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**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 September 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: 001-36294

**uniQure N.V.**

(Exact name of Registrant as specified in its charter)

**The Netherlands**

**Not applicable**

(State or other jurisdiction of incorporation or organization)

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

**Paasheuvelweg 25a,**

**1105 BP Amsterdam, The Netherlands**

(Address of principal executive offices) (Zip Code)

**+31-20-240-6000**

(Registrant's telephone number, including area code)

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

<u>Title of each class:</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Ordinary Shares	QURE	The Nasdaq Global Select Market

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

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

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

Large accelerated filer ☒

Non-accelerated filer ☐

Accelerated filer ☐

Smaller reporting company ☐

Emerging growth company ☐

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

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

As of October 23, 2019, the registrant had 43,619,926 ordinary shares, par value €0.05, outstanding.

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## **SPECIAL CAUTIONARY NOTICE REGARDING FORWARD-LOOKING STATEMENTS**

This Quarterly Report on Form 10-Q contains “forward-looking statements” as defined under federal securities laws. Forward-looking statements are based on our current expectations of future events and many of these statements can be identified using terminology such as “believes,” “expects,” “anticipates,” “plans,” “may,” “will,” “projects,” “continues,” “estimates,” “potential,” “opportunity” and similar expressions. These forward-looking statements may be found in Part II, Item 1A “Risk Factors,” Part I, Item 2 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other sections of this Quarterly Report on Form 10-Q.

Forward-looking statements are only predictions based on management’s current views and assumptions and involve risks and uncertainties, and actual results could differ materially from those projected or implied. The most significant factors known to us that could materially adversely affect our business, operations, industry, financial position or future financial performance include those discussed in Part II, Item 1A “Risk Factors,” as well as those discussed in Part I, Item 2 “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q, as well as other factors which may be identified from time to time in our other filings with the Securities and Exchange Commission (“SEC”), including our most recent [Annual Report on Form 10-K filed with the SEC on February 28, 2019](#), or in the documents where such forward-looking statements appear. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. Our actual results or experience could differ significantly from those anticipated in the forward-looking statements and from historical results, due to the risks and uncertainties described in this Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, and in our [Annual Report on Form 10-K for the year ended December 31, 2018](#), including in “Part I, Item 1A. Risk Factors,” as well as others that we may consider immaterial or do not anticipate at this time. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future or may file or furnish with the SEC. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or to reflect the occurrence of unanticipated events. All forward-looking statements attributable to us are expressly qualified in their entirety by these cautionary statements.

In addition, with respect to all of our forward-looking statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

## Part I – FINANCIAL INFORMATION

### Item 1. Financial Statements

#### uniQure N.V.

#### UNAUDITED CONSOLIDATED BALANCE SHEETS

	September 30, 2019	December 31, 2018
	(in thousands, except share and per share amounts)	
<b>Current assets</b>		
Cash and cash equivalents	\$ 403,163	\$ 234,898
Accounts receivable and accrued income from related party	101	233
Prepaid expenses	3,820	1,116
Other current assets	558	329
<b>Total current assets</b>	<b>407,642</b>	<b>236,576</b>
<b>Non-current assets</b>		
Property, plant and equipment, net of accumulated depreciation of \$26.9 million as of September 30, 2019, and \$22.9 million as of December 31, 2018 respectively.	28,278	29,179
Operating lease right-of-use assets	26,852	—
Intangible assets, net	5,424	5,201
Goodwill	482	506
Restricted cash	2,914	2,444
<b>Total non-current assets</b>	<b>63,950</b>	<b>37,330</b>
<b>Total assets</b>	<b>\$ 471,592</b>	<b>\$ 273,906</b>
<b>Current liabilities</b>		
Accounts payable	\$ 3,782	\$ 3,792
Accrued expenses and other current liabilities	12,702	8,232
Current portion of operating lease liabilities	4,436	—
Current portion of deferred rent	—	311
Current portion of deferred revenue	7,527	7,634
<b>Total current liabilities</b>	<b>28,447</b>	<b>19,969</b>
<b>Non-current liabilities</b>		
Long-term debt	35,928	35,471
Operating lease liabilities, net of current portion	31,242	—
Deferred rent, net of current portion (see note 2.5)	—	8,761
Deferred revenue, net of current portion	23,882	28,861
Derivative financial instruments related party	1,354	803
Other non-current liabilities	507	435
<b>Total non-current liabilities</b>	<b>92,913</b>	<b>74,331</b>
<b>Total liabilities</b>	<b>121,360</b>	<b>94,300</b>
<b>Commitments and contingencies</b>		
<b>Shareholders' equity</b>		
Ordinary shares, €0.05 par value: 60,000,000 shares authorized at September 30, 2019 and December 31, 2018 and 43,612,417 and 37,351,653 ordinary shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively.	2,646	2,299
Additional paid-in-capital	980,549	720,072
Accumulated other comprehensive loss	(14,682)	(7,259)
Accumulated deficit	(618,281)	(535,506)
<b>Total shareholders' equity</b>	<b>350,232</b>	<b>179,606</b>
<b>Total liabilities and shareholders' equity</b>	<b>\$ 471,592</b>	<b>\$ 273,906</b>

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND  
COMPREHENSIVE LOSS

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
	(in thousands, except share and per share amounts)		(in thousands, except share and per share amounts)	
License revenues from related party	842	2,518	3,507	7,092
Collaboration revenues from related party	204	630	1,149	2,584
<b>Total revenues</b>	<b>1,046</b>	<b>3,148</b>	<b>4,656</b>	<b>9,676</b>
<b>Operating expenses:</b>				
Research and development expenses	(23,554)	(20,541)	(68,245)	(56,092)
Selling, general and administrative expenses	(8,929)	(5,898)	(24,866)	(18,095)
<b>Total operating expenses</b>	<b>(32,483)</b>	<b>(26,439)</b>	<b>(93,111)</b>	<b>(74,187)</b>
Other income	453	557	1,332	1,737
Other expense	(342)	(490)	(1,038)	(1,252)
<b>Loss from operations</b>	<b>(31,326)</b>	<b>(23,224)</b>	<b>(88,161)</b>	<b>(64,026)</b>
Interest income	868	949	2,038	1,785
Interest expense	(960)	(515)	(2,854)	(1,496)
Foreign currency gains, net	6,041	455	7,063	2,888
Other non-operating gains / (losses), net	1,773	268	(861)	(330)
<b>Loss before income tax expense</b>	<b>(23,604)</b>	<b>(22,067)</b>	<b>(82,775)</b>	<b>(61,179)</b>
Income tax benefit / (expense)	—	32	—	(237)
<b>Net loss</b>	<b>\$ (23,604)</b>	<b>\$ (22,035)</b>	<b>\$ (82,775)</b>	<b>\$ (61,416)</b>
<b>Other comprehensive loss, net of income tax:</b>				
Foreign currency translation adjustments net of tax impact of nil and \$0.0 million for the three months ended September 30, 2019 and 2018, respectively, and nil and \$(0.2) million for the nine months ended September 30, 2019 and 2018, respectively.	(6,164)	(799)	(7,423)	(3,491)
<b>Total comprehensive loss</b>	<b>\$ (29,768)</b>	<b>\$ (22,834)</b>	<b>\$ (90,198)</b>	<b>\$ (64,907)</b>
Basic and diluted net loss per ordinary share	\$ (0.58)	(0.59)	\$ (2.14)	\$ (1.75)
Weighted average shares used in computing basic and diluted net loss per ordinary share	40,738,938	37,247,193	38,757,898	35,074,531

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

uniQure N.V.

UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY  
FOR THE THREE-MONTH PERIOD ENDED SEPTEMBER 30

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive (loss)/income	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
<b>Balance at June 30, 2018</b>	<b>37,126,741</b>	<b>\$ 2,288</b>	<b>\$ 712,399</b>	<b>\$ (4,688)</b>	<b>\$ (491,583)</b>	<b>\$ 218,416</b>
Loss for the period	—	—	—	—	(22,035)	(22,035)
Other comprehensive loss	—	—	—	(799)	—	(799)
Exercise of share options	148,458	6	1,644	—	—	1,650
Share-based compensation expense	—	—	2,620	—	—	2,620
<b>Balance at September 30, 2018</b>	<b>37,275,199</b>	<b>\$ 2,294</b>	<b>\$ 716,663</b>	<b>\$ (5,487)</b>	<b>\$ (513,618)</b>	<b>\$ 199,852</b>
<b>Balance at June 30, 2019</b>	<b>37,839,833</b>	<b>\$ 2,327</b>	<b>\$ 732,924</b>	<b>\$ (8,518)</b>	<b>\$ (594,677)</b>	<b>\$ 132,056</b>
Loss for the period	—	—	—	—	(23,604)	(23,604)
Other comprehensive loss	—	—	—	(6,164)	—	(6,164)
Follow-on public offering	5,625,000	311	242,480	—	—	242,791
Exercise of share options	119,225	6	1,172	—	—	1,178
Restricted and performance share units distributed during the period	26,211	2	(2)	—	—	—
Share-based compensation expense	—	—	3,876	—	—	3,876
Issuance of ordinary shares relating to employee stock purchase plan	2,148	—	99	—	—	99
<b>Balance at September 30, 2019</b>	<b>43,612,417</b>	<b>\$ 2,646</b>	<b>\$ 980,549</b>	<b>\$ (14,682)</b>	<b>\$ (618,281)</b>	<b>\$ 350,232</b>

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

**uniQure N.V.**

**UNAUDITED CONSOLIDATED STATEMENTS OF SHAREHOLDERS' EQUITY  
FOR THE NINE-MONTH PERIOD ENDED SEPTEMBER 30**

	Ordinary shares		Additional paid-in capital	Accumulated other comprehensive (loss)/income	Accumulated deficit	Total shareholders' equity
	No. of shares	Amount				
	(in thousands, except share and per share amounts)					
<b>Balance at December 31, 2017</b>	<b>31,339,040</b>	<b>\$ 1,947</b>	<b>\$ 566,530</b>	<b>\$ (3,800)</b>	<b>\$ (475,318)</b>	<b>\$ 89,359</b>
Cumulative effect of retroactive implementation of ASC 606						
Revenue recognition	—	—	—	1,802	23,116	24,918
Loss for the period	—	—	—	—	(61,416)	(61,416)
Other comprehensive loss	—	—	—	(3,489)	—	(3,489)
Follow-on public offering	5,175,000	309	138,182	—		138,491
Exercise of share options	416,211	18	4,619	—	—	4,637
Restricted and performance share units distributed during the period	344,948	20	(20)	—	—	—
Share-based compensation expense	—	—	7,352	—	—	7,352
<b>Balance at September 30, 2018</b>	<b>37,275,199</b>	<b>\$ 2,294</b>	<b>\$ 716,663</b>	<b>\$ (5,487)</b>	<b>\$ (513,618)</b>	<b>\$ 199,852</b>
<b>Balance at December 31, 2018</b>	<b>37,351,653</b>	<b>\$ 2,299</b>	<b>\$ 720,072</b>	<b>\$ (7,259)</b>	<b>\$ (535,506)</b>	<b>\$ 179,606</b>
Loss for the period	—	—	—	—	(82,775)	(82,775)
Other comprehensive loss	—	—	—	(7,423)	—	(7,423)
Follow-on public offering	5,625,000	311	242,480	—	—	242,791
Hercules warrants exercise	37,175	2	1,271	—	—	1,273
Exercise of share options	355,879	20	3,714	—	—	3,734
Restricted and performance share units distributed during the period	235,692	14	(14)	—	—	—
Share-based compensation expense	—	—	12,762	—	—	12,762
Issuance of ordinary shares relating to employee stock purchase plan	7,018	—	264	—	—	264
<b>Balance at September 30, 2019</b>	<b>43,612,417</b>	<b>\$ 2,646</b>	<b>\$ 980,549</b>	<b>\$ (14,682)</b>	<b>\$ (618,281)</b>	<b>\$ 350,232</b>

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

**uniQure N.V.**

**UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS**

	<b>Nine months ended September 30,</b>	
	<b>2019</b>	<b>2018</b>
	<b>(in thousands)</b>	
<b>Cash flows from operating activities</b>		
Net loss	\$ (82,775)	\$ (61,416)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation, amortization and impairment losses	4,932	10,447
Share-based compensation expense	12,762	7,352
Change in fair value of derivative financial instruments and contingent consideration	862	(3,516)
Unrealized foreign exchange gains	(6,692)	(4,210)
Change in deferred taxes	-	237
Change in lease incentives	-	(284)
Changes in operating assets and liabilities:		
Accounts receivable and accrued income, prepaid expenses and other current assets	(2,879)	768
Accounts payable	(418)	1,289
Accrued expenses, other liabilities and operating leases	4,934	(1,559)
Deferred revenue	(3,403)	(7,142)
Net cash used in operating activities	(72,677)	(58,034)
<b>Cash flows from investing activities</b>		
Purchases of intangible assets	(996)	(1,698)
Purchases of property, plant and equipment	(3,688)	(1,805)
Net cash used in investing activities	(4,684)	(3,503)
<b>Cash flows from financing activities</b>		
Proceeds from issuance of shares related to employee stock option and purchase plans	3,998	4,637
Proceeds from public offering of shares, net of issuance costs	243,013	138,480
Proceeds from exercise of warrants	500	-
Net cash generated from financing activities	247,511	143,117
Currency effect cash, cash equivalents and restricted cash	(1,415)	(1,431)
Net increase in cash, cash equivalents and restricted cash	168,735	80,149
Cash, cash equivalents and restricted cash at beginning of period	237,342	161,851
<b>Cash, cash equivalents and restricted cash at the end of period</b>	<b>\$ 406,077</b>	<b>\$ 242,000</b>
Cash and cash equivalents	\$ 403,163	\$ 239,546
Restricted cash related to leasehold and other deposits	\$ 2,914	\$ 2,454
<b>Total cash, cash equivalents and restricted cash</b>	<b>\$ 406,077</b>	<b>\$ 242,000</b>
<b>Supplemental cash flow disclosures:</b>		
Cash paid for interest	\$ (2,330)	\$ (1,498)
Non-cash increases in accounts payables related to purchase of intangible assets and property, plant and equipment	\$ 583	\$ 12

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



## **1 General business information**

uniQure (the “Company”) was incorporated on January 9, 2012 as a private company with limited liability (*besloten vennootschap met beperkte aansprakelijkheid*) under the laws of the Netherlands. The Company is a leader in the field of gene therapy and seeks to deliver to patients suffering from rare and other devastating diseases single treatments with potentially curative results. The Company’s business was founded in 1998 and was initially operated through its predecessor company, Amsterdam Molecular Therapeutics (AMT) Holding N.V. (“AMT”). In 2012, AMT undertook a corporate reorganization, pursuant to which uniQure B.V. acquired the entire business and assets of AMT and completed a share-for-share exchange with the shareholders of AMT. Effective February 10, 2014, in connection with its initial public offering, the Company converted into a public company with limited liability (*naamloze vennootschap*) and changed its legal name from uniQure B.V. to uniQure N.V.

