and obligations of a Lender and (iii) the assignor shall be released from its obligations hereunder to a corresponding extent of the assignment, and no further consent or action by any party shall be required, but shall continue to be entitled to the benefits of Article V.

(d) In the event there are multiple Lenders, all payments of principal, interest, fees and any other amounts payable pursuant to the Loan Documents shall be allocated on a *pro rata* basis among Lenders according to their proportionate interests in the Loan.

(e) Borrower and Lender shall, from time to time at the request of the other party hereto, execute and deliver any documents that are necessary to give full force and effect to an assignment permitted hereunder, including a new Note in exchange for the Note held by Lender.

Section 12.02 Successors and Assigns. Subject to the provisions of **Section 12.01** and **Schedule 12.01**, this Agreement shall be binding upon, inure to the benefit of and be enforceable by, the parties hereto and their respective permitted successors and assigns.

Section 12.03 Notices. All Notices and other communications under this Agreement to a party hereto shall be in writing and shall be sent by email with PDF attachment, internationally recognized overnight delivery service or personal delivery to the following address of such party, or to such other address as shall be designated from time to time by such party in accordance with this **Section 12.03**:

(a) If to Borrower:

c/o REGENXBIO Inc.
9804 Medical Center Drive
Rockville, MD 20850
Attention: Chief Legal Officer
Email: legal@regenxbio.com

With a copy (which shall not constitute notice) to:

Covington & Burling LLP
The New York Times Building
620 Eighth Avenue
New York, NY 10018
Attention: Peter A. Schwartz
Email: pschwartz@cov.com

(b) If to Lender:

c/o HCRX Investments HoldCo, L.P.
300 Atlantic Street, Suite 600
Stamford, CT 06901
Attention: Clarke Futch;
Anthony Rapsomanikis
Email: Clarke.Futch@hcrx.com;
Anthony.Rapsomanikis@hcrx.com;

with a copy (which shall not constitute notice) to:

c/o HCRX Investments HoldCo, L.P.
300 Atlantic Street, Suite 600
Stamford, CT 06901
Attention: Chief Legal Officer
Email: royalty-legal@hcroyalty.com

with a copy (which shall not constitute notice) to:

Morgan, Lewis & Bockius LLP
2222 Market Street
Philadelphia, PA 19103
Attention: Andrew Mariniello;
Conor F. Larkin
Email: andrew.mariniello@morganlewis.com;
conor.larkin@morganlewis.com

Any Party may change its address for the receipt of Notices at any time by giving Notice thereof to the other Party. Except as otherwise provided herein, any Notice authorized or required to be given by this Agreement shall be effective when received.

Section 12.04 Entire Agreement. This Agreement, including the Exhibits and Schedules attached to this Agreement, together with the Loan Documents, sets forth the entire agreement and understanding between the parties hereto as to the subject matter hereof. All express or implied agreements, promises, assurances, arrangements, representations, warranties and understandings as to the subject matter hereof, whether oral or written, heretofore made are superseded by this Agreement.

Section 12.05 Modification. No Loan Document or provision thereof may be waived, amended or modified except, in the case of this Agreement, by an agreement or agreements in writing executed by Borrower and Lender or, in the case of any other Loan Document, by an agreement or agreements in writing entered into by the parties thereto with the prior written consent of Lender.

Section 12.06 No Delay; Waivers; etc. No delay on the part of Lender in exercising any power or right hereunder shall operate as a waiver thereof nor shall any single or partial exercise of any power or right hereunder preclude other or further exercise thereof or the exercise of any other power or right. No Lender shall not be deemed to have waived any rights hereunder unless such waiver shall be in writing and signed by Lender.

Section 12.07 Severability. If any term or provision of this Agreement is held to be invalid, illegal or unenforceable by a court, arbitrator or Governmental Entity of competent jurisdiction, such invalidity, illegality or unenforceability shall not affect any other term or provision of this Agreement, which shall remain in full force and effect, and the parties hereto shall replace such term or provision with a new term or provision permitted by applicable Law and

having an economic effect as close as possible to the invalid, illegal or unenforceable term or provision. The holding of a term or provision to be invalid, illegal or unenforceable in a jurisdiction shall not have any effect on the application of such term or provision in any other jurisdiction.

Section 12.08 Determinations. Each determination or calculation by Lender hereunder shall, in the absence of manifest error, be conclusive and binding on the Parties.

