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, 2019

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 001-33497

Amicus Therapeutics, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**71-0869350**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification Number)
1 Cedar Brook Drive, Cranbury, NJ	**08512**
(Address of Principal Executive Offices)	(Zip Code)

(609) 662-2000

(Registrant's Telephone Number, Including Area Code)

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	FOLD	NASDAQ Global Market

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

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

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

Large accelerated filer ☒	Accelerated filer ☐
Non-accelerated filer ☐	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 ☒

The number of shares outstanding of the registrant's common stock, $0.01 par value per share, as of July 30, 2019 was 254,626,063 shares.

AMICUS THERAPEUTICS, INC.

Form 10-Q for the Quarterly Period Ended June 30, 2019

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks, uncertainties, and assumptions. Forward-looking statements are all statements, other than statements of historical facts, that discuss our current expectations and projections relating to our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans, and objectives of management. These statements may be preceded by, followed by, or include the words "aim," "anticipate," "believe," "can," "could," "estimate," "expect," "forecast," "intend," "likely," "may," "outlook," "plan," "potential," "predict," "project," "seek," "should," "will," "would," the negatives or plurals thereof, and other words and terms of similar meaning, although not all forward-looking statements contain these identifying words.

We have based these forward-looking statements on our current expectations and projections about future events. Although we believe that our assumptions made in connection with the forward-looking statements are reasonable, we cannot assure you that the assumptions and expectations will prove to be correct. You should understand that the following important factors could affect our future results and could cause those results or other outcomes to differ materially from those expressed or implied in our forward-looking statements:

- the progress and results of our preclinical and clinical trials of our drug candidates;
- the cost of manufacturing drug supply for our clinical and preclinical studies, including the cost of manufacturing Pompe Enzyme Replacement Therapy ("ERT") and gene therapies;
- the scope, progress, results, and costs of preclinical development, laboratory testing, and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of lysosomal storage disorders and gene therapies for the treatment of rare genetic metabolic diseases;
- the future results of on-going preclinical research and subsequent clinical trials for cyclin-dependent kinase-like 5 ("CDKL5") deficiency, including our ability to obtain regulatory approvals and commercialize CDKL5 therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales, and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to successfully commercialize Galafold® ("migalastat HCl");
- our ability to manufacture or supply sufficient clinical or commercial products;
- our ability to obtain reimbursement for Galafold®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold®;
- our ability to obtain market acceptance of Galafold®;
- the costs of preparing, filing, and prosecuting patent applications and maintaining, enforcing, and defending intellectual property-related claims;
- the extent to which we acquire or invest in businesses, products, and technologies;
- our ability to successfully integrate our acquired products and technologies into our business, including the possibility that the expected benefits of the transactions will not be fully realized by us or may take longer to realize than expected;
- our ability to establish collaborations and obtain milestone, royalty, or other payments from any such collaborators;
- our ability to adjust to changes in European and United Kingdom markets as the United Kingdom leaves the European Union;
- fluctuations in foreign currency exchange rates; and
- changes in accounting standards.

In light of these risks and uncertainties, we may not actually achieve the plans, intentions, or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions, and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in Part I Item 1A — Risk Factors of the Annual Report on Form 10-K for the fiscal year ended December 31, 2018, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Those factors and the other risk factors described therein are not necessarily all of the important factors that could cause actual results or developments to differ materially from those expressed in any of our forward-looking statements. Other unknown or unpredictable factors also could harm our results. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, collaborations, or investments we may make. Consequently, there can be no assurance that actual results or developments anticipated by us will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, us. Given these uncertainties, investors are cautioned not to place undue reliance on such forward-looking statements.

You should read this Quarterly Report on Form 10-Q in conjunction with our Annual Report on Form 10-K for the fiscal year ended December 31, 2018 completely and with the understanding that our actual future results may be materially different from what we expect. These forward-looking statements speak only as of the date of this report. We undertake no obligation, and specifically decline any obligation, to publicly update or revise any forward-looking statements, even if experience or future developments make it clear that projected results expressed or implied in such statements will not be realized, except as may be required by law.

PART I. FINANCIAL INFORMATION

ITEM 1. CONSOLIDATED FINANCIAL STATEMENTS AND NOTES (UNAUDITED)

Amicus Therapeutics, Inc.
Consolidated Balance Sheets
(Unaudited)
(in thousands, except share and per share amounts)

	June 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 220,578	$ 79,749
Investments in marketable securities	355,078	424,403
Accounts receivable	28,709	21,962
Inventories	10,395	8,390
Prepaid expenses and other current assets	20,116	16,592
Total current assets	634,876	551,096
Operating lease right-of-use assets, less accumulated amortization of $2,641 and $0 at June 30, 2019 and December 31, 2018, respectively	35,052	—
Property and equipment, less accumulated depreciation of $16,890 and $15,671 at June 30, 2019 and December 31, 2018, respectively	15,273	11,375
In-process research & development	23,000	23,000
Goodwill	197,797	197,797
Other non-current assets	12,035	6,683
Total Assets	$ 918,033	$ 789,951
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable, accrued expenses, and other current liabilities	$ 84,119	$ 80,625
Deferred reimbursements	2,750	5,500
Operating lease liabilities	2,678	—
Total current liabilities	89,547	86,125
Deferred reimbursements	11,406	10,156
Convertible notes	2,070	175,006
Senior secured term loan	146,994	146,734
Contingent consideration payable	21,247	19,700
Deferred income taxes	6,465	6,465
Operating lease liabilities	36,259	—
Other non-current liabilities	3,987	2,853
Total liabilities	317,975	447,039
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value, 500,000,000 shares authorized, 254,513,522 and 189,383,924 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	2,589	1,942
Additional paid-in capital	2,201,447	1,740,061
Accumulated other comprehensive loss:		
Foreign currency translation adjustment	352	495
Unrealized gain (loss) on available-for-sale securities	355	(427)
Warrants	12,387	13,063
Accumulated deficit	(1,617,072)	(1,412,222)
Total stockholders' equity	600,058	342,912
Total Liabilities and Stockholders' Equity	$ 918,033	$ 789,951

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Operations
(Unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Revenue:				
Net product sales	$ 44,130	$ 21,309	$ 78,176	$ 38,005
Cost of goods sold	5,367	3,135	9,422	5,750
Gross profit	38,763	18,174	68,754	32,255
Operating expenses:				
Research and development	70,981	34,660	135,574	75,458
Selling, general, and administrative	42,578	29,172	86,881	56,568
Changes in fair value of contingent consideration payable	480	300	1,863	1,400
Depreciation and amortization	1,154	973	2,145	1,942
Total operating expenses	115,193	65,105	226,463	135,368
Loss from operations	(76,430)	(46,931)	(157,709)	(103,113)
Other income (expense):				
Interest income	2,599	2,913	5,238	4,650
Interest expense	(4,625)	(4,560)	(11,079)	(9,048)
Loss on exchange of convertible notes	(4,501)	—	(40,624)	—
Change in fair value of derivatives	—	(7,600)	—	(2,739)
Other income (expense)	(877)	(5,316)	209	(2,554)
Loss before income tax	(83,834)	(61,494)	(203,965)	(112,804)
Income tax (expense) benefit	(717)	(339)	(885)	1,053
Net loss attributable to common stockholders	$ (84,551)	$ (61,833)	$ (204,850)	$ (111,751)
Net loss attributable to common stockholders per common share — basic and diluted	$ (0.36)	$ (0.33)	$ (0.91)	$ (0.61)
Weighted-average common shares outstanding — basic and diluted	238,089,824	188,621,423	225,848,013	182,303,128

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Comprehensive Loss
(Unaudited)
(in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Net loss	$ (84,551)	$ (61,833)	$ (204,850)	$ (111,751)
Other comprehensive (loss) gain:				
Foreign currency translation adjustment gain (loss), net of tax impact of $(30), $(109), $(30) and $(109), respectively	1,881	2,308	77	503
Unrealized (loss) gain on available-for-sale securities, net of tax impact of $(220), $0, $(220) and $0, respectively	(22)	422	562	(19)
Other comprehensive income (loss)	$ 1,859	$ 2,730	$ 639	$ 484
Comprehensive loss	$ (82,692)	$ (59,103)	$ (204,211)	$ (111,267)

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Changes in Stockholders' Equity
(Unaudited)
(in thousands, except share amounts)

Six Months Ended June 30, 2019

	Common Stock		Additional Paid-In Capital	Warrants	Other Comprehensive Gain (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2018	189,383,924	$ 1,942	$ 1,740,061	$ 13,063	$ 68	$ (1,412,222)	$ 342,912
Stock issued from exercise of stock options, net	578,451	6	3,947	—	—	—	3,953
Restricted stock tax vesting	301,058	—	(1,940)	—	—	—	(1,940)
Stock issued for contingent consideration	771,804	8	9,308	—	—	—	9,316
Stock-based compensation	—	—	12,744	—	—	—	12,744
Warrants exercised	101,787	1	1,487	(676)	—	—	812
Equity component of the convertible notes	39,043,690	390	190,368	—	—	—	190,758
Termination of capped call confirmations	—	—	14,632	—	—	—	14,632
Unrealized holding gain on available-for-sale securities	—	—	—	—	584	—	584
Foreign currency translation adjustment	—	—	—	—	(1,804)	—	(1,804)
Net loss	—	—	—	—	—	(120,299)	(120,299)
Balance at March 31, 2019	230,180,714	$ 2,347	$ 1,970,607	$ 12,387	$ (1,152)	$ (1,532,521)	$ 451,668
Stock issued from exercise of stock options, net	561,177	5	2,802	—	—	—	2,807
Stock issued from equity financing	18,720,930	187	188,807	—	—	—	188,994
Restricted stock tax vesting	99,252	—	(615)	—	—	—	(615)
Stock-based compensation	—	—	9,935	—	—	—	9,935
Equity component of the convertible notes	4,951,449	50	24,668	—	—	—	24,718
Termination of capped call confirmations	—	—	5,243	—	—	—	5,243
Unrealized holding loss on available-for-sale securities	—	—	—	—	(22)	—	(22)
Foreign currency translation adjustment	—	—	—	—	1,881	—	1,881
Net loss	—	—	—	—	—	(84,551)	(84,551)
Balance at June 30, 2019	254,513,522	$ 2,589	$ 2,201,447	$ 12,387	$ 707	$ (1,617,072)	$ 600,058

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Changes in Stockholders' Equity
(Unaudited)
(in thousands, except share amounts)

Six Months Ended June 30, 2018

	Common Stock		Additional Paid-In Capital	Warrants	Other Comprehensive Gain (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2017	166,989,790	$ 1,721	$ 1,400,758	$ 16,076	$ (2,095)	$ (1,063,610)	$ 352,850
Stock issued from exercise of stock options, net	560,721	6	3,972	—	—	—	3,978
Stock issued from equity financing	20,239,839	202	294,382	—	—	—	294,584
Restricted stock tax vesting	181,868	—	(1,912)	—	—	—	(1,912)
Stock-based compensation	—	—	7,478	—	—	—	7,478
Reclassification upon ASU 2018-02 adoption	—	—	—	—	(383)	383	—
Change in fair value of derivatives	—	—	(83,199)	—	—	—	(83,199)
Unrealized holding loss on available-for-sale securities	—	—	—	—	(441)	—	(441)
Foreign currency translation adjustment	—	—	—	—	(1,805)	—	(1,805)
Net loss	—	—	—	—	—	(49,916)	(49,916)
Balance at March 31, 2018	187,972,218	$ 1,929	$ 1,621,479	$ 16,076	$ (4,724)	$ (1,113,143)	$ 521,617
Stock issued from exercise of stock options, net	542,056	5	3,597	—	—	—	3,602
Restricted stock tax vesting	85,726	—	(115)	—	—	—	(115)
Stock-based compensation	—	—	6,341	—	—	—	6,341
Warrants exercised	453,214	5	6,625	(3,013)	—	—	3,617
Change in fair value of derivatives	—	—	85,938	—	—	—	85,938
Unrealized holding gain on available-for-sale securities	—	—	—	—	422	—	422
Foreign currency translation adjustment	—	—	—	—	2,308	—	2,308
Net loss	—	—	—	—	—	(61,833)	(61,833)
Balance at June 30, 2018	189,053,214	$ 1,939	$ 1,723,865	$ 13,063	$ (1,994)	$ (1,174,978)	$ 561,895

See accompanying notes to consolidated financial statements

Amicus Therapeutics, Inc.
Consolidated Statements of Cash Flows
(Unaudited)
(in thousands)

	Six Months Ended June 30,	
	2019	**2018**
Operating activities		
Net loss	$ (204,850)	$ (111,751)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of debt discount and deferred financing	2,104	5,273
Depreciation and amortization	2,145	1,942
Stock-based compensation	22,679	13,819
Loss on exchange of convertible debt	40,624	—
Change in fair value of derivatives	—	2,739
Non-cash changes in the fair value of contingent consideration payable	1,863	1,400
Foreign currency remeasurement loss	530	2,107
Changes in operating assets and liabilities:		
Accounts receivable	(6,829)	(6,043)
Inventories	(1,908)	(3,449)
Prepaid expenses and other current assets	(3,523)	4,584
Accounts payable and accrued expenses	12,453	(11,801)
Other non-current assets and liabilities	(819)	(336)
Deferred reimbursements	(1,500)	(5,000)
Net cash used in operating activities	$ (137,031)	$ (106,516)
Investing activities		
Sale and redemption of marketable securities	261,425	210,239
Purchases of marketable securities	(191,318)	(380,234)
Capital expenditures	(5,110)	(1,881)
Net cash provided by (used in) investing activities	$ 64,997	$ (171,876)
Financing activities		
Proceeds from issuance of common stock, net of issuance costs	188,994	294,584
Payment of finance leases	(98)	(142)
Purchase of vested restricted stock units	(2,555)	(2,027)
Proceeds from termination of capped call confirmations	19,875	—
Proceeds from exercise of stock options	6,760	7,580
Proceeds of exercise of warrants	812	3,617
Net cash provided by financing activities	$ 213,788	$ 303,612
Effect of exchange rate changes on cash, cash equivalents, and restricted cash	$ (941)	$ (1,016)
Net increase in cash, cash equivalents, and restricted cash	140,813	24,204
Cash, cash equivalents, and restricted cash at beginning of period	$ 82,375	$ 51,237
Cash, cash equivalents, and restricted cash at end of period	$ 223,188	$ 75,441
Supplemental disclosures of cash flow information		
Cash paid during the period for interest	$ 9,302	$ 3,774
Capital expenditures, unpaid	$ 837	$ —
Capital expenditures funded by capital lease	$ —	$ 81
Payment of contingent consideration in shares	$ 9,316	$ —

See accompanying notes to consolidated financial statements

Note 1. Business

Amicus Therapeutics, Inc. (the "Company") is a global patient-dedicated biotechnology company engaged in the discovery, development, and commercialization of a diverse set of novel treatments for patients living with rare metabolic diseases. With one medicine for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease in the clinic, and an industry leading rare disease gene therapy portfolio.

The cornerstone of the Company's portfolio is Galafold® (also referred to as "migalastat"), the first and only approved oral precision medicine for people living with Fabry disease who have amenable genetic variants. Migalastat is currently approved under the trade name Galafold® in the United States ("U.S."), European Union ("E.U."), and Japan, with additional approvals granted and applications pending in several other geographies. During the third quarter of 2018, the Company initiated the commercial launch of Galafold® in the U.S. for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant.

The lead biologics program of the Company's pipeline is Amicus Therapeutics GAA ("AT-GAA", also known as ATB200/AT2221), a novel, clinical-stage, potential best-in-class treatment paradigm for Pompe disease. In February 2019, the U.S. Food and Drug Administration ("FDA") granted Breakthrough Therapy Designation to AT-GAA for the treatment of late onset Pompe disease.

The Company has established an industry leading gene therapy portfolio of potential therapies for people living with rare metabolic diseases, through a license with Nationwide Children's Hospital ("NCH") and a recently expanded collaboration with the University of Pennsylvania ("Penn"). The Company's pipeline includes gene therapy programs in rare, neurologic lysosomal disorders ("LDs") with programs in CLN6, CLN3, and CLN8 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD"), Niemann-Pick Type C (“NPC”), Mucopolysaccharidosis Type IIIB ("MPSIIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPSIIIA"). This expanded collaboration with Penn also provides the Company with exclusive disease-specific access and option rights to develop potentially disruptive new gene therapy platform technologies and programs for most LDs and a broader portfolio of rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystophy, and select other muscular dystrophies.

During the second quarter of 2019, the Company completed an underwritten equity offering and issued 18.7 million shares of its common stock at $10.75 per share, inclusive of the fully exercised option to purchase additional shares from the initial offering. This transaction resulted in net proceeds of $189.0 million, after deducting underwriting discounts and commissions and offering expenses.

During the six months ended June 30, 2019, the Company entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with a limited number of holders (the "Holders") of the unsecured Convertible Senior Notes due in 2023 ("Convertible Notes"). Under the terms of the Exchange Agreements, the Holders agreed to exchange an aggregate principal amount of $247.2 million of Convertible Notes held by them in exchange for an aggregate of approximately 44.0 million shares of the Company common stock, par value $0.01 per share.

The Company had an accumulated deficit of $1.6 billion as of June 30, 2019 and anticipates incurring losses through the fiscal year ending December 31, 2019 and beyond. The Company has historically funded its operations through stock offerings, debt issuances, Galafold® revenues, collaborations, and other financing arrangements.

The current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe, and gene therapy program operations into 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact the Company's future capital requirements.

Note 2. Summary of Significant Accounting Policies

Basis of Presentation

The Company has prepared the accompanying unaudited consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") for interim financial information and with the instructions to Form 10-Q and Article 10-01 of Regulation S-X. Accordingly, they do not include all of the information and disclosures required by U.S. GAAP for complete financial statements. In the opinion of management, the accompanying unaudited financial statements reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's interim financial information.

The accompanying unaudited consolidated financial statements and related notes should be read in conjunction with the Company's financial statements and related notes as contained in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2018. For a complete description of the Company's accounting policies, please refer to the Annual Report on Form 10-K for the fiscal year ended December 31, 2018.

Consolidation

The consolidated financial statements include the accounts of the Company and its subsidiaries. Intercompany accounts and transactions have been eliminated in consolidation.

Foreign Currency Transactions

The functional currency for most of the Company's foreign subsidiaries is their local currency. For non-U.S. subsidiaries that transact in a functional currency other than the U.S. dollar, assets and liabilities are translated at current rates of exchange at the balance sheet date. Income and expense items are translated at the average foreign exchange rates for the period. Adjustments resulting from the translation of the financial statements of the Company's foreign operations into U.S. dollars are excluded from the determination of net income and are recorded in accumulated other comprehensive income, a separate component of stockholders' equity.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting periods. Actual results could differ from those estimates.

Reclassification

Certain prior year amounts have been reclassified for comparative purposes. The reclassifications did not affect results of operations, net assets, or cash flows.

Cash, Cash Equivalents, Marketable Securities, and Restricted Cash

The Company considers all highly liquid investments purchased with a maturity of three months or less at the date of acquisition to be cash equivalents. Marketable securities consist of fixed income investments with a maturity of greater than three months and other highly liquid investments that can be readily purchased or sold using established markets. These investments are classified as available-for-sale and are reported at fair value on the Consolidated Balance Sheets. Unrealized holding gains and losses are reported within comprehensive income (loss) in the Consolidated Statements of Comprehensive Loss. Fair value is based on available market information including quoted market prices, broker or dealer quotations, or other observable inputs.

Restricted cash consists primarily of funds held to satisfy the requirements of certain agreements that are restricted in their use and is included in non-current assets on the Consolidated Balance Sheets.

Concentration of Credit Risk

The Company's financial instruments that are exposed to concentration of credit risk consist primarily of cash, cash equivalents, and marketable securities. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company invests its marketable securities in high-quality commercial financial instruments. The Company has not recognized any losses from credit risks on such accounts during any of the periods presented. The Company believes it is not exposed to significant credit risk on cash and cash equivalents or its marketable securities.

The Company is subject to credit risk from its accounts receivable related to its product sales of Galafold®. The Company's accounts receivable at June 30, 2019 have arisen from product sales primarily in the E.U. and U.S. The Company will periodically assess the financial strength of its customers to establish allowances for anticipated losses, if any. For accounts receivable that have arisen from named patient sales, the payment terms are predetermined and the Company evaluates the creditworthiness of each customer on a regular basis. As of June 30, 2019, the Company recorded an allowance for doubtful accounts of $0.2 million.

Revenue Recognition

The Company's net product sales consist of sales of Galafold® for the treatment of Fabry disease. The Company has recorded revenue on sales where Galafold® is available either on a commercial basis or through a reimbursed early access program ("EAP"). Orders for Galafold® are generally received from distributors and pharmacies with the ultimate payor often a government authority.

The Company recognizes revenue when its performance obligations to its customers have been satisfied, which occurs at a point in time when the pharmacies or distributors obtain control of Galafold®. The transaction price is determined based on fixed consideration in the Company's customer contracts and is recorded net of estimates for variable consideration, which are third party discounts and rebates. The identified variable consideration is recorded as a reduction of revenue at the time revenue from the sale of Galafold® is recognized. The Company recognizes revenue to the extent that it is probable that a significant revenue reversal will not occur in a future period. These estimates may differ from actual consideration received. The Company evaluates these estimates each reporting period to reflect known changes.

The following table summarizes the Company's net product sales from Galafold® disaggregated by geographic area:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	**2019**	**2018**	**2019**	**2018**
U.S.	$ 12,181	$ —	$ 21,249	$ —
Ex-U.S.	31,949	21,309	56,927	38,005
Total net product sales	$ 44,130	$ 21,309	$ 78,176	$ 38,005

Inventories and Cost of Goods Sold

Inventories are stated at the lower of cost and net realizable value, determined by the first-in, first-out method. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on projected sales activity as well as product shelf-life. In evaluating the recoverability of inventories produced, the probability that revenue will be obtained from the future sale of the related inventory is considered and inventory value is written down for inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of goods sold in the Consolidated Statements of Operations.

Cost of goods sold includes the cost of inventory sold, manufacturing and supply chain costs, product shipping and handling costs, provisions for excess and obsolete inventory, as well as royalties payable. A portion of the inventory available-for-sale was expensed as research and development costs prior to regulatory approval and as such the cost of goods sold and related gross margins are not necessarily indicative of future cost of goods sold and gross margin.

Leases

The Company primarily enters into lease agreements for office space, equipment, and vehicles. The leases have varying terms, some of which could include options to renew, extend, and early terminate. The Company determines if an arrangement is a lease at contract inception. Operating leases are included in right-of-use ("ROU") assets and lease liabilities on the Consolidated Balance Sheets.

ROU assets represent the Company's right to control the use of an explicitly or implicitly identified fixed asset for a period of time and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. The Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments.

Lease payments included in the measurement of the lease liability are comprised of fixed payments. Variable lease payments are excluded from the ROU asset and lease liability and are recognized in the period in which the obligation for those payments is incurred. Variable lease payments are presented in the Consolidated Statements of Operations in the same line item as expenses arising from fixed lease payments for operating leases. The Company has lease agreements that include lease and non-lease components, which the Company accounts for as a single lease component for all underlying asset categories.

The lease term for all of the Company's leases include the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Leases with an initial term of 12 months or less are not recorded on the Consolidated Balance Sheets. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. For additional information, see " —Note 9. Leases" and "—Note 2. Summary of Significant Accounting Policies, Recent Accounting Developments - Guidance Adopted in 2019."

Recent Accounting Developments - Guidance Adopted in 2019

ASU 2016-02 - In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)* ("ASU 2016-02"). ASU 2016-02 requires the recognition of lease assets and lease liabilities on the balance sheet for all lease obligations and disclosing key information about leasing arrangements. ASU 2016-02 requires the recognition of lease assets and lease liabilities by lessees for those leases classified as operating leases under previous generally accepted accounting principles. In August 2018, the FASB issued ASU 2018-11, *Leases (Topic 842): Targeted Improvements*, ("ASU 2018-11"). ASU 2018-11 provided entities with an additional transition method for adoption, whereby, an entity initially applies the new leases standard at the adoption date and recognizes a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. Effective January 1, 2019 the Company adopted ASU 2016-02, along with the amendments issued in 2017 and 2018, and elected the transition method in ASU 2018-11. The Company elected the package of transition provisions available for expired or existing contracts, which allowed the Company to carry forward its historical assessments of (i) whether contracts are or contain leases, (ii) lease classification and (iii) initial direct costs. In addition, the Company applied the short-term lease recognition exemption for leases with terms at inception not greater than 12 months and will apply the practical expedient not to separate lease and non-lease components for new and modified leases commencing after adoption. The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. Upon adoption, the Company recorded a lease liability with a corresponding right-of-use asset of $17.6 million. The adoption did not have a material impact on the Consolidated Statements of Operations and the Consolidated Statements of Cash Flows.

In August 2018, the Securities Exchange Commission ("SEC") issued Final Rule 33-10532, *Disclosure Update and Simplification*, which amends certain disclosure requirements that were redundant, duplicative, overlapping, or superseded by other SEC disclosure requirements. The amendments generally eliminated or otherwise reduced certain disclosure requirements of various SEC rules and regulations. However, in some cases, the amendments require additional information to be disclosed, including changes in stockholders' equity in interim periods. The rule was effective 30 days after its publication in the Federal Register. The rule was posted on October 4, 2018. On September 25, 2018, the SEC released guidance advising it will not object to a registrant adopting the requirement to include changes in stockholders' equity in the Form 10-Q for the first quarter beginning after the effective date of the rule. The Company adopted the guidance for the period ended March 31, 2019.

Recent Accounting Developments - Guidance Not Yet Adopted

ASU 2018-13— In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework-Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"). The amendments modify the disclosure requirements in Topic 820. ASU 2018-13 is effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The amendments on changes (i) in unrealized gains and losses, (ii) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and (iii) the narrative description of measurement uncertainty should be applied prospectively for only the most recent interim or annual period presented in the initial fiscal year of adoption. All other amendments should be applied retrospectively to all periods presented upon their effective date. Early adoption is permitted. An entity is permitted to early adopt any removed or modified disclosures upon issuance of ASU 2018-13 and delay adoption of the additional disclosures until their effective date. The Company is currently assessing the impact that this standard will have on its consolidated financial statements upon adoption.

ASU 2017-04 — In January 2017, the FASB issued ASU 2017-04, *Intangibles - Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment* ("ASU 2017-04"). ASU 2017-04 simplifies the recognition and measurement of a goodwill impairment loss by eliminating Step 2 of the quantitative goodwill impairment test. The guidance requires a one-step impairment test in which an entity compares the fair value of a reporting unit with its carrying amount and recognizes an impairment charge for the amount by which the carrying amount exceeds the reporting unit's fair value, if any. ASU 2017-04 is effective for fiscal years beginning after December 15, 2019 and should be applied on a prospective basis. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The Company is currently assessing the impact that this standard will have on its consolidated financial statements upon adoption.

ASU 2016-13 — In June 2016, the FASB issued ASU 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). ASU 2016-13 requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected and amends guidance on the impairment of financial instruments. ASU 2016-13 is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. The Company is currently assessing the impact that this standard will have on its consolidated financial statements upon adoption.

Note 3. Cash, Cash Equivalents, Marketable Securities, and Restricted Cash

As of June 30, 2019, the Company held $220.6 million in cash and cash equivalents and $355.1 million of available-for-sale debt securities which are reported at fair value on the Company's Consolidated Balance Sheets. Unrealized holding gains and losses are reported within accumulated other comprehensive loss in the Statements of Comprehensive Loss. If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other-than-temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge.

The Company regularly invests excess operating cash in deposits with major financial institutions, money market funds, notes issued by the U.S. government, as well as fixed income investments and U.S. bond funds, both of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated as many of these securities are either government backed or of the highest credit rating. Investments that have original maturities greater than three months but less than one year are classified as current, while investments that have maturities greater than one year are classified as long-term.