The Company is registered in the trade register of the Chamber of Commerce (*Kamer van Koophandel*) in Amsterdam, the Netherlands under number 54385229. The Company’s headquarters are in Amsterdam, the Netherlands, and its registered office is located at Paasheuvelweg 25a, Amsterdam 1105 BP, the Netherlands and its telephone number is +31 20 240 6000.

The Company’s ordinary shares are listed on the NASDAQ Global Select Market and trades under the symbol “QURE”.

## **2 Summary of significant accounting policies**

### **2.1 Basis of preparation**

The Company prepared these unaudited consolidated financial statements in compliance with generally accepted accounting principles in the United States of America (“U.S. GAAP”) and applicable rules and regulations of the SEC regarding interim financial reporting. Any reference in these notes to applicable guidance is meant to refer to authoritative U.S. GAAP as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”).

The unaudited consolidated financial statements are presented in U.S. dollars, except where otherwise indicated. Transactions denominated in currencies other than U.S. dollars are presented in the transaction currency with the U.S. dollar amount included in parenthesis, converted at the foreign exchange rate as of the transaction date.

### **2.2 Unaudited interim financial information**

The interim financial statements and related disclosures are unaudited, have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for a fair statement of the financial position, results of operations and changes in financial position for the period presented.

Certain information and footnote disclosures normally included in annual financial statements prepared in accordance with U.S. GAAP have been omitted. The results of operations for the nine months ended September 30, 2019, are not necessarily indicative of the results to be expected for the full year ending December 31, 2019, or for any other future year or interim period. The accompanying financial statements should be read in conjunction with the audited financial statements and the related notes thereto included in the Company’s [Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019](#).

### **2.3 Use of estimates**

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

## 2.4 Accounting policies

The principal accounting policies applied in the preparation of these unaudited consolidated financial statements are described in the Company's audited financial statements as of and for the year ended December 31, 2018, and the notes thereto, which are included in the Company's [Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019](#). There have been no material changes in the Company's significant accounting policies during the nine months ended September 30, 2019, other than the adoption of accounting pronouncements discussed below.

## 2.5 Recent accounting pronouncements

### *Recently Adopted Accounting Pronouncements*

#### ASC 842 – Leases

In February 2016, the FASB issued ASU 2016-02, "Leases (Topic 842)". In July 2018, the FASB issued ASU No. 2018-10, "Codification Improvements to Topic 842, Leases" (ASU 2018-10), which provides narrow amendments to clarify how to apply certain aspects of the new lease standard, and ASU No. 2018-11, "Leases (Topic 842) – Target Improvements" (ASU 2018-11), which address implementation issues related to the new lease standard. The standard is effective for interim and annual reporting periods beginning after December 15, 2018. Under the new standard, lessees are required to recognize the right-of-use assets and lease liabilities that arise from operating leases on the Consolidated balance sheet. The Company adopted the standard using the modified retrospective approach with an effective date as of the beginning of the Company's fiscal year, January 1, 2019, to operating leases that existed on that date. Prior year comparative financial information was not recast under the new standard and continues to be presented under ASC 840. The Company elected to utilize the package of practical expedients available for expired or existing contracts which allowed the Company to carryforward historical assessments of (1) whether contracts are or contain leases, (2) lease classification, and (3) initial direct costs. The Company performed an assessment and identified the lease facilities as material leases to be accounted for under ASC 842 as of January 1, 2019. The Company elected to implement ASC 842 by applying the modified retrospective approach, which allows the Company to restrict the application of the new guidance to operating leases as of January 1, 2019. The impact of implementing ASC 842 is summarized below:

- Recognized a \$19.0 million operating right-of-use asset and a \$28.1 million operating lease liability in relation to the facilities leased at the Amsterdam and Lexington sites in the Consolidated balance sheet as of January 1, 2019;
- Presented deferred rent of \$9.1 million as of December 31, 2018, as a reduction of the right-of-use asset as from January 1, 2019 onwards in the Consolidated balance sheet and as a change within operating cash flows within accrued expense, other liabilities and operating leases;

The Company measured the lease liability at the present value of the future lease payments as of January 1, 2019. The Company used an incremental borrowing rate to discount the lease payments. The Company derived the discount rate, adjusted for differences in the term and payment patterns, from the Company's loan from Hercules Capital, which was refinanced immediately prior to the January 1, 2019 adoption date in December 2018. The right-of-use asset is valued at the amount of the lease liability reduced by the remaining December 31, 2018 balance of lease incentives received. The lease liability is subsequently measured at the present value of the future lease payments as of the reporting date with a corresponding adjustment to the right-to-use asset. Absent a lease modification, the Company will continue to utilize the January 1, 2019, incremental borrowing rate.

The Company will continue to recognize lease cost on a straight-line basis and will continue to present these costs as operating expenses within the Consolidated statements of operations and comprehensive loss. The Company will continue to present lease payments and landlord incentive payments within cash flows from operations within the Consolidated statements of cash flows.

The financial results for the three and nine months ended September 30, 2019, are presented under the new standard, while the comparative periods presented are not adjusted and continue to be reported in accordance with the Company's historical accounting policy.

Refer to note 5, "Right-of-use asset and lease liabilities" for further information.

In March 2019, the FASB issued ASU 2019-01, “Codification Improvements” to Leases (Topic 842). This pronouncement did not have a material impact on the Company.

#### ***Recent Accounting Pronouncements Not Yet Effective***

There have been no new accounting pronouncements or changes to accounting pronouncements during the nine months ended September 30, 2019, as compared to the recent accounting pronouncements described in Note 2.3.23 of the Company’s [Annual Report on Form 10-K for the year ended December 31, 2018](#), which could be expected to materially impact the Company’s unaudited consolidated financial statements.

### **3 Collaboration arrangements and concentration of credit risk**

#### ***BMS collaboration***

In May 2015, the Company entered into a collaboration and license agreement (the “BMS CLA”) and various related agreements with Bristol-Myers Squibb Company (“BMS”) that provide BMS with exclusive access to the Company’s gene therapy technology platform for the research, development and commercialization of therapeutics aimed at multiple targets in cardiovascular and other diseases (“Collaboration Targets”). During the initial research term of the BMS CLA, the Company supported BMS in discovery, non-clinical, analytical and process development efforts in respect of the Collaboration Targets. For any Collaboration Targets that may be advanced, the Company will be responsible for manufacturing of clinical and commercial supplies using the Company’s vector technologies and industrial, proprietary insect-cell based manufacturing platform. BMS reimbursed the Company for all its research and development costs in support of the collaboration during the initial research term, and will lead development, regulatory and commercial activities for any Collaboration Targets that may be advanced. The BMS CLA provides that the companies may collaborate on up to ten Collaboration Targets in total. The Company has agreed to certain restrictions on its ability to work independently of the collaboration, either directly or indirectly through any affiliate or third party, on certain programs that would be competitive with the collaboration programs.

BMS initially designated four Collaboration Targets, including S100A1 for congestive heart failure (“AMT-126”). In October 2018, the Company and BMS completed a heart function proof-of-concept study of AMT-126 in a pre-clinical, diseased animal model. The study demonstrated deoxyribonucleic acid delivery and expression of S100A1 in the myocardium, thereby validating the Company’s vector delivery platform in the animal model. The data did not show a benefit on heart function at six months and, consequently, the Joint Steering Committee for the collaboration decided to discontinue work on S100A1. The Company impaired a \$5.4 million acquired research and development asset associated with the program and released a contingent liability of \$3.8 million related to the acquisition of the asset to income in the three-month period ended September 30, 2018. In April 2019, BMS designated a new cardiovascular Collaboration Target to replace S100A1. As a result, BMS has designated a total of four Collaboration Targets.

The initial four-year research term under the collaboration terminated on May 21, 2019. In February 2019, BMS requested a one-year extension of the research term. In April 2019, following an assessment of the progress of this collaboration and the Company’s expanding proprietary programs, the Company notified BMS that the Company did not intend to agree to an extension of the research term but rather preferred to restructure the collaboration to reduce or eliminate certain of the Company’s obligations under it.

Accordingly, the Company is currently in discussions with BMS potentially to restructure the BMS CLA and other related agreements. It is currently uncertain whether a change to the BMS CLA will be agreed and, if agreed, what the specific terms of any such change may be. As a consequence, the Company has not taken into account the impact of such change, if any, on the timing of recognition of the prepaid License Revenue. The Company will account for potential changes in the timing of recognition of the prepaid License Revenue if and when the BMS CLA and other related agreements have been restructured. The final resolution of these discussions may result in material changes to the Company’s collaboration with BMS.

The Company evaluated the BMS CLA and determined that its performance obligations are as follows:

- (i) Providing access to its technology and know-how in the field of gene therapy as well as actively contributing to the selection of Collaboration Targets, the collaboration as a whole, the development during the pre-clinical and the clinical phase through participating in joint steering committee and other governing bodies (“License Revenue”);

- (ii) Providing pre-clinical Collaboration Target specific, non-clinical, analytical and process development services during the initial research term, which ended on May 21, 2019 (“Collaboration Revenue”); and
- (iii) Providing clinical and commercial manufacturing services for Collaboration Targets (“Manufacturing Revenue”). To date the Company has not generated any Manufacturing Revenue.

During the aforementioned discussions with BMS potentially to restructure the BMS CLA and other related agreements, which may be terminated by the Company or BMS at any time, the Company has agreed to continue providing support of the pre-clinical Collaboration Targets, and any related costs will be reimbursed by BMS.

Amounts owed by BMS in relation to Collaboration Revenue are as follows:

	September 30, 2019	December 31, 2018
	(in thousands)	
Bristol Myers Squibb	\$ 101	\$ 233
<b>Total</b>	<b>\$ 101</b>	<b>\$ 233</b>

#### *License Revenue*

The Company recognized \$0.8 million and \$3.5 million of License Revenue for the three and nine months ended September 30, 2019, respectively, compared to \$2.5 million and \$7.1 million during the same periods in 2018 in relation to a \$60.1 million upfront payment recorded on May 21, 2015, as well as \$15.0 million received in relation to the designation of the second, third and fourth Collaboration Targets in August 2015.

The Company would be entitled to an aggregate \$16.5 million in target designation payments upon the selection of the fifth to tenth Collaboration Targets. The Company would also be eligible to receive research, development and regulatory milestone payments of up to \$254.0 million for a lead Collaboration Target and up to \$217.0 million for each of the other selected Collaboration Targets, if defined milestones are achieved. The Company would include the variable consideration related to the selection of the fifth to tenth Collaboration Targets, or any of the milestones, in the transaction price once it is considered probable that including these payments in the transaction price would not result in the reversal of cumulative revenue recognized. The Company would recognize significant amounts of License Revenue for services performed in prior periods if and when the Company considers this probable. Due to the significant uncertainty surrounding the development of gene-therapy product candidates and the dependence on BMS’s performance and decisions, the Company does not currently consider this probable.

Additionally, the Company is eligible to receive net sales-based milestone payments and tiered mid-single to low double-digit royalties on product sales. The royalty term is determined on a licensed-product-by-licensed-product and country-by-country basis and begins on the first commercial sale of a licensed product in a country and ends on the expiration of the last to expire of specified patents or regulatory exclusivity covering such licensed product in such country or, with a customary royalty reduction, ten years after the first commercial sale if there is no such exclusivity. These revenues will be recognized when performance obligations are satisfied.

The Company recognizes License Revenue over the expected performance period based on its measure of progress towards the completion of certain activities related to its services. The Company determines such progress by comparing activities performed at the end of each reporting period with total activities expected to be performed. The Company estimates total expected activities using a number of unobservable inputs, such as the probability of BMS designating additional targets, the probability of successfully completing each phase and estimated time required to provide services during the various development stages. If available, the Company uses product candidate-specific research and development plans. Alternatively, the Company assumes that completion of the pre-clinical phase requires an average of four years and that clinical development and commercial launch on average require 8.5 years.

The estimation of total services at the end of each reporting period involves considerable judgement. The estimated number of Collaboration Targets that BMS will pursue significantly impacts the amount of License Revenue the Company recognizes. For example, if the Company would increase the probability of all additional Collaboration Targets being designated by 10% then the revenue for the nine months ended September 30, 2019 would have decreased by approximately \$1.8 million, as the Company would be required to render more services in relation to the consideration received.

#### 4 Fair value measurement

The Company measures certain assets and liabilities at fair value, either upon initial recognition or for subsequent accounting or reporting. U.S. GAAP requires disclosure of methodologies used in determining the reported fair values, and establishes a hierarchy of inputs used when available. The three levels of the fair value hierarchy are described below:

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

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The carrying amount of cash and cash equivalents, accrued income from related parties, prepaid expenses, other assets, accounts payable, accrued expenses and other current liabilities reflected in the Consolidated balance sheets approximate their fair values due to their short-term maturities.

The following table sets forth the Company's assets and liabilities that are required to be measured at fair value on a recurring basis as of September 30, 2019, and December 31, 2018:

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total	Classification in Consolidated balance sheets
(in thousands)					
<b>At December 31, 2018</b>					
Assets:					
Cash, cash equivalents and restricted cash	\$ 237,342	\$ —	\$ —	\$ 237,342	Cash and cash equivalents; restricted cash
Total assets	<u>\$ 237,342</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 237,342</u>	
Liabilities:					
Derivative financial instruments - debt	\$ —	\$ —	\$ 572	\$ 572	Accrued expenses and other current liabilities
Derivative financial instruments - related party	—	—	803	803	
Total liabilities	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 1,375</u>	<u>\$ 1,375</u>	
<b>At September 30, 2019</b>					
Assets:					
Cash, cash equivalents and restricted cash	\$ 406,077	\$ —	\$ —	\$ 406,077	Cash and cash equivalents; restricted cash
Total assets	<u>\$ 406,077</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 406,077</u>	
Liabilities:					
Derivative financial instruments - related party	\$ —	\$ —	\$ 1,354	\$ 1,354	
Total liabilities	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 1,354</u>	<u>\$ 1,354</u>	

Changes in Level 3 items during the nine months ended September 30, 2019, are as follows

	Derivative financial instruments (in thousands)
<b>Balance at December 31, 2018</b>	<b>\$ 1,375</b>
Net losses recognized in profit or loss	862
Exercise of warrants	(770)
Currency translation effects	(113)
<b>Balance at September 30, 2019</b>	<b>\$ 1,354</b>

### *Derivative financial instruments*

The Company issued derivative financial instruments related to its collaboration with BMS and in relation to the issuance of the Hercules Technology Growth Corp. (“Hercules”) loan facility. The Hercules warrants were exercised as of February 1, 2019. The Company issued 37,175 ordinary shares at \$34.25 following the exercise of all Hercules warrants and receipt of \$0.5 million from Hercules.

The fair value of the BMS derivative financial instruments (“BMS warrants”) as of September 30, 2019, was \$1.4 million compared to a fair value of both the BMS and Hercules derivative financial instruments of \$1.4 million as of December 31, 2018. Changes in the fair value of the BMS warrants are primarily impacted by changes in the Company’s share price, whereby an increase in share price generally results in an increase of the fair value. These BMS warrants are described in more detail below.