Section 12.09 Replacement of Note. Upon the loss, theft, destruction, or mutilation of the Note and (a) in the case of loss, theft or destruction, upon receipt by Borrower of indemnity or security reasonably satisfactory to it (except that if the holder of such Note is Lender or any other financial institution of recognized responsibility, the holder's own agreement of indemnity shall be deemed to be satisfactory) or (b) in the case of mutilation, upon surrender to Borrower of any mutilated Note, Borrower shall execute and deliver in lieu thereof a new Note, dated the Closing Date, in the same Principal Amount.

Section 12.10 Governing Law. This Agreement shall be governed by and construed and interpreted in accordance with the Laws of State of New York without regard to the conflicts of Laws principles thereof to the extent that such principles would require or permit the application of the Laws of a jurisdiction other than the State of New York.

Section 12.11 Jurisdiction. Each party hereto irrevocably submits to the exclusive jurisdiction of (a) the courts of the State of New York located in New York County, New York and (b) the U.S. District Court for the Southern District of New York for the purposes of any suit, action or other proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby. Each party hereto agrees to commence any action, suit or other proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby in the U.S. District Court for the Southern District of New York or if such suit, action or other proceeding may not be brought in such court for jurisdictional reasons, in the courts of the State of New York located in New York County, New York. Each party hereto irrevocably and unconditionally waives any objection to the laying of venue of any action, suit or other proceeding arising out of, relating to or in connection with this Agreement or any transaction contemplated hereby in (i) the courts of the State of New York located in New York County, New York or (ii) the U.S. District Court for the Southern District of New York, and hereby further irrevocably and unconditionally waives, and shall not assert by way of motion, defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper, or that this Agreement and the transactions contemplated hereby and thereby may not be enforced in or by any of the above-named courts.

Section 12.12 Waiver of Jury Trial. EACH PARTY HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY AND ALL RIGHT TO TRIAL BY JURY IN ANY ACTION, PROCEEDING, CLAIM OR COUNTERCLAIM ARISING OUT OF OR RELATING TO ANY TRANSACTION DOCUMENT OR THE TRANSACTIONS CONTEMPLATED

UNDER ANY TRANSACTION DOCUMENT (WHETHER BASED ON CONTRACT, TORT OR ANY OTHER THEORY). THIS WAIVER SHALL APPLY TO ANY SUBSEQUENT AMENDMENTS, RENEWALS, SUPPLEMENTS OR MODIFICATIONS TO ANY TRANSACTION DOCUMENT. EACH PARTY HERETO (A) CERTIFIES THAT NO REPRESENTATIVE, AGENT OR ATTORNEY OF THE OTHER PARTY HERETO HAS REPRESENTED, EXPRESSLY OR OTHERWISE, THAT THE OTHER PARTY HERETO WOULD NOT, IN THE EVENT OF LITIGATION, SEEK TO ENFORCE THE FOREGOING WAIVER AND (B) ACKNOWLEDGES THAT IT AND THE OTHER PARTY HERETO HAVE BEEN INDUCED TO ENTER INTO THIS AGREEMENT BY, AMONG OTHER THINGS, THE MUTUAL WAIVERS AND CERTIFICATIONS IN THIS SECTION 12.12.

Section 12.13 Waiver of Immunity. To the extent that Borrower has or hereafter may be entitled to claim or may acquire, for itself or any of its assets, any immunity from suit, jurisdiction of any court or from any legal process (whether through service or notice, attachment prior to judgment, attachment in aid of execution, or otherwise) with respect to itself or any of its property, Borrower hereby irrevocably waives such immunity in respect of its obligations hereunder and under the Note to the fullest extent permitted by law.

Section 12.14 Counterparts. This Agreement may be executed in any number of counterparts and by the parties hereto in separate counterparts, each of which when so executed shall be deemed to be an original and all of which taken together shall constitute one and the same agreement. Copies of executed counterparts transmitted by email with PDF attachment shall be considered original executed counterparts.

Section 12.15 Limitation on Rights of Others. Except for the Indemnitees referred to in Section 11.01, no Person other than a Party shall have any legal or equitable right, remedy or claim under or in respect of this Agreement.

Section 12.16 Survival.

(a) The obligations of Borrower contained in Sections 4.06, 4.07, Article V, Article XI and this Section 12.16 shall survive the repayment of the Loan, assignment of the Loan by Lender and the cancellation of the Note and the termination of the other obligations of Borrower hereunder.

(b) All representations and warranties by Borrower, whether with respect to Borrower, the Company, any respective Affiliate or any asset or property, contained in this Agreement shall survive the execution, delivery and acceptance thereof by the Parties and the closing of the transactions described in this Agreement and continue in effect until payment of all amounts due to Lender under the Loan Documents.

Section 12.17 Confidentiality.