Cash, cash equivalents and marketable securities are classified as current unless mentioned otherwise below and consisted of the following:

	As of June 30, 2019			
(in thousands)	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	$ 220,578	$ —	$ —	$ 220,578
Corporate debt securities	169,425	148	(15)	169,558
Commercial paper	111,872	156	(2)	112,026
Asset-backed securities	73,025	68	—	73,093
Money market	350	—	—	350
Certificates of deposit	51	—	—	51
	$ 575,301	$ 372	$ (17)	$ 575,656
Included in cash and cash equivalents	$ 220,578	$ —	$ —	$ 220,578
Included in marketable securities	354,723	372	(17)	355,078
Total cash, cash equivalents, and marketable securities	$ 575,301	$ 372	$ (17)	$ 575,656

	As of December 31, 2018			
(in thousands)	Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Cash and cash equivalents	$ 79,749	$ —	$ —	$ 79,749
Corporate debt securities	240,969	7	(250)	240,726
Commercial paper	115,245	—	(104)	115,141
Asset-backed securities	68,215	4	(84)	68,135
Money market	350	—	—	350
Certificates of deposit	51	—	—	51
	$ 504,579	$ 11	$ (438)	$ 504,152
Included in cash and cash equivalents	$ 79,749	$ —	$ —	$ 79,749
Included in marketable securities	424,830	11	(438)	424,403
Total cash, cash equivalents, and marketable securities	$ 504,579	$ 11	$ (438)	$ 504,152

For the six months ended June 30, 2019 there were no realized gains. For the fiscal year ended December 31, 2018, there were nominal realized gains. The cost of securities sold is based on the specific identification method.

Unrealized loss positions in the available-for-sale debt securities as of June 30, 2019 and December 31, 2018 reflect temporary impairments that have been in a loss position for less than twelve months and as such are recognized in other comprehensive gain (loss). The fair value of these available-for-sale debt securities in unrealized loss positions was $38.5 million and $403.1 million as of June 30, 2019 and December 31, 2018, respectively.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the Consolidated Balance Sheets that sum to the total of the same such amounts shown in the Consolidated Statements of Cash Flows.

(in thousands)	June 30, 2019	December 31, 2018	June 30, 2018	December 31, 2017
Cash and cash equivalents	$ 220,578	$ 79,749	$ 73,311	$ 49,060
Restricted cash	2,610	2,626	2,130	2,177
Cash, cash equivalents, and restricted cash shown in the Consolidated Statements of Cash Flows	$ 223,188	$ 82,375	$ 75,441	$ 51,237

Note 4. Inventories

Inventories consist of raw materials, work-in-process, and finished goods related to the manufacture of Galafold®. The following table summarizes the components of inventories:

(in thousands)	June 30, 2019	December 31, 2018
Raw materials	$ 4,018	$ 1,291
Work-in-process	3,320	3,485
Finished goods	3,057	3,614
Total inventories	$ 10,395	$ 8,390

The Company recorded a reserve for inventory of $0.2 million as of June 30, 2019 and December 31, 2018.

Note 5. Debt

Senior Secured Term Loan due 2023

In September 2018, the Company entered into a loan agreement with BioPharma Credit PLC as the lender. The loan agreement provides for a $150 million senior secured term loan ("Senior Secured Term Loan") with an interest rate equal to the 3-month LIBOR plus 7.50% per annum and matures 5 years from the maturity date. The Senior Secured Term Loan will be repaid in four quarterly payments equal to 12.50% thereof starting on the forty-eight month anniversary of the date of the first credit extension with the balance due on the Maturity Date. Interest is payable quarterly in arrears. The Senior Secured Term Loan contains certain customary representations and warranties, affirmative and negative covenants, and events of default applicable to the Company and certain of its subsidiaries, but does not include any financial covenants relating to the achievement or maintenance of revenue or cash flow. If an event of default occurs and is continuing, the lender may declare all amounts outstanding under the Senior Secured Term Loan to be immediately due and payable. The Company received net proceeds of $146.6 million in September 2018, after deducting fees and estimated expenses payable by the Company.

Convertible Notes due 2023

In December 2016, the Company issued at par value $250 million aggregate principal amount of Convertible Notes, which included the exercise in full of the $25 million over-allotment option granted to the initial purchasers of the Convertible Notes in a private offering to qualified institutional buyers pursuant to Rule 144A under the Securities Act (the "Note Offering"). Interest is payable semiannually on June 15 and December 15 of each year, beginning on June 15, 2017. The Convertible Notes will mature on December 15, 2023, unless earlier repurchased, redeemed, or converted in accordance with their terms. The Convertible Notes are convertible at the option of the Holders, under certain circumstances and during certain periods, into cash, shares of the Company's common stock or a combination thereof. The net proceeds from the Note Offering were $243.0 million, after deducting fees and estimated expenses payable by the Company. In addition, the Company used $13.5 million of the net proceeds from the issuance of the Convertible Notes to pay the cost of the capped call transactions ("Capped Call Confirmations") that the Company entered into in connection with the issuance of the Convertible Notes. In accounting for the issuance of the Convertible Notes, the Company separated the Convertible Notes into liability and equity components based on their relative values. The Convertible Notes were initially convertible into 40.8 million shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 163.3987 shares per $1,000 principal amount of Convertible Notes, which represents a conversion price of $6.12 per share of the Company's common stock, subject to adjustment under certain conditions.

On February 15, 2018, the Company entered into an underwriting agreement relating to an underwritten public offering of 19.4 million shares of the Company's common stock. Under the terms of the underwriting agreement, the Company granted the underwriters an option, exercisable for 30 days after February 16, 2018, to purchase up to an additional 2.9 million shares of the Company's common stock, which was exercised with respect to 885,000 shares of the Company's common stock.

Subsequent to the underwritten public offering on February 15, 2018, the Company did not have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. As a result, the Company accounted for the portion of the bifurcated conversion feature and of the Capped Call Confirmations that would not be able to be net share settled as a current derivative liability and as a derivative asset, respectively. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations at February 15, 2018 was determined to be $507.4 million and $13.6 million, respectively, of which the portion that was determined to not be able to be net share settled was recorded with a corresponding impact to additional-paid-in-capital. Subsequent changes to fair value of the derivatives were recorded through earnings on the Consolidated Statements of Operations resulting in a change in fair value of derivatives for the three and six months ended June 30, 2018 of $(7.6) million and $(2.7) million, respectively.

Following the approval by the stockholders of the Company on June 7, 2018, to increase the authorized shares of common stock to 500,000,000, the Company has sufficient unissued authorized shares to cover a conversion of the Convertible Notes. As a result, the derivative liability and derivative asset were reclassified into additional-paid-in-capital. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations at June 7, 2018 was determined to be $88.3 million and $2.4 million, respectively.

During the six months ended June 30, 2019, the Company entered into separate, privately negotiated Exchange Agreements with the Holders of the Convertible Notes. Under the terms of the Exchange Agreements , the Holders agreed to exchange an aggregate principal amount of $247.2 million of Convertible Notes held by them in exchange for an aggregate of approximately 44.0 million shares of Company common stock, par value $0.01 per share. In addition, pursuant to the Exchange Agreements, the Company made aggregate cash payments of $1.3 million during the six months ended June 30, 2019, to the Holders to satisfy accrued and unpaid interest to the closing date of the transaction, along with cash in lieu of fractional shares. The transaction resulted in $215.0 million in additional paid-in-capital and common stock of $0.4 million in the Consolidated Balance Sheets as of June 30, 2019. Additionally, the Company recognized a net loss on the exchange of debt of $36.1 million and $4.5 million in the Consolidated Statements of Operations for the quarters ended March 31, 2019 and June 30, 2019, respectively.

The last reported sale price of the Company's common stock was equal to or more than 130% of the conversion price of the Convertible Notes for at least 20 trading days of the 30 consecutive trading days ending on the last day of the second quarter. As a result, the remaining Convertible Notes are currently convertible into the Company's common stock.

During the six months ended June 30, 2019, the Company also terminated the Capped Call Confirmations related to the exchange of the Convertible Notes for proceeds of $19.9 million.

The Convertible Notes and Senior Secured Term Loan consist of the following:

Liability component (in thousands)	June 30, 2019	December 31, 2018
Principal	$ 152,825	$ 400,000
Less: debt discount [1]	(3,373)	(74,145)
Less: deferred financing [1]	(388)	(4,115)
Net carrying value of the debt	$ 149,064	$ 321,740

[1] Included in the Consolidated Balance Sheets within Convertible Notes and Senior Secured Term Loan and amortized to interest expense over the remaining life of the Convertible Notes and Senior Secured Term Loan using the effective interest rate method.

The following table sets forth total interest expense recognized related to the Convertible Notes and Senior Secured Term Loan for the three and six months ended June 30, 2019 and 2018, respectively:

	Three Months Ended June 30,		Six Months Ended June 30,	
Interest component (in thousands)	2019	2018	2019	2018
Contractual interest expense	$ 4,146	$ 1,888	$ 8,959	$ 3,775
Amortization of debt discount	423	2,539	1,982	5,011
Amortization of deferred financing	39	133	122	262
Total	$ 4,608	$ 4,560	$ 11,063	$ 9,048

Note 6. Stockholders' Equity

During the six months ended June 30, 2019, 101,787 warrants were exercised at $7.98 per share of common stock resulting in gross cash proceeds of $0.8 million.

As discussed in "— Note 1. Business" during the second quarter of 2019, the Company completed an underwritten equity offering and issued 18.7 million shares of its common stock at $10.75 per share, inclusive of the fully exercised option to purchase additional shares from the initial offering. This transaction resulted in net proceeds of $189.0 million, after deducting underwriting discounts and commissions and offering expenses.

As discussed in "— Note 5. Debt" during the six months ended June 30, 2019, the Company entered into separate, privately negotiated Exchange Agreements with the Holders of the Convertible Notes. Under the terms of the Exchange Agreements, the Holders agreed to exchange an aggregate principal amount of $247.2 million of Convertible Notes held by them in exchange for an aggregate of approximately 44.0 million shares of Company common stock, par value $0.01 per share.

As discussed in "— Note 8. Assets and Liabilities Measured at Fair Value", the Company reached a clinical milestone, which was the dosing of the first patient in a Phase 3 study, related to the contingent consideration from the acquisition of Callidus. The milestone for this event was $9.0 million, which was paid in Company common stock in the first quarter of 2019, and resulted in a $9.3 million impact on stockholder's equity.

Note 7. Share-Based Compensation

The Company's Equity Incentive Plans consist of the Amended and Restated 2007 Equity Incentive Plan (the "Plan") and the 2007 Director Option Plan (the "2007 Director Plan"). The Plan provides for the granting of restricted stock units and options to purchase common stock in the Company to employees, directors, advisors, and consultants at a price to be determined by the Company's Board of Directors. The Plan is intended to encourage ownership of stock by employees and consultants of the Company and to provide additional incentives for them to promote the success of the Company's business. The 2007 Director Plan is intended to promote the recruiting and retention of highly qualified eligible directors and strengthen the commonality of interest between directors and stockholders by encouraging ownership of common stock of the Company. The Board of Directors, or its committee, is responsible for determining the individuals to be granted options, the number of options each individual will receive, the option price per share, and the exercise period of each option.

Stock Option Grants

The fair value of the stock options granted is estimated on the date of grant using a Black-Scholes option pricing model with the following weighted-average assumptions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Expected stock price volatility	74.2%	81.1%	74.2%	81.2%
Risk free interest rate	2.0%	2.8%	2.4%	2.4%
Expected life of options (years) [1]	5.68	5.62	5.68	5.62
Expected annual dividend per share	$ —	$ —	$ —	$ —

[1] The average expected life is determined using actual historical data.

A summary of the Company's stock options for the six months ended June 30, 2019 were as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Options outstanding, December 31, 2018	15,810	$ 8.63		
Granted	3,683	$ 10.36		
Exercised	(1,139)	$ 5.93		
Forfeited	(445)	$ 11.02		
Expired	(60)	$ 13.61		
Options outstanding, June 30, 2019	17,849	$ 9.09	6.8 years	$ 68.7
Vested and unvested expected to vest, June 30, 2019	16,975	$ 8.99	6.7 years	$ 66.8
Exercisable at June 30, 2019	10,624	$ 8.14	5.5 years	$ 50.1

As of June 30, 2019, the total unrecognized compensation cost related to non-vested stock options granted was $40.8 million and is expected to be recognized over a weighted average period of three years.

Restricted Stock Units and Performance-Based Restricted Stock Units (collectively "RSUs")

RSUs awarded under the Plan are generally subject to graded vesting and are contingent on an employee's continued service. RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse. A summary of non-vested RSU activity under the Plan for the six months ended June 30, 2019 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value
	(in thousands)			(in millions)
Non-vested units as of December 31, 2018	3,712	$ 10.59		
Granted	3,262	$ 11.02		
Vested	(788)	$ 9.28		
Forfeited	(301)	$ 10.32		
Non-vested units as of June 30, 2019	5,885	$ 11.09	2.7 years	$ 73.5

All non-vested units granted as of June 30, 2019 are expected to vest over their normal term. As of June 30, 2019, there was $47.5 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of three years.

Compensation Expense Related to Equity Awards

The following table summarizes information related to compensation expense recognized in the Consolidated Statements of Operations related to the equity awards:

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Equity compensation expense recognized in:				
Research and development expense	$ 3,952	$ 2,641	$ 8,984	$ 5,698
Selling, general, and administrative expense	5,983	3,700	13,695	8,121
Total equity compensation expense	$ 9,935	$ 6,341	$ 22,679	$ 13,819

Note 8. Assets and Liabilities Measured at Fair Value

The Company's financial assets and liabilities are measured at fair value and classified within the fair value hierarchy, which is defined as follows:

Level 1 — Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2 — Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly.

Level 3 — Inputs that are unobservable for the asset or liability.

A summary of the fair value of the Company's recurring assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of June 30, 2019 are identified in the following table:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	$ 112,026	$ 112,026
Asset-backed securities	73,093	73,093
Corporate debt securities	169,558	169,558
Money market funds	4,224	4,224
	$ 358,901	$ 358,901

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$ —	$ 21,247	$ 21,247
Deferred compensation plan liability	3,874	—	3,874
	$ 3,874	$ 21,247	$ 25,121

A summary of the fair value of the Company's recurring assets and liabilities aggregated by the level in the fair value hierarchy within which those measurements fall as of December 31, 2018 are identified in the following table:

(in thousands)	Level 2	Total
Assets:		
Commercial paper	$ 115,141	$ 115,141
Asset-backed securities	68,135	68,135
Corporate debt securities	240,726	240,726
Money market funds	3,082	3,082
	$ 427,084	$ 427,084

	Level 2	Level 3	Total
Liabilities:			
Contingent consideration payable	$ —	$ 19,700	$ 19,700
Deferred compensation plan liability	2,732	—	2,732
	$ 2,732	$ 19,700	$ 22,432

The Company's Convertible Notes fall into the Level 2 category within the fair value level hierarchy. The fair value was determined using broker quotes in a non-active market for valuation. The fair value of the debt at June 30, 2019 was $6.1 million.

The Company's Senior Secured Term Loan fall into the Level 2 category within the fair value level hierarchy and the fair value was determined using quoted prices for similar liabilities in active markets, as well as inputs that are observable for the liability (other than quoted prices), such as interest rates that are observable at commonly quoted intervals. The carrying value of the Senior Secured Term Loan approximates the fair value.

The Company did not have any Level 3 assets as of June 30, 2019 or December 31, 2018.

Cash, Money Market Funds, and Marketable Securities

The Company classifies its cash within the fair value hierarchy as Level 1 as these assets are valued using quoted prices in an active market for identical assets at the measurement date. The Company considers its investments in marketable securities as available-for-sale debt securities and classifies these assets and the money market funds within the fair value hierarchy as Level 2 primarily utilizing broker quotes in a non-active market for valuation of these securities. No changes in valuation techniques or inputs occurred during the six months ended June 30, 2019. No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the six months ended June 30, 2019.

Contingent Consideration Payable

The contingent consideration payable resulted from the acquisition of Callidus Biopharma, Inc. ("Callidus") in November 2013. The most recent valuation was determined using a probability weighted discounted cash flow valuation approach. Using this approach, expected future cash flows are calculated over the expected life of the agreement, are discounted, and then exercise scenario probabilities are applied. The valuation is performed quarterly. Gains and losses are included in the Consolidated Statements of Operations.

The contingent consideration payable for Callidus has been classified as a Level 3 recurring liability as its valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approach, the estimated fair value could be significantly higher or lower than the fair value the Company determined. The Company may be required to record losses in future periods.

The following significant unobservable inputs were used in the valuation of the contingent consideration payable of Callidus for the ATB200 Pompe program:

Contingent Consideration Liability	Fair Value as of June 30, 2019	Valuation Technique	Unobservable Input	Range
	(in thousands)			
Clinical and regulatory milestones	$20,999	Probability weighted discounted cash flow	Discount rate	10.1%
			Probability of achievement of milestones	75%-78%
			Projected year of payments	2021-2022

Contingent consideration liabilities are remeasured to fair value each reporting period using discount rates, probabilities of payment, and projected payment dates. Projected contingent payment amounts related to clinical and regulatory based milestones are discounted back to the current period using a discounted cash flow model. Increases in discount rates and the time to payment may result in lower fair value measurements. Increases or decreases in any of those inputs together, or in isolation, may result in a significantly lower or higher fair value measurement. There is no assurance that any of the conditions for the milestone payments will be met.

The Company reached a clinical milestone, which was the dosing of the first patient in a Phase 3 study, related to the contingent consideration from the acquisition of Callidus. The milestone for this event was $9.0 million, which was paid in Company common stock in the first quarter of 2019, resulting in $9.3 million impact on stockholder's equity.

The following table shows the change in the balance of contingent consideration payable for the three and six months ended June 30, 2019 and 2018, respectively:

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Balance, beginning of the period	$ 20,767	$ 26,500	$ 28,700	$ 25,400
Payment of contingent consideration in stock	—	—	(9,316)	—
Changes in fair value during the period, included in the Consolidated Statements of Operations	480	300	1,863	1,400
Balance, end of the period	$ 21,247	$ 26,800	$ 21,247	$ 26,800

Deferred Compensation Plan - Investment and Liability

The Deferred Compensation Plan (the "Deferral Plan") provides certain key employees and members of the Board of Directors with an opportunity to defer the receipt of such participant's base salary, bonus, and director's fees, as applicable. Deferral Plan assets are classified as trading securities and recorded at fair value with changes in the investment's fair value recognized in the period they occur. The asset investments consist of market exchanged mutual funds. The Company considers its investments in marketable securities as available-for-sale and classifies these assets and related liability within the fair value hierarchy as Level 2, primarily utilizing broker quotes in a non-active market for valuation of these securities.

Note 9. Leases

The Company currently has operating leases for office and research laboratory space, equipment, and vehicles under agreements expiring at various dates through 2044, which include renewal options on leases which the Company is reasonably certain to exercise.

For the three and six months ended June 30, 2019, operating lease expense was $2.7 million and $4.8 million, respectively. For the six months ended June 30, 2019, the Company paid $2.4 million for amounts included in the measurement of operating lease liabilities and recorded $0.2 million of right-of-use assets obtained in exchange for new operating lease liabilities.

Commitments under finance leases are not significant.

Supplemental balance sheet information related to operating leases was as follows:

(in thousands, except year and discount rate amounts)	June 30, 2019
Operating lease ROU asset	$ 35,052
Current portion of the operating lease liabilities	$ 2,678
Non-current portion of the operating lease liabilities	36,259
Total operating lease liability	$ 38,937
Weighted-average remaining lease terms (years)	14.8
Weighted-average discount rate	12.9%

At June 30, 2019, the future minimum lease payments were as follows:

(in thousands)	Operating Leases
2019 (excludes the six months ended June 30, 2019)	$ 2,781
2020	9,647
2021	10,566
2022	10,411
2023	10,789
Thereafter	174,673
Total lease payments	$ 218,867
Less lease incentives	(28,939)
Less imputed interest	(150,991)
Total operating lease liability	$ 38,937

At December 31, 2018, the future minimum lease payments were as follows:

(in thousands)	Operating Leases
2019	$ 6,244
2020	4,063
2021	3,560
2022	3,371
2023	3,611
Thereafter	10,038
Total lease payments	$ 30,887

Note 10. Basic and Diluted Net Loss per Common Share

The following table provides a reconciliation of the numerator and denominator used in computing basic and diluted net loss attributable to common stockholders per common share:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands, except per share amounts)	2019	2018	2019	2018
Numerator:				
Net loss attributable to common stockholders	$ (84,551)	$ (61,833)	$ (204,850)	$ (111,751)
Denominator:				
Weighted average common shares outstanding — basic and diluted	238,089,824	188,621,423	225,848,013	182,303,128

Dilutive common stock equivalents would include the dilutive effect of common stock options, convertible debt units, RSUs, and warrants for common stock equivalents. Potentially dilutive common stock equivalents were excluded from the diluted earnings per share denominator for all periods because of their anti-dilutive effect.

The table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method:

	As of June 30,	
(in thousands)	**2019**	**2018**
Options to purchase common stock	17,849	15,869
Convertible notes	462	40,850
Outstanding warrants, convertible to common stock	2,555	2,657
Unvested restricted stock units	5,885	3,556
Vested restricted stock units, unissued	227	111
Total number of potentially issuable shares	26,978	63,043

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

Overview

We are a global patient-dedicated biotechnology company engaged in the discovery, development, and commercialization of a diverse set of novel treatments for patients living with rare metabolic diseases. With one medicine for Fabry disease that has achieved widespread global approval, a differentiated biologic for Pompe disease in the clinic, and an industry leading rare disease gene therapy portfolio.

The cornerstone of our portfolio is Galafold® (also referred to as "migalastat"), the first and only approved oral precision medicine for people living with Fabry disease who have amenable genetic variants. Migalastat is currently approved under the trade name Galafold® in the United States ("U.S."), European Union ("E.U."), and Japan, with additional approvals granted and applications pending in several geographies. During the third quarter of 2018, we initiated the commercial launch of Galafold® in the U.S. for the treatment of adult patients with a confirmed diagnosis of Fabry disease and an amenable genetic variant.

The lead biologics program of our pipeline is Amicus Therapeutics GAA ("AT-GAA", also known as ATB200/AT2221), a novel, clinical-stage, potential best-in-class treatment paradigm for Pompe disease. In February 2019, the U.S. Food and Drug Administration ("FDA") granted Breakthrough Therapy designation to AT-GAA for the treatment of late onset Pompe disease.

We have established an industry leading gene therapy portfolio of potential therapies for people living with rare metabolic diseases, through a license with Nationwide Children's Hospital ("NCH") and a recently expanded collaboration with the University of Pennsylvania ("Penn"). Our pipeline includes gene therapy programs in rare, neurologic lysosomal disorders ("LDs") with programs in CLN6, CLN3, and CLN8 Batten disease, Pompe disease, Fabry disease, CDKL5 deficiency disorder ("CDD"), Niemann-Pick Type C (“NPC”), Mucopolysaccharidosis Type IIIB ("MPSIIIB"), as well as a next generation program in Mucopolysaccharidosis Type IIIA ("MPSIIIA"). This expanded collaboration with Penn also provides us with exclusive disease-specific access and the option rights to develop potentially disruptive new gene therapy platform technologies and programs for most LDs and a broader portfolio of rare diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystophy, and select other muscular dystrophies.

Our Strategy

Our strategy is to create, manufacture, test, and deliver the highest quality medicines for people living with rare metabolic diseases through internally developed, acquired, or in-licensed products and product candidates that have the potential to obsolete current treatments, provide significant benefits to patients, and be first- or best-in-class. In addition to our programs in Fabry and Pompe, we have begun to leverage our global capabilities to develop and expand our robust pipeline through our recent entry into genomic medicine. We have made significant progress toward fulfilling our vision to build a leading global biotechnology company focused on rare metabolic diseases.

Highlights of our progress in the first six months of 2019 include:

- *Commercial and regulatory success in Fabry disease*. During the six months ended June 30, 2019, Galafold® revenue totaled $78.2 million, an increase of $40.2 million compared to the same period in the prior year. We continue to see strong momentum in new markets including in the U.S. and Japan, as well as in our more mature markets. In the countries we have been operating the longest, such as Germany and the United Kingdom, we see an increasing proportion of previously untreated patients come onto Galafold®. In the U.S., we continue to see a significant increase in patients from a growing and very wide prescriber base. Across all markets, we see a high rate of compliance and adherence to this oral treatment option.
- *Pompe clinical program milestones*. We reported positive data from a Phase 1/2 clinical study to evaluate Pompe disease patients treated with our novel treatment paradigm AT-GAA for up to 24 months. The U.S. FDA also granted Breakthrough Therapy designation for AT-GAA for the treatment of late-onset Pompe disease. We are currently enrolling a global pivotal study of AT-GAA (ATB200-03, also known as PROPEL) which is on track to enroll approximately 100 participants with late-onset Pompe disease at up to 90 global sites.
- *Pipeline growth*. With our recent gene therapy program expansion, we have established an industry leading gene therapy portfolio of medicines for people living with rare metabolic diseases. Through our license with NCH, we acquired worldwide development and commercial rights for ten gene therapy programs in rare, neurologic LDs with programs in CLN6, CLN3, and CLN8 Batten disease. Additionally, four programs were added to the pipeline through

ongoing collaborations with Penn to pursue research and development of novel gene therapies for Pompe disease, Fabry disease, CDD, NPC, MPSIIIB, as well as a next generation program in MPSIIIA.

- *Manufacturing*. We successfully scaled up manufacturing of our Pompe biologic to commercial scale (1,000L) for our pivotal PROPEL study and commercial supply. Our supply agreement with WuXi Biologics and current capacity are expected to produce sufficient quantities to serve the entire Pompe population as quickly as possible after receipt of applicable regulatory approvals. For gene therapy, we have recently entered into strategic partnerships with two best-in-class contract development and manufacturing organizations: Catalent Biologics and Thermo Fisher Scientific. Catalent Biologics will support our clinical manufacturing capabilities and capacity for multiple active preclinical lysosomal disorder programs that are currently in development in collaboration with Penn. Thermo Fisher will assist with late-stage clinical and commercial-scale capabilities and provides us with immediate clinical and commercial manufacturing capabilities and capacity for the Amicus intrathecal AAV Batten disease gene therapy programs.
- *Financial strength*. Total cash, cash equivalents, and marketable securities of $575.7 million at June 30, 2019 compared to $504.2 million at December 31, 2018. The current cash position, including expected Galafold® revenues, is sufficient to fund ongoing Fabry, Pompe, and gene therapy program operations into 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact our future capital requirements.

Our Commercial Product and Product Candidates

Galafold® (Migalastat HCl) for Fabry Disease

Our oral precision medicine Galafold® was granted accelerated approval by the FDA in August 2018 under the brand name Galafold ® for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene ("GLA") variant based on in vitro assay data. The FDA approved Galafold® for 348 amenable GLA variants. Galafold® was approved in the E.U. in May 2016 as a first-line therapy for long-term treatment of adults and adolescents, aged 16 years and older, with a confirmed diagnosis of Fabry disease and who have an amenable variant. The approved E.U. label includes 367 Fabry-causing variants, which represent up to half of all patients with Fabry disease. Approvals have also been granted in Australia, Canada, Israel, Japan, South Korea, and Switzerland, with additional applications pending in other geographies. We have been granted pricing and reimbursement in 24 countries. We plan to continue to launch Galafold® in additional countries during 2019.

As an orally administered monotherapy, Galafold® is designed to bind to and stabilize an endogenous alpha-galactosidase A ("alpha-Gal A") enzyme in those patients with genetic variants identified as amenable in a GLP cell-based amenability assay. Galafold® is an oral precision medicine intended to treat Fabry disease in patients who have amenable genetic variants and, at this time, it is not intended for concomitant use with ERT.

Gene Therapy for Fabry Disease

We are committed to continued innovation for all people living with Fabry disease. For people living with Fabry disease who have non-amenable variants, which are not suitable for Galafold® as a monotherapy, our strategy is to develop a Fabry gene therapy. In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for Fabry disease.

Novel ERT for Pompe Disease

We are leveraging our biologics capabilities to develop AT-GAA, a novel treatment paradigm for Pompe disease. AT-GAA consists of a uniquely engineered rhGAA enzyme, ATB200, with an optimized carbohydrate structure to enhance lysosomal uptake, administered in combination with a pharmacological chaperone, AT2221, to improve activity and stability. We initiated a global Phase 3 clinical study ("ATB200-03", or "PROPEL") of AT-GAA in adult patients with late onset Pompe disease in 2018, with the first patient dosed in December 2018.

The pharmacological chaperone, AT2221 is not an active ingredient that contributes directly to GAA substrate reduction but instead acts to stabilize ATB200. The small molecule pharmacological chaperone AT2221 binds and stabilizes ATB200 to improve the uptake of active enzyme in key disease-relevant tissues, resulting in increased clearance of accumulated substrate, glycogen.