### BMS warrants

Pursuant to the BMS CLA, the Company granted BMS two warrants:

- A warrant allowing BMS to purchase a specific number of the Company’s ordinary shares such that its ownership will equal 14.9% immediately after such purchase. The warrant can be exercised on the later of (i) the date on which the Company receives from BMS the Target Designation Fees (as defined in the BMS CLA) associated with the first six new targets (a total of seven Collaboration Targets); and (ii) the date on which BMS designates the sixth new target (the seventh Collaboration Target).
- A warrant allowing BMS to purchase a specific number of the Company’s ordinary shares such that its ownership will equal 19.9% immediately after such purchase. The warrant can be exercised on the later of (i) the date on which the Company receives from BMS the Target Designation Fees associated with the first nine new targets (a total of ten Collaboration Targets); and (ii) the date on which BMS designates the ninth new target (the tenth Collaboration Target).

Pursuant to the terms of the BMS CLA, the exercise price in respect of each warrant is equal to the greater of (i) the product of (A) \$33.84, multiplied by (B) a compounded annual growth rate of 10% (or approximately \$51.15 as of September 30, 2019) and (ii) the product of (A) 1.10 multiplied by (B) the VWAP for the 20 trading days ending on the date that is five trading days prior to the date of a notice of exercise delivered by BMS.

As of September 30, 2019, BMS had designated a total of four Collaboration Targets, and as such, the warrants were not exercisable. The Company estimated the exercise of warrants to occur within two and four years after the balance sheet date.

The Company conducted a sensitivity analysis to assess the impact on changes in assumptions on the fair value. Specifically, the Company examined the impact on the fair market value of the warrants by increasing the volatility by 10% to 82.5%. A further sensitivity analysis was performed assuming the warrants would be exercised a year later than currently estimated. The table below illustrates the impact on the fair market valuation associated with these changes in assumptions as of September 30, 2019.

	<b>Total warrants</b>
	<b>(in thousands)</b>
Base case	\$ 1,354
Increase volatility by 10% to 82.5%	375
Extend exercise dates by one year	41

## 5 Right-of-use asset and lease liabilities

The Company adopted ASU 2016-02 “Leases (Topic 842)” as well as ASU 2018-10 and ASU 2018-11, which both relate to improvements to ASC 842. The Company adopted the standard using the modified retrospective approach with an effective date as of the beginning of the Company’s fiscal year, January 1, 2019. The standard requires the balance sheet recognition for leases. Prior year interim periods were not recast under the new standard and therefore, those amounts are not presented below. The Company elected to utilize practical expedients available for expired or existing contracts which allowed the Company to carryforward historical assessments of (1) whether contracts are or contain leases, (2) lease classification, and (3) initial direct costs.

The Company’s most significant leases relate to office and laboratory space under the following operating lease agreements:

### *Lexington, Massachusetts / United States*

In July 2013, the Company entered into a lease for a facility in Lexington, Massachusetts, United States. The term of the lease commenced in November 2013, was set for 10 years starting from the 2014 rent commencement date and is non-cancellable. Originally, the lease for this facility had a termination date of 2024. In November 2018, the term was expanded by five years to June 2029. The lease continues to be renewable for two subsequent five-year terms. Additionally, the lease was expanded to include an additional 30,655 square feet within the same facility and for the same term. The lease of the expansion space commenced on June 1, 2019.

The contractually fixed annual increases of lease payments through 2029 for both the extension and expansion lease have been included in the lease payments.

### *Amsterdam / The Netherlands*

In March 2016, the Company entered into a 16-year lease for a facility in Amsterdam, the Netherlands, and amended this agreement in June 2016. The lease for this facility terminates in February 2032, with an option to extend in increments of five-year periods. The lease contract includes variable lease payments related to annual increases in payments based on a consumer price index.

On December 1, 2017, the Company entered into an agreement to sub-lease three of the seven floors of its Amsterdam facility for a ten-year term ending on December 31, 2027, with an option for the sub-lessee to extend until December 31, 2031. The fixed lease payments to be received during the remaining term amount to \$8.5 million (EUR 7.8 million) as of September 30, 2019.

### *Operating lease liabilities*

As no implicit rate in relation to the three above facility leases and other equipment leases was readily available, the Company used an incremental borrowing rate to discount the lease payments. The Company derived the discount rates, adjusted for differences in the term and payment patterns, from the Company’s loan from Hercules Capital, which was refinanced in December 2018.

The components of lease cost were as follows:

	Three months ended September 30, 2019	Nine months ended September 30, 2019
	(in thousands)	
Operating lease cost	\$ 1,264	\$ 3,183
Variable lease cost	131	313
Sublease income	(260)	(792)
<b>Total lease cost</b>	<b>\$ 1,135</b>	<b>\$ 2,704</b>

The rent expense for the three and nine months ended September 30, 2018 was \$0.7 million and \$2.1 million, respectively.

The table below presents the lease-related assets and liabilities recorded on the Consolidated balance sheet.

	September 30, 2019 (in thousands)
<b>Assets</b>	
Operating lease right-of-use assets	<b>\$ 26,852</b>
<b>Liabilities</b>	
<b>Current</b>	
Current operating lease liabilities, net of \$0.9 million landlord incentive payments expected to be collected within 12 months	4,436
<b>Non-current</b>	
Non-current operating lease liabilities	31,242
<b>Total lease liabilities</b>	<b>\$ 35,678</b>

*Other information*

The weighted-average remaining lease term as of September 30, 2019 is 10.5 years and the weighted-average discount rate as of this date is 11.32%.

The table below presents supplemental cash flow and non-cash information related to leases.

	Three months ended September 30, 2019 (in thousands)	Nine months ended September 30, 2019 (in thousands)
<b>Cash paid for amounts included in the measurement of lease liabilities</b>		
Operating cash flows for operating leases <sup>1)</sup>	\$ 1,761	\$ 3,777
<b>Right-of-use asset obtained in exchange for lease obligation</b>		
Operating lease <sup>2)</sup>	\$ 368	\$ 9,002

<sup>(1)</sup> The Company has received \$0.5 million of landlord incentive payments as of September 30, 2019, which are not included in the cash paid amounts.)

<sup>(2)</sup> The Company capitalized \$19.0 million of operating right-of-use assets upon adoption of the new lease standard on January 1, 2019 that are not included in the movement for three and the nine months ended September 30, 2019.)



### Undiscounted cash flows

The table below reconciles the undiscounted cash flows as of September 30, 2019, for each of the first five years and the total of the remaining years to the operating lease liabilities recorded on the Consolidated balance sheet as of September 30, 2019.

	Lexington	Amsterdam <sup>(1)</sup>	Other <sup>(1)</sup>	Total
	(in thousands)			
2019 (three months remaining)	\$ 825	\$ 460	\$ 59	\$ 1,344
2020	3,360	1,841	197	5,398
2021	3,455	1,841	138	5,434
2022	3,552	1,841	-	5,393
2023	3,650	1,841	-	5,491
Thereafter	24,891	14,577	-	39,468
<b>Total lease payments</b>	<b>\$ 39,733</b>	<b>\$ 22,401</b>	<b>\$ 394</b>	<b>\$ 62,528</b>
Less: amount of lease payments representing interest payments	(16,580)	(10,247)	(23)	(26,850)
Present value of lease payments	23,153	12,154	371	35,678
Less: current operating lease liabilities	(2,398)	(1,841)	(197)	(4,436)
<b>Non-current operating lease liabilities</b>	<b>\$ 20,755</b>	<b>\$ 10,313</b>	<b>\$ 174</b>	<b>\$ 31,242</b>

<sup>(1)</sup> Payments are due in EUR and have been translated at the foreign exchange rate as of September 30, 2019, of \$1.09 / €1.00)

As of December 31, 2018, aggregate minimum lease payments under the historical accounting standard ASC 840 for the calendar years were as follows:

	Lexington	Amsterdam <sup>(1)</sup>	Total
	(in thousands)		
2019	\$ 2,707	\$ 1,963	\$ 4,670
2020	3,360	1,970	5,330
2021	3,455	1,970	5,425
2022	3,552	1,970	5,522
2023	3,650	1,970	5,620
Thereafter	24,892	16,085	40,977
<b>Total minimum lease payments</b>	<b>\$ 41,616</b>	<b>\$ 25,926</b>	<b>\$ 67,544</b>

<sup>(1)</sup> Payments are due in EUR and have been translated at the foreign exchange rate as of December 31, 2018, of \$1.14 / €1.00)

## 6 Accrued expenses and other current liabilities

Accrued expenses and other current liabilities include the following items:

	September 30, 2019	December 31, 2018
	(in thousands)	
Accruals for services provided by vendors-not yet billed	\$ 6,175	\$ 1,999
Personnel related accruals and liabilities	6,527	5,688
Derivative financial liability warrants (see note 4)	—	545
<b>Total</b>	<b>\$ 12,702</b>	<b>\$ 8,232</b>

## 7 Long-term debt

On June 14, 2013, the Company entered into a venture debt loan facility with Hercules, which was amended and restated on June 26, 2014, and again on May 6, 2016 (“2016 Amended Facility”). The 2016 Amended Facility extended the maturity date from June 30, 2018, to May 1, 2020. The interest rate was adjustable and was the greater of (i) 8.25% or (ii) 8.25% plus the prime rate less 5.25%. Under the 2016 Amended Facility, the interest rate initially was 8.25% per annum. The interest-only payment period was extended by 12 months to November 30, 2018 as a result of raising more than \$50.0 million in equity financing in October 2017.

On December 6, 2018, the Company signed an amendment to the Second Amended and Restated Loan and Security Agreement that both refinanced the existing \$20 million 2016 Amended Facility and provided an additional commitment of \$30 million (of which \$15 million is subject to the discretion of Hercules) (the “2018 Amended Facility”). At signing, the Company drew down an additional \$15 million for a total of \$35 million outstanding. The Company has the right to draw another \$15 million through June 30, 2020 subject to the terms of the 2018 Amended Facility. The 2018 Amended Facility extends the loan’s maturity date from May 1, 2020 until June 1, 2023. The interest-only period was initially extended from November 2018 to January 1, 2021. The interest-only period was further extended to January 1, 2022 as a result of meeting the provision in the 2018 Amended Facility of raising more than \$90.0 million in equity financing. The Company met this provision as a result of the follow-on public offering completed in September 2019. The Company is required to repay the facility in equal monthly installments of principal and interest between the end of the interest-only period and the maturity date. The interest rate continues to be adjustable and is the greater of (i) 8.85% or (ii) 8.85% plus the prime rate less 5.50% per annum.

Under the 2018 Amended Facility, the Company paid a facility fee of 0.50% of the \$35 million outstanding as of signing and owes a back-end fee of 4.95% of the outstanding debt. In addition, in May 2020 the Company owes a back-end fee of 4.85% of \$20 million, which is the amount of debt raised under the 2016 Amended Facility.

The amortized cost (including interest due presented as part of accrued expenses and other current liabilities) of the 2018 Amended Facility was \$36.2 million as September 30, 2019, compared to \$35.7 million as of December 31, 2018, and is recorded net of discount and debt issuance costs. The foreign currency loss on the loan in the three and nine months ended September 30, 2019, was \$1.5 million and \$1.7 million, respectively, compared to a foreign currency loss of \$0.2 million and \$0.7 million during the same periods in 2018.

Interest expense associated with the 2018 Amended Facility during the three and nine months ended September 30, 2019 was \$0.9 million and \$2.8 million, respectively, compared to \$0.5 million and \$1.4 million associated with the 2016 Amended Facility during the same periods in 2018.

As a covenant in the 2018 Amended Facility, the Company has periodic reporting requirements and is required to keep a minimum cash balance deposited in bank accounts in the United States, equivalent to the lesser of 65% of the outstanding balance of principal due or 100% of worldwide cash and cash equivalents. This restriction on cash and cash equivalents only relates to the location of the cash and cash equivalents, and such cash and cash equivalents can be used at the discretion of the Company. In combination with other covenants, the 2018 Amended Facility restricts the Company’s ability to, among other things, incur future indebtedness and obtain additional debt financing, to make investments in securities or in other companies, to transfer assets, to perform certain corporate changes, to make loans to employees, officers and directors, and to make dividend payments and other distributions. The Company secured the facilities by pledging the shares in its subsidiaries, substantially all its receivables, moveable assets as well as the equipment, fixtures, inventory and cash of uniQure Inc.

The 2018 Amended Facility contains provisions that include the occurrence of a material adverse effect, as defined therein, which would entitle Hercules to declare all principal, interest and other amounts owed by the Company immediately due and payable. As of September 30, 2019, the Company was in compliance with all covenants and provisions.

## 8 Shareholders' Equity

On September 10, 2019, the Company completed a follow-on public offering of 4,891,305 ordinary shares at a public offering price of \$46.00 per ordinary share, and on September 13, 2019, the Company completed the sale of an additional 733,695 ordinary shares at a public offering price of \$46.00 per ordinary share pursuant to the exercise by the underwriters of the option to purchase additional ordinary shares, resulting in total gross proceeds to the Company of \$258.8 million. The net proceeds to the Company from this offering were \$242.8 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by the Company. The Company deducted \$0.4 million of expenses incurred related to this offering from additional paid-in capital in the accompanying Consolidated balance sheets. Of the \$0.4 million incurred, \$0.2 million has been paid and reflected within the proceeds from public offering of shares, net of issuance costs within the cash flows from financing activities.

On May 7, 2018, the Company completed a follow-on public offering of 5,175,000 ordinary shares at a public offering price of \$28.50 per ordinary share, resulting in gross proceeds to the Company of \$147.5 million. The net proceeds to the Company from this offering were \$138.5 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by the Company. The Company deducted \$0.2 million of expenses incurred related to this offering from additional paid-in capital in the accompanying Consolidated balance sheets.

## 9 Share-based compensation

The Company's share-based compensation plans include the 2014 Amended and Restated Share Option Plan (the "2014 Plan") and inducement grants under Rule 5653(c)(4) of the NASDAQ Global Select Market with terms similar to the 2014 Plan (together the "2014 Plans"). At the annual general meeting of shareholders in June 2018, the Company's shareholders approved amendments of the 2014 Plan, increasing the shares authorized for issuance by 3,000,000 to a total of 8,601,471. The Company previously had a 2012 Equity Incentive Plan ("2012 Plan"). As of September 30, 2019, 14,000 fully vested share options are outstanding (December 31, 2018: 32,567) under the 2012 Plan.

### a) 2014 Plan

Share-based compensation expense recognized by classification included in the Consolidated statements of operations and comprehensive loss in relation to the 2014 Plan was as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
	(in thousands)			
Research and development	\$ 1,922	\$ 895	\$ 6,021	\$ 2,694
Selling, general and administrative	1,936	1,724	6,691	4,657
<b>Total</b>	<b>\$ 3,858</b>	<b>\$ 2,619</b>	<b>\$ 12,712</b>	<b>\$ 7,351</b>

Share-based compensation expense for the nine months ended September 30, 2019 increased by \$5.4 million when compared to nine months ended September 30, 2018 primarily as a result of the appreciation of the share price and increase in number of grants. Share-based compensation expense for the three months ended September 30, 2019 increased by \$1.2 million when compared to three months ended September 30, 2018 primarily as a result of the appreciation of the share price and increase in number of grants.