(a) Until the payment of all amounts required pursuant to Section 3.01, and for a period of three years thereafter, each Party shall maintain in strict confidence all Confidential

Information and materials disclosed or provided to it by the other Party, except as approved in writing in advance by the disclosing Party, and shall not use or reproduce the disclosing Party's Confidential Information for any purpose other than as required to carry out its obligations and exercise its rights pursuant to this Agreement. Notwithstanding the foregoing, the obligations of confidentiality and non-use set forth in this Section 12.17 shall not apply to the extent that the receiving Party or its Affiliates: (a) discloses such Confidential Information solely on a "need to know basis" to its employees, consultants and Affiliates as well as any actual or potential acquirers, merger partners, licensees, permitted assignees, collaborators (including licensees), subcontractors, investment bankers, investors, limited partners, partners, lenders, or other financial partners, and its and their respective directors, employees, contractors and agents, on a confidential basis to the extent requested by an authorized representative of a U.S. or foreign tax authority, or (b) discloses Confidential Information in response to a routine audit or examination by, or a blanket document request from, a Governmental Entity. A Party receiving any such Confidential Information hereunder agrees to institute measures to protect the Confidential Information in a manner consistent with the measures it uses to protect its own most sensitive proprietary and confidential information, which in any event must not be less than a reasonable standard of care. Each Party shall be responsible for the breach of this Section 12.17 by its employees, consultants or Third Parties to whom such disclosure is made pursuant to this Section 12.17. Each Party shall immediately notify the other Party upon discovery of any loss or unauthorized disclosure of the other Party's Confidential Information.

(b) The obligations of confidentiality and non-use set forth in Section 12.17(a) shall not apply to the extent that the receiving Party or its Affiliates is required to disclose Confidential Information pursuant to: (i) an order of a court of competent jurisdiction; (ii) Applicable Laws; (iii) regulations or rules of a securities exchange; or (iv) requirement of a Governmental Entity.

(c) Effective upon the date hereof, the Confidentiality Agreement shall terminate and be of no further force or effect, and shall be superseded by the provisions of this Section 12.17.

Section 12.18 Patriot Act Notification. Lender hereby notifies Borrower that pursuant to the requirements of the Patriot Act, regulations promulgated thereunder and under other Applicable Law, Lender is required to obtain, verify and record information that identifies each Borrower and the Company, which information includes the name and address of each such party and other information that will allow Lender to identify each such party in accordance with the Patriot Act. The Company and Borrower agree to, promptly following a request by Lender, provide all such other documentation and information that Lender requests in order to comply with its ongoing obligations under applicable "know your customer" and anti-money laundering rules and regulations, including the Patriot Act.

Section 12.19 Electronic Execution; Counterparts. This Agreement, any Transaction Document and any other Notice, including Notice required to be in writing, may be in the form of an Electronic Record and may be executed using Electronic Signatures. This Agreement may be executed in any number of counterparts and by the parties hereto in separate counterparts, each of which when so executed shall be deemed to be an original and all of which

taken together shall constitute one and the same agreement. Copies of executed counterparts transmitted by email with PDF attachment shall be considered original executed counterparts.

[Signature page follows.]

IN WITNESS WHEREOF, the Parties have duly executed this Agreement as of the day and year first above written.

HCR RGNX ROYALTY SPV, LP,
as Lender

By: HCR RGNX ROYALTY SPV GP, LLC,
its general partner

By: /s/ Clarke B. Futch
Name: Clarke B. Futch
Title: Chief Executive Officer

REGENXBIO RS LLC,
as Borrower

By: /s/ Curran Simpson
Name: Curran Simpson
Title: Chief Executive Officer

CERTIFICATION

I, Curran Simpson, certify that:

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

Date: August 7, 2025

/s/ Curran Simpson

Curran Simpson
President and Chief Executive Officer
(Principal Executive Officer)

CERTIFICATION

I, Mitchell Chan, certify that:

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

Date: August 7, 2025

/s/ Mitchell Chan

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

CERTIFICATION

In connection with the Quarterly Report of REGENXBIO Inc. (the "Registrant") on Form 10-Q for the quarter ended June 30, 2025 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Curran Simpson, President, Chief Executive Officer and Director of the Registrant, and Mitchell Chan, Chief Financial Officer of the Registrant, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to their respective knowledge:

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

Date: August 7, 2025

/s/ Curran Simpson

Curran Simpson
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 7, 2025

/s/ Mitchell Chan

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

This certification is made solely for the purposes of 18 U.S.C. Section 1350, subject to the knowledge standard contained therein, and not for any other purpose. A signed original of this written statement required by Section 906 has been provided to the Registrant and will be retained by the Registrant and furnished to the United States Securities and Exchange Commission or its staff upon request.

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933 or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.