Our strategy is to enhance the body of clinical data for AT-GAA in ongoing clinical studies, including the pivotal study (PROPEL) to deliver this potential new therapy to as many people living with late onset Pompe disease as soon as possible. Based

on regulatory feedback from both the U.S. FDA and the European Medicines Agency ("EMA"), the PROPEL study is expected to support approval for a broad indication, including ERT-switch and treatment-naïve patients, if the results are favorable.

In February 2019, we reported additional interim data from our clinical study ATB200-02 at the 15th Annual WORLDSymposium™. Highlights included muscle function, safety and tolerability data in patients as well as pharmacodynamic data (muscle damage biomarker, creatine kinase, and disease substrate biomarker, urine hexose tetrasaccharide). Muscle function improved in 16 out of 17 patients who have available data for up to 21 or 24 months. Mean six-minute walk test ("6MWT") improved in both ERT-naïve and ERT-switch patients with continued benefit observed out to month 24. All 5 ERT-naive patients showed increases from baseline in 6MWT distance at all time points out to month 21. To date, adverse events have been generally mild and transient. AT-GAA has resulted in a low rate of infusion-associated reactions ("IARs") following over 1,110 infusions (16 events of IARs in six patients). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data. Treatment with AT-GAA resulted in persistent and durable reductions in creatine kinase and urine hexose tetrasaccharide across all patient cohorts up to month 24.

Gene Therapy for Pompe Disease

As part of our long-term commitment to provide multiple solutions to address the significant unmet needs of the Pompe community, we are also advancing a next-generation gene therapy treatment for Pompe disease. In October 2018, we further expanded our gene therapy portfolio through a collaboration agreement with Penn to pursue research and development of novel gene therapies for, among other indications, Pompe disease.

In April 2019, we presented initial preclinical data from our investigational adeno-associated viral ("AAV") gene therapy program for Pompe disease. This initial preclinical study in Pompe knockout mice administered a single high dose of AAV gene therapy with either natural unmodified hGAA ("natural hGAA") or an Amicus/Penn engineered hGAA transgene with a Lysosomal-Targeting Cell receptor binding motif ("engineered hGAA"). The Amicus/Penn engineered hGAA AAV gene therapy demonstrated more uniform cellular uptake and lysosomal targeting compared to natural hGAA AAV gene therapy, as well as, robust glycogen reduction in all key tissues in Pompe disease that were assessed. In the central nervous system, the engineered hGAA AAV gene therapy showed robust glycogen reduction in neuronal cells, suggesting this may be an effective way to address neuronal aspects of Pompe disease. Natural hGAA AAV gene therapy did not show glycogen reduction in neuronal cells. This initial preclinical study provides initial validation for combining Amicus-engineered transgenes with Penn's AAV gene therapy technologies.

Batten Disease Product Candidates

We are researching potential first-in-class gene therapies for multiple forms of Batten disease. Batten disease is the common name for a broad class of rare, fatal, inherited disorders of the nervous system also known as neuronal ceroid lipofuscinoses, or NCLs. In these diseases, a defect in a specific gene triggers a cascade of problems that interferes with a cell's ability to recycle certain molecules. Each gene is called CLN (ceroid lipofuscinosis, neuronal) and given a different number designation as its subtype. There are 13 known forms of Batten disease often referred to as CLN1-8; 10-14. The various types of Batten disease have similar features and symptoms but vary in severity and age of onset.

The two clinical stage gene therapies are in CLN3 and CLN6 Batten disease. The CLN6 Batten disease Phase 1/2 study completed target enrollment, with twelve patients receiving a single administration of adeno-associated virus serotype 9 AAV-CLN6 gene therapy. In August 2019, we reported positive interim clinical data from the first eight patients in the study. The AAV-CLN6 gene therapy demonstrated a positive impact on motor and language function. Seven out of eight patients maintained stable Hamburg Motor and Language scores or had an initial change (+1 to -1 points) followed by stabilization.

In the CLN3 Batten disease study, a total of three patients were dosed in the low dose group with no serious adverse events after up to 5 months following a single administration of AAV-CLN3 gene therapy. Based on the safety profile to date, the data safety monitoring board cleared Amicus to begin enrollment in the high dose cohort of up to three additional patients.

CDKL5 Deficiency Disorder

We are researching a potential first-in-class protein replacement therapy approach for CDD. In addition, through our collaboration with Penn, we are researching a gene therapy for CDD. CDKL5 is a gene on the X-chromosome encoding the CDKL5 protein that regulates the expression of several essential proteins for normal brain development. Genetic mutations in the CDKL5 gene result in CDKL5 protein deficiency and CDD. This disorder manifests clinically as persistent seizures starting in infancy, followed by severe impairment in neurological development. Most children affected by CDD cannot walk or care for themselves and may also suffer from scoliosis, visual impairment, sensory issues, and gastrointestinal complications.

Strategic Alliances and Arrangements

We will continue to evaluate business development opportunities as appropriate that build stockholder value and provide us with access to the financial, technical, clinical, and commercial resources necessary to develop and market technologies or products with a focus on rare metabolic diseases. We are exploring potential collaborations, alliances, and other business development opportunities on a regular basis. These opportunities may include the acquisition of preclinical-stage, clinical-stage, or marketed products so long as such transactions are consistent with our strategic plan to develop and provide therapies to patients living with rare and orphan diseases.

Consolidated Results of Operations

Three Months Ended June 30, 2019 compared to June 30, 2018

The following table provides selected financial information for the Company:

	Three Months Ended June 30,		
(in thousands)	**2019**	**2018**	**Change**
Net product sales	$ 44,130	$ 21,309	$ 22,821
Cost of goods sold	5,367	3,135	2,232
Cost of goods sold as a percentage of net product sales	12.2%	14.7%	(2.5)%
Operating expenses:			
Research and development	70,981	34,660	36,321
Selling, general, and administrative	42,578	29,172	13,406
Changes in fair value of contingent consideration payable	480	300	180
Depreciation and amortization	1,154	973	181
Other income (expense):			
Interest income	2,599	2,913	(314)
Interest expense	(4,625)	(4,560)	(65)
Loss on exchange of convertible notes	(4,501)	—	(4,501)
Change in fair value of derivatives	—	(7,600)	7,600
Other income (expense)	(877)	(5,316)	4,439
Income tax expense	(717)	(339)	(378)
Net loss attributable to common stockholders	$ (84,551)	$ (61,833)	$ (22,718)

Net Product Sales. Net product sales increased $22.8 million during the three months ended June 30, 2019 compared to the same period in the prior year. The increase was primarily due to Galafold® being approved for sale in the U.S. in the third quarter of 2018, as well as continued growth in the E.U. and Japan markets.

Cost of Goods Sold. Cost of goods sold includes manufacturing costs as well as royalties associated with sales of our product. Cost of goods sold as a percentage of net product sales was 12.2% during the three months ended June 30, 2019 compared to 14.7% during the same period in the prior year primarily due to the proportion of sales in countries subject to a higher royalty burden.

Research and Development Expense. The following table summarizes our principal product development programs for each product candidate in development, and the out-of-pocket, third party expenses incurred with respect to each product candidate:

(in thousands)	Three Months Ended June 30,	
Projects	**2019**	**2018**
Third party direct project expenses		
Migalastat (Fabry Disease)	$ 4,253	$ 3,726
AT-GAA (Pompe Disease)	29,638	9,538
Gene therapy programs	7,753	—
Pre-clinical and other programs	256	142
Total third party direct project expenses	41,900	13,406
Other project costs		
Personnel costs	18,821	13,967
Other costs	10,260	7,287
Total other project costs	29,081	21,254
Total research and development costs	$ 70,981	$ 34,660

The increase in research and development costs was primarily due to increases in clinical research and manufacturing costs with the advancement and enrollment of clinical studies in the Pompe program and an increase in gene therapy programs driven by the pipeline growth. There were also increases in personnel and other costs associated with the advancement and enrollment of clinical studies and investments in manufacturing.

Selling, General, and Administrative Expense. Selling, general, and administrative expense increased $13.4 million primarily due to the expanded geographic scope of the ongoing commercial launch of Galafold® and related operational costs of our global business, including establishing commercial organizations and related teams in the U.S and Japan.

Change in Fair Value of Derivatives. Subsequent to the underwritten public offering on February 15, 2018, we did not have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations was determined and subsequent changes to the fair value of the derivatives were recorded through earnings on the Consolidated Statements of Operations resulting in a change in fair value of derivatives for the three months ended June 30, 2018 of $7.6 million.

Loss on Exchange of Convertible Notes. During the three months ended June 30, 2019, the Company entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with a limited number of holders of the Convertible Notes. As a result of this exchange, the Company recognized a loss on exchange of debt of $4.5 million in the Consolidated Statements of Operations and $24.7 million in additional paid-in-capital in the Consolidated Balance Sheets for the three months ended June 30, 2019.

Other Income (Expense). The $4.4 million increase was primarily due to the variance of unrealized losses on foreign exchange transactions.

Six Months Ended June 30, 2019 compared to June 30, 2018

The following table provides selected financial information for the Company:

	Six Months Ended June 30,		
(in thousands)	**2019**	**2018**	**Change**
Net product sales	$ 78,176	$ 38,005	$ 40,171
Cost of goods sold	9,422	5,750	3,672
Cost of goods sold as a percentage of net product sales	12.1%	15.1%	(3.0)%
Operating expenses:			
Research and development	135,574	75,458	60,116
Selling, general, and administrative	86,881	56,568	30,313
Changes in fair value of contingent consideration payable	1,863	1,400	463
Depreciation and amortization	2,145	1,942	203
Other income (expense):			
Interest income	5,238	4,650	588
Interest expense	(11,079)	(9,048)	(2,031)
Loss on exchange of convertible notes	(40,624)	—	(40,624)
Change in fair value of derivatives	—	(2,739)	2,739
Other income (expense)	209	(2,554)	2,763
Income tax (expense) benefit	(885)	1,053	(1,938)
Net loss attributable to common stockholders	$ (204,850)	$ (111,751)	$ (93,099)

Net Product Sales. Net product sales increased $40.2 million during the six months ended June 30, 2019 compared to the same period in the prior year. The increase was primarily due to the approval of Galafold® for sale in the U.S. and Japan in the third quarter of 2018 and second quarter of 2018, respectively, as well as continued growth in the E.U. market.

Cost of Goods Sold. Cost of goods sold includes manufacturing costs as well as royalties associated with sales of our product. Cost of goods sold as a percentage of net product sales was 12.1% during the six months ended June 30, 2019 compared to 15.1% during the same period in the prior year primarily due to the proportion of sales in countries subject to a higher royalty burden.

Research and Development Expense. The following table summarizes our principal product development programs for each product candidate in development, and the out-of-pocket, third party expenses incurred with respect to each product candidate:

(in thousands)	Six Months Ended June 30,	
Projects	**2019**	**2018**
Third party direct project expenses		
Migalastat (Fabry Disease)	$ 8,609	$ 7,434
AT-GAA (Pompe Disease)	57,924	25,052
Gene therapy programs	9,945	—
Pre-clinical and other programs	716	800
Total third party direct project expenses	77,194	33,286
Other project costs		
Personnel costs	38,455	28,916
Other costs	19,925	13,256
Total other project costs	58,380	42,172
Total research and development costs	$ 135,574	$ 75,458

The increase in research and development costs was primarily due to increases in clinical research and manufacturing costs with the advancement and enrollment of clinical studies in the Pompe program and an increase in gene therapy programs driven by the pipeline growth. There were also increases in personnel and other costs with the advancement and enrollment of clinical studies and investments in manufacturing.

Selling, General, and Administrative Expense. Selling, general, and administrative expense increased $30.3 million primarily due to the expanded geographic scope of the ongoing commercial launch of Galafold® and related operational costs of our global business, including establishing commercial organizations and related teams in the U.S and Japan.

Change in Fair Value of Derivatives. Subsequent to the underwritten public offerings in February 2018, we did not have sufficient unissued authorized shares to cover a conversion of the Convertible Notes. The fair value of the derivative liability for the conversion feature and derivative asset for the Capped Call Confirmations was determined and subsequent changes to the fair value of the derivatives were recorded through earnings on the Consolidated Statements of Operations resulting in a change in fair value of derivatives for the six months ended June 30, 2018 of $2.7 million.

Loss on Exchange of Convertible Notes. During the first six months of 2019, the Company entered into separate, privately negotiated Exchange Agreements with a limited number of holders of the Convertible Notes. As a result of this exchange, the Company recognized a loss on exchange of debt of $40.6 million in the Consolidated Statements of Operations, and $215.0 million in additional paid-in-capital and common stock of $0.4 million in the Consolidated Balance Sheets for the six months ended June 30, 2019.

Other Income (Expense). The $2.8 million increase was primarily due to unrealized gain on foreign exchange transactions.

Income Tax (Expense) Benefit. The income tax expense for the six months ended June 30, 2019 was $0.9 million. We are subject to income taxes in the United States, although currently not a taxpayer, and in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions. The income tax benefit for the six months ended June 30, 2018 of $1.1 million was primarily due to a discrete tax item.

Liquidity and Capital Resources

As a result of our significant research and development expenditures, as well as expenditures to build a commercial organization to support the launch of Galafold®, we have not been profitable and have generated operating losses since we were incorporated in 2002. We have historically funded our operations through stock offerings, debt issuances, Galafold® revenues, collaborations, and other financing arrangements.

Sources of Liquidity

During the six months ended June 30, 2019, we entered into separate, privately negotiated Exchange Agreements with a limited number of holders of the Convertible Notes. Under the terms of the Exchange Agreements, the limited number of holders agreed to exchange an aggregate principal amount of $247.2 million of Convertible Notes held by them in exchange for an aggregate of approximately 44.0 million shares of our common stock, par value $0.01 per share. Additionally, we terminated the Capped Call Confirmations related to the exchange of the Convertible Notes for cash proceeds of $19.9 million.

During the second quarter of 2019, we completed an underwritten equity offering and issued 18.7 million shares of common stock at $10.75 per share, inclusive of the fully exercised option to purchase additional shares from the initial offering. This transaction resulted in net proceeds of $189.0 million, after deducting underwriting discounts, commissions and offering expenses.

Cash Flow Discussion

As of June 30, 2019, we had cash, cash equivalents, and marketable securities of $575.7 million. We invest cash in excess of our immediate requirements with regard to liquidity and capital preservation in a variety of interest-bearing instruments, including obligations of U.S. government agencies and money market accounts. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. Although we maintain cash balances with financial institutions in excess of insured limits, we do not anticipate any losses with respect to such cash balances. For more details on the cash, cash equivalents, and marketable securities, refer to "—Note 3. Cash, Cash Equivalents, Marketable Securities, and Restricted Cash," in our Notes to Consolidated Financial Statements.

Net Cash Used in Operating Activities

Net cash used in operations for the six months ended June 30, 2019 was $137.0 million. The components of net cash used in operations included the net loss for the six months ended June 30, 2019 of $204.9 million and the net change in operating assets and liabilities of $2.1 million. The change in operating assets was primarily due to an increase in accounts receivable by $6.8 million due to increased commercial sales of Galafold®, an increase in prepaid and other current assets of $3.5 million to support commercial activities for Galafold® launch and an increase in inventory of $1.9 million. The net cash used in operations was also impacted by an increase in accounts payable and accrued expenses of $12.5 million, mainly related to program expenses and support for the commercial launch of Galafold®, partially offset by a decrease in deferred reimbursement of $1.5 million due to payment of a milestone.

Net cash used in operations for the six months ended June 30, 2018 was $106.5 million. The components of net cash used in operations included the net loss for the six months ended June 30, 2018 of $111.8 million and the net change in operating assets and liabilities of $22.0 million. The change in operating assets was primarily due to an increase in accounts receivable by $6.0 million and an increase in inventory of $3.4 million due to commercial sales of Galafold®, partially offset by a decrease in prepaid and other current assets of $4.6 million to support commercial activities for Galafold® launch. The net cash used in operations was also impacted by a decrease in accounts payable and accrued expenses of $11.8 million, mainly related to program expenses and support for the commercial launch of Galafold®, partially offset by a decrease in deferred reimbursement of $5.0 million due to payment of a milestone.

Net Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities for the six months ended June 30, 2019 was $65.0 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash provided by investing activities reflects $261.4 million for the sale and redemption of marketable securities, partially offset by $191.3 million for the purchase of marketable securities, and $5.1 million for the acquisition of property and equipment.

Net cash used in investing activities for the six months ended June 30, 2018 was $171.9 million. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. Net cash used in investing activities reflects $380.2 million for the purchase of marketable securities, and $1.9 million for the acquisition of property and equipment, partially offset by $210.2 million for the sale and redemption of marketable securities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2019 was $213.8 million. Net cash provided by financing activities primarily reflects $189.0 million from the issuance of common stock, net of issuance costs paid, $19.9 million from partial termination of capped call and $7.6 million from the exercise of stock options and warrants, partially offset by $2.6 million from the purchase of vested restricted stock units.

Net cash provided by financing activities for the six months ended June 30, 2018 was $303.6 million. Net cash provided by financing activities primarily reflects $294.6 million from the issuance of common stock, net of issuance costs, and $11.2 million from the exercise of stock options and warrants, partially offset by $2.0 million from the purchase of vested restricted stock units.

Funding Requirements

We expect to incur losses from operations for the foreseeable future primarily due to research and development expenses, including expenses related to conducting clinical trials. Our future capital requirements will depend on a number of factors, including:

- the progress and results of our preclinical and clinical trials of our drug candidates and gene therapy candidates;
- the cost of manufacturing drug and gene therapy supply for our clinical and preclinical studies, including the significant cost of manufacturing Pompe ERT and gene therapies;
- the scope, progress, results, and costs of preclinical development, laboratory testing and clinical trials for our product candidates including those testing the use of pharmacological chaperones co-formulated and co-administered with ERT and for the treatment of LDs and gene therapies for the treatment of rare genetic metabolic diseases;
- the future results of on-going preclinical research and subsequent clinical trials for CDD, including our ability to obtain regulatory approvals and commercialize CDKL5 therapies and obtain market acceptance for such therapies;
- the costs, timing, and outcome of regulatory review of our product candidates;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales, and distribution;
- the emergence of competing technologies and other adverse market developments;
- our ability to successfully commercialize Galafold® ("migalastat HCl");
- our ability to manufacture or supply sufficient clinical or commercial products;
- our ability to obtain reimbursement for Galafold®;
- our ability to satisfy post-marketing commitments or requirements for continued regulatory approval of Galafold®;
- our ability to obtain market acceptance of Galafold®;
- the costs of preparing, filing, and prosecuting patent applications and maintaining, enforcing, and defending intellectual property-related claims;
- the extent to which we acquire or invest in businesses, products, and technologies;
- our ability to successfully integrate our acquired products and technologies into our business, including the possibility that the expected benefits of the transactions will not be fully realized by us or may take longer to realize than expected;
- our ability to establish collaborations and obtain milestone, royalty, or other payments from any such collaborators;
- our ability to adjust to changes in European and United Kingdom markets as the United Kingdom leaves the E.U.; and
- fluctuations in foreign currency exchange rates; and changes in accounting standards.

While we continue to generate revenue from product sales, in the absence of additional funding, we expect our continuing operating losses to result in increases in our cash used in operations over the next several quarters and years. We may seek additional funding through public or private financings of debt or equity. We believe that our current cash position, including expected Galafold ® revenues, is sufficient to fund ongoing Fabry, Pompe, and gene therapy program operations into 2021. Potential future business development collaborations, pipeline expansion, and investment in manufacturing capabilities could impact our future capital requirements.

Financial Uncertainties Related to Potential Future Payments

Milestone Payments / Royalties

Celenex - With our acquisition of Celenex in 2018, we agreed to pay up to an additional $15 million in connection with the achievement of certain development milestones, $262 million in connection with the achievement of certain regulatory approval milestones across multiple programs and up to $75 million in tiered sales milestone payments.

NCH - Celenex has an exclusive license agreement with NCH. Under this license agreement, NCH is eligible to receive development and sales based milestones of up to $7.8 million from us for each product.

Penn - Under our expanded collaboration agreement with Penn, Penn is eligible to receive certain milestone, royalty and discovery research payments with respect to licensed products for each indication. Milestone payments are payable following the achievement of certain development and commercial milestone events in each indication, up to an aggregate of $86.5 million per indication. Royalty payments are based on net sales of licensed products on a licensed product-by-licensed product and country-by-country basis. We will provide $10.0 million each year during the five year agreement to fund the discovery research program.

MSSM - We acquired exclusive worldwide patent rights to develop and commercialize migalastat and other pharmacological chaperones for the prevention or treatment of human diseases or clinical conditions by increasing the activity of wild-type and mutant enzymes pursuant to a license agreement with Mount Sinai School of Medicine ("MSSM"). This agreement expired upon expiration of the last of the licensed patent rights, which occurred in 2018 in the U.S. and 2019 in Europe and Japan for monotherapy.

GSK - In November 2013, we entered into the Revised Agreement (the "Revised Agreement") with GlaxoSmithKline ("GSK"), pursuant to which we have obtained global rights to develop and commercialize migalastat as a monotherapy and in combination with ERT for Fabry disease. The Revised Agreement amends and replaces in its entirety the earlier agreement entered into between us and GSK in July 2012 (the "Original Collaboration Agreement"). Under the terms of the Revised Agreement, there was no upfront payment from us to GSK. For migalastat monotherapy, GSK is eligible to receive post-approval and sales-based milestones up to $40 million, as well as tiered royalties in the mid-teens in eight major markets outside the United States. In addition, because we reacquired worldwide rights to migalastat, we are no longer eligible to receive any milestones or royalties we would have been eligible to receive under the Original Collaboration Agreement.

Under our license agreements, if we owe royalties on net sales for one of our products to more than one of the above licensors, we have the right to reduce the royalties owed to one licensor for royalties paid to another. The amount of royalties to be offset is generally limited in each license and can vary under each agreement. For the six months ended June 30, 2019, under the MSSM and GSK license and collaboration agreements, we paid $7.2 million in royalties and $1.5 million in sales-based milestones.

Critical Accounting Policies and Significant Judgments

The discussion and analysis of our financial condition and results of operations are based on our financial statements, which we have prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

There were no significant changes during the six months ended June 30, 2019 to the items that we disclosed as our significant accounting policies and estimates described in "—Note 2. Summary of Significant Accounting Policies" to the Company's financial statements as contained in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2018, except as it relates to the adoption of ASU 2016-02, Leases (Topic 842), which is described below.

Leases

The Company primarily enters into lease agreements for office space, equipment, and vehicles. The leases have varying terms, some of which could include options to renew, extend, and early terminate. The Company determines if an arrangement is a lease at contract inception. Operating leases are included in right-of-use ("ROU") assets and lease liabilities on the Consolidated Balance Sheets.

ROU assets represent the Company's right to control the use of an explicitly or implicitly identified fixed asset for a period of time and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. ROU assets and liabilities are recognized at commencement date based on the present value of lease payments over the lease term. The Company uses its incremental borrowing rate based on the information available at commencement date in determining the present value of lease payments.

Lease payments included in the measurement of the lease liability are comprised of fixed payments. Variable lease payments are excluded from the ROU asset and lease liability and are recognized in the period in which the obligation for those payments is incurred. Variable lease payments are presented in the Consolidated Statements of Operations in the same line item as expenses arising from fixed lease payments for operating leases. The Company has lease agreements that include lease and non-lease components, which the Company accounts for as a single lease component for all underlying asset categories.

The lease term for all of the Company's leases include the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Leases with an initial term of 12 months or less are not recorded on the Consolidated Balance Sheets. The Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

The information presented for the periods prior to January 1, 2019 has not been restated and is reported under the accounting standard in effect for those periods. For additional information, see " —Note 9. Leases" and "—Note 2. Summary of Significant Accounting Policies, Recent Accounting Developments - Guidance Adopted in 2019."

Recent Accounting Pronouncements

Please refer to "—Note 2. Summary of Significant Accounting Policies," in our Notes to Consolidated Financial Statements.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Market risk is the risk of change in fair value of a financial instrument due to changes in interest rates, equity prices, creditworthiness, financing, exchange rates, or other factors. Our primary market risk exposure relates to changes in interest rates in our cash, cash equivalents, and marketable securities. We place our investments in high-quality financial instruments, primarily money market funds, corporate debt securities, asset backed securities, and U.S. government agency notes with maturities of less than one year, which we believe are subject to limited interest rate and credit risk. The securities in our investment portfolio are not leveraged, are classified as available-for-sale and, due to the short-term nature, are subject to minimal interest rate risk. We believe that a 1% (100 basis points) change in average interest rates would either increase or decrease the market value of our investment portfolio by $1.6 million as of June 30, 2019. We currently do not hedge interest rate exposure and consistent with our investment policy, we do not use derivative financial instruments in our investment portfolio.

We are exposed to interest rate risk with respect to variable rate debt. At December 31, 2018, we had $150 million aggregate principal amount of variable rate debt through our Senior Secured Term Loan. We do not currently hedge our variable interest rate debt. The average variable interest rate for our variable rate debt as of June 30, 2019 was 10.1%. A hypothetical 100 basis point increase or decrease in the average interest rate on our variable rate debt would not result in a material change in the interest expense.

We face foreign exchange risk as a result of entering into transactions denominated in currencies other than U.S. dollars. We are not currently engaged in any foreign currency hedging activities. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, and net product sales denominated in foreign currencies. Both positive and negative impacts to our international product sales from movements in foreign currency exchange rates may be partially mitigated by the natural, opposite impact that foreign currency exchange rates have on our international operating expenses. A hypothetical 10% change in foreign exchange rates during any of the periods presented would not have had a material impact on our consolidated financial statements.

For information regarding our exposure to certain market risks, see Item 7A, Quantitative and Qualitative Disclosures About Market Risk, in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018. There have been no material changes in our financial instrument portfolio or market risk exposures since our fiscal year ended December 31, 2018.

ITEM 4. CONTROLS AND PROCEDURES

As of the end of the period covered by this Quarterly Report on Form 10-Q, an evaluation of the effectiveness of our disclosure controls and procedures (pursuant to Rule 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act") was carried out under the supervision of our Principal Executive Officer and Principal Financial Officer, with the participation of our management. Based on that evaluation, the Principal Executive Officer and the Principal Financial Officer concluded that, as of the end of such period, our disclosure controls and procedures are effective in recording, processing, summarizing and reporting, on a timely basis, information required to be disclosed by us in the reports that we file or submit under the Exchange Act and are effective in ensuring that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our Principal Executive Officer and Principal Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

During the fiscal quarter covered by this report, there has been no change 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 last fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are not currently a party to any legal proceedings.

ITEM 1A. RISK FACTORS

There have been no material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018.

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

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

None.

ITEM 6. EXHIBITS

Exhibit Number	Description
10.1	Form of Capped Call Transaction Termination Agreement
10.2	Amended and Restated Research, Collaboration & License Agreement, dated May 28, 2019, by and between Amicus Therapeutics, Inc. and the Trustees of the University of Pennsylvania.*
10.3	Form of Exchange Agreement Relating to Company's 3.00% Convertible Senior Notes due 2023, incorporated by reference to Exhibit 10.1 of the Current Report on Form 8-K filed with the Securities and Exchange Commission on June 19, 2019.
31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rules 13a-14 and 15d-14 promulgated pursuant to the Securities Exchange Act of 1934, as amended
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document

* Certain confidential portions of this exhibit were omitted in accordance with Item 601(b)(10) of Regulation S-K.

SIGNATURES

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

AMICUS THERAPEUTICS, INC.

Date:	August 8, 2019 By:	/s/ John F. Crowley
		John F. Crowley
		Chairman and Chief Executive Officer
		(Principal Executive Officer)
Date:	August 8, 2019 By:	/s/ Daphne Quimi
		Daphne Quimi
		Chief Financial Officer
		(Principal Financial Officer)

TERMINATION AGREEMENT

This TERMINATION AGREEMENT (this "**Termination Agreement**") with respect to the Call Option Confirmations (as defined below) is made as of [●] between [●] ("**Dealer**") and Amicus Therapeutics, Inc. ("**Counterparty**"), a Delaware corporation.