Share-based compensation expense recognized by award type was as follows:

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
	(in thousands)			
<b>Award type</b>				
Share options	\$ 2,025	\$ 1,160	\$ 6,105	\$ 3,315
Restricted share units ("RSUs")	1,075	667	3,000	1,953
Performance share units ("PSUs")	758	792	3,607	2,083
<b>Total</b>	<b>\$ 3,858</b>	<b>\$ 2,619</b>	<b>\$ 12,712</b>	<b>\$ 7,351</b>

As of September 30, 2019, the unrecognized share-based compensation expense related to unvested awards under the 2014 Plans were:

Award type	Unrecognized share-based compensation expense	Weighted average remaining period for recognition
	(in thousands)	(in years)
Share options	\$ 20,776	2.93
Restricted share units	8,614	2.08
Performance share units	5,370	1.76
<b>Total</b>	<b>\$ 34,760</b>	<b>2.54</b>

The Company satisfies the exercise of share options and vesting of RSUs and PSUs through newly issued ordinary shares.

#### Share options

The following table summarizes option activity for the nine months ended September 30, 2019:

	Options	
	Number of ordinary shares	Weighted average exercise price
<b>Outstanding at December 31, 2018</b>	<b>2,673,712</b>	<b>\$ 15.09</b>
Granted	571,026	\$ 36.95
Forfeited	(46,278)	\$ 12.68
Expired	(543)	\$ 12.26
Exercised	(337,312)	\$ 10.89
<b>Outstanding at September 30, 2019</b>	<b>2,860,605</b>	<b>\$ 19.96</b>
Fully vested and exercisable at September 30, 2019	1,321,258	\$ 13.65
Outstanding and expected to vest at September 30, 2019	1,539,347	\$ 25.38
Total weighted average grant date fair value of options issued during the period (in \$ millions)		\$ 12.4
Proceeds from option sales during the period (in \$ millions)		\$ 3.7

Share options are priced on the date of grant and, except for certain grants made to non-executive directors, vest over a period of four years, the first 25% vests after one year from the grant date and the remainder vests in equal quarterly installments, over years two, three and four. Any options that vest must be exercised by the tenth anniversary of the grant date.

The fair value of each option issued is estimated at the respective grant date using the Hull & White option pricing model with the following weighted-average assumptions:

Assumptions	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
Expected volatility	75%	80%	75%	80%
Expected terms	10 years	10 years	10 years	10 years
Risk free interest rate	1.92%	3.10%	1.92% - 2.87%	2.67% - 3.10%
Expected dividend yield	0%	0%	0%	0%

### Restricted share units

The following table summarizes the RSUs activity for the nine months ended September 30, 2019:

	RSU	
	Number of ordinary shares	Weighted average grant-date fair value
<b>Non-vested at December 31, 2018</b>	<b>412,321</b>	<b>\$ 16.49</b>
Granted	198,504	\$ 38.63
Vested	(205,583)	\$ 15.31
Forfeited	(17,657)	\$ 14.00
<b>Non-vested at September 30, 2019</b>	<b>387,585</b>	<b>\$ 28.57</b>
Total weighted average grant date fair value of RSUs granted during the period (in \$ millions)		
		\$ 7.7

RSUs vest over one to three years. RSUs granted to non-executive directors will vest one year from the date of grant.

### Performance share units

The following table summarizes the PSUs activity for the nine months ended September 30, 2019:

	PSU	
	Number of ordinary shares	Weighted average grant-date fair value
<b>Non-vested at December 31, 2018</b>	<b>377,169</b>	<b>\$ 16.73</b>
Granted	132,362	\$ 31.71
Vested	(30,109)	\$ 11.83
<b>Non-vested at September 30, 2019</b>	<b>479,422</b>	<b>\$ 21.17</b>
PSUs awarded but not yet earned	96,389	\$ 39.36
Total non-vested and discretionary PSUs	575,811	\$ 24.22
Total weighted average grant date fair value of PSUs granted and awarded during the period (in \$ millions)		
		\$ 8.0

In January 2019, the Company awarded PSUs to its executives and other members of senior management. These PSUs are earned based on the Board's assessment of the level of achievement of agreed upon performance targets through December 31, 2019.

#### b) Employee Share Purchase Plan ("ESPP")

In June 2018, the Company's shareholders adopted and approved an ESPP allowing the Company to issue up to 150,000 ordinary shares. The ESPP is intended to qualify under Section 423 of the Internal Revenue Code of 1986. Under the ESPP, employees are eligible to purchase ordinary shares through payroll deductions, subject to any plan limitations. The purchase price of the shares on each purchase date is equal to 85% of the lower of the closing market price on the offering date or the closing market price on the purchase date of each three-month offering period. During the nine months ended September 30, 2019, 7,018 shares were issued. As of September 30, 2019, a total of 140,391 ordinary shares remains available for issuance under the ESPP plan.

## 10 Income taxes

Deferred tax assets and deferred tax liabilities are recognized based on the expected future tax consequences of temporary differences between the financial statement carrying amounts and the income tax basis of assets and liabilities, using current statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all the deferred tax assets will not be realized. Due to the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets.

## 11 Basic and diluted earnings per share

Diluted earnings per share are calculated by adjusting the weighted average number of ordinary shares outstanding, assuming conversion of all potentially dilutive ordinary shares. As the Company has incurred a loss, all potentially dilutive ordinary shares would have an antidilutive effect, if converted, and thus have been excluded from the computation of loss per share.

The potentially dilutive ordinary shares are summarized below:

	September 30,	
	2019	2018
	(ordinary shares)	
BMS warrants	8,890,000	7,570,000
Stock options under 2014 Plans	2,860,605	2,707,538
Non-vested RSUs and earned PSUs	867,007	849,612
Stock options under 2012 Plan	14,000	32,567
Hercules warrants (exercised February 1, 2019)	—	37,175
Employee share purchase plan	1,020	—
<b>Total potential dilutive ordinary shares</b>	<b>12,632,632</b>	<b>11,196,892</b>

## 12 Related party transaction

In August 2019, the Company promoted Sander van Deventer, M.D., Ph.D., to Executive Vice President, Research and Product Development, and Alex Kuta, Ph.D., to Executive Vice President, Operations. Dr. van Deventer, in addition to his responsibilities to being responsible for research, will now also be responsible for the Company's product development. Dr. Kuta, in addition to regulatory affairs, will now also be responsible for global quality as well as GMP manufacturing at uniQure's state-of-the-art facility in Lexington, Massachusetts. As a result of these changes, the Company eliminated the Chief Operating Officer role, and Scott McMillan, Ph.D. retired from uniQure.

In August 2019, the Company entered into an Amended and Restated Agreement Collaboration and License Agreement ("Amended CLA") as well as an additional new Collaboration and License Agreement ("New CLA") with its related party 4DMT Molecular Therapeutics, Inc. ("4DMT"). In the Amended CLA, the Company received from 4DMT an exclusive, sublicensable, worldwide license under certain 4DMT intellectual property rights to research, develop, make, use, and commercialize previously selected AAV capsid variants and certain associated products using 4DMT proprietary AAV technology for delivery of gene therapy constructs to cells in the central nervous system and the liver ("the Field"). In the New CLA, the parties agreed to research and develop at 4DMT's cost new AAV capsid variants using 4DMT proprietary AAV technology for delivery of up to six additional transgene constructs in the Field that will be selected by the Company.

## 13 Subsequent event

None.

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

*The following Management's Discussion and Analysis of Financial Condition and Results of Operations ("MD&A") is intended to help the reader understand our results of operations and financial condition. This MD&A is provided as a supplement to, and should be read in conjunction with, our unaudited consolidated financial statements and the accompanying notes thereto and other disclosures included in this Quarterly Report on Form 10-Q, including the disclosures under Part II, Item 1A "Risk Factors", and our audited financial information and the notes thereto included in our [Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the Securities and Exchange Commission \(the "SEC"\), on February 28, 2019](#). Our unaudited consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the U.S. ("U.S. GAAP") and unless otherwise indicated are presented in U.S. dollars.*

### Overview

We are a leader in the field of gene therapy, seeking to develop single treatments with potentially curative results for patients suffering from genetic and other devastating diseases. We are advancing a focused pipeline of innovative gene therapies. We have established clinical proof-of-concept in our lead indication, hemophilia B, and achieved preclinical proof-of-concept in Huntington's disease. We believe our validated technology platform and manufacturing capabilities provide us distinct competitive advantages, including the potential to reduce development risk, cost and time to market. We produce our adeno-associated virus ("AAV")-based gene therapies in our own facilities with a proprietary, commercial-scale, current good manufacturing practices ("cGMP")-compliant, manufacturing process. We believe our Lexington, Massachusetts-based facility is one of the world's leading, most versatile, gene therapy manufacturing facilities.

### Business Developments

Below is a summary of our recent significant business developments:

#### Hemophilia B program - Etranacogene dezaparvovec (AMT-061)

Etranacogene dezaparvovec is our lead gene therapy candidate and includes an AAV5 vector incorporating the Factor IX-Padua variant. We are currently conducting a pivotal study in patients with severe and moderately-severe hemophilia B. Etranacogene dezaparvovec has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration ("FDA") and access to the PRIME initiative by the European Medicines Agency ("EMA").

In June 2018, we initiated our Phase III HOPE-B pivotal trial of Etranacogene dezaparvovec. The trial is a multinational, multi-center, open-label, single-arm study to evaluate the safety and efficacy of Etranacogene dezaparvovec. After a six-month lead-in period, patients will receive a single intravenous administration of Etranacogene dezaparvovec. The primary endpoint of the study will be based on the Factor IX ("FIX") activity level achieved following the administration of Etranacogene dezaparvovec, and the secondary endpoints will measure annualized FIX replacement therapy usage, annualized bleed rates and safety. Patients enrolled in the HOPE-B trial will be tested for the presence of pre-existing neutralizing antibodies to AAV5 but will not be excluded from the trial based on their titers.

In January 2019, we dosed the first patient in our Phase III HOPE-B hemophilia B pivotal trial and in September 2019, we completed the enrollment of 62 patients in the lead-in phase of the study.

In August 2018, we initiated a Phase IIb dose-confirmation study of Etranacogene dezaparvovec. The Phase IIb study is an open-label, single-dose, single-arm, multi-center trial being conducted in the United States. The objective of the study was to evaluate the safety and tolerability of Etranacogene dezaparvovec and confirm the dose based on FIX activity at six weeks after administration. Three patients with severe hemophilia were enrolled in this study and received a single intravenous infusion of  $2 \times 10^{13}$  gc/kg.

In February, May and July 2019, we presented updated data from our Phase IIb dose-confirmation study of Etranacogene dezaparvovec. Data from the Phase IIb study of Etranacogene dezaparvovec show that all three patients experienced increasing and sustained FIX levels after a one-time administration of Etranacogene dezaparvovec, with two of the three patients maintaining FIX activity in the normal range. Mean FIX activity was 45% of normal at 36 weeks of follow-up, exceeding threshold FIX levels generally considered sufficient to significantly reduce the risk of bleeding events. Specifically, the first patient achieved FIX activity of 54% of normal, the second patient achieved FIX activity of 30% of normal, and the third patient achieved FIX activity of 51% of normal. Through 36 weeks of follow-up, no patient experienced a material loss of FIX activity, reported any bleeding events or required any

infusions of FIX replacement therapy for bleeds. One patient underwent hip surgery due to a pre-existing condition and was treated perioperatively with short-acting factor replacement. This was reported by the investigator as a serious adverse event unrelated to Etranacogene dezaparvovec.

In July 2019, we also presented three-and-a-half-year follow-up data related to our first-generation hemophilia B program, AMT-060, which incorporated a wild-type Factor IX gene. All 10 patients enrolled in the Phase I/II study continue to show long-term meaningful clinical impact, including sustained increases in FIX activity and improvements in their disease state as measured by reduced usage of FIX replacement therapy and decreased bleeding frequency. At up to 3.5 years of follow-up, AMT-060 continues to be safe and well-tolerated, with no new serious adverse events and no development of inhibitors since the last reported data.

All five patients in the second dose cohort of  $2 \times 10^{13}$  gc/kg continue to be free of routine FIX replacement therapy at up to three years after treatment. During the last 12 months of observation, the mean annualized bleeding rate was 0.7 bleeds, representing an 83% reduction compared with the year prior to treatment. During this same period, the usage of FIX replacement therapy declined 96% compared with the year prior to treatment. Steady state mean yearly FIX activity at three years was 7.9%, compared with 7.1% in the first year and 8.4% in the second year.

#### Huntington's disease program (AMT-130)

AMT-130 is our novel gene therapy candidate for the treatment of Huntington's disease. AMT-130 utilizes our miQURE™ proprietary, gene-silencing platform and incorporates an AAV vector carrying a microRNA ("miRNA") specifically designed to silence the huntingtin gene and the potentially highly toxic exon 1 protein fragment. AMT-130 has received orphan drug and fast track designations from the FDA and Orphan Medicinal Product Designation from the EMA.

In January 2019, our Initial New Drug ("IND") application for AMT-130 was cleared by the FDA, thereby enabling us to initiate our planned Phase I/II clinical study. The Phase I/II study is expected to be a randomized, double-arm, blinded, imitation surgery-controlled trial conducted at three surgical sites in the U.S., and at least five non-surgical sites. The primary objective of the study is to evaluate the safety, tolerability and efficacy of AMT-130 at two doses.

In October 2019, we initiated patient screening activities and expect to enroll the first patient later this year or in early 2020. Additionally, cGMP clinical material has been manufactured at our Lexington facility and has been released for shipment.

In January 2019, the U.S. Patent and Trademark Office issued U.S. Patent 10,174,321 and in May 2019 the European Patent Office issued EP 3237618, both with granted claims that cover the RNA constructs specifically designed to target exon1 and the embedding of these Huntington's disease RNA sequences into the miR451 scaffold, which we exclusively license from Cold Spring Harbor Laboratory (CSHL). The claims also cover certain expression cassettes comprising the RNA constructs and the use of gene therapy vectors including AAV vectors encompassing the described expression cassettes.

In February 2019, we presented new preclinical data at the 14th Annual CHDI Huntington's disease Therapeutics Conference that illustrate the therapeutic potential of AMT-130 in restoring function of damaged brain cells in Huntington's disease and providing a safe and sustained reduction of mutant huntingtin protein.

#### Hemophilia A program (AMT-180)

AMT-180 is our novel gene therapy candidate for the treatment of hemophilia A that utilizes an AAV vector incorporating a proprietary, exclusively licensed, modified Factor IX gene that has been demonstrated in preclinical studies to convey Factor VIII-independent activity and circumvent inhibitors to Factor VIII. In May 2019, we presented preclinical proof-of-concept data at the American Society of Gene and Cell Therapy ("ASGCT") Annual Meeting, demonstrating that AMT-180 induced clinically relevant thrombin activation, and up to 29% of Factor VIII-independent activity, in FVIII-depleted human plasma. The studies further demonstrated that a single intravenous administration of AMT-180 resulted in sustained, dose-dependent hemostatic effect as measured by one-stage clotting assay, and that AMT-180 shows activation kinetics similar to native FIX and is not hyperactive. A pilot study in non-human primates demonstrated that administration of AMT-180 resulted in sufficient FIX protein expression that translates to clinically relevant Factor VIII-independent activity in humans. No elevation of coagulation activation markers or signs of thrombi formation were observed.

We are currently conducting safety and toxicology studies of AMT-180 to support the submission of a clinical trial application in 2020.



### Spinocerebellar Ataxia Type 3 (AMT-150)

AMT-150 is our novel gene therapy candidate for the treatment of Spinocerebellar Ataxia Type 3 (“SCA3”), also known as Machado-Joseph disease, which is caused by a CAG-repeat expansion in the ATXN3 gene that results in an abnormal form of the protein ataxin-3. At the 2019 American Academy of Neurology (AAN) Annual Meeting, we presented preclinical data on AMT-150 demonstrating mechanistic proof-of-concept of the non-allele-specific ataxin-3 protein-silencing approach by using artificial microRNA candidates engineered to target the ataxin-3 gene in a SCA3 knock-in mouse model. The proof-of-concept study demonstrated that a single AMT-150 injection in the cerebrospinal fluid resulted in strong AAV transduction and significant mutant ataxin-3 lowering at the primary sites of disease neuropathology, the cerebellum (up to 53%) and the brainstem (up to 65%).

We are currently preparing to initiate safety and toxicology studies of AMT-150 to support the submission of an IND application.

### Fabry program (AMT-190)

AMT-190 is our novel gene therapy candidate for the treatment of Fabry disease that comprises of an AAV vector incorporating a proprietary, exclusively licensed, modified NAGA (ModNAGA) variant. ModNAGA may have several advantages over other therapies for Fabry disease, including higher stability in blood, circumvention of inhibitors, better biodistribution in the target organs, secondary toxic metabolite reduction and improved cross-correction of neighboring cells.

At the ASGCT Annual Meeting in May 2019, we presented data from in vitro and in vivo studies showing that AMT-190 has the potential to become a one-time treatment option that could be an improvement upon the enzyme replacement standard of care with more efficient uptake in the kidney and heart and an improved immunogenicity profile. In particular, data from a study in wild-type mice showed a single intravenous administration of AMT-190 resulted in a ten- to twenty-fold higher alpha-galactosidase (“GLA”) activity in the plasma compared to the control group. Additionally, data from a study in a diseased mouse model demonstrated significantly increased GLA activity in plasma and significantly reduced globotriaosylsphingosine (“Lyso-Gb3”) in target organs after a single dose of AMT-190. In silico and in vitro studies also showed that the modifications introduced into NAGA are believed to pose a very low immunogenicity risk.

We are currently conducting additional preclinical studies to identify a lead candidate for further safety testing.

### BMS collaboration

We entered into a collaboration and license agreement with BMS in May 2015. We have been supporting BMS in the discovery, non-clinical, analytical and process development efforts of Collaboration Targets. For any Collaboration Targets that are advanced, we will be responsible for manufacturing of clinical and commercial supplies using our vector technologies and industrial, proprietary insect-cell based manufacturing platform. BMS has been reimbursing us for all our research and development costs in support of the collaboration during the initial research term. BMS will lead the development, regulatory and commercial activities for all four currently active Collaboration Targets as well as additional Collaboration Targets that may be advanced.

In February 2019, BMS requested a one-year extension of the research term. In April 2019, following an assessment of the progress of this collaboration and our expanding proprietary programs, we notified BMS that we did not intend to agree to an extension of the research term. Accordingly, the initial four-year research term under the collaboration terminated on May 21, 2019. We are currently in discussions with BMS potentially to restructure the collaboration and license agreement and other related agreements following the expiration of the research term. Although such discussions are ongoing and may not result in any change to these arrangements, we believe that the final resolution of these discussions may result in material changes to our collaboration with BMS.

### Padua mutation in human Factor IX patent family

We own a patent family, including patents and patent applications, directed to the use of the Padua mutation in human Factor IX for gene therapy. A patent cooperation treaty application was filed on September 15, 2009, and patents have been issued in the United States, Europe, and Canada. Further applications are pending in the United States, Europe, and Hong Kong. The issued patents include claims directed to FIX protein with a leucine at the R338 position of the protein sequence, nucleic acid sequences coding for this protein, and therapeutic applications, including gene therapy. The standard 20-year patent term of patents in this family will expire in 2029.

On July 31, 2019 we received a notice of allowance from the U.S. Patent and Trademark Office for U.S. application number 15/989,665, a third U.S. family member in the FIX-Padua patent family. The claims as allowed cover any AAV comprising a nucleic acid encoding a FIX-Padua protein, and promoter sequences, transcription termination and control elements. The claims also cover FIX-Padua variants with at least 70% sequence identity to FIX-R338L.

On June 13, 2018, we were granted European Patent 2337849 directed to a FIX polypeptide protein. The opposition period with respect to such patent expired on March 13, 2019, by which time five parties had filed an opposition. On July 25, 2019, we submitted responses to such oppositions with the European Patent Office, or EPO, and expect that oral proceeding with respect to such oppositions will take place in the first half of 2020. In addition, on May 15, 2019, a divisional European patent application in the FIX-Padua family, EP 3252157, was refused. In September 2019, we filed a notice of appeal with respect to such refusal. We are also pursuing a European divisional patent application that was filed on May 14, 2019.

## Financing

On September 10, 2019, we completed a follow-on public offering of 4,891,305 ordinary shares at a public offering price of \$46.00 per ordinary share, and on September 13, 2019, we completed the sale of an additional 733,965 ordinary shares at a public offering price of \$46.00 per ordinary share, pursuant to the exercise by the underwriters of the option to purchase additional ordinary shares, resulting in gross proceeds to us of \$258.8 million. The net proceeds to us from this offering were \$242.8 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We deducted \$0.4 million of expenses incurred related to this offering from additional paid-in capital.

## Organization

In August 2019 we promoted Sander van Deventer, M.D., Ph.D., to Executive Vice President, Research and Product Development, and Alex Kuta, Ph.D., to Executive Vice President, Operations. Dr. van Deventer, in addition to his responsibilities to being responsible for research, will now also be responsible for our product development. Dr. Kuta, in addition to regulatory affairs, will now also be responsible for global quality as well as GMP manufacturing at uniQure's state-of-the-art facility in Lexington, Massachusetts. As a result of these changes, we eliminated the Chief Operating Officer role, and Scott McMillan, Ph.D. retired from uniQure.

## Related Party Transaction

In August 2019, we entered into an Amended and Restated Agreement Collaboration and License Agreement ("Amended CLA") as well as an additional new Collaboration and License Agreement ("New CLA") with our related party 4DMT Molecular Therapeutics, Inc. ("4DMT"). In the Amended CLA, we received from 4DMT an exclusive, sublicensable, worldwide license under certain 4DMT intellectual property rights to research, develop, make, use, and commercialize previously selected AAV capsid variants and certain associated products using 4DMT proprietary AAV technology for delivery of gene therapy constructs to cells in the central nervous system and the liver ("the Field"). In the New CLA, the parties agreed to research and develop at 4DMT's cost new AAV capsid variants using 4DMT proprietary AAV technology for delivery of up to six additional transgene constructs in the Field that will be selected by us.

## Financial Overview

Key components of our results of operations include the following:

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
	(in thousands)		(in thousands)	
Total revenues	\$ 1,046	\$ 3,148	\$ 4,656	\$ 9,676
Research and development expenses	(23,554)	(20,541)	(68,245)	(56,092)
Selling, general and administrative expenses	(8,929)	(5,898)	(24,866)	(18,095)
Net loss	(23,604)	(22,035)	(82,775)	(61,416)

As of September 30, 2019, and December 31, 2018, we had cash and cash equivalents of \$403.2 million and \$234.9 million, respectively. We had a net loss of \$23.6 million and \$82.8 million in the three and nine months ended September 30, 2019, respectively, compared to \$22.0 million and \$61.4 million for the same periods in 2018. As of September 30, 2019, and December 31, 2018, we had accumulated deficits of \$618.3 million and \$535.5 million, respectively. We anticipate that our loss from operations will increase in the future as we:

- Build-out our commercial infrastructure and seek marketing approval for any product candidates (including Etranacogene dezaparvovec) that successfully complete clinical trials;
- Advance the clinical development of AMT-130 for our Huntington's disease gene therapy program;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed and central nervous system ("CNS") diseases;
- Continue to build-out our clinical, medical and regulatory capabilities;
- Continue to expand, enhance and optimize our technology platform, including our manufacturing capabilities, next-generation viral vectors and promoters, and other enabling technologies;
- Acquire or in-license rights to new therapeutic targets or product candidates; and
- Maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties.

See "Results of Operations" below for a discussion of the detailed components and analysis of the amounts above.

### **Critical Accounting Policies and Estimates**

In preparing our consolidated financial statements in accordance with U.S. GAAP and pursuant to the rules and regulations promulgated by the SEC, our management makes assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. On an ongoing basis, we evaluate our estimates and judgments, including those related to recognition of License Revenue in accordance with ASC 606, BMS warrants and share-based payments. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not clear from other sources. Actual results may differ from these estimates under different assumptions or conditions. In making estimates and judgments, management employs critical accounting policies. With the exception of ASC 842 Leases, during the nine months ended September 30, 2019, there were no material changes to our critical accounting policies as reported in our [Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 28, 2019](#).

We believe that the assumptions, judgments and estimates involved in the recognition of License Revenue in accordance with ASC 606, BMS warrants and share-based payments to be our critical accounting policies during the year ended December 31, 2018, as well as during the three and nine months ended September 30, 2019. We also consider the accounting for our operating leases to be among our critical accounting policies.

#### **Adoption of ASC 842 Leases on January 1, 2019**

On January 1, 2019, we adopted ASC 842, "Leases (Topic 842)". We adopted the standard using the modified retrospective approach with an effective date as of the beginning of the fiscal year, January 1, 2019, to operating leases that existed on that date. Prior year comparative financial information was not recast under the new standard and continues to be presented under ASC 840. We elected to utilize the package of practical expedients available for expired or existing contracts which allowed us to carryforward historical assessments of (1) whether contracts are or contain leases, (2) lease classification, and (3) initial direct costs. We performed an assessment and identified the lease facilities as material leases to be accounted for under ASC 842 as of January 1, 2019. The impact of implementing ASC 842 is summarized below:

- Recognized a \$19.0 million operating right-of-use asset and a \$28.1 million operating lease liability in relation to the facilities leased at the Amsterdam and Lexington sites in the Consolidated balance sheet as of January 1, 2019;
- Presented deferred rent of \$9.1 million as of December 31, 2018, as a reduction of the right-of-use asset as from January 1, 2019 onwards in the Consolidated balance sheet and as a change within operating cash flows within accrued expense, other liabilities and operating leases;

We measured the lease liability at the present value of the future lease payments as of January 1, 2019. We used an incremental borrowing rate to discount the lease payments. We derived the discount rate, adjusted for differences in the term and payment patterns, from our Hercules loan which was refinanced immediately prior to the January 1, 2019 adoption date in December 2018. We valued the right-of-use asset at the amount of the lease liability reduced by the remaining December 31, 2018 balance of lease incentives received. We subsequently measure the lease liability at the present value of the future lease payments as of the reporting date with a corresponding adjustment to the right-to-use asset. Absent a lease modification we will continue to utilize the January 1, 2019, incremental borrowing rate.

We will continue to recognize lease cost on a straight-line basis and will continue to present these costs as operating expenses within our Consolidated statements of operations and comprehensive loss. We will continue to present lease payments and landlord incentive payments within cash flows from operations within our Consolidated statements of cash flows.

### **Revenues**

We recognize Collaboration Revenues associated with pre-clinical Collaboration Target specific, non-clinical, analytical and process development activities that are reimbursable by BMS under our collaboration agreement during the initial research term (that ended on May 21, 2019). We are currently in discussions with BMS potentially to restructure the collaboration and license agreement and other related agreements following the expiration of the research term. During these discussions, which may be terminated by us or BMS at any time, we have agreed to continue providing support of the pre-clinical Collaboration Targets, and any related costs will be reimbursed by BMS.

We recognize License Revenues associated with the amortization of the non-refundable upfront payment, target designation fees and research and development milestone payments we received or might receive from BMS. The timing of these cash payments may differ from the recognition of revenue, as revenue is deferred and recognized over the duration of the performance period. We recognize other revenue, such as sales milestone payments, when earned.

### **Research and development expenses**

We expense research and development costs ("R&D") as incurred. Our R&D expenses generally consist of costs incurred for the development of our target candidates, which include:

- Employee-related expenses, including salaries, benefits, travel and share-based compensation expense;
- Costs incurred for laboratory research, preclinical and nonclinical studies, clinical trials, statistical analysis and report writing, and regulatory compliance costs incurred with clinical research organizations and other third-party vendors;
- Costs incurred to conduct consistency and comparability studies;
- Costs incurred for the validation of our Lexington facility;
- Costs incurred for the development and improvement of our manufacturing processes and methods;
- Costs associated with our research activities for our next-generation vector and promoter platform;
- Changes in the fair value of the contingent consideration related to our acquisition of InoCard (up to September 30, 2018) as well as the impairment of in process research and development acquired (in the three-month period ended September 30, 2018);
- Facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and
- Amortization of intangible assets.

Our research and development expenses primarily consist of costs incurred for the research and development of our product candidates, which include:

- *Etranacogene dezaparvovec (hemophilia B)*. We have incurred costs related to the research, development and production of Etranacogene dezaparvovec for the treatment of hemophilia B. In June 2018, we initiated a pivotal study. We completed enrollment of this pivotal study in September 2019. In September 2018, we completed patient dosing in our Phase IIb dose-confirmation study.
- *AMT-130 (Huntington's disease)*. We have incurred costs related to preclinical and nonclinical studies of AMT-130 and started incurring costs related to our Phase I/II trial from February 2019 onwards;
- *Preclinical research programs*. We incur costs related to the research of multiple preclinical gene therapy product candidates with the potential to treat certain rare and other serious medical conditions; and
- *Technology platform development and other related research*. We incur significant research and development costs related to manufacturing and other enabling technologies that are applicable across all our programs.

Our research and development expenses may vary substantially from period to period based on the timing of our research and development activities, including manufacturing campaigns, regulatory submissions and enrollment of patients in clinical trials. The successful development of our product candidates is highly uncertain. Estimating the nature, timing or cost of the development of any of our product candidates involves considerable judgement due to numerous risks and uncertainties associated with developing gene therapies, including the uncertainty of:

- the scope, rate of progress and expense of our research and development activities;
- our ability to successfully manufacture and scale-up production;
- clinical trial protocols, speed of enrollment and resulting data;
- the effectiveness and safety of our product candidates;
- the timing of regulatory approvals; and
- our ability to agree to ongoing development budgets with collaborators who share the costs of our development programs.