WHEREAS, Counterparty issued $250,000,000 principal amount of 3.00% Convertible Senior Notes due 2023 (the "**Convertible Notes**") pursuant to an Indenture dated as of December 21, 2016 between Counterparty and Wilmington Trust, National Association, as trustee;

WHEREAS, Dealer and Counterparty are parties to the base capped call option transaction (as amended, modified or supplemented, the "**Base Call Option Transaction**") evidenced by the letter agreement between Dealer and Counterparty, dated as of December 15, 2016 (as amended, modified or supplemented, the "**Base Call Option Confirmation**"), and the additional capped call option transaction (as amended, modified or supplemented, the "**Additional Call Option Transaction**" and, together with the Base Call Option Transaction, the "**Call Option Transactions**") evidenced by the letter agreement between Dealer and Counterparty, dated as of December 19, 2016 (as amended, modified or supplemented, the "**Additional Call Option Confirmation**" and, together with the Base Call Option Confirmation, the "**Call Option Confirmations**"); and

WHEREAS, the parties partially terminated both the Base Call Option Transaction and the Additional Call Option Transaction on [●] (the "**Partial Unwind**");

WHEREAS, the Counterparty has requested the full termination of both the Base Call Option Transaction and the Additional Call Option Transaction.

NOW, THEREFORE, in consideration of their mutual covenants contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto, intending to be legally bound, hereby mutually covenant and agree as follows:

1. *Defined Terms*. Any capitalized term not otherwise defined herein shall have the meaning set forth for such term in the Call Option Confirmations.

2. *Termination of Transactions and Confirmations*. Notwithstanding anything to the contrary in the Call Option Confirmations, Counterparty and Dealer agree that, effective on the date hereof, (i) the Base Call Option Transaction shall automatically terminate and all of the respective rights and obligations of the parties under the Base Call Option Confirmation shall be terminated, cancelled and extinguished, (ii) the Additional Call Option Confirmation shall automatically terminate and all of the respective rights and obligations of the parties under the Additional Call Option Confirmation shall be terminated, cancelled and extinguished and (iii) in consideration of the foregoing, Dealer shall pay to Counterparty, in immediately available funds to the account specified below, cash in US Dollars in an amount equal to the Cash Settlement Amount no later than 4:00 p.m. (New York City time) on the first Clearance System Business Day following the conclusion of the Hedge Unwind Period. "**Cash Settlement Amount**" and "**Hedge Unwind Period**" shall have the meanings assigned to such terms in Exhibit A hereto.

3. *Representations and Warranties of Counterparty*. Counterparty represents and warrants to Dealer on the date hereof that:

(a) it is duly organized and validly existing under the laws of the jurisdiction of its organization or incorporation and, if relevant under such laws, in good standing;

(b) it has the power to execute this Termination Agreement and any other documentation relating to this Termination Agreement, to deliver this Termination Agreement and each such other document relating thereto (if any) and to perform its obligations hereunder and thereunder (as applicable) and has taken all necessary action to authorize such execution, delivery and performance;

(c) such execution, delivery and performance do not violate or conflict with any law applicable to it, any provision of its constitutional documents, any order or judgment of any court or other agency of government applicable to it or any of its assets or any contractual restriction binding on or affecting it or any of its assets;

(d) all governmental and other consents that are required to have been obtained by it with respect to this Termination Agreement have been obtained and are in full force and effect and all conditions of any such consents have been complied with;

(e) its obligations under this Termination Agreement constitute its legal, valid and binding obligations, enforceable in accordance with their respective terms (subject to applicable bankruptcy, reorganization, insolvency, moratorium or similar laws affecting creditors' rights generally and subject, as to enforceability, to equitable principles of general application (regardless of whether enforcement is sought in a proceeding in equity or at law));

(f) it is not aware of any material nonpublic information regarding Counterparty or the Shares;

(g) it (A) is capable of evaluating investment risks independently, both in general and with regard to all transactions and investment strategies involving a security or securities; (B) will exercise independent judgment in evaluating the recommendations of any broker-dealer or its associated persons, unless it has otherwise notified the broker-dealer in writing; and (C) has total assets of at least $50 million;

(h) it is not entering into this Termination Agreement to create actual or apparent trading activity in the Shares (or any security convertible into or exchangeable for the Shares) or to raise or depress or otherwise manipulate the price of the Shares (or any security convertible into or exchangeable for the Shares) or otherwise in violation of the Exchange Act; and

(i) on the date hereof, it remains a party to the Call Option Transactions to the full extent as on the date of execution thereof (after taking into account the Partial Unwind) and it has not assigned, purported to assign or made any attempt to assign, any interest in the Call Option Transactions to any third party.

4. *Representations and Warranties of Dealer*. Dealer represents and warrants to Counterparty on the date hereof that:

(a) it is duly organized and validly existing under the laws of the jurisdiction of its organization or incorporation and, if relevant under such laws,

in good standing;

(b) it has the power to execute this Termination Agreement and any other documentation relating to this Termination Agreement to which it is a party, to deliver this Termination Agreement and each such other document (if any) and to perform its obligations hereunder and thereunder (as applicable) and has taken all necessary action to authorize such execution, delivery and performance;

(c) such execution, delivery and performance do not violate or conflict with any law applicable to it, any provision of its constitutional documents, any order or judgment of any court or other agency of government applicable to it or any of its assets or any contractual restriction binding on or affecting it or any of its assets;

(d) all governmental and other consents that are required to have been obtained by it with respect to this Termination Agreement have been obtained and are in full force and effect and all conditions of any such consents have been complied with;

(e) its obligations under this Termination Agreement constitute its legal, valid and binding obligations, enforceable in accordance with their respective terms (subject to applicable bankruptcy, reorganization, insolvency, moratorium or similar laws affecting creditors' rights generally and subject, as to enforceability, to equitable principles of general application (regardless of whether enforcement is sought in a proceeding in equity or at law)); and

(f) on the date hereof, it remains a party to the Call Option Transactions to the full extent as on the date of execution thereof (after taking into account the Partial Unwind) and it has not assigned, purported to assign or made any attempt to assign, any interest in the Call Option Transactions to any third party.

5. *Notices*. For purposes of this Termination Agreement, the addresses for notices or communications to the parties shall be:

(a) Counterparty:

Amicus Therapeutics, Inc.
1 Cedar Brook Drive
Cranbury, NJ 08512
Attention: **[●]**
Telephone No.: **[●]**
Email.: **[●]**

With a copy to:

Attention: **[●]**
Telephone No.: **[●]**
Email: **[●]**

(b) Dealer:

Attention: [●]
Telephone: [●]
Facsimile: [●]
Email: [●]

With a copy to:

Attention: [●]
Telephone: [●]
Email: [●]

And email notification to the following address:
[●]

6. *Account Details*. For purposes of this Termination Agreement, the account for payments to Counterparty shall be:

Bank: [●]
ABA#: [●]
Acct No.: [●]
Beneficiary: [●]
SWIFT ID: [●]

7. *Disclosure*. Notwithstanding anything provided in this Termination Agreement or the Call Option Confirmations, effective from the date of commencement of discussions concerning the Call Option Transactions, Counterparty and each of its employees, representatives, or other agents may disclose to any and all persons, without limitation of any kind, the tax treatment and tax structure of the Call Option Transactions and this Termination Agreement and all materials of any kind (including opinions or other tax analyses) that are provided to Counterparty relating to such tax treatment and tax structure.

8. *No Reliance, etc*. Counterparty hereby confirms that it has relied on the advice of its own counsel and other advisors (to the extent it deems appropriate) with respect to any legal, tax, accounting, or regulatory consequences of this Termination Agreement, that it has not relied on Dealer or its affiliates in any respect in connection therewith, and that it will not hold Dealer or its affiliates accountable for any such consequences.

9. *[Reserved]*

10. *Designation by Dealer*. Notwithstanding any other provision in this Termination Agreement to the contrary requiring or allowing Dealer to purchase, sell, receive or deliver any Shares or other securities to or from Counterparty, Dealer may designate any of its affiliates to purchase, sell, receive or deliver such shares or other securities and otherwise to perform Dealer obligations in respect of the transactions contemplated by this Termination Agreement and any such designee may assume such obligations. Dealer shall be discharged of its obligations to Counterparty to the extent of any such performance.

11. *Governing Law*. THIS TERMINATION AGREEMENT AND ALL MATTERS ARISING IN CONNECTION WITH THIS TERMINATION AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED AND ENFORCED IN ACCORDANCE WITH, THE LAWS OF [●] (WITHOUT REFERENCE TO ITS CHOICE OF LAW DOCTRINE, [●]).

12. *Waiver of Trial by Jury*. EACH OF COUNTERPARTY AND DEALER HEREBY IRREVOCABLY WAIVES (ON ITS OWN BEHALF AND, TO THE EXTENT PERMITTED BY APPLICABLE LAW, ON BEHALF OF ITS STOCKHOLDERS) ALL RIGHT TO TRIAL BY JURY IN ANY ACTION, PROCEEDING OR COUNTERCLAIM (WHETHER BASED ON CONTRACT, TORT OR OTHERWISE) ARISING OUT OF OR RELATING TO THE TRANSACTION OR THE ACTIONS OF DEALER OR ITS AFFILIATES IN THE NEGOTIATION, PERFORMANCE OR ENFORCEMENT HEREOF.

13. *Amendment*. This Termination Agreement may not be modified, amended or supplemented, except in a written instrument signed by each party hereto.

14. *Counterparts*. This Termination Agreement may be executed in several counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument.

15. *Agreements and Acknowledgements Regarding Hedging*. Counterparty acknowledges and agrees that:

(a) on or following the date hereof, Dealer and its Affiliates may buy or sell Shares or other securities or buy or sell options or futures contracts or enter into swaps or other derivative securities in order to adjust its hedge position with respect to this Termination Agreement;

(b) Dealer and its Affiliates also may be active in the market for Shares other than in connection with hedging activities in relation to this Termination Agreement;

(c) Dealer shall make its own determination as to whether, when or in what manner any hedging or market activities in Counterparty's securities shall be conducted and shall do so in a manner that it deems appropriate to hedge its price and market risk; and

(d) any market activities of Dealer and its Affiliates with respect to Shares may affect the market price and volatility of Shares each in a manner that may be adverse to Counterparty.

[*The remainder of page intentionally left blank*]

IN WITNESS WHEREOF the parties have executed this Termination Agreement with effect from the date specified on the first page of this Termination Agreement.

[●]

By: _____

Name:

Title:

Amicus Therapeutics, Inc.

By: _____
Name:
Title:

EXHIBIT A

VWAP Price	Cash Settlement Amount
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]
$[●]	$[●]

$[●]	$[●]

"**Cash Settlement Amount**" means the amount set forth in the table above opposite the applicable VWAP Price (as defined below). If the VWAP Price is not specified on the table above, the Cash Settlement Amount shall be the straight-line interpolation between the VWAP Prices or extrapolation from the VWAP Prices (as the case may be) specified on the table above. Dealer shall notify Counterparty of the applicable Cash Settlement Amount as soon as reasonably practicable after 5:00pm (New York City time) on the last Scheduled Trading Day of the Hedge Unwind Period.

"**VWAP Price**" means the arithmetic average of the Daily Prices for all Exchange Business Days in the Hedge Unwind Period, as determined by Dealer in good faith and a commercially reasonable manner.

"**Daily Price**" means, for any Exchange Business Day, the per Share volume-weighted average price as displayed under the heading "Bloomberg VWAP" on Bloomberg Page "FOLD <Equity> AQR" (or any successor thereto) for each such Exchange Business Day in respect of the period from the scheduled opening time of the Exchange to the Scheduled Closing Time of the Exchange on such Exchange Business Day (or, if such volume-weighted average price is unavailable at such time, the market value of one Share on such Exchange Business Day, as determined by the Calculation Agent using, if practicable, a volume-weighted average method). The VWAP Price will be determined without regard to after-hours trading or any other trading outside of the regular trading session trading hours.

"**Hedge Unwind Period**" means the Scheduled Trading Day occurring on [●]; *provided* that, notwithstanding anything to the contrary in this Termination Agreement, Dealer may postpone such Scheduled Trading Day or extend the Hedge Unwind Period by one or more Scheduled Trading Days if Dealer determines, in its reasonable discretion, that such postponement or extension is reasonably necessary or appropriate to preserve Dealer's hedge unwind activity hereunder in light of existing liquidity conditions or to enable Dealer (or any of its affiliates) to effect transactions of Shares in connection with its hedge unwind activity hereunder in a manner that would, if Dealer (or such affiliate) were Counterparty or an affiliated purchaser of Counterparty, be in compliance with applicable legal, regulatory or self-regulatory requirements, or with related policies and procedures applicable to Dealer.

CERTAIN INFORMATION IDENTIFIED WITH THE MARK "[***]" HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE SUCH INFORMATION IS BOTH (I) NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

Execution Version

AMENDED AND RESTATED

RESEARCH, COLLABORATION & LICENSE AGREEMENT DATED AS OF MAY 28, 2019

BY AND BETWEEN

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA AND

AMICUS THERAPEUTICS, INC.

UNIVERSITY OF PENNSYLVANIA

AMENDED AND RESTATED RESEARCH, COLLABORATION & LICENSE AGREEMENT

This Amended and Restated Research, Collaboration & License Agreement (this "**Agreement**") is dated as of May 28, 2019 (the "**New Effective Date**") by and between The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation ("**Penn**"), and Amicus Therapeutics, Inc., a corporation organized under the laws of the state of Delaware ("**Licensee**"). Penn and Licensee may be referred to herein as a "**Party**" or, collectively, as "**Parties**".

RECITALS:

WHEREAS, Licensee is a biopharmaceutical company with expertise in the development, manufacture and commercialization of human therapeutic products for treatment of genetic disorders.

WHEREAS, Penn, through Dr. James Wilson and the Wilson Lab, have technology and expertise in the research and development of gene therapy products.

WHEREAS, the Research Programs contemplated by this Agreement are of mutual interest to Licensee and Penn and furthers the educational, scholarship and research objectives of Penn as a nonprofit, tax-exempt, educational university, and may benefit Licensee and Penn through the creation or discovery of new inventions and the development and commercialization of Licensed Products (as defined below).

WHEREAS, Penn and Licensee are parties to that certain Research, Collaboration & License Agreement dated as of October 8, 2018 (the "**Effective Date**") pursuant to which the Parties established a collaboration regarding the research and development of gene therapy products for certain indications (the "**Original Agreement**").

WHEREAS, Penn and Licensee desire to amend and restate the Original Agreement as of the New Effective Date to include certain mutually agreed amendments to the terms of the Original Agreement, all as set forth in more detail in the Agreement below.

NOW, THEREFORE, in consideration of the various promises and undertakings set forth herein, the Parties agree as follows:

ARTICLE 1 DEFINITIONS

Unless otherwise specifically provided herein, the following terms shall have the following meanings:

1.1 "**AAV**" means adeno-associated virus.

1.2 "**Achievement Date**" means with respect to a Diligence Event, the corresponding date such Diligence Event is to be achieved as provided in Sections 7.8 and 7.9 below.

1.3 "**Affiliate**" means with respect to a Person, any corporation or other business entity that controls, is controlled by or is under common control with such Person, but only for so long as such control exists. For the purposes of this Section 1.3, the word "control" (including, with correlative meaning, the terms "controlled by" or "under the common control with") means the

affirmative power, either directly or indirectly through one or more intermediaries, to direct the management and policies of such Person or entity, whether by the ownership of more than fifty percent (50%) of the voting stock of such entity, or by contract or otherwise.

1.4 "**BLA**" means (a) a Biologics License Application as defined in the FD&C Act and the regulations promulgated thereunder, (b) a Marketing Authorization Application ("**MAA**") in the European Union, or (b) any equivalent or comparable application, registration or certification in any other country or region.

1.5 "**Calendar Quarter**" mean the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31 of each Calendar Year.

1.6 "**Calendar Year**" means each successive period of twelve (12) months commencing on January 1 and ending on December 31.

1.7 **"cGLP"** means the current good laboratory practice regulations promulgated by the FDA, published at 21 U.S C.F.R. § 58, and equivalent non-United States regulations and standards in the Territory, as applicable, as such current laboratory practices, regulations and standards may be amended from time to time.

1.8 **"cGMP"** means those current practices, as amended from time to time, related to the manufacture of pharmaceutical products and any precursors thereto promulgated in guidelines and regulations of standard compilations including the GMP Rules of the World Health Organization, the United States Code of Federal Regulations, the Guide to Inspection of Bulk Pharmaceutical Chemicals (established by the United States Department of Health and Human Services), the Pharmaceutical Inspection Convention, and the European Community Guide to Good Manufacturing Practice in the production of pharmaceutical products, and equivalent guidelines, regulations and standards in the Territory, as such guidelines, regulations and standards may be amended from time to time.

1.9 "**Challenge**" means Licensee or a Sublicensee will be deemed to have made a "Challenge" of the Penn Patent Rights if Licensee or a Sublicensee: (a) institutes or voluntarily joins as a party to, or causes its counsel to institute on Licensee's or such Sublicensee's behalf, any interference, opposition, re-examination, post-grant review or similar proceeding with respect to any Penn Patent Right with the U.S. Patent and Trademark Office or any foreign patent office; or (b) files or voluntarily joins as a party to any legal proceeding, or causes its counsel to institute or voluntarily join as a party to any legal proceeding on Licensee's or such Sublicensee's behalf, with a court or other Governmental Body (including, without limitation, the U.S. Patent and Trademark Office or any foreign patent office) having authority to determine the validity, enforceability or scope of the Penn Patent Rights, in which one or more claims in such legal proceeding challenges the validity or enforceability of any Penn Patent Right.

1.10 "**Change of Control**" means the occurrence of any of the following events: (a) any party becomes the owner, directly or indirectly, of more than fifty percent (50%) of the total voting power (on an as converted basis) of the equity units or other interests of Licensee then outstanding that are normally entitled to vote in the election of directors of Licensee other than in connection with a financing or series of financing transactions; (b) the merger, consolidation or amalgamation of Licensee with or into any other party, other than any transaction in which the holders of the outstanding voting securities of Licensee immediately prior to the transaction own, directly or indirectly, not less than fifty percent (50%) of the total voting power (on an as converted basis) of the voting securities of the party surviving such merger, consolidation or amalgamation; or (c) the sale of all or substantially all of the assets of Licensee.

1.11 "**Clinical Study**" means (a) a Phase 1 Study, Phase 1/2 Study, Phase 2 Study, or Phase 3 Study, or (b) such other study in humans that is conducted in accordance with good clinical practices and is designed to generate data in support or maintenance of an application for Regulatory Approval.

1.12 "**Commercially Reasonable Efforts**" means [***].

1.13 "**Compulsory License**" means a compulsory license under Penn Patent Rights obtained by a Third Party through the order, decree, or grant of a competent Governmental Body or court, authorizing such Third Party to develop, make, have made, use, sell, offer to sell or import a Licensed Product in any country.

1.14 "**Confidential Information**" of a Party, means (i) confidential or proprietary information or materials relating to the business, operations, technology or products of a Party or any of its Affiliates, including any know-how, that such Party discloses to the other Party under this Agreement, or otherwise makes available to the other Party under this Agreement, and (ii) the terms of this Agreement; provided that Confidential Information shall not include information that:

(a) is or becomes generally available to the public other than as a result of disclosure by the recipient in breach of this Agreement;

(b) is already known by or in the possession of the recipient at the time of disclosure by the disclosing Party;

(c) is independently developed by recipient without use of or reference to the disclosing Party's Confidential Information; or

(d) is obtained by recipient from a Third Party that has not breached any obligations of confidentiality to the disclosing party.

1.15 "**Controlled**" means, with respect to intellectual property rights, that a Party or one of its Affiliates owns or has a license or sublicense to such intellectual property rights and has the ability to provide to, grant a license or sublicense to, or assign its right, title and interest in and to, such intellectual property rights as provided for in the Agreement without violating the terms of any other agreement or other arrangement with any Third Party.

1.16 "**Designated Product**" means a Licensed Product arising from a Research Program [***]

1.17 "**Development Transition Point**" or "**DTP**" means on a Licensed Product-by-Licensed Product basis the date on which the IND enabling studies for a Pre-Designation Product under a Research Program have been successfully completed and immediately prior to filing of the IND, unless otherwise agreed by the Parties.

1.18 **"Diligence Event"** means each of the events that Licensee is expected to accomplish in the development of a Licensed Product in each Indication set forth in Sections 7.8 and 7.9.

1.19 "**Discovery Patent Rights**" means [***].

1.20 "**Discovery Plan**" means the plan for the Tasks or the portions of Tasks set forth in Exhibit F hereto, as may be updated in accordance with Section 3.1.

1.21 "**Discovery Product**" means [***].

1.22 "**Discovery Product Proceeds**" means [***].

1.23 "**Discovery Program**" means the discovery research conducted at Penn solely by the Wilson Lab during the Discovery Term to [***].

1.24 "**Discovery Program Extension**" means [***].

1.25 "**Discovery Program Period**" means the period beginning [***] and ending on [***].

1.26 "**Discovery Program Quarter**" means each successive period of three (3) consecutive months during the period beginning on the New Effective Date and thereafter for the remainder of the Discovery Term. The first Discovery Program Quarter will commence on the New Effective Date.

1.27 "**Discovery Program Year** " means each successive period of twelve (12) consecutive months during the period beginning on the New Effective Date and thereafter for the remainder of the Discovery Term. The first Discovery Program Year will commence on the New Effective Date.

1.28 "**Discovery Results**" means all any and all ideas, information, inventions, developments, animate and inanimate materials, including live animals, discoveries, software, know-how, methods, techniques, formulae, data, processes, methodologies, techniques, biological materials, software and works of authorship, whether patentable or copyrightable, that are first conceived, discovered, developed, reduced to practice, or generated in the performance of the Discovery Program by the Wilson Lab, including any unpatentable inventions discovered, developed or conceived in the conduct of the Discovery Program. Discovery Results expressly excludes any such items covered by Penn Patent Rights and Joint Patent Rights.

1.29 "**Discovery Term**" means the Discovery Program Period plus the period of any Discovery Program Extension(s).

1.30 "**DRG Technology**" means [***].

1.31 "**EMA**" means the European Medicines Agency and any successor entity thereto.

1.32 "**Exploratory Indication**" means [***].

1.33 "**FDA**" means the United States Food and Drug Administration and any successor entity thereto.

1.34 "**FD&C Act**" means the United States Federal Food, Drug and Cosmetic Act, as amended.

1.35 "**Field of Use**" means all research, prophylactic, diagnostic and therapeutic uses in or for humans. For clarity, except for any uses in non-humans intended to support development for prophylactic, diagnostic and/or therapeutic use in humans, any and all uses in non-humans, including any and all veterinary uses in companion animals and livestock species, is excluded from the Field of Use.

1.36 "**FIH**" means, on a Licensed Product-by-Licensed Product basis, the first dosing of the first patient in a Clinical Study.

1.37 "**First Commercial Sale**" means, on a country-by-country basis, the first commercial transfer or disposition for monetary value of Licensed Product in such country for use or consumption by a Third Party end user by Licensee, or any of its Affiliates or Sublicensees, in each case, after all Regulatory Approvals have been obtained for such country and where such disposition or transfer results in a recordable Net Sale in accordance with Licensee's, or its Affiliate's or Sublicensee's, applicable accounting practices (consistently applied). Sales prior to receipt of Regulatory Approval of a Licensed Product such as so-called "treatment IND sales," "named patient sales," "compassionate use sales" or expanded access programs, shall not be considered a First Commercial Sale.

1.38 "**Force Majeure Event**" means any circumstance beyond the affected Party's reasonable control to foresee, including, without limitation, labor disturbances or labor disputes of any kind, unforeseeable acts, omissions or delays in acting by any Governmental Body required for full performance (except to the extent such delay results from a breach by the affected Party of a term of this Agreement), civil disorders or commotions, strikes, acts of war, terrorism, acts of God, energy or other conservation measures imposed by law or regulation, explosions, failure of utilities, mechanical breakdowns, material shortages, or disease.

1.39 "**FPFD**" means, on a Licensed Product-by Licensed Product basis with respect to each Clinical Study, the first dosing of the first patient in such Clinical Study.

1.40 "**Funded Discovery Patent Rights**" means [***].

1.41 "**GAAP**" means United States generally accepted accounting principles applied on a consistent basis.

1.42 "**Gene Editing Technologies**" means nucleic acid polymers that encode proteins whose primary recognized enzymatic activity is to (i) selectively induce double or single stranded breaks in a DNA or RNA sequence, or (ii) substitute, replace or delete a particular base or set of bases of a DNA or RNA sequence in the absence of a double or single stranded break in the DNA or RNA. Gene Editing Technologies include, but are not limited to: CRISPR-Cas systems (including different Cas nucleases), Zinc finger nucleases, meganucleases, TALENS or base editors. For clarity, "Gene Editing Technologies" does not include chromosomal integration of a transgene introduced by a parvovirus vector in the absence of exogenous nucleases.

1.43 "**Generic Product**" means, with respect to a particular Licensed Product in a country, a generic or biosimilar pharmaceutical product, that is not licensed or owned by Licensee, any of its Affiliates or Sublicensees, that is approved for use in such country by a Regulatory Authority by referencing the prior approval, in whole or part, or safety and efficacy data submitted in support of the prior approval, of such Licensed Product.

1.44 "**Governmental Body**" means any: (a) nation, principality, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (b) federal, provincial, state, local, municipal, foreign or other government; (c) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative, organization, unit, body or entity and any court or other tribunal); (d) multi- national or supranational organization or body; or (e) individual, entity, or body exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

1.45 "**IND**" means an Investigational New Drug Application as defined in the FD&C Act and the regulations promulgated thereunder, or (b) an equivalent application to an equivalent Regulatory Authority in any other regulatory jurisdiction, including a Clinical Trial Authorization ("**CTA**") to the European Medicines Agency, the filing of which is necessary to initiate or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.

1.46 "**Indication**" means each of (a) CDKL5 deficiency [***] ("**CDKL5 Deficiency Disorder**"), (b) Pompe disease ("**Pompe Disease**"), (c) Fabry disease ("**Fabry Disease**"), (d) Niemann Pick Type C ("**NPC**"), (e) Mucopolysaccharidosis Type IIIA ("**MPS IIIA**"), (f) Mucopolysaccharidosis Type IIIB ("**MPS IIIB**") and (g) [***].

1.47 "**Joint Patent Rights**" means (a) any Patent Rights covering an invention conceived and reduced to practice jointly by the Wilson Lab and Licensee in the conduct of a Research Program, (b) any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues of any of the Patent Rights in (a), including divisions, but excluding continuations-in-part except to the extent of claims solely supported in the specification and entitled to the priority date of the parent application for any of the foregoing, and (c) any corresponding Patent Rights to the foregoing outside of the United States.

1.48 "**Know-How**" means intellectual property, data, results, pre-clinical and clinical protocols and study data, chemical structures, chemical sequences, information, inventions, formulas, techniques, methods, processes, procedures and developments. "Know-How" does not include any of the foregoing claimed in a Penn Patent Right or Patent Right Controlled by Licensee.

1.49 "**Law**" or "**Laws**" means all applicable laws, statutes, rules, regulations, ordinances and other pronouncements having the binding effect of law of any Governmental Body.

1.50 "**Licensed Discovery Know-How**" means all Know-How that is Controlled by Penn as of the Effective Date or during the Discovery Term and developed by the Wilson Lab under the Discovery Program and is necessary or reasonably useful to develop, make, use, sell, offer for sale or import a Designated Product for an Indication.

1.51 "**Licensed Know-How**" means all Know-How that is Controlled by Penn as of the Effective Date or during the Research Term and (a) developed by the Wilson Lab as of the Effective Date of the Agreement, or (b) developed by the Wilson Lab under a Research Program, and in each case (a) and (b) is necessary or reasonably useful to develop, make, use, sell, offer for sale or import a Licensed Product for an Indication in the Field of Use.

1.52 "**Licensed Product**" means any (a) article, composition, apparatus, substance, chemical or any other material covered by a Program Valid Claim or whose manufacture, import, use, offer for sale or sale would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Program Valid Claim or would infringe a Program Valid Claim once issued; (b) article, composition, apparatus, chemical, substance or any other material made, used or sold by or utilizing or practicing a Method, (c) article, composition, apparatus, substance, chemical or any other material that incorporates, uses or is made through the use of any Licensed Know-How and is an parvovirus gene therapy for an Indication or (d) any parvovirus gene therapy for an Indication conceived during or tested in a Research Program. Notwithstanding the foregoing, "Licensed Product" shall not include a product, to the extent it would have solely been a Licensed Product pursuant to subsection (c) above, that is, or was prior to the Effective Date, (i)

acquired or licensed by Licensee or any of its Affiliates (including pursuant to a Change of Control) or (ii) controlled by a Third Party acquirer of Licensee or any of its Affiliates (whether by merger or acquisition of all or substantially all of the stock or assets of Licensee or its Affiliate or a similar transaction) and, in each case ((i) and (ii)), that has been independently developed by a Third Party and for which [***] prior to the closing of such acquisition or license by Licensee or any of its Affiliates (with respect to clause (i) above) or such merger, acquisition or similar transaction (with respect to clause (ii) above).

1.53 "**Lock-Up Term**" [***].

1.54 "**Lysosomal Storage Disease**" means any disease, state or condition inherited in an autosomal or x-linked recessive manner caused by the loss of function of a lysosomal protein (including lysosomal membrane proteins) that are characterized by a progressive accumulation of molecular substrates in the lysosome provoking cellular dysfunction and clinical manifestations.

1.55 "**Lysosomal Storage Disease Indication**" or "**LSD Indication**" means any indication that is a Lysosomal Storage Disease; provided that each of the following will not be included as a Lysosomal Storage Disease Indication as of the New Effective Date: (a) any Lysosomal Storage Disease obligated to a Third Party by Penn through any contractual mechanism entered into by Penn prior to the New Effective Date as set forth in Schedule 1.55, (b) [***], (c) any Potential Indication and (d) [***], but in each case, (a) and (d), shall each automatically be included as a Lysosomal Storage Disease Indication under this Agreement if the Third Party to whom Penn granted an option to license certain intellectual property controlled by Penn for use in connection with a Lysosomal Storage Disease described in clause (a) or (d) of this Section 1.55, as applicable, releases its rights with respect thereto. If and when such Third Party releases such rights, Penn will promptly notify Licensee. [***].

1.56 "**Major Markets**" means [***].

1.57 "**Manufacturing Patent Rights**" means, [***], any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues thereof, including divisions, but excluding continuations-in-part except to the extent of claims solely supported in the specification and entitled to the priority date of the parent application, and (c) any corresponding foreign Patent Rights to the foregoing.

1.58 "**Method**" means process or method covered by a Program Valid Claim or whose use or practice would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Program Valid Claim, or would infringe a Program Valid Claim once issued.

1.59 "**Net Sales**" means the gross amounts billed, invoiced or received by Licensee or any of its Affiliates or Sublicensees for Sales of Licensed Product (including any cash amounts plus the fair market value of any other forms of consideration), less the following deductions to the extent reasonable customary, and actually deducted:

1.59.1 [***];

1.59.2 [***];

1.59.3 [***];

1.59.4 [***];

1.59.5 [***].

Even if there is overlap between any of deductions described above, each individual item shall only be deducted once in the overall Net Sales calculation. Net Sales shall not include sales or other transfers or dispositions of Licensed Products between or among Licensee, Sublicensees or their Affiliates. [***].

1.60 "**Next Generation Capsid**" means a specific parvovirus capsid identified by sequence that is discovered, developed or engineered in the Discovery Program.

1.61 "**Next Generation Capsid Data Package**" means a written data package prepared by the Wilson Lab with respect to a Next Generation Capsid containing: [***].

1.62 "**Patent Rights**" means (a) patents and patent applications, together with any unlisted patents and patent applications claiming priority thereto, and any continuations, continuations-in-part (to the extent related directly to the subject matter of the parent application or containing new information developed pursuant to a Research Program), reissues, reexamination certificates, substitutions, divisionals, supplementary protection certificates, renewals, registrations, extensions including all confirmations, revalidations, patents of addition, PCTs, and pediatric exclusivity periods and all foreign counterparts thereof, and any patents issued or issuing with respect to any of the foregoing and (b) all official correspondence relating to the foregoing.

1.63 "**Parvovirus Gene Therapy Product**" means a product (or proposed or prospective product) that inserts one or more functional genes into a patient's cells using a parvovirus vector to treat an indication [***].

1.64 "**Payee**" means the Party owed or receiving a payment under this Agreement.

1.65 "**Payor**" means the Party owing or making a payment under this Agreement.

1.66 "**Penn Patent Rights**" means Penn Patent Rights A (including Penn's interest in the Joint Patent Rights), Discovery Patent Rights (including Penn Patent Rights B), Penn Patent Rights C, and Manufacturing Patent Rights, collectively.

1.67 "**Penn Patent Rights A**" means [***] any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues of any of the Patent Rights in (a) or (b), including divisions, but excluding continuations-in-part except to the extent of claims solely supported in the specification and entitled to the priority date of the parent application for any of the foregoing, and (d) any corresponding foreign Patent Rights to the foregoing.

1.68 "**Penn Patent Rights B**" means [***], (b) any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues thereof, including divisions, but excluding continuations-in-part except to the extent of claims entirely solely supported in the specification and entitled to the priority date of the parent application for any of the foregoing, and (c) any corresponding foreign Patent Rights to the foregoing.

1.69 "**Penn Patent Rights C**" means [***], any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or

reissues thereof, including divisions, but excluding continuations-in-part except to the extent of claims solely supported in the specification and entitled to the priority date of the parent application, and (c) any corresponding foreign Patent Rights to the foregoing.

1.70 "**Person**" means any natural person, corporation, firm, business trust, joint venture, association, organization, company, partnership or other business entity, or any government or agency or political subdivision thereof.

1.71 "**Phase 1 Study**" means a clinical study of a drug candidate in patients with the primary objective of characterizing its safety, tolerability, and pharmacokinetics and identifying a recommended dose and regimen for future studies as described in 21 C.F.R. 312.21(a), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States. The drug candidate can be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.72 "**Phase 1/2 Study**" means a clinical study of a drug candidate in diseased patients that satisfies the requirements of a Phase 1 Study and a Phase 2 Study.

1.73 "**Phase 2 Study**" means a clinical study of a drug candidate in patients with the primary objective of characterizing its activity in a specific disease state as well as generating more detailed safety, tolerability, and pharmacokinetics information as described in 21 C.F.R. 312.21(b), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States including a human clinical trial that is also designed to satisfy the requirements of 21 C.F.R. 312.21(a) or corresponding foreign regulations and is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. 312.21(b) (or corresponding foreign regulations) or otherwise to enable a Phase 3 Clinical Study (e.g., a phase 1/2 trial). The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.74 "**Phase 3 Study**" means a clinical study of a drug candidate in patients that incorporates accepted endpoints for confirmation of statistical significance of efficacy and safety in order to obtain Regulatory Approval in any country, as further described in 21 C.F.R. 312.21(c) with respect to the United States, or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States. The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.75 "**Pilot Study**" means, with respect to an Exploratory Indication, a research program for the generation and early pre-clinical testing of a development candidate ("**DC**") for such Exploratory Indication. Each Pilot Study shall be designed not to take more than [***] to complete.

1.76 "**Pivotal Study**" means Phase 3 Study or other clinical study of a drug candidate in human patients with the disease being studied, in each case, the principal purpose of which is to achieve a determination of efficacy and safety and is designed and intended to provide the basis for obtaining Regulatory Approval to market the applicable product for patients with the indication being studied or where a Clinical Study subsequently is deemed to achieve efficacy and safety for the applicable product and indication for the purpose of obtaining Regulatory Approval.

1.77 "**Potential Indications**" means [***], in each case using a Parvovirus Gene Therapy Product that delivers nucleic acid polymers [***].

1.78 "**Pre-Designation Product**" means a parvovirus gene therapy product for an Indication arising from a Research Program for which the time period within which Licensee must provide notice to Penn under Section 2.7 has not yet expired.

1.79 "**Program Valid Claim**" means a claim of (a) an issued and unexpired patent in Penn Patent Rights A or Penn Patent Rights B which claim has not been revoked or held unenforceable or invalid by a decision of a court of governmental agency of competent jurisdiction from which no further appeal can be taken or has been taken within the time allowed for appeal, and has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer; or (b) a pending patent application that is included in Penn Patent Rights A or Penn Patent Rights B which was filed and is being prosecuted, and has not been abandoned or finally disallowed without the possibility of appeal or re-filing of the application and has not been [***].

1.80 "**Proposed Task**" means a discovery research task proposed to be conducted in the Wilson Lab under the Discovery Program.

1.81 "**Regulatory Approval**" means, with respect to a product in any regulatory jurisdiction, approval from the applicable Regulatory Authority sufficient for the manufacture, distribution, use, marketing and sale of such pharmaceutical product in such jurisdiction in accordance with Laws (including, where applicable, any pricing or reimbursement approvals). "Regulatory Approval" does not include authorization by a Regulatory Authority to conduct named patient, compassionate use or other similar activities.

1.82 "**Regulatory Authority**" means any governmental authority, including the FDA, EMA or MHLW, or any successor agency thereto, that has responsibility for granting any licenses or approvals or granting pricing or reimbursement approvals necessary for the marketing and sale of a pharmaceutical product in any country.

1.83 "**Regulatory Exclusivity**" means with respect to any country or jurisdiction, any exclusive marketing rights or data exclusivity protection conferred by an applicable Regulatory Authority or other Regulatory Authority in such country or jurisdiction with respect to a compound or bio- pharmaceutical product, including any regulatory data protection exclusivity (including any orphan drug designation or pediatric exclusivity).

1.84 "**Research Plan**" means the research plan setting forth the Parties' roles and responsibilities for a Research Program, respectively, and as may be amended from time to time with written approval of the JSC or updated in accordance with Section 2.4 or Section 2.5, as applicable. The Research Plans for the Indications identified as of the New Effective Date are set forth in Exhibit B hereto.

1.85 "**Research Program**" means a research and pre-clinical development program of [***].

1.86 "**Research Results**" means all any and all ideas, information, inventions, developments, animate and inanimate materials, including live animals, discoveries, software, know-how, methods, techniques, formulae, data, processes, methodologies, techniques, biological materials, software and works of authorship, whether patentable or copyrightable, that are first conceived, discovered, developed, reduced to practice, or generated in the performance of a Research Program by the Wilson Lab, including any unpatentable inventions discovered, developed or conceived in the conduct of a Research Program. Research Results expressly excludes any such items covered by Penn Patent Rights and Joint Patent Rights.

1.87 "**Research Term**" means the period beginning on the Effective Date and ending on the completion of activities under the Research Plan for each Indication, or the termination of the last Research Program, whichever occurs first.

1.88 "**Sale**" means any transaction for which consideration is received or expected by Licensee, its Affiliates or Sublicensees for sale, use, lease, transfer or other disposition of a Licensed Product to or for the benefit of a Third Party. For clarity, sale, use, lease, transfer or other disposition of a Licensed Product by Licensee or any of its Affiliates or Sublicensees to another of these entities for resale by such entity to a Third Party shall not be deemed a Sale.

1.89 "**Service Center Cores**" means the following core laboratories at Penn that report directly to Dr. James Wilson, all science cores, including the Animal Models Core, the Vector Core, the Immunology Core, the Cell Morphology Core, the Biostatistics Core and the Integrated Technology Core.

1.90 "**Sublicensee**" means a Third Party to which a Sublicense is granted pursuant to the terms of Section 5.6.

1.91 "**Sublicense Documents**" means any and all agreements, amendments or written understandings entered into with a Sublicensee (including any of its Affiliates) pertaining to a Sublicense, Penn Patent Rights or Licensed Product. For clarity, a development agreement or distribution agreement for a Licensed Product is a Sublicense Document.

1.92 "**Sublicense Income**" means payments received by Licensee or its Affiliates from a Sublicensee in consideration for a Sublicense or other agreement providing the right to negotiate or obtain a Sublicense. Sublicense Income includes payments received from a Sublicensee in the form of license issue fees, milestone payments and the like, but specifically excludes [***].

1.93 "**Task**" means [***].

1.94 "**Tax**" means all taxes, duties, fees, premiums, assessments, imposts, levies, rates, withholdings, dues, government contributions and other charges of any kind whatsoever, whether direct or indirect, together with all interest, penalties, fines, additions to tax or other additional amounts, imposed by any Governmental Body.

1.95 "**Third Party**" means any Person other than Penn, Licensee or any of their respective Affiliates.

1.96 "**United States**" or "**US**" means the United States of America, its territories and possessions.

1.97 "**USD**" or "**$**" means the lawful currency of the United States of America.

1.98 "**Valid Claim**" means a claim of (a) an issued and unexpired patent in Penn Patent Rights which claim has not been revoked or held unenforceable or invalid by a decision of a court of governmental agency of competent jurisdiction from which no further appeal can be taken or has been taken within the time allowed for appeal, and has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer; or (b) a pending patent application that is included in Penn Patent Rights which was filed and is being prosecuted, and has not been abandoned or finally disallowed without the possibility of appeal or re-filing of the application and has not [***].

1.99 "**Wilson Lab**" means Dr. James M. Wilson and all individuals who are under the direct supervision or control of Dr. James M. Wilson or his successor as Director of the Wilson Gene Therapy Program at Penn, provided that the Service Center Cores are not included in the Wilson Lab, including any personnel of the Service Center Cores.

1.100 **Other Terms**. The definition of each of the following terms is set forth in the section of the Agreement indicated below:

Defined Term	Section
Abandoned Discovery Rights	8.1.5
Advance Payment	8.2.3
Agreement	Introductory Clause
Amicus Technology	5.3
Bankruptcy Action	12.3.4
Budget	2.3.1
Capsid Notice	2.6.2
Carve-Out Patent Rights	8.1.2
CDKL5 Deficiency Disorder	1.46
Commercial Milestone	6.2.2(a)
Commercial Milestone Payment	6.2.2(a)
CTA	1.45
DC	1.75
Development Milestone	6.2.1(a)
Development Milestone Payment	6.2.1(a)
Disclosing Party	9.1
Discovery Product License	1.22
Discovery Support Amount	3.1.1
Discovery Extension Support Amount	3.1.2
Effective Date	Introductory Clause
Election Notice	2.8.2
Exclusivity Period	2.8
Exploratory Indication Option	2.5.3
Exploratory Notice Period	2.5.2
Exploratory Option Period	2.5.1
Exploratory Program Plan/Budget	2.5.3
Extension Event	7.9
Fabry Disease	1.46

Failed Indication	2.10
Failed Indication Notice	2.10
Financial Report	6.7
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ARTICLE 2 COLLABORATION PROGRAMS

2.1 **Overall Project**. The Parties desire to collaborate with respect to the pre-clinical development of a parvovirus gene therapy product, as set forth in more detail in this Article 2, for each Indication within the Field of Use, with the goal of designating one Licensed Product for clinical development and commercialization for each Indication in the Field of Use. Penn will be

responsible for preclinical development activities, including all IND-enabling non-clinical studies and research grade manufacturing, and all activities allocated to Penn as set forth in a Research Plan. Licensee will be responsible for those activities allocated to Licensee in a Research Plan and for regulatory strategy and operations, clinical development, cGMP manufacture, and commercialization of all Licensed Product(s).

2.2 Research.

2.2.1 During the Research Term, subject to the terms and conditions of this Agreement, Licensee shall provide to Penn $[***] ("**Research Support Amount**") based on the Research Plans for research and development funding to fund the Research Programs for the following Indications identified as of the New Effective Date: (a) CDKL5 Deficiency Disorder (b) Pompe Disease, (c) Fabry Disease, (d) NPC, (e) MPS IIIA, and
(f) MPS IIIB. Such Research Support Amount shall be inclusive of Penn's standard indirect charges. Licensee shall remit such funds in each year of the Research Term in accordance with Section 2.3 below and such funds will be allocated and utilized solely to support each of the Research Programs as set forth in the Research Plan for such Research Program. The Parties acknowledge and agree that the Research Support Amount includes $[***] paid by Licensee to Penn prior to the New Effective Date in accordance with Section 2.3.1 below.

2.2.2 Penn will conduct each Research Program in accordance with the Research Plan for such Research Program and the other terms and conditions of this Agreement. Without limiting the foregoing, within each Indication, Penn will be responsible for the completion of the Research Plan for such Indication for the research and development work up to completion of IND enabling studies, including animal model development, and IND supporting preclinical work (toxicology and pharmacokinetics) and manufacturing to support preclinical development of a Licensed Product for such Indication under the Research Program for such Indication through DTP.

2.2.3 The JSC shall review each Research Plan at least once per Calendar Year. The JSC may amend a Research Plan at any time, including amendments to include further activities, including corresponding revisions to the budget.

2.2.4 Penn shall maintain records of the activities conducted under and the results of each Research Program (including the Research Results) in sufficient detail and in good scientific manner appropriate for patent purposes to properly reflect all work done and results achieved. Penn will provide task-based, scientific reports of the progress and results of each Research Program on the schedule specified in the Research Plan for such Research Program or on another schedule to be agreed in writing by the Parties. Penn shall maintain reasonable and accurate records of the use of the funds provided by Licensee under this Agreement and shall make such records available to Licensee (or its designee) upon reasonable notice during Penn's normal business hours, but not more frequently once each Calendar Year. All Research Results shall be solely and exclusively owned by Penn. For the avoidance of doubt, Research Results will constitute Licensed Know-How and will be included within the scope of the Licenses granted by Penn to Licensee under this Agreement.

2.2.5 Each Party will have the right to engage Third Party subcontractors to perform certain of its obligations under this Agreement. Any subcontractor to be engaged by a Party to perform a Party's obligations set forth in the Agreement will meet the qualifications

typically required by such Party for the performance of work similar in scope and complexity to the subcontracted activity and will enter into an appropriate agreement with such Party consistent with such Party's standard practices which agreement shall be as least as protective as the nondisclosure and nonuse of confidential information obligations set forth herein and requiring the assignment or license of Know-How and other intellectual property

generated in the course of the subcontracted work (including, with respect to any such Know-How and other intellectual property licensed to Penn, the right to grant and authorize Sublicenses under such Know-How and other intellectual property as contemplated herein in the same manner and of the same scope as required for intellectual property generated solely by the Wilson Lab). Any Party engaging a subcontractor hereunder will remain responsible and obligated for the acts and omissions of such subcontractor and will not grant rights to such subcontractor that would interfere with, limit or diminish the rights of the other Party under this Agreement.

2.3 Funding of the Research Program.

2.3.1 The initial budget for the Research Programs, broken down by Calendar Year, is set forth in Exhibit C (the "**Budget**", including as subsequently amended, pursuant to this Section 2.3.1). On or before November 1 of each year, the Parties, through the JSC, will agree on an updated budget for the remainder of each Research Program, also broken down by Calendar Year. Subject to the terms and conditions of this Agreement, Licensee shall pay Penn the applicable portion of the Research Support Amount in advance on a Calendar Quarter basis (in accordance with the payment schedule in the Budget) to cover the cost of the performance of each Research Plan by Penn (including reasonable and documented direct external expenses incurred by Licensee in accordance with such Research Plan and as agreed to by the Parties through the JSC).

2.3.2 If at any time Penn determines that it will require additional funds for a Research Program, it will promptly notify Licensee through the JSC and provide a good faith estimate and itemized budget of the additional amount. Notwithstanding the foregoing, changes to the scope of or budget for a Research Plan in a Calendar Year will require approval of the JSC if the budget impact for the applicable Calendar Year (in the aggregate) is greater than the higher of [***].

2.3.3 Title to any equipment, laboratory animals, or any other tangible materials made or acquired with funds provided under this Agreement will vest in Penn, and such equipment, animals, or tangible materials will remain the property of Penn following termination or expiration of this Agreement (but subject to any license grants to Licensee hereunder).

2.4 New Indication Option.

2.4.1 If Licensee has interest to include [***] Potential Indications in a Research Program, Licensee shall formally notify Penn in writing within [***] after the New Effective Date of such interest by specifying which of the indication(s) in the Potential Indications for which it is willing to fund preclinical development work at Penn.

2.4.2 If Licensee has interest to include an Exploratory Indication in a Research Program, Licensee shall formally notify Penn in writing prior to the expiration of the Discovery

Term of such interest by specifying which indication in the Exploratory Indications for which it is willing to fund preclinical development work at Penn.

2.4.3 With respect to Sections 2.4.1 and 2.4.2 above, Penn will then develop and propose within thirty (30) days of such written request a work plan and budget for the preclinical development activities and costs through completion of IND enabling studies to be conducted at Penn for a Licensed Product for each such indication subject to the reasonable review and approval by Licensee ("**New Program Plan/Budget**"). Within thirty (30) days of Licensee's receipt of a New Program Plan/Budget for a Potential Indication or Exploratory Indication, Licensee will decide whether to exercise its option to such indication (each, a "**New Indication Option**"). If Licensee exercises its New Indication Option with respect to such Potential Indication or Exploratory Indication by written notice to Penn, then a) the research program with respect to such indication will become a Research Program, b) the Research Support Amount will be increased by the amount of the agreed budget within the New Program Plan/Budget, c) Licensee will pay a New Indication Option Fee and d) the definition of "Indication" will include such indication.

2.4.4 For the avoidance of doubt, (i) if Licensee does not exercise its option for a Potential Indication that is the subject of a New Program Plan/Budget, then Licensee's New Indication Option for such Potential Indication will continue for the remainder of such [***] period remaining after the New Effective Date and (ii) if Licensee does not exercise its option for an Exploratory Indication that is the subject of a New Program Plan/Budget, then, subject to Section 3.3.1, Licensee's New Indication Option for such Exploratory Indication will continue for the remainder of the Exploratory Option Period.

2.4.5 [***].

2.5 Exploratory Indication Options; Pilot Studies.

2.5.1 During the period beginning on the New Effective Date and thereafter for the remainder of the Discovery Term (the "**Exploratory Option Period**"), Licensee has the exclusive option to initiate Pilot Studies for the Exploratory Indications. If Licensee has interest to initiate a Pilot Study for an Exploratory Indication, Licensee shall formally notify Penn of such interest in writing within the Exploratory Option Period by identifying the Exploratory Indications with respect to which it wishes to initiate a Pilot Study. Penn will then develop and propose, within thirty (30) days of such written request, a plan and budget for a Pilot Study for such Exploratory Indication subject to the reasonable review and approval by Licensee ("**Pilot Study Plan/Budget**"). Within thirty (30) days of Licensee's receipt of a Pilot Study Plan/Budget for an Exploratory Indication, Licensee will notify Penn whether it approves such Pilot Study Plan/Budget, and if so approved by Licensee, Licensee will pay Penn for the amount budgeted in such Pilot Study Plan/Budget within thirty (30) days thereafter. For the avoidance of doubt, if Licensee does not elect to initiate a Pilot Study that is the subject of a Pilot Study Plan/Budget,

then Licensee's exclusive option to initiate Pilot Studies for the Exploratory Indication that is the subject of such Pilot Study Plan/Budget will continue for the remainder of the Exploratory Option Period.

2.5.2 Upon the earlier of sixty (60) days after (a) the completion (as determined by the JSC) of a Pilot Study for an Exploratory Indication or (b) the date on which Penn notifies Licensee of DC identification for an Exploratory Indication ("**Exploratory Notice Period**"), Licensee must provide to Penn either (i) a preliminary notice of interest to include such Exploratory Indication in a Research Program or (ii) notice that Licensee declines to include such Exploratory Indication in a Research Program.

2.5.3 If Licensee provides to Penn a preliminary notice of interest to include an Exploratory Indication in a Research Program, and the Wilson Lab reasonably believes that the current research supports the selection of such Exploratory Indication, Penn will then develop and propose within thirty (30) days of such written request a work plan and budget for the preclinical development activities and costs through completion of IND enabling studies to be conducted at Penn for a Licensed Product for such indication subject to the reasonable review and approval by Licensee ("**Exploratory Program Plan/Budget**"). Within thirty (30) days of Licensee's receipt of an Exploratory Program Plan/Budget for an Exploratory Indication, Licensee will decide whether to (i) exercise its option to such indication (each, an "**Exploratory Indication Option**"), or (ii) decline to include such Exploratory Indication in a Research Program at such time. If Licensee exercises its Exploratory Indication Option with respect to such Exploratory Indication by written notice to Penn, then (a) the research program with respect to such indication will become a Research Program, (b) the Research Support Amount will be increased by the amount of the agreed budget within the Exploratory Program Plan/Budget, (c) Licensee will pay a New Indication Option Fee and (d) the definition of "Indication" will include such indication.

2.5.4 If Licensee (a) within the applicable Exploratory Notice Period, fails to notify Penn of its interest to include an Exploratory Indication in a Research Program, (b) prior to the expiration of the applicable thirty (30) day time period following Licensee's receipt of the relevant Exploratory Program Plan/Budget set forth in Section 2.5.3, fails to exercise an Exploratory Indication Option, or (c) at any time notifies Penn that it declines to include an Exploratory Indication in a Research Program at such time, then Licensee's exclusive option to initiate Pilot Studies for the Exploratory Indication that is the subject of such Pilot Study Plan/Budget and Licensee's Exploratory Indication Option for such Exploratory Indication will each continue for the remainder of the Exploratory Option Period.

2.6 Next Generation Option

2.6.1 **Generally**. It is anticipated that Licensed Product development under the Research Program for an Indication may either [***].

2.6.2 **Next Generation Capsid Data Packages**. During the Research Term until a Designated Product for each Indication has been determined, the Wilson Lab will notify Licensee [***] of any available Next Generation Capsids for a Licensed

Product for each Indication in the Field of Use. For each such Next Generation Capsid, the Wilson Lab will at the time of such notification provide Licensee a Next Generation Capsid Data Package and thereafter upon Licensee's written request, provide any additional information regarding such Next Generation Capsid not previously provided. If Licensee expresses interest in any Next Generation Capsid for a Licensed Product for a specific Indication and provides Penn with written notice thereof after Licensee's receipt of the relevant Next Generation Capsid Data Package during the Research Term (a "**Capsid Notice**"), Penn will reserve such Next Generation Capsid for the Licensed Product for such Indication until such time as Licensee determines the Designated Product for such Indication ("**Reserved Capsid**"). License may only reserve one Next Generation Capsid per Indication but may substitute another Next Generation Capsid for an Indication ("**Substitute Capsid**") by providing Penn written notice of such substitution during the Research Term and prior to Designated Product selection for such Indication. Upon Penn's timely receipt of such notice, the Substitute Capsid will become the Reserved Capsid for such Indication.

Following Penn's receipt of a Capsid Notice, Penn will provide Licensee with a list of Penn Patent Rights B, Penn Patent Rights C and Manufacturing Patent Rights related to such Reserved Capsid. Licensee shall be responsible for paying a pro rata share (based on the number of licensees for such Penn Patent Rights) of documented Historic Patent Costs and Ongoing Patent Costs (as such terms are defined in Section 8.2) for Penn Patent Rights B, Penn Patent Rights C and Manufacturing Patent Rights covering a Reserved Capsid for each Indication following Penn's receipt of a Capsid Notice.

2.6.3 **Updates Regarding Next Generation Capsid**. With regard to a Reserved Capsid or a Next Generation Capsid for a Designated Product, Penn will keep Licensee apprised, on a confidential basis, of material regulatory communications or other interactions to or from the FDA or EMA, which Penn is aware, with respect to such Next Generation Capsid, including any material safety issues with respect to any of the foregoing, in each case, to the extent such disclosure is not prohibited by a Third Party arrangement to which Penn is a party.

2.7 **Designated Product Selection.** For each Indication within [***] of the completion of the DTP of a Parvovirus Gene Therapy Product for such Indication and the receipt by Licensee of the Research Results with respect to such Indication, Licensee will provide written notice to Penn of its selection of the parvovirus capsid and the transgene sequence for such Pre-Designation Product for further development by Licensee, at which time such Pre-Designation Product (including such parvovirus capsid and transgene sequence) will become a Designated Product. With respect to the parvovirus capsid to be used in the Designated Product for each Indication, Licensee may designate that it will use [***].

2.8 **[***]**.

2.8.1 [***].

2.8.2 [***].

2.8.3 [***].

2.8.4 [***].

2.9 **Lock-Up**. On an Indication-by-Indication basis, [***].

2.10 **Expanded Collaboration.** [***].

2.11 **Program Failure**. Should any Licensed Product development program for any Indication fail at a key decision point during the Research Program for such Indication, as such failure is defined in the Research Plan for such Research Program, and a decision is subsequently made by the Licensee to discontinue further development under the Research Program with respect to such Indication ("**Failed Indication**"), any remaining Research Support Amount allocated for the Failed Indication program pursuant to the then-current agreed budget for such Research Program (minus wind-down and non-cancellable expenses with respect to the activities under the research plan for such Failed Indication) will be reallocated to activities to be conducted under one or more Research Programs for one or more remaining Indications. Such Failed Indication will be removed promptly from the Indication definition of the Agreement, with written confirmation of such termination of rights promptly provided by Licensee to Penn ("**Failed Indication Notice**"). In addition, Penn's obligations pursuant to the Limited Exclusivity Covenant in Section 2.8 shall terminate as of the date of such Failed Indication Notice; and any licenses and rights granted by Licensee to Penn under the Amicus Technology with respect to such Failed Indication shall also automatically terminate, effective as of the date of the applicable Failed Indication Notice.