A change in the outcome of any of these variables with respect to our product candidates that we may develop could mean a significant change in the expenses and timing associated with the development of such product candidate.

#### ***Selling, general and administrative expenses***

Our general and administrative expenses consist principally of employee, office, consulting, legal and other professional and administrative expenses. We incur expenses associated with operating as a public company, including expenses for personnel, legal, accounting and audit fees, board of directors' costs, directors' and officers' liability insurance premiums, NASDAQ listing fees, expenses related to investor relations and fees related to business development and maintaining our patent and license portfolio. Our selling costs include employee expenses as well as professional fees related to the preparation of a commercial launch of Etranacogene dezaparvovec.

#### ***Other items, net***

Our other income consists of payments to subsidize our research and development efforts as well as income from the subleasing of our Amsterdam facility.

Our other expense consists of expenses we incur in relation to our subleasing income.

## Results of Operations

### Comparison of the three months ended September 30, 2019 and 2018

The following table presents a comparison of the three months ended September 30, 2019 and 2018.

	Three months ended September 30,		
	2019	2018 (in thousands)	2019 vs 2018
Total revenues	\$ 1,046	\$ 3,148	\$ (2,102)
<b>Operating expenses:</b>			
Research and development expenses	(23,554)	(20,541)	(3,013)
Selling, general and administrative expenses	(8,929)	(5,898)	(3,031)
<b>Total operating expenses</b>	<b>(32,483)</b>	<b>(26,439)</b>	<b>(6,044)</b>
Other income	453	557	(104)
Other expense	(342)	(490)	148
<b>Loss from operations</b>	<b>(31,326)</b>	<b>(23,224)</b>	<b>(8,102)</b>
Other non-operating items, net	7,722	1,157	6,565
<b>Loss before income tax benefit</b>	<b>(23,604)</b>	<b>(22,067)</b>	<b>(1,537)</b>
Income tax benefit	—	32	(32)
<b>Net loss</b>	<b>\$ (23,604)</b>	<b>\$ (22,035)</b>	<b>\$ (1,569)</b>

#### Revenue

Our revenue for the three months ended September 30, 2019 and 2018 was as follows:

	Three months ended September 30,		
	2019	2018 (in thousands)	2019 vs 2018
License Revenue	\$ 842	\$ 2,518	\$ (1,676)
Collaboration Revenue	204	630	(426)
<b>Total revenues</b>	<b>\$ 1,046</b>	<b>\$ 3,148</b>	<b>\$ (2,102)</b>

We recognize License Revenue related to upfront payments and target designation fees received from BMS in 2015. We recognized \$0.8 million License Revenue in the three months ended September 30, 2019, compared to \$2.5 million for the same period in 2018. The reduction in License Revenue is primarily a result of additional services we expect to provide as a result of BMS designating a replacement target for S100A1.

We recognized \$0.2 million Collaboration Revenue in the three months ended September 30, 2019, compared to \$0.6 million for the same period in 2018. The decrease in Collaboration Revenue was primarily related to the discontinuation of S100A1 research in the fourth quarter of 2018.

#### Research and development expenses

Research and development expenses for the three months ended September 30, 2019 were \$23.6 million, compared to \$20.5 million for the same period in 2018.

- We incurred \$9.8 million in personnel and consulting expenses in the three months ended September 30, 2019, compared to \$8.2 million for the same period in 2018. Our costs during the three months ended September 30, 2019 increased by \$1.6 million as a result of the recruitment of personnel to support the preclinical and clinical development of our product candidates;
- We incurred \$2.0 million in share-based compensation expenses in the three months ended September 30, 2019, compared to \$0.9 million for the same period in 2018 primarily driven by the appreciation of our share price and increase in number of grants;
- We incurred \$7.0 million in external services and costs related to the development of our product candidates in the three months ended September 30, 2019, compared to \$6.6 million in the same period in 2018. The increase was a result of the advancement of our ongoing Phase III and Phase IIb trials of Etranacogene dezaparvovec and

- additional activities in support of our pre-clinical product candidates, partially offset by a decrease in disposable costs;
- We incurred \$4.0 million in operating expenses and depreciation expenses related to our rented facilities in the three months ended September 30, 2019, compared to \$3.1 million in the same period in 2018. Our costs during the three months ended September 30, 2019 increased as a result of extending and expanding (as from June 2019) the lease of our Lexington facility;
- We recorded no change in the fair value of the contingent consideration owed to the sellers of InoCard business in the three months ended September 30, 2019, compared to income of \$3.8 million recorded in the same period in 2018; and
- We incurred no impairment losses in the three months ended September 30, 2019, compared to an impairment loss of \$5.4 million on the in-process research and development asset acquired in the InoCard business combination in the same period in 2018.

#### *Selling, general and administrative expenses*

Selling, general and administrative expenses for the three months ended September 30, 2019 were \$8.9 million, compared to \$5.9 million for the same period in 2018.

- We incurred \$2.5 million in personnel and consulting expenses in the three months ended September 30, 2019, compared to \$2.0 million in the same period in 2018;
- We incurred \$1.9 million in share-based compensation expenses in the three months ended September 30, 2019, compared to \$1.7 million in the same period in 2018. The increase was primarily driven by the appreciation of our share price and increase in number of grants; and
- We incurred \$2.2 million in professional fees in the three months ended September 30, 2019, compared to \$0.9 million in the same period in 2018. The increase was primarily driven by expenses related to the preparation of a commercial launch of Etranacogene dezaparvovec and various corporate activities.

#### *Other items, net*

We recognized \$0.1 million in income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the three months ended September 30, 2019, compared to \$0.3 million for the same period in 2018.

#### *Other non-operating items, net*

We recognize interest income associated with our cash and cash equivalents.

We hold monetary items and enter into transactions in foreign currencies, predominantly in euros and U.S. dollars. We recognize foreign exchange results related to changes in these foreign currencies.

We issued warrants to Hercules in 2013 and to BMS in 2015. We recognize changes in the fair value of these warrants within other non-operating (expense) / income. Following the exercise of the warrants by Hercules in February 2019 we no longer recognize changes in the fair value of these warrants within other non-operating (expense) / income.

Our other non-operating items, net, for the three months ended September 30, 2019 and 2018 were as follows:

	Three months ended September 30,		
	2019	2018	2019 vs 2018
	(in thousands)		
Interest income	\$ 868	\$ 949	\$ (81)
Interest expense - Hercules long-term debt	(960)	(515)	(445)
Foreign currency gains, net	6,041	455	5,586
Other non-operating gains	1,773	268	1,505
<b>Total other non-operating income, net</b>	<b>\$ 7,722</b>	<b>\$ 1,157</b>	<b>\$ 6,565</b>



We recognized a net foreign currency gain related to our borrowings from Hercules and our cash and cash equivalents of \$6.0 million during the three months ended September 30, 2019, compared to a net gain of \$0.5 million during the same period in 2018.

In the three months ended September 30, 2019, we recognized an income of \$1.8 million related to changes of fair value of warrants compared to an income of \$0.3 million for the same period in 2018. The increase is primarily driven by reduction in fair value of the BMS warrants.

### **Comparison of the nine months ended September 30, 2019, and 2018**

The following table presents a comparison of the nine months ended September 30, 2019 and 2018.

	Nine months ended September 30,		
	2019	2018	2019 vs 2018
	(in thousands)		
Total revenues	\$ 4,656	\$ 9,676	\$ (5,020)
<b>Operating expenses:</b>			
Research and development expenses	(68,245)	(56,092)	(12,153)
Selling, general and administrative expenses	(24,866)	(18,095)	(6,771)
<b>Total operating expenses</b>	<b>(93,111)</b>	<b>(74,187)</b>	<b>(18,924)</b>
Other income	1,332	1,737	(405)
Other expense	(1,038)	(1,252)	214
<b>Loss from operations</b>	<b>(88,161)</b>	<b>(64,026)</b>	<b>(24,135)</b>
Non-operating items, net	5,386	2,847	2,539
<b>Loss before income tax expense</b>	<b>(82,775)</b>	<b>(61,179)</b>	<b>(21,596)</b>
Income tax expense	—	(237)	237
<b>Net loss</b>	<b>\$ (82,775)</b>	<b>\$ (61,416)</b>	<b>\$ (21,359)</b>

### *Revenue*

Our revenue for the nine months ended September 30, 2019 and 2018 was as follows:

	Nine months ended September 30,		
	2019	2018	2019 vs 2018
	in thousands		
License revenue	\$ 3,507	\$ 7,092	\$ (3,585)
Collaboration revenue	1,149	2,584	(1,435)
<b>Total revenues</b>	<b>\$ 4,656</b>	<b>\$ 9,676</b>	<b>\$ (5,020)</b>

We recognize License Revenue related to upfront payments and target designation fees received from BMS in 2015. We recognized \$3.5 million License Revenue in the nine months ended September 30, 2019, compared to \$7.1 million for the same period in 2018. The reduction in License Revenue is primarily a result of additional services we expect to provide as a result of BMS designating a replacement target for S100A1.

We recognized \$1.1 million in Collaboration Revenue in the nine months ended September 30, 2019, compared to \$2.6 million for the same period in 2018. The decrease in Collaboration Revenue was primarily related to the discontinuation of S100A1 research in the fourth quarter of 2018.

### *Research and development expenses*

Research and development expenses for the nine months ended September 30, 2019 were \$68.2 million, compared to \$56.1 million for the same period in 2018.

- We incurred \$27.8 million in personnel and consulting expenses in the nine months ended September 30, 2019, compared to \$23.6 million for the same period in 2018. Our costs during the nine months ended September 30, 2019 increased by \$4.2 million as a result of the recruitment of personnel to support the preclinical and clinical development of our product candidates;



- We incurred \$6.1 million in share-based compensation expenses in the nine months ended September 30, 2019, compared to \$2.7 million for the same period in 2018 primarily driven by the appreciation of our share price and increase in number of grants;
- We incurred \$21.8 million in external services and costs related to the development of our product candidates in the nine months ended September 30, 2019, compared to \$18.5 million in the same period in 2018. The increase was a result of the advancement of our Phase III and Phase IIb trials of Etranacogene dezaparvovec, as well as increased activities associated with our pre-clinical product candidates, partially offset by a decrease in disposable costs;
- We incurred \$11.0 million in operating expenses and depreciation expenses related to our rented facilities in the nine months ended September 30, 2019, compared to \$9.0 million in the same period in 2018. Our costs during the nine months ended September 30, 2019 increased as a result of extending and expanding (as of June 2019) the lease of our Lexington facility;
- We recorded no results related to a change in the fair value of the contingent consideration owed to the sellers of InoCard business in the nine months ended September 30, 2019, compared to income of \$4.0 million recorded in the same period in 2018; and
- We incurred no impairment losses in the nine months ended September 30, 2019, compared to an impairment loss of \$5.4 million on the in-process research and development asset acquired in the InoCard business combination in the same period in 2018.

*Selling, general and administrative expenses*

Selling, general and administrative expenses for the nine months ended September 30, 2019 were \$24.9 million, compared to \$18.1 million for the same period in 2018.

- We incurred \$7.6 million in personnel and consulting expenses in the nine months ended September 30, 2019, compared to \$6.4 million in the same period in 2018 driven by increase in personnel related and consulting related expenses;
- We incurred \$6.7 million in share-based compensation expenses in the nine months ended September 30, 2019, compared to \$4.7 million in the same period in 2018. The increase was primarily driven by the appreciation of our share price and increase in number of grants; and
- We incurred \$5.1 million in professional fees in the nine months ended September 30, 2019, compared to \$3.1 million in the same period in 2018. The increase was primarily driven by expenses related to various corporate activities as well as the preparation of a commercial launch of Etranacogene dezaparvovec.

*Other items, net*

We recognized \$0.4 million in income from payments received from European authorities to subsidize our research and development efforts in the Netherlands in the nine months ended September 30, 2019, compared to \$0.8 million for the same period in 2018.

*Other non-operating items, net*

We recognize interest income associated with our cash and cash equivalents.

We hold monetary items and enter into transactions in foreign currencies, predominantly in euros and U.S. dollars. We recognize foreign exchange results related to changes in these foreign currencies.

We issued warrants to Hercules in 2013 and to BMS in 2015. We recognize changes in the fair value of these warrants within other non-operating (expense) / income. Following the exercise of the warrants by Hercules in February 2019 we no longer recognize changes in the fair value of these warrants within other non-operating (expense) / income.

Our other non-operating items, net, for the nine months ended September 30, 2019 and 2018 were as follows:

	Nine months ended September 30,		
	2019	2018	2019 vs 2018
	(in thousands)		
Interest income	\$ 2,038	\$ 1,785	\$ 253
Interest expense - Hercules debt	(2,854)	(1,496)	(1,358)
Foreign currency gains, net	7,063	2,888	4,175
Other non-operating expense	(861)	(330)	(531)
<b>Total non-operating income, net</b>	<b>\$ 5,386</b>	<b>\$ 2,847</b>	<b>\$ 2,539</b>

We recognized a net foreign currency gain related to our borrowings from Hercules and our cash and cash equivalents of \$7.1 million during the nine months ended September 30, 2019, compared to a net gain of \$2.9 million during the same period in 2018.

In the nine months ended September 30, 2019, we recognized a loss of \$0.9 million related to changes of fair value of warrants compared to a loss of \$0.3 million for the same period in 2018.

### Financial Position, Liquidity and Capital Resources

As of September 30, 2019, we had cash, cash equivalents and restricted cash of \$406.1 million. We currently expect that our cash and cash equivalents will be sufficient to fund operations into mid-2022. The table below summarizes our consolidated cash flow data for the nine months ended September 30, 2019, and 2018.

	Nine months ended September 30,	
	2019	2018
	(in thousands)	
Cash, cash equivalents and restricted cash at the beginning of the period	\$ 237,342	\$ 161,851
Net cash used in operating activities	(72,677)	(58,034)
Net cash used in investing activities	(4,684)	(3,503)
Net cash generated from financing activities	247,511	143,117
Foreign exchange impact	(1,415)	(1,431)
<b>Cash, cash equivalents and restricted cash at the end of period</b>	<b>\$ 406,077</b>	<b>\$ 242,000</b>

We have incurred losses and cumulative negative cash flows from operations since our business was founded by our predecessor entity AMT Therapeutics (“AMT”) Holding N.V. in 1998. We had a net loss of \$23.6 million and \$82.8 million during the three and nine months ended September 30, 2019, respectively, compared to a net loss of \$22.0 million and \$61.4 million during the same periods in 2018, respectively. As of September 30, 2019, we had an accumulated deficit of \$618.3 million.

### Sources of liquidity

From our first institutional venture capital financing in 2006 through September 30, 2019, we funded our operations primarily through private and public placements of equity securities and convertible and other debt securities as well as payments from our collaboration partners.