2.12 **Data Ownership**. All data generated by Penn under a Research Program or the Discovery Program shall be owned by Penn including all rights, title and interest ("**Penn Data**"). All data generated by Licensee under a Research Program shall be owned by Licensee ("**Licensee Data**"). For the avoidance of doubt, Penn Data will constitute Research Results and Licensed Know-How and will be included within the scope of the License.

3.1 Discovery Program.

ARTICLE 3 DISCOVERY PROGRAM

3.1.1 During the period beginning on the New Effective date and thereafter for the remainder of the Discovery Program Period, subject to the terms and conditions of this Agreement, Licensee shall pay [***] in research and development funding to Penn to fund the Discovery Program ("**Discovery Support Amount**"). Such Discovery Support Amount shall be inclusive of Penn's standard indirect charges. Licensee shall remit such funds in each Discovery Program Year of the Discovery Program Period in accordance with Section 3.2.1 below and such funds will be allocated and utilized solely to support the Discovery Program as set forth in the Discovery Plan. [***].

3.1.2 The Parties may through mutual agreement extend the term of the Discovery Program for [***].

3.1.3 Penn will conduct the Discovery Program in accordance with the Discovery Plan and the other terms and conditions of this Agreement. Subject to the terms and conditions of this Agreement, [***].

3.1.4 The JSC shall review the Discovery Plan and the Tasks at least once per Calendar Year during the period beginning on the New Effective Date and thereafter for the remainder of the Discovery Term. Penn or Licensee may recommend to the JSC that a Proposed Task be added to the Discovery Program (and upon such addition become a Task) or a Task be removed from the Discovery Program. If approved by consensus by the JSC, the Proposed Task will be added to the Discovery Program (and upon such addition become a Task) or Task will be removed from the Discovery Program. As a condition of adding a Task to the Discovery Program, Penn may require that [***].

3.1.5 If a Proposed Task is recommended by Penn to be included in the Discovery Program, and not approved by Licensee (a "**Rejected Task**"), (a) Penn may conduct such Rejected Task, (b) Penn may not apply any of the Discovery Support Amount or Discovery Extension Support Amount to the conduct of such Rejected Task and (c) any Patent Rights that result, in whole or in part, from the conduct of such Rejected Task will not be included in the Discovery Patent Rights.

3.1.6 If a Proposed Task is recommended by Licensee to be included in the Discovery Program and Licensee agrees to provide adequate additional funding in order to conduct such Proposed Task, if necessary, and the Proposed Task is not approved by Penn (a) the

Wilson Lab may not conduct such Rejected Task during the Discovery Term, (b) Penn may not apply any of the Discovery Support Amount or Discovery Extension Support Amount to the conduct of such Rejected Task and (c) any Patent Rights that result, in whole or in part, from the conduct of such Rejected Task (if conducted in violation of clause (a) of this Section 3.1.6) will be included in the Discovery Patent Rights.

3.1.7 Penn shall maintain records of the activities conducted under and the results of the Discovery Program (including the Discovery Results) in sufficient detail and in good scientific manner appropriate for patent purposes to properly reflect all work done and results achieved. At least once every [***] during the period beginning on the New Effective Date and thereafter for the remainder of the Discovery Term, Penn will notify Licensee and the JSC of any available Discovery Patent Rights and/or Discovery Results and will provide to Licensee and the JSC task-based, scientific reports of the progress and results of the Discovery Program. All Discovery Results, shall be solely and exclusively owned by Penn ("**Penn Discovery Results**"). For the avoidance of doubt, Penn Discovery Results will constitute Licensed Discovery Know-How and will be included within the scope of the Licenses granted by Penn to Licensee under this Agreement.

3.2 Funding of the Discovery Program.

3.2.1 Subject to the terms and conditions of this Agreement, Licensee shall pay Penn the Discovery Support Amount in equal quarterly installments as follows: with respect to the first Discovery Program Quarter, Licensee shall pay Penn such installment within [***] after the New Effective Date and with respect to the second and each subsequent Discovery Program Quarter during the Discovery Program Period, Licensee shall pay Penn such installment in advance, at least sixty (60) days prior to the beginning of the applicable Discovery Program Quarter.

3.2.2 Subject to the terms and conditions of this Agreement, if Licensee elects to extend the Discovery Program as set forth in Section 3.1.2, Licensee shall pay Penn the Discovery Extension Support Amount in equal quarterly installments in advance, at least sixty (60) days prior to the beginning of each Discovery Program Quarter during each Discovery Program Extension.

3.2.3 Upon the expiration of the Discovery Term, in the event that any Discovery Support Amount or Discovery Extension Support amount remains unexpended, subject to the permitted wind-down and payment of all non-cancellable costs by Penn for the Discovery Program, Penn shall promptly return any remaining amount to Licensee within sixty (60) days after the expiration of the Discovery Term.

3.2.4 Subject to the terms and conditions of this Agreement, Penn may utilize Penn funding or collaborate with and receive funding from any Third Party to support the Discovery Program so long as Penn does not, in connection with any such collaboration or the receipt or use of any such funding or otherwise, grant to any Third Party any right or license that would conflict with the rights and licenses granted to Licensee under this Agreement. Schedule 3.2.4 sets forth a complete and accurate list and description, as of the New Effective Date, of all Third Parties with which Penn collaborates, or from which Penn has received or has entered into an agreement to receive funding, in connection with the Discovery Program. Following the New Effective Date, Penn will promptly disclose

to Licensee any additional Third Party from which Penn has received or is entitled to receive funding, for the Discovery Program.

3.3 Limited Discovery Exclusivity during the Discovery Term.

3.3.1 [***].

3.3.2 [***].

ARTICLE 4 GOVERNANCE.

4.1 **Joint Steering Committee**.

4.1.1 Formation; Composition. Within sixty (60) days of the Effective Date, the Parties will establish a joint steering committee (the "**Joint Steering Committee**" or "**JSC**") comprised of four (4) representatives from each Party with sufficient seniority within the applicable Party to make decisions arising within the scope of the JSC's responsibilities. The JSC may change its size from time to time by mutual consent of its members, provided that the JSC will consist at all times of an equal number of representatives of each of Penn and Licensee. Each Party may replace its JSC representatives at any time upon written notice to the other Party.

4.1.2 Specific Responsibilities. The JSC will:

(a) oversee the Research Program and the Discovery Program;

(b) review and discuss the Discovery Program;

(c) on or before November 1 of each year, approve an updated budget for each Research Program in accordance with Section 2.3.1;

(d) approve any amendments to a Research Plan (including any changes to the budget that are greater than the higher of [***];

(e) review and discuss Proposed Tasks and the Discovery Plan and approve any amendments to the Discovery Program to include such Proposed Tasks as Tasks, as described in Section 3.1.4;

(f) determine whether Penn or Licensee will contract directly with subcontractors;

(g) establish appropriate reporting procedures for each Research Program and the Discovery Program, including the scope and content of reports to be provided by each Party to the other Party (specifically for Penn under Section 2.2.4 and Section 3.1.7) and in order to provide Licensee with sufficient information regarding the Research Results for each Indication to allow Licensee to select a Designated Product pursuant to Section 2.6;

(h) review, coordinate and discuss the Penn MPS Activities, including serving as a forum for the exchange of information with respect thereto;

(i) endeavor to resolve any disagreement between the Parties relating to a Research Program, a Research Plan, the Discovery Program or the Discovery Plan;

(j) establish such additional subcommittees as it deems necessary to achieve the objectives and intent of each Research Program and the Discovery Program; and

(k) conduct such other activities as specifically assigned to the JSC under this Agreement.

4.1.3 Reporting. Each Party shall keep the JSC informed on the status and progress of the activities under each Research Program then currently ongoing under a Research Plan and Penn shall keep the JSC informed on the status and progress of the activities under the Discovery Program, including delivering quarterly written updates of its progress under each Research Program and/or the Discovery Program, as applicable, to the JSC at least one (1) week in advance of each JSC meeting.

4.1.4 Meetings. During the performance of a Research Program and/or the Discovery Program by Penn, the JSC will meet at least quarterly. Following the completion of Penn's performance of all of the Research Programs and the Discovery Program, the Parties may agree to meet to discuss items previously addressed by the JSC. The JSC may meet in person, by videoconference or by teleconference. Notwithstanding the foregoing, at least two (2) meetings per Calendar Year will be in person unless the parties mutually agree in writing to waive such requirement. In-person JSC meetings will be held at locations alternately selected by Penn and by Licensee; provided, however, that Licensee shall reimburse Penn for its JSC representatives' reasonable out-of-pocket travel costs in connection with attending such in-person JSC meeting at a location other than in Philadelphia. Meetings of the JSC will be effective only if at least one representative from each Party is present or participating in such meeting. The JSC shall keep accurate minutes of its deliberations which shall record all proposed decisions and all actions recommended or taken. The secretary of the JSC (as appointed by the members of the JSC) shall be responsible for the preparation of draft minutes. Draft minutes shall be sent to all members of the JSC within ten (10) working days after each meeting and shall be

approved, if appropriate, at the next meeting. All records of the JSC shall at all times be available to both Penn and Licensee.

4.1.5 Decision-Making. The representatives from each Party on the JSC will have, collectively, one (1) vote on behalf of that Party, and all decision making will be by unanimous consent of both Parties. If the JSC is unable to reach agreement on any issue or matter within the scope of the JSC's decision-making authority, such disputed matter will be escalated to Licensee's Chief Executive Officer and Penn's Dean of Medicine or his designee, for discussion in good faith. Except with respect to the addition or removal of Tasks to or from the Discovery Program, if the JSC (after escalation pursuant to this Section 4.1.5) is unable to reach agreement on any issue pertaining to the Discovery Program, Penn shall have the final decision making authority with respect thereto.

4.2 Joint Intellectual Property Committee.

4.2.1 Formation; Composition. Within thirty (30) days of the Effective Date, the Parties will establish a joint intellectual property oversight committee (the "**Joint Intellectual Property Committee**" or "**JIPC**") comprised of an equal number of representatives from each Party. The JIPC may change its size from time to time by mutual consent of its members, provided that the JIPC will consist at all times of an equal number of representatives of each of Penn and Licensee, with at least one representative of Penn from the Penn Center for Innovation. Each Party may replace its JIPC representatives at any time upon written notice to the other Party.

4.2.2 Specific Responsibilities. The JIPC will (a) discuss and make recommendations with respect to the intellectual property activities of the Parties related to Licensed Product research and development under each Research Plan in a manner that is consistent with the other terms of this Agreement, specifically with respect to the prosecution, maintenance, defense and enforcement of the Penn Patent Rights A and Joint Patent Rights, and prosecution and maintenance of Penn Patent Rights B (excluding Patent Rights listed on Exhibit G) and (b) conduct such other activities as specifically assigned to the JIPC under this Agreement.

4.2.3 Meetings. The JIPC will meet at twice annually, unless the Parties mutually agree in writing to a different frequency. The JIPC may meet in person, by videoconference, or by teleconference. In-person JIPC meetings will be held at locations alternately selected by Penn and by Licensee; provided, however, that Licensee shall reimburse Penn for its JIPC representatives' costs in connection with attending such in-person JSC meeting at a location other than Penn. Meetings of the JIPC will be effective only if at least one representative of each Party is present or participating in such meeting.

4.2.4 Decision-Making. The representatives from each Party on the JIPC will have, collectively, one (1) vote on behalf of that party, and all decision making will be by unanimous consent by the Parties. Disputes at the JIPC will be referred to the JSC for resolution.

4.3 Scope of Authority.

Each Party shall retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers or discretion shall be delegated or vested in the JSC (or the JIPC) unless expressly provided in this Agreement or otherwise agreed by the Parties in writing. The JSC

(and JIPC), including pursuant to any Party's exercise of its final decision making authority, shall not have the power to amend, modify or waive this Agreement or compliance with the terms of this Agreement. No decision of the JSC (or JIPC) shall conflict with the terms of this Agreement nor be in contravention of applicable law in any material respect.

ARTICLE 5 LICENSES AND OTHER RIGHTS

5.1 **Grant of License**. Subject to the terms and conditions of this Agreement, Penn hereby grants to Licensee (the below rights under (a) through (d), the "**License**").

(a)(i) an exclusive, worldwide, royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under Penn Patent Rights A and (ii) a non-exclusive, world-wide royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under Licensed Know-How, in each case ((i) and (ii)), to make, have made, use, sell, offer for sale, and import Licensed Products for the Indications in the Field of Use during the Term;

(b) (i) except as set forth below with respect to Patent Rights within the DRG Technology, an exclusive, worldwide, royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under Penn Patent Rights B and (ii) a non-exclusive, world-wide royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6), under (x) Licensed Discovery Know-How and (y) Patent Rights under the DRG Technology included in Penn Patent Rights B, in each case ((i) and (ii)), to make, have made, use, sell, offer for sale, and import Designated Products for the Indications in the Field of Use during the Term;

(c) a non-exclusive, world-wide, royalty bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6) under Penn Patent Rights C and Manufacturing Patent Rights, in each case, to make, have made, use, sell, offer for sale, and import Designated Products for the Indication (on an elected Indication-by-Indication basis) in the Field of Use during the Term; and

(d) an exclusive, world-wide, royalty-bearing right and license, with the right to sublicense (subject to the provisions of Section 5.6) under Penn's interest in Joint Patent Rights to make, have made, use, sell, offer for sale, and import products for any purposes within the Indications in the Field of Use during the Term.

5.2 **Joint Patent Rights**. Each Party hereby grants to the other Party a non-exclusive, world-wide, royalty-free, fully paid up, perpetual, irrevocable right and license, with the right to freely sublicense under the Joint Patent Rights, to make, have made, use, sell, offer for sale and import products and services other than products and services for the Indications in the Field of Use.

5.3 **Research License to Penn**. Licensee will grant to Penn a non-exclusive, non-transferable, non- sublicensable license (a) under [***] the "**Amicus Technology**") solely for purposes of performing Penn's obligations under a Research Program in accordance with the Research Plan for such Research Program and the terms of this Agreement and (b) under (i) the Licensee Data and (ii) new inventions reduced to practice by

Licensee in the performance of a Research Program, in the case of each of (i) and (ii), to the extent Controlled by Licensee and solely during the Exclusivity Period and solely for purposes of performing internal, non-commercial research in the Wilson Lab. Notwithstanding the foregoing, Amicus Technology expressly excludes any Joint Patent Rights. For clarity, internal, non- commercial research includes performance of activities funded by a government entity or non- commercial Third Party (so long as such non-commercial Third Party does not obtain any commercial right in or to any data, results, inventions or other intellectual property arising in connection with the relevant funded activities). Amicus Technology will remain the exclusive property of Licensee. Penn shall use the Amicus Technology solely in the conduct of the Research Program for which such Amicus Technology is provided in accordance with the Research Plan for such Research Program and the terms of this Agreement and, for clarity, shall not use any Amicus Technology in the conduct of the Discovery Program. Penn shall not reverse engineer, decompile or disassemble any Amicus Technology, nor attempt or assist any Third Party to do the foregoing. Amicus Technology is provided "as is" with no warranty, express, implied or statutory, including without limitation warranties of merchantability, title, non- infringement, exclusivity or fitness for a particular purpose.

5.4 **Retained Rights**. Notwithstanding the License, Penn retains the right under the Penn Patent Rights to (a) conduct educational, research and clinical / patient care activities itself (including sponsored research) and (b) authorize non-commercial third parties to conduct education, non- commercial research and clinical / patient activities, in each case of subpart (a) and (b) related to the Indications in the Field of Use; provided however that Penn shall not have the right to use or authorize the use of any Licensed Product for an Indication that is under active clinical testing or being sold by Licensee in the conduct of clinical or patient care activities without Licensee's consent.

5.5 **U.S. Government Rights**. The License is expressly subject to all applicable provisions of any license to the United States Government executed by Penn and is subject to any overriding obligations to the United States Federal Government under 35 U.S.C. §§200-212, applicable governmental implementing regulations, and the U.S. Government sponsored research agreement or other guidelines, including that products that result from intellectual property funded by the United States Federal Government that are sold in the United States be substantially manufactured in the United States. If so requested by Licensee, Penn shall reasonably cooperate with and assist Licensee to seek and obtain a waiver from the appropriate Regulatory Authorities with respect to such manufacturing requirement.

5.6 Grant of Sublicense by Licensee.

5.6.1 Penn grants to Licensee the right to grant and authorize sublicenses in whole or in part, under the License (each, a "**Sublicense**") subject to the terms and conditions of this Agreement and specifically this Section 5.6. The term Sublicense shall include any grant of rights under the License by a Sublicensee to any downstream Third Party to develop, manufacture, use or sell a Licensed Product, such downstream Third Party shall also be considered a Sublicensee for the purposes of this Agreement.

5.6.2 Licensee will have the right to extend any and all of its rights under this Agreement to its Affiliate (subject to such Affiliate agreeing in writing with Licensee to be bound by the terms and conditions of this Agreement to the extent applicable to such Affiliate) without the consent of Penn; provided that Licensee will be responsible for the conduct of any such Affiliate under the Agreement to the same extent as if such activities had been undertaken by Licensee itself.

5.6.3 Licensee will have the right to grant and authorize Sublicenses to Third Parties (and their Affiliates), without the consent of Penn. For clarity, except for Sublicenses granted to Service Provider Sublicensees pursuant to Section 5.6.4 below, this provision permits only a single-tier of sublicensing to a Third Party (and its Affiliates) for Sale of a Licensed Product.

5.6.4 Licensee, Sublicensee and each of their respective Affiliates may also, without Penn's consent, engage Third Party service providers (and grant Sublicenses within the scope of the License to such Persons) solely to perform activities for the benefit of or on behalf of Licensee or such Sublicensee or Affiliate, as the case may be (each a "**Service Provider Sublicensee**"). Licensee shall remain responsible to Penn for all activities of such Service Provider Sublicensee to the same extent as if such activities had been undertaken by Licensee itself.

5.6.5 Each Sublicense Document will (a) be issued in writing, (b) to the extent applicable, include all of the rights of Penn and require the performance of obligations due to Penn (and, if applicable, the U.S. Government under 35 U.S.C. §§200-212) contained in this Agreement and (c) to the extent applicable, include the following terms and conditions:

(a) Reasonable record keeping, audit and reporting obligations sufficient to enable Licensee and Penn to reasonably verify the payments due to Penn as a result of such Sublicense and to reasonably monitor such Sublicensee's progress in developing and/or commercializing Licensed Product.

(b) Infringement and enforcement provisions that do not conflict with the restrictions and procedural requirements imposed on Licensee and do not provide greater rights to Sublicensee than as provided in Section 8.3.

(c) Confidentiality provisions with respect to Confidential Information of Penn provided to a Sublicensee consistent with the obligations on Licensee in Article 10 of this Agreement.

(d) Covenants by Sublicensee that are equivalent to those made by Licensee in Section 10.3.

(e) A requirement of indemnification of Penn by Sublicensee that is equivalent to the indemnification of Penn by Licensee under Section 11.1 of this Agreement.

(f) A requirement of obtaining and maintaining insurance by Sublicensee that is equivalent to the insurance requirement of Licensee under Section 11.2 of this Agreement, including coverage under such insurance of Penn as provided in Section 11.2.

(g) Restriction on use of Penn's names etc. consistent with Section 13.4 of this Agreement.

(h) A requirement of antidiscrimination by Sublicensee no less stringent than that provided in Section 13.5 of this Agreement.

(i) A requirement that Penn is a third party beneficiary of such Sublicense solely with respect to the rights of Penn and the performance obligations owed to Penn as required hereunder.

Notwithstanding the foregoing, with respect to Service Provider Sublicensees, the items set forth in subsections (a), (b), (d), (e), (f), (g), (h) and (i) may need not be included in the relevant Sublicense Document to the extent such is not applicable.

5.6.6 Within thirty (30) days after of the execution of a Sublicense Document, Licensee shall provide a complete and accurate copy of such Sublicense Document (which may be redacted with respect to matters unnecessary to show compliance herewith, provided that in no event will any financial information be redacted) to Penn, in the English Language. Penn's receipt of a Sublicense Document, however, will constitute neither an approval nor disapproval of the Sublicense Document nor a waiver of any right of Penn or obligation of Licensee under this Agreement. Notwithstanding the foregoing, upon Penn's request, Licensee will provide an unredacted copy of any Sublicense Document to Penn's outside counsel to confirm compliance herewith, and such outside counsel shall not provide such Sublicense Document to Penn.

5.7 **No Implied License.** Each Party acknowledges that the rights and licenses granted in this Agreement are limited to the scope expressly granted. Accordingly, except for the rights expressly granted under this Agreement, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by either Party to the other Party. All rights with respect to any know-how, patent or other intellectual property right rights that are not specifically granted herein are reserved to the owner thereof.

ARTICLE 6 FINANCIAL PROVISIONS

6.1 Payments

6.1.1 **Issue Fee.** In partial consideration of the rights and licenses granted to Licensee under this Agreement, within [***] following the Effective Date, Licensee paid Penn a non-refundable and non-creditable license issue fee of Seven Million US Dollars ($7,000,000). Such payment was made by wire transfer of immediately available funds into the account specified in Section 6.6. For the avoidance of doubt, no amount shall be payable pursuant to this Section 6.1.1 on or after the New Effective Date.

6.1.2 **Alliance Management Fee**. During the Research Term, Licensee shall pay the Penn Center for Innovation an annual alliance management fee of [***] per year within three [***] following the Effective Date and each one-year anniversary thereof. For clarity, the amount of the alliance management fee will be paid only once per year (not once per Indication) and will not exceed [***] in any year and would not be payable in any year in which a License Maintenance Fee is also payable. The Parties acknowledge and agree that the first alliance management fee has been paid by Licensee in accordance with this Section 6.1.2.

6.1.3 **License Maintenance Fee**. Following expiration of the Research Term and until the expiration of the first Royalty Period in the first Major Market for a Licensed Product, Licensee shall pay Penn a non-refundable and annual maintenance fee of [***] for the first and second year after the anniversary of the Research Term expiration date and [***] for the third year after the anniversary of the Research Term expiration date and on each anniversary thereafter ("**License Maintenance Fee**"). The License Maintenance Fee payment obligation shall only be creditable against royalties owed to Penn in the year such License Maintenance Fee was paid (there shall be no carry forward credit on License Maintenance Fees paid). For clarity, the amount of the License Maintenance Fee will be paid only once per year (not once per Indication).

6.1.4 **New Indication Option Payment.** Within [***] following Licensee's exercise of a New Indication Option, Licensee will pay to Penn a non- refundable, non-creditable payment in the amount of [***] by wire transfer of immediately available funds ("**New Indication Option Fee**") pursuant to Section 6.6 below. For the avoidance of doubt, Licensee will be deemed to have exercised a New Indication Option for each of NPC, MPS IIIA and MPS IIIB as of the New Effective Date and will pay to Penn a New Indication Option Fee for each such Indication within [***] following the New Effective Date.

6.2 Milestone Payments.

6.2.1 **Development Milestones**.

(a) As additional consideration for the License, Licensee will pay Penn the following milestone payments (each, a "**Development Milestone Payment**") upon the achievement of the first Licensed Product to achieve the corresponding milestone for each Indication (each, a "**Development Milestone**"), whether achieved by Licensee or an Affiliate or Sublicensee. Licensee shall promptly notify Penn in writing of the achievement of any such Development Milestone and Licensee shall pay Penn in full the corresponding Development Milestone Payment within [***] of such achievement. For clarity, each Development Milestone Payment is non-refundable, non-creditable and is not an advance against Royalties due to Penn or any other amounts due to Penn.

Development Milestone (payable once per Indication)	Milestone Payment (in U.S. dollars)
[***]	$[***]
[***]	$[***]
[***]	$[***]
[***]	$[***]
[***]	$[***]
[***]	$[***]
Total Development Milestones per Indication	**$[***]**

(b) Each time a Development Milestone [***] in the table above is achieved for a Licensed Product for an Indication, then any other Development Milestone Payments with respect to earlier Development Milestones (i.e., Development Milestones [***] in the table above, as applicable) for that Indication that have not yet been paid will be due and payable together with the Development Milestone Payment for the relevant Development Milestone that is actually achieved. If Development Milestone [***] is achieved prior to the achievement any of Development Milestones [***], then Milestone Payments for Development Milestones [***] shall be due to the extent not previously paid.

6.2.2 Commercial Milestone Payments.

(a) As additional consideration for the License, Licensee will pay Penn the following commercial milestone payments (each, a "**Commercial Milestone Payment**") upon the achievement of the corresponding milestone (each, a "**Commercial Milestone**"), whether achieved by Licensee or an Affiliate or Sublicensee, or a combination of Licensee, Affiliate or Sublicensee, when cumulative worldwide Net Sales of Licensed Product(s) for an Indication reach the respective thresholds indicated below. Licensee shall notify Penn in writing of the achievement of any such Commercial Milestone within [***] following [***] in which such Commercial Milestone is achieved and Licensee shall pay Penn in full the corresponding Commercial Milestone Payment together with such notice. For clarity, each Commercial Milestone Payment is non-refundable, non-creditable and is not an advance against Royalties due to Penn or any other amounts due to Penn.

Commercial Milestone (payable once per Indication)	Milestone Payment
Cumulative Net Sales of Licensed Product for an Indication reaches $[***]	$[***]
Cumulative Net Sales of Licensed Product for an Indication reaches $[***]	$[***]
Cumulative Net Sales for Licensed Product for an Indication reaches $[***]	$[***]
Total Commercial Milestone Payments to Penn for each Indication	**$[***]**

6.3 Royalties.

6.3.1 **Royalty**. As further consideration for the License, on a Licensed Product-by-Licensed Product basis during the applicable Royalty Period Licensee shall pay to Penn a non- refundable, non-creditable royalty on worldwide Net Sales of Licensed Product ("**Royalty**") as set forth below:

Annual Worldwide Net Sales of a Licensed Product	Royalty Rate
Less than $[***]	[***]
Greater than or equal to $[***] and less than or equal to $[***]	[***]
Greater than $[***]	[***]

For such purposes, "Annual Worldwide Net Sales" means the total Net Sales of the applicable Licensed Product in all countries in a particular Calendar Year.

6.3.2 **Royalty Term**. Licensee's obligation to pay Penn the Royalty will continue on a country-by-country and Licensed Product-by-Licensed Product basis from the date of First Commercial Sale of such Licensed Product in a country until the latest of (a) the expiration or abandonment of the last Valid Claim within the Penn Patent Rights covering such Licensed Product in such country, (b) [***] after First Commercial Sale of such Licensed Product in such country, (c) the expiration of the Regulatory Exclusivity with respect to such Licensed Product; [***] (such royalty period, the "**Royalty Period**").

6.3.3 Royalty Reductions.

(a) [***].

(b) [***].

(i) [***].

(ii) [***].

(iii) [***].

(iv) [***].

6.3.4 **Calculations**. Licensee must pay Royalties owed to Penn on a Calendar Quarter basis on or before the following dates:

(a) [***] for any Sales that took place on or before the last day of the Calendar Quarter ending December 31, of the prior Calendar Year;

(b) [***] for any Sales that took place on or before the last day of the Calendar Quarter ending March 31 of such Calendar Year;

(c) [***] for any Sales that took place on or before the last day of the Calendar Quarter ending June 30 of such Calendar Year; and

(d) [***] for any Sales that took place on or before the last day of the Calendar Quarter ending September 30 of such Calendar Year.

6.4 Penn Sublicense Income.

6.4.1 On a Licensed Product-by-Licensed Product basis, Licensee will pay to Penn the following percentage of Sublicense Income ("**Penn Sublicense Income**") received by Licensee from a Sublicensee:

Stage in Licensed Product development for the applicable Indication at which Sublicense is granted by Licensee	Percent of Sublicense Income payable to Penn
Prior to [***] for the first Licensed Product for such Indication	[***]
After [***] for the first Licensed Product for such Indication and prior to [***] for the first Licensed Product for such Indication	[***]
After [***] for the first Licensed Product for an Indication	[***]

[***].

6.4.2 Licensee will make such payment to Penn on or before the following dates:

(a) [***] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending December 31, of the prior Calendar Year;

(b) [***] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending March 31 of such Calendar Year;

(c) [***] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending June 30 of such Calendar Year; and

(d) [***] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending September 30 of such Calendar Year.