On December 6, 2018, we signed an amendment to the Second Amended and Restated Loan and Security Agreement with Hercules Technology Growth Capital Inc. (“Hercules”) that both refinanced our existing \$20 million credit facility and provided us with an additional commitment of \$30 million (of which \$15 million is subject to the discretion of Hercules) (the “2018 Amended Facility”). At signing, we drew down an additional \$15 million, for a total outstanding amount of \$35 million. We have the right to draw another \$15 million through June 30, 2020 subject to the terms of the 2018 Amended Facility.

The 2018 Amended Facility extends the loan's maturity date until June 1, 2023. The interest-only period was initially extended from November 2018 to January 1, 2021. The interest-only period was further extended to January 1, 2022 as a result of raising more than \$90.0 million in equity financing in September 2019. As of September 30, 2019, and December 31, 2018, \$35 million was outstanding. We are required to repay the facility in equal monthly installments of principal and interest between the end of the interest-only period and the maturity date. The variable interest rate is equal to the greater of (i) 8.85% or (ii) 8.85% plus the prime rate less 5.50%. Under the 2018 Amended Facility, we paid a facility fee equal to 0.50% of the \$35 million loan outstanding and will owe a back-end fee of 4.95% of the outstanding debt. In addition, in May 2020 the Company owes a back-end fee of 4.85% of \$20 million, which is the amount of debt raised under the 2016 Amended Facility.

On September 10, 2019, we completed a follow-on public offering 4,891,305 ordinary shares at a public offering price of \$46.00 per ordinary share, and on September 13, 2019, we completed the sale of an additional 733,695 ordinary shares at a public offering price of \$46.00 per ordinary share, pursuant to the exercise by the underwriters of the option to purchase additional ordinary shares, resulting in gross proceeds to us of \$258.8 million. The net proceeds to us from this offering were \$242.8 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We deducted \$0.4 million of expenses incurred related to this offering from additional paid-in capital.

On May 7, 2018, we completed a follow-on public offering of 5,175,000 ordinary shares at a public offering price of \$28.50 per ordinary share, resulting in gross proceeds to us of \$147.5 million. The net proceeds to us from this offering were \$138.5 million, after deducting underwriting discounts and commissions and other estimated offering expenses payable by us. We deducted \$0.2 of million expenses incurred related to this offering from additional paid-in capital.

We expect to continue to incur losses and to generate negative cash flows. We have no firm sources of additional funding. Until such time, if ever, as we can generate substantial cash flows from successfully commercializing our product candidates, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution and licensing arrangements.

We are subject to covenants under our Loan Agreement with Hercules and may become subject to covenants under any future indebtedness that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business. In addition, our pledge of assets as collateral to secure our obligations under the Hercules loan agreement may limit our ability to obtain debt financing. To the extent we need to finance our cash needs through equity offerings or debt financings, such financing may be subject to unfavorable terms including without limitation, the negotiation and execution of definitive documentation, as well as credit and debt market conditions, and we may not be able to obtain such financing on terms acceptable to us or at all. If financing is not available when needed, including through debt or equity financings, or is available only on unfavorable terms, we may be unable to meet our cash needs. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, which could have a material adverse effect on our business, financial conditions, results of operations and cash flows.

### ***Net Cash used in operating activities***

Net cash used in operating activities was \$72.7 million for the nine months ended September 30, 2019, and consisted of a net loss of \$82.8 million adjusted for non-cash items, including depreciation and amortization expense of \$4.9 million, share-based compensation expense of \$12.8 million, fair value loss of derivative financial instruments of \$0.9 million, unrealized foreign exchange gain of \$6.7 million, and a decrease in unamortized deferred revenue of \$3.4 million. Net cash used in operating activities also included changes in operating assets and liabilities of \$1.6 million. These changes primarily related to a net increase in accounts receivable and accrued income, prepaid expenses and other current assets of \$2.9 million and a net increase in accounts payable, accrued expenses and other liabilities of \$4.5 million primarily related to our Etranacogene dezaparvovec and AMT-130 clinical trials.

Net cash used in operating activities was \$58.0 million for the nine months ended September 30, 2018, and consisted of a net loss of \$61.4 million adjusted for non-cash items, including depreciation and amortization expense of \$10.4 million, share-based compensation expense of \$7.4 million, fair value gain of derivative financial instruments and contingent consideration of \$3.5 million, unrealized foreign exchange gain of \$4.2 million, deferred tax expense of \$0.2 million, a decrease in lease incentive of \$0.3 million, and a decrease in unamortized deferred revenue of \$7.1 million. Net cash used in operating activities also included changes in operating assets and liabilities of \$0.5 million.

### ***Net cash used in investing activities***

In the nine months ended September 30, 2019, we used \$4.7 million in our investing activities compared to \$3.5 million for the same period in 2018.

	Nine months ended September 30,	
	2019	2018
	(in thousands)	
Build out of Lexington site	\$ (2,754)	\$ (1,208)
Build out of Amsterdam site	(934)	(597)
Acquisition of licenses and patents	(996)	(1,698)
<b>Total investments</b>	<b>\$ (4,684)</b>	<b>\$ (3,503)</b>

Investments in our Lexington site in 2019 primarily related to leasehold improvements for the expansion as well as changes to our existing Lexington facility.

### ***Net cash generated from financing activities***

During the nine months ended September 30, 2019, we received \$4.0 million from the exercise of options to purchase ordinary shares in relation to our share incentive plans compared to \$4.6 million in the same period 2018.

We received net proceeds of \$243.0 million associated with our follow-on offering in September 2019 and received net proceeds of \$138.5 million associated with our follow-on offering in May 2018.

### ***Funding requirements***

We believe our cash and cash equivalents as of September 30, 2019 will enable us to fund our operating expenses including our debt repayment obligations as they become due and capital expenditure requirements into mid- 2022. Our future capital requirements will depend on many factors, including but not limited to:

- the cost and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution of any of our product candidates for which we receive marketing approval in the future;
- the amount and timing of revenue, if any, we receive from commercial sales of any product candidates for which we, or our collaboration partner, receives marketing approval in the future;
- the scope, timing, results and costs of our current and planned clinical trials, including those for Etranacogene dezaparvovec in hemophilia B and AMT-130 in Huntington's disease;
- the scope, timing, results and costs of preclinical development and laboratory testing of our additional product candidates;
- the need for additional resources and related recruitment costs to support the preclinical and clinical development of our product candidates;

- the need for any additional tests, studies, or trials beyond those originally anticipated to confirm the safety or efficacy of our product candidates and technologies;
- the cost, timing and outcome of regulatory reviews associated with our product candidates;
- our ability to enter into collaboration arrangements in the future;
- the costs and timing of preparing, filing, expanding, acquiring, licensing, maintaining, enforcing and prosecuting patents and patent applications, as well as defending any intellectual property-related claims;
- the repayments of the principal amount of our venture debt loan with Hercules, which will contractually start in January 2022 and will run through June 2023;
- the extent to which we acquire or in-license other businesses, products, product candidates or technologies; and
- the costs associated with maintaining quality compliance and optimizing our manufacturing processes, including the operating costs associated with our Lexington, Massachusetts manufacturing facility.

### **Contractual obligations and commitments**

The table below sets forth our contractual obligations and commercial commitments as of September 30, 2019, that are expected to have an impact on liquidity and cash flows in future periods.

	Less than 1 year	Between 1 and 2 years	Between 2 and 5 years (in thousands)	Over 5 years	Total
Debt obligations (including \$12.3 million interest payments)	\$ 4,119	\$ 3,141	\$ 40,054	\$ —	\$ 47,314
Operating lease obligations	5,411	5,506	16,761	35,305	62,983
<b>Total</b>	<b>\$ 9,530</b>	<b>\$ 8,647</b>	<b>\$ 56,815</b>	<b>\$ 35,305</b>	<b>\$ 110,297</b>

We also have obligations to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a Biologics License Application, approval by the FDA or product launch). We have not included these commitments on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed and determinable.

We enter into contracts in the normal course of business with clinical research organizations (“CROs”) for preclinical research studies and clinical trials, research supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

### **Off-Balance Sheet Arrangements**

As of September 30, 2019, we did not have any off-balance sheet arrangements as defined in Item 303(a) (4) of Regulation S-K.

**Item 3. Quantitative and Qualitative Disclosures about Market Risk**

We are exposed to a variety of financial risks in the normal course of our business, including market risk (including currency, price and interest rate risk), credit risk and liquidity risk. Our overall risk management program focuses on preservation of capital and the unpredictability of financial markets and has sought to minimize potential adverse effects on our financial performance and position.

Our market risks and exposures to such market risks during the nine months ended September 30, 2019, have not materially changed from our market risks and our exposure to market risk discussed in Part II, Item 7A of our [Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 28, 2019](#).

**Item 4. Controls and Procedures**

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

Our management, with the participation of our chief executive and chief financial officer (“CEO”), evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the “Exchange Act”)) as of September 30, 2019. Based on such evaluation, our CEO has concluded that as of September 30, 2019, our disclosure controls and procedures were effective to ensure that information required to be disclosed by it in reports the Company files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC, and that such material information is accumulated and communicated to the Company’s management, including its Principal Executive Officer and Principal Financial Officer, to allow timely decisions regarding required disclosure. Because of the inherent limitations in all control systems, any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management necessarily is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Furthermore, the Company’s controls and procedures can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of such control, and misstatements due to error or fraud may occur and not be detected on a timely basis.

***Changes in Internal Control over Financial Reporting***

During the third quarter of 2019, there was no change in our internal control over financial reporting (as defined in Rule 13a-15(f) under the Exchange Act) that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## Part II – OTHER INFORMATION

### Item 1. Legal Proceedings

None.

### Item 1A. Risk Factors

*An investment in our ordinary shares involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our financial statements and related notes thereto, and the risk factors discussed in Part I, Item 1A “Risk Factors” in our [Annual Report on Form 10-K for the year ended December 31, 2018, filed with the SEC on February 28, 2019](#), before deciding to invest in our ordinary shares. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.*

#### **Risks Related to the Development of Our Product Candidates**

***None of our product candidates have been approved for commercial sale and they might never receive regulatory approval or become commercially viable. We have never generated any revenue from product sales and may never be profitable.***

All of our product candidates are in research or development. We have not generated any revenues from the sale of products and do not expect to do so for at least the next several years. Our lead product candidates, Etranacogene dezaparovec (also known as AMT-061) and AMT-130, and any of our other potential product candidates will require extensive preclinical and/or clinical testing and regulatory approval prior to commercial use. Our research and development efforts may not be successful. Even if our clinical development efforts result in positive data, our product candidates may not receive regulatory approval or be successfully introduced and marketed at prices that would permit us to operate profitably.

***We may encounter substantial delays in and impediments to the progress of our clinical trials or fail to demonstrate the safety and efficacy of our product candidates.***

Clinical and non-clinical development is expensive, time-consuming and uncertain as to outcome. Our product candidates are in different stages of clinical or preclinical development, and there is a significant risk of failure or delay in each of these programs. We cannot guarantee that any preclinical tests or clinical trials will be completed as planned or completed on schedule, if at all. A failure of one or more preclinical tests or clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include, but are not limited to:

- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations (“CROs”) and clinical trial sites;
- delays in receiving regulatory authorization to conduct the clinical trials or a regulatory authority decision that the clinical trial should not proceed;
- delays in obtaining required IRB approval at each clinical trial site;
- imposition of a clinical hold by regulatory agencies after an inspection of our clinical trial operations or trial sites;
- failure by CROs, other third parties or us to adhere to clinical trial requirements or otherwise properly manage the clinical trial process, including meeting applicable timelines, properly documenting case files, including the retention of proper case files, and properly monitoring and auditing clinical sites;
- failure of sites or clinical investigators to perform in accordance with Good Clinical Practice or applicable regulatory guidelines in other countries;
- difficulty or delays in patient recruiting into clinical trials;
- delays or deviations in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;

- delays in having patients' complete participation in a study or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a study;
- occurrence of serious adverse events associated with a product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols, undertaking additional new tests or analyses or submitting new types or amounts of clinical data.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Such trials and regulatory review and approval take many years. It is impossible to predict when or if any of our clinical trials will demonstrate that product candidates are effective or safe in humans.

If the results of our clinical trials are inconclusive, or fail to meet the level of statistical significance required for approval or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in or altogether prevented from obtaining marketing approval for our product candidates;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Because of the nature of the gene therapies we are developing, regulators may also require us to demonstrate long-term gene expression, clinical efficacy and safety, which may require additional or longer clinical trials, and which may not be able to be demonstrated to the regulatory authorities' standards.

Our ability to recruit patients for our trials is often reliant on third parties, such as clinical trial sites. Clinical trial sites may not have the adequate infrastructure established to handle gene therapy products or may have difficulty finding eligible patients to enroll into a trial.

In addition, we or any collaborators we may have may not be able to locate and enroll enough eligible patients to participate in these trials as required by the FDA, the EMA or similar regulatory authorities outside the United States and the European Union. This may result in our failure to initiate or continue clinical trials for our product candidates, or may cause us to abandon one or more clinical trials altogether. Because our programs are focused on the treatment of patients with rare or orphan or ultra-orphan diseases, our ability to enroll eligible patients in these trials may be limited or slower than we anticipate considering the small patient populations involved and the specific age range required for treatment eligibility in some indications. In addition, our potential competitors, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions, may seek to develop competing therapies, which would further limit the small patient pool available for our studies. Also, patients may be reluctant to enroll in gene therapy trial where there are other therapeutic alternatives available or that may become available, which may be for various reasons including uncertainty about the safety or effectiveness of the therapeutic and the possibility that treatment with the therapeutic would preclude future gene therapy treatments.

Any inability to successfully initiate or complete preclinical and clinical development could result in additional costs to us or impair our ability to receive marketing approval, to generate revenues from product sales or obtain regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, including changes in the vector or manufacturing process used, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may materially harm our business, financial conditions and results of operations.



***Our progress in early-stage clinical trials may not be indicative of long-term efficacy in late-stage clinical trials, and our progress in trials for one product candidate may not be indicative of progress in trials for other product candidates.***

Study designs and results from previous studies are not necessarily predictive of our future clinical study designs or results, and initial results may not be confirmed upon full analysis of the complete study data. Our product candidates may fail to show the required level of safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies. In 2017, we announced our plans to advance Etranacogene dezaparvovec, which includes an AAV5 vector carrying the FIX-Padua transgene, into a pivotal study. While we believe Etranacogene dezaparvovec and AMT-060, our product candidate that was previously studied in a Phase I/II study, have been demonstrated to be materially comparable in nonclinical studies and manufacturing quality assessments, it is possible that ongoing or future clinical studies of Etranacogene dezaparvovec may show unexpected differences from AMT-060. Should these differences have an unfavorable impact on clinical outcomes, they may adversely impact our ability to achieve regulatory approval or market acceptance of Etranacogene dezaparvovec.