6.5 Discovery Product Proceeds.

6.5.1 During the Term of this Agreement and thereafter, [***].

6.5.2 [***] on or before the following dates:

(a) [***] for any Discovery Product Proceeds received by Penn on or before the last day of the Calendar Quarter ending December 31, of the prior Calendar Year;

(b) [***] for any Discovery Product Proceeds received by Penn on or before the last day of the Calendar Quarter ending March 31 of such Calendar Year;

(c) [***] for any Discovery Product Proceeds received by Penn on or before the last day of the Calendar Quarter ending June 30 of such Calendar Year; and

(d) [***] for any Discovery Product Proceeds received by Penn on or before the last day of the Calendar Quarter ending September 30 of such Calendar Year.

6.5.3 Within [***] after [***] in which Penn receives Discovery Product Proceeds, Penn shall deliver to Licensee a report ("**Penn Financial Report**") setting out sufficient details necessary to calculate the Discovery Product Proceeds received by Penn under this Article 6 in such Calendar Quarter, [***].

6.6 Mode of Payment and Currency.

6.6.1 All payments to Penn hereunder shall be made by deposit of USD in the requisite amount to the "The Trustees of the University of Pennsylvania" and will be made by delivery to any one of the following:

For funding of the performance of a Research Program or the Discovery Program by Penn:

By ACH/Wire:
[***]
[***] (domestic wires)
[***]
(international wires only)
Account Number:
[***]

For all other payments to Penn under this Agreement:

By ACH/Wire:	**By Check (direct mail):**	**By Check (lockbox):**
[***]	The Trustees of the	The Trustees of the
(domestic wires)	University of Pennsylvania	University of Pennsylvania

[***]	c/o Penn Center for Innovation	c/o Penn Center for Innovation
(international wires only)	Attention: Financial	PO Box 785546
Account Number: [***]	Coordinator	Philadelphia, PA 19178-5546
	3600 Civic Center Blvd. 9th Floor Philadelphia, PA 19104	

6.6.2 All payments to Licensee hereunder shall be made by deposit of USD in the requisite amount to such bank account as Licensee may from time to time designate by written notice to Penn.

6.6.3 All amounts stated in and payments due under this Agreement shall be in USD. All Royalties, Sublicense Income and/or [***] payable shall be calculated first in the currency of the jurisdiction in which payment was made, and if not in the United States, then converted into USD. The exchange rate for such conversion shall be the average of the rate quoted in The Wall Street Journal for the last business day of each month in the Calendar Quarter for such Royalty, Sublicense Income and/or [***] payment made.

6.7 **Royalty and Penn Sublicense Income Reports**. Within [***] after the end of each Calendar Quarter, Licensee shall deliver to Penn a report ("**Licensee Financial Report**") setting out sufficient details necessary to calculate the Royalty and Penn Sublicense Income due under this Article 6 for such Calendar Quarter, including:

6.7.1 Number of each Licensed Product Sold by Licensee, its Affiliates and Sublicensees in each country, the corresponding name of each such Licensed Product;

6.7.2 Gross sales, Net Sales of each Licensed Product made by Licensee, its Affiliates and Sublicensees;

6.7.3 Royalties due for the applicable period pursuant to Section 6.3;

6.7.4 Sublicense Income due for the applicable period pursuant to Section 6.4 and the calculation of Penn Sublicense Income;

6.7.5 The method and currency exchange rates (if any) used to calculate the Royalties and Penn Sublicense Income;

6.7.6 [***];

6.7.7 [***]; and

6.7.8 Date of First Commercial Sale of each Licensed Product in the United States (this need only be reported in the first royalty report following such First Commercial Sale in the United States).

6.8 **Late Payments**. In addition to any other remedies available to Payee, including the right to terminate this Agreement, any failure by Payor to make a payment within [***] after the date when due shall obligate Payor to pay computed interest, the interest period commencing on the due date and ending on the actual payment date, to Payee at a rate per annum equal to [***], or the highest rate allowed by Law, whichever is lower.

6.9 **Default Payment**. In the event of default in payment of any payment owing to Payee under the terms of this Agreement, and if it becomes necessary for Payee to undertake legal action to collect said payment, Payor shall pay reasonable, documented legal fees and costs incurred in connection therewith.

6.10 **Accounting**. Each Party shall calculate all amounts, and perform other accounting procedures required, under this Agreement and applicable to it in accordance with GAAP.

6.11 **Books and Records**. Licensee will keep accurate books and records of all Licensed Products developed, manufactured, used or sold and all Sublicenses entered into by Licensee with respect to Penn Patent Rights. Licensee will preserve these books and records for at least [***] from the date of the Licensee Financial Report to which they pertain. Penn will keep accurate books and records of all [***]. Penn will preserve these books and records for at least [***] from the date of the Penn Financial Report to which they pertain. Upon reasonable notice, not less than [***] prior to the proposed date of review, books and records pertaining to the calculation of [***] any Milestones, Royalties and Penn Sublicense Income due to Penn under this Agreement [***] will be made reasonably available and will be open to examination by up to two (2) representatives or agents of Payee reasonably acceptable to Payor (and, to the extent such are not employees of Payee, each of

whom has executed an appropriate confidentiality agreement reasonably acceptable to Payor that requires the representative or agent to keep any information learned by it confidential except as needed to report its audit conclusions to Payee) for no longer than one (1) business day during regular office hours to determine the accuracy of such books and records and assess Payor's compliance with the terms of this Agreement, provided that Payor shall not have an obligation to provide such access more than once in any given twelve (12) month period nor more than [***] after the date of any record to be audited.

6.12 **Audits**. Payee, at its own cost, through an independent auditor reasonably acceptable to Payor (and who has executed an appropriate confidentiality agreement reasonably acceptable to Payor that requires the auditor to keep any information learned by it confidential except as needed to report its audit conclusions to Payee), may inspect and audit the relevant records of Payor pertaining to the calculation of [***] any Milestones, Royalties and Penn Sublicense Income due to Penn under this Agreement [***]. Payor shall provide such auditors with access to the records at Payor's principal place of business during reasonable business hours. Such access need not be given to any such set of records more often than once each Calendar Year nor more than [***] after the date of any report to be audited. Payee shall provide Payor with written notice of its election to inspect and audit the records related to [***] the Milestones and Royalties [***], due hereunder not less than [***] prior to the proposed date of review of Payor's records by Payee's auditors. Should the auditor establish any underpayment of Milestones, Royalties or Penn Sublicense Income by Licensee [***], Payor shall (a) promptly pay Payee the amount of such underpayment;
(b) shall reimburse Payee for the cost of the audit, if such underpayment equals or exceeds [***] of amounts paid to Payee hereunder during the time period audited. If the auditor finds overpayment by Payor, then Payor shall have the right to deduct the overpayment from any future milestones or royalties due to Penn by Licensee [***] or deduct the overpayment from [***] or, if no such future milestones or royalties [***], as applicable, are payable, then Payee shall refund the overpayment to Payor within [***] after Payee receives the audit report. Payor may designate competitively sensitive information which such auditor may see and review but which it may not disclose to Payee; provided, however, that such designation shall not restrict the auditor's investigation or conclusions.

6.13 **Taxes**. All payments made by Payor to Payee under the Agreement shall be made free and clear of and without any deduction for or on account of any Taxes on or with respect to such payments.

ARTICLE 7
CLINICAL DEVELOPMENT, REGULATORY AFFAIRS; COMMERCIALIZATION

7.1 **Development Plan**. Until the First Commercial Sale in a Major Market of the first Licensed Product for each Indication, Licensee shall provide Penn with a development plan for a Licensed Product for such Indication no later than December 1st of each year during the Term, commencing with the Calendar Year after the expiration of the Research Term. The development plan shall include a timeline for material clinical activities to be conducted by Licensee, its Affiliates and Sublicensees to support obtaining Regulatory Approvals for a Licensed Product in the Major Markets in each Indication.

7.2 **Clinical**. Licensee will consider in good faith using Penn as a study site for one or more Clinical Studies for a Licensed Product where Penn can reasonably demonstrate that Penn's capabilities

and costs are reasonably comparable to other potential study sites. If Penn (in its sole discretion) is willing and able to conduct a Clinical Study for a Licensed Product developed under a Research Program, the Parties will negotiate a separate clinical trial agreement and a separate clinical trial budget prior to initiation of such clinical trial. For clarity, any Clinical Study funding by Licensee shall be separate and in addition to the Research Support Amount.

7.3 **MPS Activities**. With respect to MPS IIIA, MPS IIIB and/or MPS VII (if Licensee has exercised a New Indication Option with respect thereto), [***].

7.4 **Commercialization**. As between the Parties, Licensee will have sole responsibility for and sole decision-making over all commercialization activities of the Licensed Products for the Indications in the Field of Use, and will be solely responsible for the associated costs of such commercialization activities.

7.5 **Manufacturing**. Except as otherwise provided in this Agreement or in a Research Plan, as between the Parties, Licensee will have responsibility for and decision-making authority over all manufacturing activities and associated costs for the clinical development (including cGMP manufacturing for clinical trials) and commercialization of the Licensed Products for the Indications in the Field of Use post-DTP for each such Licensed Product. Penn will have sole responsibility and sole decision-making authority over manufacturing activities for pre-clinical manufacturing, at Licensee's cost.

7.6 Regulatory.

7.6.1 As between the Parties, Licensee will have responsibility for and decision-making over regulatory activities for the Licensed Products for the Indications in the Field of Use. As between the Parties, Licensee will have the right to conduct all communications with Regulatory Authorities, including all meetings, conferences and discussions (including advisory committee meetings), with regard to Licensed Products for the Indications in the Field of Use. Licensee will lead and have control over preparing and submitting all INDs, BLAs and other material regulatory filings related to the Licensed Products for the Indications in the Field of Use, including all applications for Regulatory Approval,

provided, however, that Licensee shall provide Penn with copies of all such applications for Regulatory Approval prior to submission. As between the Parties, Licensee will own any and all applications for Regulatory Approvals (including INDs), Regulatory Approvals, and other regulatory filings related to the Licensed Products for the Indications in the Field of Use which will be held in the name of Licensee or its designees.

7.6.2 At Licensee's reasonable request and expense, Penn (through Dr. Wilson and other Wilson Lab personnel) shall reasonably cooperate with and assist Licensee (or its designee) in connection with interactions with Regulatory Authorities relating to Licensed Products for the Indications in the Field of Use. In addition, Licensee will keep Penn reasonably informed of the progress of such regulatory interactions and, upon request but not more than twice per Calendar Year, Licensee will provide to Dr. Wilson (or another designated Wilson Lab personnel), on a confidential basis, a copy of any material regulatory filings or correspondence to or from the FDA or EMA with respect to a Licensed Product for an Indication. Licensee will consider any reasonable comments provided on a timely basis by Dr. Wilson (or such Wilson Lab personnel).

7.7 **General Diligence**. Licensee (itself and/or through its Affiliates or Sublicensees) shall use Commercially Reasonable Efforts to actively develop and, following Regulatory Approval, to commercialize, in the Major Markets one Licensed Product for each Indication in the Field of Use.

7.8 **Structured Development Diligence Events.** Licensee shall achieve each of the following Diligence Events for each Indication by the corresponding Achievement Date:

Diligence Event	Achievement Date
[***] for a Licensed Product for each Indication	[***] after DTP for a Licensed Product for the applicable Indication
[***] for a Licensed Product for each Indication	[***] after DTP for a Licensed Product for the applicable Indication
[***] for a Licensed Product for each Indication [***].	[***] after DTP for a Licensed Product for the applicable Indication

7.9 Licensee may extend any Achievement Date for a Diligence Event (and all subsequent Diligence Events) by [***], but not more than [***] per Indication, by making a [***] payment per extension to Penn prior to the expiration of the Achievement Date for such Diligence Event. [***].

7.10 Progress Reports.

7.10.1 After performance of the Research Plans by Penn but prior to the First Commercial Sale of a Licensed Product for an Indication, Licensee on an annual basis, but in no event later than June 1st of each Calendar Year, shall submit to Penn a progress report (each, a "**Progress Report**") summarizing Licensee's (and any Affiliates' and Sublicensees') material activities related to the development of all Licensed Products for each Indication directed to obtaining of Regulatory Approvals necessary for commercialization of Licensed Products in the Major Markets.

7.10.2 Each Progress Report must include all of the following for each annual period:

(a) Update on the status of material pre-clinical work and Clinical Studies involving a Licensed Product, as well as the status of any IND and/or BLA filings for a Licensed Product; and

(b) Anticipated dates for receipt of Regulatory Approval for a Licensed Product in the Major Markets.

ARTICLE 8 INTELLECTUAL PROPERTY

8.1 **Patent Filing Prosecution and Maintenance**.

8.1.1 Penn will use diligent efforts to file, and thereafter prosecute in good faith and maintain, a Patent Right(s), which would be included in Penn Patent Rights A, claiming the Designated Product for each Indication and the use of such Designated Product for the Indication. Penn Patent Rights will be held in the name of Penn and obtained with counsel selected by Penn and reasonably acceptable to Licensee ("**Patent Counsel**"). Penn shall control all actions and decisions with respect to the filing, prosecution and maintenance of Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) and Joint Patent Rights in close coordination with Licensee via discussions at the JIPC and, in any event, Penn will consider any reasonable comments or suggestions by Licensee with respect to same; provided, however, that with respect to Penn Patent Rights A claiming solely a Designated Product applicable to the Indications in the Field of Use, Joint Patent Rights, and Discovery Patent Rights for which there is no Third Party licensee, Penn shall have an obligation to consider in good faith and implement any reasonable comments provided by Licensee. Penn will instruct Patent Counsel to copy Licensee on all correspondence related to Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G), Joint Patent Rights and any other Discovery Patent Rights for which there is no Third Party licensee (including copies of each patent application, office action, response to office action, request for terminal disclaimer, and request for reissue or reexamination of any patent or patent application) and to interact with Licensee with respect to the preparation, filing, prosecution and maintenance of

Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) and Joint Patent Rights. Penn has the right to take action to preserve rights and minimize cost whether or not Licensee has commented, and will use reasonable efforts to not abandon or allow to lapse (a) any Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) or Joint Patent Rights for which Licensee is licensed and is underwriting its share of the Patent Costs nor (b) any other Discovery Patent Rights for which Licensee is underwriting its share of the Patent Costs, if any, in each case ((a) and (b)) without Licensee's written authorization under this Agreement, except for filing of continuations, divisionals, or the like that substitute for the lapsed application, provided that, Penn shall have no requirement to file, prosecute, or maintain Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) or Joint Patent Rights if Licensee is not current with its Patent Cost obligations with respect to such Patent Right as set forth in this Agreement. For the purposes of this Agreement, "maintenance" of the Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) and Joint Patent Rights includes inter parties patent review proceedings before the USPTO or a similar patent administration outside the US. For further clarity, validity challenges raised in infringement litigation will be handled per Section 8.3, Infringement.

8.1.2 The Parties shall discuss and agree at the JIPC the countries in which Patent Rights within the Penn Patent Rights A, Penn Patent Rights B (excluding Patent Rights on Exhibit G) and Joint Patent Rights will be filed. Licensee has the right to request any additional country filing for Penn Patent Rights A, Penn Patent Rights B and Joint Patent Rights via a written request to Penn ninety (90) days prior to the deadline set by the patent office in the territory in which filing is to take place ("**Prosecution Request**"). The absence of a given Prosecution Request by such deadline will be considered an election not to secure the Patent Rights associated with the specific phase of patent prosecution in such country, and such patent application(s) and patent(s) in such country ("**Carve-Out Patent Rights**") will not be part of Penn Patent Rights and therefore not subject to this Agreement, including the License, and Licensee will have no further rights or license to them.

8.1.3 For Penn Patent Rights B listed on Exhibit G and any other Discovery Patent Rights for which there is a Third Party licensee, Penn Patent Rights C and Manufacturing Patent Rights, Penn will instruct Patent Counsel to copy Licensee on all correspondence (including copies of each patent application, office action, response to office action, request for terminal disclaimer, and request for reissue or reexamination of any patent or patent application), to interact with Licensee with respect to the preparation, filing, prosecution and maintenance, and to consider any reasonable comments or suggestions by Licensee with respect to same.

8.1.4 Licensee shall also have the right, on a Penn Patent Right-by-Penn Patent Right and country-by-country basis, to (i) elect not to fund at the time of disclosure, or (ii) elect not to continue to fund, in each case (i) and (ii), its pro rata share (as determined pursuant to Section 8.2) of the Patent Costs with respect to any Penn Patent Right(s) in a particular country, which election may be made by Licensee upon sixty (60) days prior written notice to Penn ("**Election Notice**"). Within fifteen (15) business days after receipt of an Election Notice from Licensee, Penn shall notify Licensee in writing whether (a) any Third Party is obligated to fund any portion of the Patent Costs with respect to any Penn Patent Right identified in such Election Notice in any country identified in such Election Notice or (b) Penn and/or Dr. James Wilson will fund such Patent Costs or will allow such Penn Patent Right to lapse or become abandoned in such country. Within five (5)

business days after receipt of any such notice from Penn, Licensee shall have the right to cancel its Election Notice and fund or continue to fund, as applicable, its pro rata share of the Patent Costs with respect to such Penn Patent Right in such country. If Licensee delivers an Election Notice to Penn (and does not cancel such Election Notice, pursuant to the preceding sentence), following the expiration of such sixty (60) day period, Licensee shall have no further obligation to pay Ongoing Patent Costs with respect to any Penn Patent Right identified in such Election Notice in any country identified in such Election Notice and any such Patent Right in any such country shall thereafter be excluded from the Penn Patent Rights.

8.1.5 Notwithstanding the foregoing, on a Funded Discovery Patent Right-by-Funded Discovery Patent Right and country-by-country basis, if any Patent Right for which Licensee elects not to fund its share of Patent Costs in a country pursuant to Section 8.1.4, is a Funded Discovery Patent Right and Licensee has funded its share of the Patent Costs for such Funded Discovery Patent Right in such country through the expiration of the Discovery Term, such Patent Right in such country shall be treated as a Funded Discovery Patent Right in such country for the purpose of revenue sharing. If Licensee chooses (pursuant to Section 8.1.4) not to fund its share of the Patent Costs for a Funded Discovery Patent Right in a country following completion of the Discovery Term ("**Abandoned Discovery Rights**"), [***].

8.2 **Patent Costs**.

8.2.1 Subject to Section 8.2.3, within 30 days after the New Effective Date, Licensee will reimburse Penn for all documented out-of-pocket costs for the filing, prosecution and maintenance of Penn Patent Rights and Joint Patent Rights, including all accrued and documented attorney fees, expenses, official and filing fees ("**Patent Costs**"), incurred prior to the New Effective Date or the date at which such Patent Rights are added to the License (as applicable), which have not otherwise been reimbursed by Licensee or other licensees of such Penn Patent Rights ("**Historic Patent Costs**"). Historic Patent Costs for the Discovery Patent Rights that were incurred prior to the New Effective Date shall be excluded from Licensee's reimbursement obligation to Penn. Notwithstanding the first sentence of this Section 8.2.1, for Penn Patent Rights or Joint Patent Rights licensed by Penn to more than one licensee, Licensee shall be responsible for payment to Penn of a pro rata share of such documented Historic Patent Costs based on the number of licensees for such Penn Patent Rights or Joint Patent Rights.

8.2.2 Licensee will bear (a) all Patent Costs incurred during the Term, for Penn Patent Rights (other than Discovery Patent Rights) and Joint Patent Rights, (b) for Discovery Patent Rights, all Patent Costs incurred during the period beginning on the New Effective Date and thereafter until the expiration of the last Discovery Patent Right (collectively, "**Ongoing Patent Costs**"). Notwithstanding the foregoing, for Penn Patent Rights or Joint Patent Rights licensed by Penn to more than one licensee, Licensee shall be responsible for payment to Penn of a pro rata share of such documented Ongoing Patent Costs based on the number of licensees for such Penn Patent Rights or Joint Patent Rights. No later than sixty (60) days prior to the end of each Calendar Year during the Term, Penn shall provide to Licensee, a good faith estimate and budget for the Ongoing
Patent Costs anticipated to be incurred for the next Calendar Year and, to the extent

applicable, Licensee's proportionate share of such Ongoing Patent Costs. This Section 8.2.2 is subject to Section 8.1.4 above.

8.2.3 Licensee shall pay in advance the Patent Counsel's estimated costs for undertaking material patent actions with respect to Penn Patent Rights and Joint Patent Rights before Penn authorizes the Patent Counsel to proceed ("**Advance Payment**"). Notwithstanding whether Licensee makes an Advance Payment for any patent action, Licensee shall bear its pro rata share (based on the number of licensees for such Penn Patent Rights or Joint Patent Rights) of all Patent Costs with respect to Penn Patent Rights and Joint Patent Rights as set forth in Section 8.2.1 or Section 8.2.2 and shall pay such amounts within [***] of receipt of invoice for such patent actions. For clarity, the term "Patent Costs" means and includes Historic Patent Costs and Ongoing Patent Costs.

8.3 Infringement.

8.3.1 If either Party believes that an infringement by a Third Party with respect to any Penn Patent Right is occurring or may potentially occur, the knowledgeable Party will provide the other Party with (a) written notice of such infringement or potential infringement and
(b) evidence of such infringement or potential infringement (the "**Infringement Notice**"). During the period in which, and in the jurisdiction where, Licensee has exclusive rights under this Agreement, subject to Licensee's right to institute suit for patent infringement pursuant to Section 8.3.2 if infringing activity of potential commercial

significance has not been abated within [***] following the date the Infringement Notice for such activity was provided, neither Penn or Licensee will notify such a Third Party (including the infringer) of infringement or put such Third Party on notice of the existence of Penn Patent Rights without first obtaining the written consent of the other Party. If Licensee puts such infringer on notice of the existence of any Penn Patent Right without the prior written consent of Penn prior to the expiration of such [***], then Licensee's right to initiate a suit under Section 8.3.2 below will terminate immediately without the obligation of Penn to provide notice to Licensee. Both Penn and Licensee will use their diligent efforts to cooperate with each other to terminate any such infringement without litigation.

8.3.2 With respect to Penn Patent Rights A, if infringing activity of potential commercial significance has not been abated within [***] following the date the Infringement Notice for such activity was provided, then during the period in which, and in the jurisdiction where, Licensee is the sole licensee for certain Penn Patent Rights A and the infringement is a competing product to a Licensed Product for an Indication, Licensee may institute suit for patent infringement of such Penn Patent Rights A against the infringer. With respect to Penn Patent Rights B, if infringing activity of potential commercial significance has not been abated within [***] following the date the Infringement Notice for such activity was provided, then during the period in which, and in the jurisdiction where, Licensee is the sole licensee for certain Penn Patent Rights B and the infringement is a competing product to a Designated Product for an Indication, the JIPC shall discuss and recommend how to handle such infringement, including whether to institute suit for patent infringement of such Penn Patent Rights B against the infringer, which Party shall have the right to initiate and control such suit and making decisions with respect to litigation strategy. If the JIPC (after escalation pursuant to Section 4.2.4 and Section 4.1.5) is unable to reach agreement, Penn shall have the final decision making authority with respect to handling

any infringement action related to Penn Patent Rights B; provided that in any event, Penn will consider any reasonable comments or suggestions by Licensee with respect to same.

8.3.3 Penn may voluntarily join such suit at its own expense, but may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Licensee's suit or any judgment rendered in such suit. If in a suit initiated by Licensee, Penn is involuntarily joined other than by Licensee, then Licensee will pay any documented costs incurred by Penn arising out of such suit, including any documented legal fees of counsel that Penn selects and retains to represent it in the suit. In any suit initiated by Licensee, Licensee shall be free to enter into a settlement, consent judgment or other voluntary disposition, provided that any settlement, consent judgment or other voluntary disposition that (i) limits the scope, validity or enforcement of Penn Patent Rights A or Penn Patent Rights B or (ii) admits fault or wrongdoing on the part of Penn must be approved in advance by Penn in writing (such approval not to be unreasonably withheld or delayed). Licensee's request for such approval shall include complete copies of proposed settlement documents, a summary of such settlement, and any other information material to such settlement that is reasonably requested by Penn. Penn shall provide Licensee notice of its approval or denial within thirty (30) days of any request for such approval by Licensee, provided that (x) in the event Penn wishes to deny such approval, such notice shall include a detailed written description of Penn's reasonable objections to the proposed settlement, consent judgment, or other voluntary disposition and (y) Penn shall be deemed to have approved of such proposed settlement, consent judgment, or other voluntary disposition in the event it fails to provide such notice within such thirty (30) day period in accordance herewith.

8.3.4 If, within [***] following the date of a request to do so from Penn, infringing activity of potential commercial significance has not been abated and if Licensee has not brought suit against the infringer, then Penn may institute suit for patent infringement against the infringer. If Penn institutes such suit, then Licensee may not join such suit without the prior written consent of Penn (which consent shall not be unreasonably withheld or delayed) and may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Penn's suit or any judgment rendered in such suit.

8.3.5 Notwithstanding Sections 8.3.2, 8.3.3 and 8.3.4, in the event that any Penn Patent Rights A or Penn Patent Rights B are infringed by a Third Party and any of the infringed Penn Patent Rights A or Penn Patent Rights B are also licensed by Penn to a Third Party, prior to any enforcement action being taken by either Party regarding such infringement, the JIPC shall discuss and recommend how to handle such infringement by such Third Party.

8.3.6 Any recovery or settlement received in connection with any suit will first be shared by Penn and Licensee equally to cover any litigation costs each incurred (to the extent not previously reimbursed) and next shall be paid to Penn or Licensee to cover any litigation costs it incurred in excess of the litigation costs of the other (to the extent not previously reimbursed). Any remaining recoveries shall be allocated as follows:

For any portion of the recovery or settlement, other than for amounts attributable and paid as enhanced damages for willful infringement:

(a) for any suit that is initiated by Licensee and in which Penn was not a party in the litigation, Penn shall receive [***] of the recovery and the Licensee shall receive the remainder; and

(b) for any suit that is initiated by the Licensee or Penn and that the other Party joins voluntarily (but only to the extent such voluntary joining is allowed under this Agreement or expressly by the other Party in a separate agreement) or involuntarily, the non-initiating party's percentage of the total litigation costs incurred by Penn and Licensee, but in no event shall the non-initiating Party receive less than [***] of such recovery, while the initiating party shall receive the remainder, and in no case shall Penn receive less than [***] of such recovery.

For any portion of the recovery or settlement paid as enhanced damages for willful infringement:

(c) for any suit that is initiated by Licensee or Penn and the other Party voluntarily but only to the extent such voluntary joining is allowed under this Agreement or expressly by the other Party in a separate agreement) or involuntarily, the initiating party shall receive [***] and the non-initiating shall receive the remainder; and

(d) for any suit that is initiated by Licensee and in which Penn was not a party in the litigation, Penn shall receive [***] and Licensee shall receive the remainder.

For any portion of the recovery or settlement received in connection with any suit that is initiated by Penn and in which Licensee was not a party in the litigation, any recovery in excess of litigation costs will belong to Penn.

8.3.7 Each Party will reasonably cooperate and assist with the other in litigation proceedings instituted hereunder but at the expense of the Party who initiated the suit (unless such suit is being jointly prosecuted by the Parties). For clarity, such requirement does not require a Party to join a suit unless otherwise specifically required under this Agreement. If Penn is subjected to third party discovery related to the Penn Patent Rights or Licensed Products licensed to Licensee hereunder, Licensee will pay Penn's documented out-of-pocket expenses with respect to same.

8.3.8 Penn shall keep Licensee reasonably informed of the initiation and status of any action to enforce any Penn Patent Rights A, Discovery Patent Rights (including Penn Patent Rights B), Penn Patent Rights C or Manufacturing Patent Rights pertaining to the Indications or a Licensed Product.

8.4 **Defense**. Each Party shall have the right to defend any adversarial legal proceeding brought against it, and the Parties shall reasonably cooperate with one another regarding such defense, provided that such right of defense does not include any right to bring infringement actions (including counterclaims) with respect to Penn Patent Rights except as expressly set forth herein or as otherwise agreed by the Parties.

8.5 **Patent Marking**. Licensee shall place in a conspicuous location on any Licensed Product (or its packaging where appropriate and practicable) made or sold under this Agreement a patent notice

in accordance with the Laws concerning the marking of patented articles where such Licensed Product is made or sold, as applicable.

8.6 **Ownership of Inventions**. Ownership of any inventions or other intellectual property generated in the conduct of a Research Program or otherwise under this Agreement will be determined in accordance with United States patent law or other applicable intellectual property law. For clarity,
(a) inventions conceived and reduced to practice solely by Penn inventors will be solely owned by Penn, (b) inventions conceived and reduced to practice solely by Licensee inventors will be solely owned by Licensee, and (c) inventions jointly conceived and reduced to practice by both Penn and Licensee inventors will be jointly owned by Penn and Licensee.

ARTICLE 9 CONFIDENTIALITY& PUBLICATION

9.1 **Confidential Information**. Licensee shall not disclose Confidential Information to Penn unless it is reasonably necessary to the performance of a Research Program or otherwise required to perform Licensee's obligations under this Agreement. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, during the Term and for ten (10) years thereafter, the receiving Party (the "**Receiving Party**") and its Affiliates will keep confidential and will not publish or otherwise disclose or use for any purpose any Confidential Information, which is disclosed to it by the other Party (the "**Disclosing Party**") or its Affiliates or otherwise made available to a Receiving Party in the course of performing its obligations or exercising its rights under this Agreement. A Receiving Party shall also have the right to disclose the disclosing Party's Confidential Information to those of the Receiving Party's and its Affiliates' employees, agents and/or consultants who have a need to know such Confidential Information to perform its obligations or exercise its rights under this Agreement; and who have entered into a written agreement with the Receiving Party (or its relevant Affiliate) to be bound by the obligations of confidentiality and non-use at least as protective of such Confidential Information as set forth in this Article 9. In the case of Licensee as the Receiving Party, Licensee and its Affiliates shall have the right to use and disclose Confidential Information of Penn: for the purpose of developing, seeking and obtaining Regulatory Approval for, making, having made, using, selling, offering for sale and/or otherwise commercializing Licensed Products under the License; and to actual and potential Third Party service providers, sublicensees, other sources of financing and/or acquirers or others on a need-to-know basis under appropriate conditions of confidentiality.

9.2 **Disclosures Required by Law**. In the event a Party is required to make a disclosure under Law or regulation, the order of a court of competent jurisdiction, or the rules of the U.S. Securities and Exchange Commission or other Governmental Body within or outside the United States (including by reason of any securities offering by Licensee), or any stock exchange or listing entity, a Receiving Party shall provide prompt written notice to the Disclosing Party and take all reasonable steps to limit the extent of the disclosure and obtain confidential treatment for any remaining required disclosure.

9.3 **Penn Intellectual Property**. In order to preserve the patentability of Penn intellectual property and to preserve Penn's publication rights, Licensee shall maintain Penn Patent Rights, Research Results and Confidential Information provided by Penn pursuant to a Research Program (whether oral or written) as confidential and shall not disclose such Confidential Information to any Third Party except as permitted under this Article 9 until the publication of such information by Penn or until Penn provides Licensee with written verification that all desirable patentable inventions have been protected, whichever occurs sooner.

9.4 **Licensee Intellectual Property**. In order to preserve the patentability of Licensee's intellectual property and otherwise to preserve Licensee's rights therein and thereto, Penn shall maintain Amicus Technology and Confidential Information provided by Licensee pursuant to a Research Program or otherwise under this Agreement as confidential and shall not disclose such information to any Third Party except as expressly permitted under this Agreement. For the avoidance of doubt, for purposes of this Agreement, all records maintained by Licensee described in Section 6.11 and all Amicus Technology, Progress Reports and Licensee Financial Reports provided by Licensee to Penn under this Agreement, as well as the sequence of any Designated Product, shall be Confidential Information of Licensee.

9.5 **Publications**. Penn shall have the first right to publish, present or otherwise disclose Research Results or other information and material resulting from a Research Program for any purpose; provided, however, that consistent with the Wilson Lab's standard operating procedures for collaborations with commercial third parties, Penn shall provide Licensee the opportunity to review and comment on any proposed manuscripts or any other proposed public disclosure describing work developed under a Research Program that has not previously been disclosed, [***] prior to its submission for publication or first public disclosure for manuscripts and [***] prior to its submission or first public disclosure for abstracts and speaking engagements to (a) determine whether such contains any Licensee Confidential Information and

(b) enable Licensee to identify any Penn intellectual property or joint intellectual property that it wishes Penn to file patent applications on or to seek other intellectual property protection for. If within the [***] review period (i) Licensee notifies Penn in writing that the Licensee requires deletion from the publication or presentation of Licensee Confidential Information, the Parties will cooperate to modify the disclosure to ensure Licensee Confidential Information is not disclosed or (ii) if Licensee requests in writing that publication or presentation be delayed to allow for patent filings or other intellectual property protection on certain items in the proposed publication or presentation, Penn shall delay the publication or presentation for up to [***] to allow for the filing of applicable patent applications.

ARTICLE 10 REPRESENTATIONS, WARRANTIES AND COVENANTS

10.1 **Mutual Representations and Warranties**. Each Party represents and warrants to the other Party that, as of the Effective Date and as of the New Effective Date:

10.1.1 such Party is duly organized and validly existing under the Laws of the jurisdiction of its incorporation or organization;

10.1.2 such Party has taken all action necessary to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement;

10.1.3 this Agreement is a legal and valid obligation of such Party, binding upon such Party and enforceable against such Party in accordance with the terms of this Agreement, except as enforcement may be limited by applicable bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other laws relating to or affecting creditors' rights generally and by general equitable principles; and

10.1.4 such Party has all right, power and authority to enter into this Agreement, to perform its obligations under this Agreement.

10.2 **Representation of Penn**. Penn hereby represents:

10.2.1 as of the Effective Date, to Penn's knowledge, Penn's performance of any Research Program and/or grant of rights to Licensee under this Agreement does not conflict with any agreement with a Third Party;

10.2.2 as of the New Effective Date, to Penn's knowledge, Penn's performance of any Research Program and/or the Discovery Program and/or grant of rights to Licensee under this Agreement does not conflict with any agreement with a Third Party;

10.2.3 other than licenses granted under the Excluded Penn IP or the arrangements with Third Parties described on Schedule 3.2.4, to Penn's knowledge, Penn has not entered into any arrangement with any Third Party pertaining to any Indication, Potential Indication or Exploratory Indication prior to the New Effective Date which is still in effect and/or pursuant to which a Third Party may have rights to any Patent Rights or Know-How conceived or reduced to practice in the Wilson Lab;

10.2.4 all information provided by or on behalf of Penn to Licensee on or before the New Effective Date in connection of this Agreement was (when provided) and is (as of the New Effective Date), to Penn's knowledge, true, accurate and complete in all material respects and, as of the New Effective Date, Penn has not knowingly failed to disclose any material information necessary to make such information that has been disclosed not misleading in any material respect; and

10.2.5 Penn has not, up through and including the New Effective Date, intentionally omitted to furnish Licensee with any information in its control or possession, or of which it is aware, concerning the Penn Patent Rights or the activities contemplated by this Agreement, which could reasonably be expected to be material to Licensee's decision to enter into this Agreement and to undertake the commitments and obligations set forth herein.

10.3 Disclaimer of Representations and Warranties.

10.3.1 Other than the representations and warranties provided in Section 10.1 above, **PENN MAKES NO REPRESENTATIONS AND WARRANTIES, WHETHER EXPRESS OR IMPLIED, AND EXPLICITLY DISCLAIMS ANY REPRESENTATION AND WARRANTY, INCLUDING WITH RESPECT TO ANY ACCURACY, COMPLETENESS, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, COMMERCIAL UTILITY, NON-INFRINGEMENT OR TITLE FOR THE INTELLECTUAL PROPERTY, PATENT RIGHTS, LICENSE AND ANY LICENSED PRODUCT**.

10.3.2 Furthermore, nothing in this Agreement will be construed as:

(a) A representation or warranty by Penn as to the validity or scope of any Penn Patent Right;

(b) A representation or warranty that anything made, used, sold or otherwise disposed of under the License is or will be free from infringement of patents, copyrights, trademarks or any other forms of intellectual property rights or tangible property rights of Third Parties;

(c) Obligating Penn to bring or prosecute actions or suits against Third Parties for patent, copyright or trademark infringement; and

(d) Conferring by implication, estoppel or otherwise any license or rights under any Patent Rights of Penn other than Penn Patent Rights as defined herein, regardless of whether such Patent Rights are dominant or subordinate to Penn Patent Rights.

10.4 Covenants of Licensee.

10.4.1 Licensee and its Affiliates will not, directly or indirectly (including where such is done by a Third Party on behalf of Licensee or its Affiliates) make any Challenge; provided, however, that if any Penn Patent Right is asserted against Licensee or its Affiliate, then such Licensee or its Affiliates is entitled to all and any defenses available to it including challenging the validity or enforceability of such Patent Right.

10.4.2 Licensee will comply in all material respects with all Laws that apply to its activities or obligations under this Agreement. For example, Licensee will comply with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the applicable agency of the United States Government and/or written assurances by Licensee that Licensee will not export data or commodities to certain foreign countries without prior approval of the agency.

10.4.3 Licensee will not grant a security interest in the License or this Agreement.

ARTICLE 11
INDEMNIFICATION; INSURANCE AND LIMITATION OF LIABILITY

11.1 **Indemnification by Licensee**.

11.1.1 Licensee shall defend, indemnify and hold Penn and its respective trustees, officers, faculty, students, employees, contractors and agents (the "**Penn Indemnitees**") harmless from and against any and all liability, damage, loss, cost or expense (including reasonable attorneys' fees), including, without limitation, bodily injury, risk of bodily injury, death and property damage (collectively, "**Liabilities**") to the extent arising out of Third Party claims or suits [***] including:

(a) the gross negligence, recklessness or wrongful intentional acts or omissions of Licensee, its Affiliates or Sublicensees and its or their respective directors, officers, employees and agents, in the performance of the Licensee's obligations or exercise of Licensee's rights under this Agreement;

(b) any material breach of this Agreement by Licensee;

(c) the development, manufacturing or commercialization of Licensed Products (including commercial manufacturing, packaging and labeling of Licensed Products, and all product liability losses of a Licensed Product by or on behalf of Licensee or its Affiliates or Sublicensees; and

(d) any enforcement action or suit brought by Licensee against a Third Party for infringement of Penn Patent Rights or Joint Patent Rights.

provided that Licensee's obligations pursuant to this Section 11.1 shall not apply to the extent such Liabilities and Third Party claims or suits result or arise from [***].

11.1.2 As a condition to a Penn Indemnitee's right to receive indemnification under this Section 11.1, Penn shall: (a) promptly notify Licensee as soon as it becomes aware of a claim or suit for which indemnification may be sought pursuant hereto; (b) fully cooperate, and cause the individual Penn Indemnitees to fully cooperate, with Licensee in the defense, settlement or compromise of such claim or suit; and (c) permit the Licensee to control the defense, settlement or compromise of such claim or suit, including the right to select defense counsel. In no event, however, may Licensee compromise or settle any claim or suit in a manner which (i) admits fault or negligence on the part of Penn or any other Penn Indemnitee; (ii) commits Penn or any other Penn Indemnitee to take, or forbear to take, any action, without the prior written consent of Penn, or (iii) grant any rights under the Penn Patent Rights except for Sublicenses permitted under Article 5. Penn shall fully cooperate, and cause the individual Penn Indemnitees to fully cooperate, with Licensee and its counsel in the course of the defense or settlement of any such suit, claim or demand, such cooperation to include without limitation providing or making available documents, information and witnesses.

11.1.3 Notwithstanding Section 11.1.2 above, a Penn Indemnitee shall be entitled to participate in, but not control, the defense of a Third Party claim or suit subject to indemnification under Section 11.1.1 above and to engage counsel of its own choice for such purpose; provided that such engagement shall be at such Penn Indemnitee's own expense unless a bona fide conflict exists between Licensee and Penn or any other Penn Indemnitee with respect to a claim or suit subject to indemnification hereunder, such that representation by Licensee and Penn or such other Penn Indemnitee by the same legal counsel due to a misalignment of interests or would be prohibited under applicable law, ethical rules or equitable principles, in which case, Licensee will either pay any reasonable, documented out-of-pocket attorney's fees and litigation expenses of such Penn Indemnitee directly or reimburse Penn within [***] of Licensee's receipt of invoices for such fees and expenses.

11.1.4 In no event shall Licensee be liable under this Section 11.1 for any settlement, compromise or other disposition of a Third Party claim or suit for which a Penn Indemnitee seeks indemnification hereunder and that is reached without the prior written consent of Licensee, such consent not to be unreasonably withheld, conditioned or delayed.

11.2 Insurance.

11.2.1 Licensee, at its sole cost and expense, must insure its activities in connection with the exercise of its rights under this Agreement and keep in force and maintain Commercial Form General Liability Insurance (contractual liability included) with at least the following limits:

(a) Each occurrence $[***];

(b) General aggregate $[***]

Prior to the commencement of clinical trials, if applicable, involving Licensed Product:

(c) Clinical trials liability insurance $[***]

Prior to the First Commercial Sale of a Licensed Product:

(d) Products liability insurance $[***]

Penn may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 11.2.1, and has the right to discuss with Licensee adjustments to such limits.

11.2.2 If the above insurance is written on a claims-made form, it shall continue for three (3) years following termination or expiration of this Agreement. The insurance shall have a retroactive date of placement prior to or coinciding with the Effective Date of this Agreement.

11.2.3 Licensee expressly understands, however, that the coverages and limits in Section 11.2.1 do not in any way limit Licensee's liability or indemnification obligations. Licensee's insurance will:

(a) Be issued by an insurance carrier with an A.M. Best rating of "A" or better;

(b) Provide for thirty (30) day advance written notice to Penn of any modification;

(c) State that Penn is endorsed as an additional insured with respect to the coverages in Section 11.2.1; and

(d) Include a provision that the coverages will be primary and will not participate with nor will be excess over any valid and collective insurance or program of self insurance carried or maintained by Penn.

11.2.4 Licensee must furnish to Penn with (a) valid certificate of insurance evidencing compliance with all requirements of this Agreement and (b) additional insured endorsements for Licensee's applicable policies naming "The Trustees of the University of Pennsylvania" as an additional insured. Licensee must furnish both documents within thirty (30) days of the Effective Date, once per year thereafter and at any time there is a modification in such insurance.

11.3 **LIMITATION OF LIABILITY**. [***], IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER OR ANY OF ITS AFFILIATES FOR SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES, INCLUDING LOSS OF PROFITS OR OPPORTUNITY, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE ARISING OUT OF OR RELATING TO THIS AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREIN OR ANY BREACH HEREOF; PROVIDED THAT NOTHING IN THIS SECTION 11.3 SHALL BE DEEMED TO LIMIT LICENSEE'S INDEMNIFICATION OBLIGATIONS UNDER SECTION 11.1.

ARTICLE 12
TERM AND TERMINATION

12.1 **Term**. The term of this Agreement (the "**Term**") shall commence on the Effective Date and, unless terminated sooner as provided below, shall continue in full force and effect on a country- by-country and Licensed Product-by-Licensed Product basis until [***]. Following expiration of the [***] (but not earlier termination) in a particular country, the license to Licensed Know-How and Licensed Discovery Know-How in such country for Licensed Products for the applicable Indication in the Field of Use as set forth in Section 5.1 will become perpetual and fully paid-up.

12.2 **Termination of the Agreement for Convenience**. Subject to Section 12.4, Licensee may, at its convenience, terminate this entire Agreement or on an Indication-by-Indication basis, by providing at least [***] prior written notice to Penn of such intention to terminate.

12.3 Termination For Cause.

12.3.1 In the event Licensee fails to achieve any Diligence Event by the applicable Achievement Date (or as extended according to the terms of Section 7.8 hereto) other than due to a an Extension Event, Penn has the right and option to terminate this Agreement upon written notice to Licensee on an Indication-by-Indication basis for the Indication for which the Diligence Event has not been achieved, if Licensee has not cured such failure within [***] of written notice from Penn.

12.3.2 In addition to all other remedies available to it, Penn may terminate this Agreement upon [***] written notice if Licensee materially fails to comply with any Laws that apply to its activities or obligations under this Agreement and that can be remedied and Licensee fails to remedy such lack of compliance within such [***] period, (b) upon [***] written notice, if Licensee grants a security interest in this Agreement or any of the rights granted herein and does not revoke such grant prior to the expiration of such [***] period, or (d) upon written notice, if Licensee breaches Section 10.3.1 and does not withdraw or discontinue the applicable Challenge within [***] of such notice.

12.3.3 If either Party materially breaches any of its material obligations under this Agreement, the non-breaching Party may give to the breaching Party a written notice specifying the nature of the default, requiring it to cure such breach, and stating its intention to terminate this Agreement. If such breach is not cured within [***] of such notice (for non-payment), and [***] of such notice for all other material breaches, such termination shall become effective upon a notice of termination by the terminating Party thereafter; provide that if there is a good faith dispute as to the existence of a material breach, such [***] period may be extended by mutual agreement of the Parties to allow the Parties additional time to continue good faith discussions to resolve the dispute. To the extent Licensee's material breach relates solely to an Indication, Penn's right to terminate the Licensee's rights under the Agreement will be limited to such Indication.

12.3.4 Either Party may terminate this Agreement, upon written notice if, at any time, the other Party files in any court or agency pursuant to any statute or regulation of any state, country or jurisdiction, a petition in bankruptcy or insolvency or for reorganization or for

an arrangement or for the appointment of a receiver or trustee of such Party or of its assets, or if such Party proposes a written agreement of composition or extension of its debts, or if such Party is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition is not dismissed within [***] after the filing thereof, or if such Party proposes or is a party to any dissolution or liquidation, or if such Party makes an assignment for the benefit of its creditors of all or substantially all its assets (in each case, "**Bankruptcy Action**").

12.4 **Effects of Termination**.

12.4.1 Notwithstanding the termination or expiration of this Agreement, the following provisions shall survive: Sections [***] and Articles [***].

12.4.2 Termination of this Agreement shall not relieve the Parties of any obligation or liability that, at the time of termination, has already accrued hereunder, or which is attributable to a period prior to the effective date of such termination. Termination of this Agreement shall not preclude either Party from pursuing all rights and remedies it may have hereunder or at Law or in equity with respect to any breach of this Agreement nor prejudice either Party's right to obtain performance of any obligation.

12.4.3 If this Agreement is terminated for any reason, all outstanding Sublicenses (including all Sublicense Documents for each Sublicense) not in default shall survive, provided that each such Sublicensee agrees in writing to be bound by the applicable terms of this Agreement with respect to the activities of such Sublicensee under such Sublicense. The duties and obligations of Penn under any surviving Sublicenses will not be greater than the duties of Penn under this Agreement, and the rights of Penn under any surviving Sublicenses will not be less than the rights of Penn under this Agreement, including all financial consideration and other rights of Penn.

12.4.4 Within [***] of termination of this Agreement or any Indication (other than termination by Licensee pursuant to Section 12.3.3 or 12.3.4), Licensee shall pay Penn
(a) any unpaid portion of the Research Support Amount, (b) all costs for commitments pertaining to the performance of a Research Plan (to the extent such costs are non- cancellable commitments incurred prior to the receipt, or issuance, by Penn of the notice of termination, and the cost of each employee, student and faculty member allocated to activities under the applicable portion(s) of a Research Plan during the Research Term, in each case, to the extent such costs are not included in the Research Support Amount) (c) any unpaid portion of the Discovery Support Amount and (d) if Licensee has elected to extend the Discovery Program pursuant to Section 3.1.2, any unpaid portion of the Discovery Extension Support Amount.

12.4.5 Upon termination of this Agreement and subject to Section 12.3.3, Licensee, its Affiliates and Sublicensees whose rights do not survive termination of this Agreement will promptly cease selling the Licensed Product(s) subject to such termination. Each Party

will return (or destroy, as directed by the other Party) all data, files, records and other materials containing or comprising the other Party's Confidential Information with respect to this Agreement, except to the extent such Confidential Information is necessary or useful to conduct activities in connection with surviving portions of or rights pursuant to this Agreement. Notwithstanding the foregoing, the Parties will be permitted to retain one copy of such data, files, records, and other materials for archival and legal compliance purposes.

ARTICLE 13 ADDITIONAL PROVISIONS

13.1 **Relationship of the Parties**. Nothing in this Agreement is intended or shall be deemed, for financial, tax, legal or other purposes, to constitute a partnership, agency, joint venture, fiduciary or employer-employee relationship between the Parties. The Parties are independent contractors and at no time will either Party make commitments or incur any charges or expenses for or on behalf of the other Party.

13.2 **Expenses**. Except as otherwise provided in this Agreement, each Party shall pay its own expenses and costs incidental to the preparation of this Agreement and to the consummation of the transactions contemplated hereby

13.3 **Use of Names**. Licensee, its Affiliates and Sublicensees may not use the name, logo, seal, trademark, or service mark (including any adaptation of them) of Penn or any Penn school, organization, employee, student or representative in any press release, advertising, promotional or sales literature, without the prior written consent of Penn. Notwithstanding the foregoing, Licensee may use the name of Penn in a non-misleading and factual manner solely in (a) executive summaries, business plans, offering memoranda and other similar documents used by Licensee for the purpose of raising financing, including for the operations of Licensee as related to a Licensed Product, or entering into commercial contracts with Third Parties, but in such case only to the extent necessary to inform a reader that the Penn Patent Rights has been licensed by Licensee from Penn, and (b) any securities reports required to be filed with the Securities and Exchange Commission or any other disclosures required under applicable Laws (including securities regulations).

13.4 **No Discrimination**. Neither Penn nor Licensee will discriminate against any employee or applicant for employment because of race, color, sex, sexual orientation, age, religion, national or ethnic origin, handicap, or veteran status.

13.5 Successors and Assignment.

13.5.1 The terms and provisions hereof shall inure to the benefit of, and be binding upon, the Parties and their respective successors and permitted assigns.

13.5.2 Neither Party may assign or transfer this Agreement or any of its rights or obligations created hereunder, by operation of law or otherwise, without the prior written consent of the other Party. Notwithstanding the foregoing, without Penn's consent, Licensee shall have the right to assign any of its rights or obligations under this Agreement, or to transfer this Agreement, to: (a) any of its Affiliates, [***]; or (b) a Third Party in connection with a merger, acquisition of all or substantially all of the business or assets of Licensee(whether by sale of stock or assets),

consolidation, change of control or other similar transaction; provided that such third party is bound by the terms of this Agreement, by operation of law or otherwise.

13.5.3 Any assignment not in accordance with this Section 13.5 shall be null and void.

13.6 **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments and to do all such other acts as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

13.7 **Entire Agreement of the Parties; Amendments**. This Agreement, the Exhibits and Appendices or Schedules hereto constitute and contain the entire understanding and agreement of the Parties respecting the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter, including the Original Agreement. No waiver, modification or amendment of any provision of this Agreement shall be valid or effective unless made in a writing referencing this Agreement and signed by a duly authorized officer of each Party.

13.8 **Governing Law**. This Agreement shall be governed by and interpreted in accordance with the laws of the Commonwealth of Pennsylvania, excluding application of any conflict of laws principles that would require application of the law of a jurisdiction outside of the Commonwealth of Pennsylvania.

13.9 **Dispute Resolution**. If a dispute arises between the Parties concerning this Agreement, then the Parties will confer, as soon as practicable, in an attempt to resolve the dispute. Prior to initiation of outside dispute resolution or termination of the Agreement for a material breach, each Party shall escalate such issue to the Chief Executive Officer of Licensee and Dean of Medicine for Penn and such parties will engage in good faith discussions with regard to the applicable dispute within fifteen (15) days. If the Parties are unable to resolve such dispute amicably through good faith discussion and such escalation within thirty (30) days, then either Party may submit to the exclusive jurisdiction of, and venue in, the state and Federal courts located in the Eastern District of Pennsylvania.

13.10 **Notices and Deliveries**. Any notice, request, approval or consent required or permitted to be given under this Agreement shall be in writing and directed to a Party at its address or facsimile number shown below or such other address or facsimile number as such Party shall have last given by notice to the other Party. A notice will be deemed received: if delivered personally, on the date of delivery; if mailed, five (5) days after deposit in the United States mail; if sent via courier, one (1) business day after deposit with the courier service; or if sent via facsimile, upon receipt of confirmation of transmission provided that a confirming copy of such notice is sent by certified mail, postage prepaid, return receipt requested.

For Penn

Penn Center for Innovation University of Pennsylvania 3600 Civic Center Blvd.
9th Floor Philadelphia, PA 19104
Attention: Managing Director

with a copy to:

University of Pennsylvania Office of General Counsel 2929 Walnut St., Suite 400
Philadelphia, PA 19104 Attention: General Counsel

For Licensee:

Amicus Therapeutics, Inc.
1 Cedar Brook Drive
Cranbury, NJ 08512
Attention: General Counsel and Corporate Secretary

with a copy to:

Wilson Sonsini Goodrich & Rosati
12235 El Camino Real
San Diego CA 92130
Attention: Miranda Biven

13.11 **Waiver**. A waiver by either Party of any of the terms and conditions of this Agreement in any instance shall not be deemed or construed to be a waiver of such term or condition for the future, or of any other term or condition hereof. All rights, remedies, undertakings, obligations and agreements contained in this Agreement shall be cumulative and none of them shall be in limitation of any other remedy, right, undertaking, obligation or agreement of either Party.

13.12 **Severability**. When possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under law, but if any provision of this Agreement is held to be prohibited by or invalid under law, such provision will be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of this Agreement. The Parties shall make a good faith effort to replace the invalid or unenforceable provision with a valid one which in its economic effect is most consistent with the invalid or unenforceable provision.

13.13 **Interpretation**. The words "include," "includes" and "including" shall be deemed to be followed by the phrase "without limitation." All references herein to Articles, Sections, Schedules and Exhibits shall be deemed references to Articles and Sections of, Schedules and Exhibits to, this Agreement unless the context shall otherwise require. Except as otherwise expressly provided herein, all terms of an accounting or financial nature shall be construed in accordance with GAAP, as in effect from time to time. Unless the context otherwise requires, countries shall include territories. References to any specific Law or article, section or other division thereof, shall be deemed to include the then-current amendments or any replacement Law thereto.

13.14 **Counterparts**. This Agreement may be executed in counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or a portable document format (PDF) copy of this Agreement, including the signature pages, will be deemed an original.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the New Effective Date.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA	**AMICUS THERAPEUTICS, INC.**
By:	By:
Name: John S. Swartley, PhD	Name: John Crowley
Title: Associate Vice Provost for Research and Managing Director, Penn Center for Innovation	Title: Chairman and CEO

Read and Acknowledged:

By:
Name: Dr. James M. Wilson
Title: Director, Gene Therapy Program

Exhibit A
Patent Rights

[***]

Exhibit B
Research Plans

[***]

Exhibit C
Research Program Budget

[***]

Exhibit D

[***]

[***]

Exhibit E
Amicus Technology

[***]

Exhibit F
Discovery Plan

[***]

Exhibit G
Funded Discovery Patent Rights Exceptions

[***]

Exhibit H
Excluded Penn IP

[***]

Schedule 1.55

LSD Indications Obligated to Third Parties

[***]

Schedule 2.8
[***]

[***]

Schedule 3.2.4
Third Party Collaborations or Funding for Discovery Program

[***]

Exhibit 31.1

CERTIFICATIONS PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY PRINCIPAL EXECUTIVE OFFICER

I, John F. Crowley, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, 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 preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2019

/s/ John F. Crowley
John F. Crowley
Chairman and Chief Executive Officer

Exhibit 31.2

CERTIFICATIONS PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002
CERTIFICATION BY PRINCIPAL FINANCIAL OFFICER

I, Daphne Quimi, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Amicus Therapeutics, 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 preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2019

/s/ Daphne Quimi
Daphne Quimi
Chief Financial Officer

Exhibit 32.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Each of the undersigned hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, in his capacity as an officer of Amicus Therapeutics, Inc. (the "Company"), that, to his knowledge, the Quarterly Report of the Company on Form 10-Q for the period ended June 30, 2019, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 (15 U.S.C. 78m or 78o(d)) and that the information contained in such report fairly presents, in all material respects, the financial condition and results of operations of the Company. This written statement is being furnished to the Securities and Exchange Commission as an exhibit to such Form 10-Q. A signed original of this statement has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Date: August 8, 2019

By: /s/ John F. Crowley

John F. Crowley

Chairman and Chief Executive Officer

Date: August 8, 2019

By: /s/ Daphne Quimi

Daphne Quimi

Chief Financial Officer