In our Phase I/II clinical study of AMT-060, we screened patients for preexisting anti-AAV5 antibodies to determine their eligibility for the trial. Three of the ten patients screened for the study tested positive for anti-AAV5 antibodies on reanalysis using a more sensitive antibody assay. Since we did not observe any ill-effects or correlation between the level of anti-AAV5 antibodies and clinical outcomes, patients who have anti-AAV5 antibodies are permitted to enroll in our planned pivotal study of Etranacogene dezaparvovec. Since we only have been able to test a limited number of patients and have limited clinical and pre-clinical data, it is possible that ongoing or future clinical studies may not confirm these results, and if so, negatively impact the outcome of our study.

In advance of treating patients in the pivotal study of Etranacogene dezaparvovec, we conducted a short study to confirm the dose expected to be used in the pivotal trial. The dose-confirmation study enrolled three patients, who were administered a single dose of  $2 \times 10^{13}$  gc/kg. We have relied on the short-term data from this study, including FIX activity and safety outcomes during the weeks following administration of Etranacogene dezaparvovec, to confirm the dose to be used in the pivotal study. Following the results of this study, our Data Monitoring Committee confirmed the dose of  $2 \times 10^{13}$  gc/kg for administration in the pivotal study. Given the limited number of patients and short follow-up period, data from this study may differ materially from the future results of our planned pivotal study of Etranacogene dezaparvovec.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results during clinical trials, if these results are not reproducible or if our products show diminishing activity over time, our product candidates may not receive approval from the FDA or EMA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections because of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in later-stage clinical trials with larger patient populations could have a material adverse effect on our business, financial condition and results of operations.

***Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy (“RMAT”) designation by the FDA, or access to the PRIME scheme by the EMA, for our product candidates may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.***

We have obtained and may in the future seek one or more of fast track designation, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition and which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA, similar to the FDA’s Breakthrough Therapy Designation, to enhance support for the development of medicines that target an unmet medical need.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten to twelve months under standard review. RMAT designations may also expedite product candidate development and approval.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the relevant criteria, the agency may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

***We may not be successful in our efforts to use our gene therapy technology platform to build a pipeline of additional product candidates.***

An element of our strategy is to use our gene therapy technology platform to expand our product pipeline and to progress these candidates through preclinical and clinical development ourselves or together with collaborators. Although we currently have a pipeline of programs at various stages of development, we may not be able to identify or develop product candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. Research programs to identify new product candidates require substantial technical, financial and human resources. We or any collaborators may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. If we do not continue to successfully develop and commercialize product candidates based upon our technology, we may face difficulty in obtaining product revenues in future periods, which could result in significant harm to our business, results of operations and financial position and materially adversely affect our share price.

***Our strategy of obtaining rights to key technologies through in-licenses may not be successful.***

We seek to expand our product pipeline from time to time in part by in-licensing the rights to key technologies, including those related to gene delivery, genes and gene cassettes. The future growth of our business will depend in significant part on our ability to in-license or otherwise acquire the rights to additional product candidates or technologies, particularly through our collaborations with academic research institutions. However, we may be unable to in-license or acquire the rights to any such product candidates or technologies from third parties on acceptable terms or at all. The in-licensing and acquisition of these technologies is a competitive area, and many more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be competitors may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our areas of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business, financial condition and prospects could suffer.

***Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain marketing approvals for our product candidates.***

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. The risk of cancer remains a concern for gene therapy, and we cannot assure that it will not occur in any of our planned or future clinical studies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

As of September 30, 2019, a total of three patients reported serious adverse events related to the treatment of AMT-060 in our Phase I/II hemophilia B trial, including one patient with a short, self-limiting fever in the first 24 hours after treatment and two patients with mild, asymptomatic elevations in liver transaminases. Additionally, one patient in our ongoing Phase IIb study of Etranacogene dezaparvovec underwent hip surgery due to a pre-existing condition and was treated perioperatively with short-acting factor replacement. This was reported by the investigator as a serious adverse event unrelated to Etranacogene dezaparvovec.

Adverse events in our clinical trials or those conducted by other parties (even if not ultimately attributable to our product candidates), and the resulting publicity, could result in increased governmental regulation, unfavorable public perception, failure of the medical community to accept and prescribe gene therapy treatments, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. If any of these events should occur, it may have a material adverse effect on our business, financial condition and results of operations.

#### **Risks Related to Our Manufacturing**

***Our manufacturing facility is subject to significant government regulations and approvals. If we fail to comply with these regulations or maintain these approvals our business will be materially harmed.***

Our manufacturing facility in Lexington is subject to ongoing regulation and periodic inspection by the FDA, EMA and other regulatory bodies to ensure compliance with current Good Manufacturing Practices (“cGMP”). Any failure to follow and document our adherence to such cGMP regulations or other regulatory requirements may lead to significant delays in the availability of products for commercial sale or clinical study, may result in the termination of or a hold on a clinical study, or may delay or prevent filing or approval of marketing applications for our products.

Failure to comply with applicable regulations could also result in the FDA, EMA or other applicable authorities taking various actions, including levying fines and other civil penalties; imposing consent decrees or injunctions; requiring us to suspend or put on hold one or more of our clinical trials; suspending or withdrawing regulatory approvals; delaying or refusing to approve pending applications or supplements to approved applications; requiring us to suspend manufacturing activities or product sales, imports or exports; requiring us to communicate with physicians and other customers about concerns related to actual or potential safety, efficacy, and other issues involving our products; mandating product recalls or seizing products; imposing operating restrictions; and seeking criminal prosecutions. Any of the foregoing could materially harm our business, financial condition and results of operations.

***Gene therapies are complex and difficult to manufacture. We could experience capacity, production or technology transfer problems that result in delays in our development or commercialization schedules or otherwise adversely affect our business.***

The insect-cell based manufacturing process we use to produce our products and product candidates is highly complex and in the normal course is subject to variation or production difficulties. Issues with any of our manufacturing processes, even minor deviations from the normal process, could result in insufficient yield, product deficiencies or manufacturing failures that result in adverse patient reactions, lot failures, insufficient inventory, product recalls and product liability claims. Additionally, we may not be able to scale up some or all of our manufacturing processes that may result in delays in regulatory approvals or otherwise adversely affect our ability to manufacture sufficient amounts of our products.

Many factors common to the manufacturing of most biologics and drugs could also cause production interruptions, including raw materials shortages, raw material failures, growth media failures, equipment malfunctions, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or acts of god beyond our control. We also may encounter problems in hiring and retaining the experienced specialized personnel needed to operate our manufacturing process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing processes or facilities could make us a less attractive collaborator for academic research institutions and other parties, which could limit our access to additional attractive development programs, result in delays in our clinical development or marketing schedules and materially harm our business.

***Our use of viruses, chemicals and other hazardous materials requires us to comply with regulatory requirements and exposes us to significant potential liabilities.***

Our development and manufacturing processes involve the use of viruses, chemicals, other (potentially) hazardous materials and produce waste products. Accordingly, we are subject to national, federal, state and local laws and regulations in the United States and the Netherlands governing the use, manufacture, distribution, storage, handling, treatment and disposal of these materials. In addition to ensuring the safe handling of these materials, applicable requirements require increased safeguards and security measures for many of these agents, including controlling access and screening of entities and personnel who have access to them, and establishing a comprehensive national database of registered entities. In the event of an accident or failure to comply with environmental, occupational health and safety and export control laws and regulations, we could be held liable for damages that result, and any such liability could exceed our assets and resources, and could result in material harm to our business, financial condition and results of operations.

**Risks Related to Regulatory Approval of Our Products**

***We cannot predict when or if we will obtain marketing approval to commercialize a product candidate.***

The development and commercialization of our product candidates, including their design, testing, manufacture, safety, efficacy, purity, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States, the EMA and other regulatory agencies of the member states of the European Union, and similar regulatory authorities in other jurisdictions. Failure to obtain marketing approval for a product candidate in a specific jurisdiction will prevent us from commercializing the product candidate in that jurisdiction.

The process of obtaining marketing approval for our product candidates in the United States, the European Union and other countries is expensive and may take many years, if approval is obtained at all. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application, may decide that our data are insufficient for approval, may require additional preclinical, clinical or other studies and may not complete their review in a timely manner. Further, any marketing approval we ultimately obtain may be for only limited indications or be subject to stringent labeling or other restrictions or post-approval commitments that render the approved product not commercially viable.

If we experience delays in obtaining marketing approval for any of our product candidates in the United States, the European Union or other countries, the commercial prospects of our other product candidates may be harmed and our ability to generate revenues will be materially impaired.

***The risks associated with the marketing approval process are heightened by the status of our products as gene therapies.***

We believe that all our current product candidates will be viewed as gene therapy products by the applicable regulatory authorities. While there are a number of gene therapy product candidates under development, in the United States, FDA has only approved a limited number of gene therapy products, to date. Accordingly, regulators, like FDA, may have limited experience with the review and approval of marketing applications for gene therapy products.

Both the FDA and EMA have demonstrated caution in their regulation of gene therapy treatments, and ethical and legal concerns about gene therapy and genetic testing may result in additional regulations or restrictions on the development and commercialization of our product candidates that are difficult to predict. The FDA and the EMA have issued various guidance documents pertaining to gene therapy products, with which we likely must comply to gain regulatory approval of any of our product candidates in the United States or European Union, respectively. The close regulatory scrutiny of gene therapy products may result in delays and increased costs, and may ultimately lead to the failure to obtain approval for any gene therapy product.

Regulatory requirements affecting gene therapy have changed frequently and continue to evolve, and agencies at both the U.S. federal and state level, as well as congressional committees and foreign governments, have sometimes expressed interest in further regulating biotechnology. In the United States, there have been a number of recent changes relating to gene therapy development. By example, FDA issued a number of new guidance documents on human gene therapy development, one of which was specific to human gene therapy for hemophilia and another of which was specific to rare diseases. Moreover, the U.S. National Institutes of Health, which also has authority over research involving gene therapy products, issued a proposed rule in October 2018, seeking to streamline the oversight

of such protocols and reduce duplicative reporting requirements that are already captured within existing regulatory frameworks. Moreover, the European Commission conducted a public consultation in early 2013 on the application of EU legislation that governs advanced therapy medicinal products, including gene therapy products, which could result in changes in the data we need to submit to the EMA for our product candidates to gain regulatory approval or change the requirements for tracking, handling and distribution of the products which may be associated with increased costs. In addition, divergent scientific opinions among the various bodies involved in the review process may result in delays, require additional resources and ultimately result in rejection. The FDA, EMA, and other regulatory authorities will likely continue to revise and further update its approach to gene therapies in the coming years. These regulatory agencies, committees and advisory groups and the new regulations and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenues to maintain our business.

***Our failure to obtain or maintain orphan product exclusivity for any of our product candidates for which we seek this status could limit our commercial opportunity, and if our competitors are able to obtain orphan product exclusivity before we do, we may not be able to obtain approval for our competing products for a significant period.***

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the relevant indication, the product is entitled to a period of market exclusivity, which precludes the FDA or EMA from approving another marketing application for the same drug for the same indication for that period. The FDA and EMA, however, may subsequently approve a similar drug for the same indication during the first product's market exclusivity if the FDA or EMA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective, or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition or if the incidence and prevalence of patients who are eligible to receive the drug in these markets materially increase. The inability to obtain or failure to maintain adequate product exclusivity for our product candidates could have a material adverse effect on our business prospects, results of operations and financial conditions.

***As appropriate, we intend to seek all available periods of regulatory exclusivity for our product candidates. However, there is no guarantee that we will be granted these periods of regulatory exclusivity or that we will be able to maintain these periods of exclusivity.***

The FDA grants product sponsors certain periods of regulatory exclusivity, during which the agency may not approve, and in certain instances, may not accept, certain marketing applications for competing drugs. For example, biologic product sponsors may be eligible for twelve years of exclusivity from the date of approval, seven years of exclusivity for drugs that are designated to be orphan drugs, and/or a six-month period of exclusivity added to any existing exclusivity period or patent life for the submission of FDA requested pediatric data. While we intend to apply for all periods of market exclusivity that we may be eligible for, there is no guarantee that we will receive all such periods of market exclusivity. Additionally, under certain circumstances, the FDA may revoke the period of market exclusivity. Thus, there is no guarantee that we will be able to maintain a period of market exclusivity, even if granted. In the case of orphan designation, other benefits, such as tax credits and exemption from user fees may be available. If we are not able to obtain or maintain orphan drug designation or any period of market exclusivity to which we may be entitled, we will be materially harmed, as we will potentially be subject to greater market competition and may lose the benefits associated with programs.

#### **Risks Related to Commercialization**

***If we are unable to successfully commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.***

Our ability to generate product revenues will depend on the successful development and eventual commercialization of our product candidates. The success of our product candidates will depend on many factors, including:

- successful completion of preclinical studies and clinical trials;
- receipt and maintenance of marketing approvals from applicable regulatory authorities;
- our ability to timely manufacture sufficient quantities according to required quality specifications;

- obtaining and maintaining patent and trade secret protection and non-patent, orphan drug exclusivity for our product candidates;
- obtaining and maintaining regulatory approvals using our manufacturing facility in Lexington, Massachusetts;
- launch and commercialization of our products, if approved, whether alone or in collaboration with others;
- identifying and engaging effective distributors or resellers on acceptable terms in jurisdictions where we plan to utilize third parties for the marketing and sales of our product candidates;
- acceptance of our products, if approved, by patients, the medical community and third-party payers;
- effectively competing with existing therapies and gene therapies based on safety and efficacy profile;
- achieve value-based pricing levels based on durability of expression, safety and efficacy;
- obtaining and maintaining healthcare coverage and adequate reimbursement;
- complying with any applicable post-approval requirements and maintaining a continued acceptable overall safety profile; and
- obtaining adequate reimbursement for the total patient population and each subgroup to sustain a viable commercial business model in U.S. and EU markets.

Failure to achieve or implement any of these elements could result in significant delays or an inability to successfully commercialize our product candidates, which could materially harm our business.

***The affected populations for our gene therapies may be smaller than we or third parties currently project, which may affect the size of our addressable markets.***

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our therapies, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunities for these therapies will ultimately depend upon many factors, including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient consent, patient access and product pricing and reimbursement.

Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, reimbursement may not be sufficient to sustain a viable business for all sub populations being studied, or new patients may become increasingly difficult to identify or access, any of which would adversely affect our results of operations and our business.

The addressable markets for AAV-based gene therapies may be impacted by the prevalence of neutralizing antibodies to the capsids, which are an integral component of our gene therapy constructs. Patients that have pre-existing antibodies to a particular capsid may not be eligible for administration of a gene therapy that includes this particular capsid. For example, Etranacogene dezaparvovec, our gene therapy candidate for hemophilia B patients, incorporates an AAV5 capsid. In our Phase I/II clinical study of AMT-060, we screened patients for preexisting anti-AAV5 antibodies to determine their eligibility for the trial. Three of the ten patients screened for the study tested positive for anti-AAV5 antibodies on reanalysis. However, we did not observe any ill-effects or correlation between the level of anti-AAV5 antibodies and clinical outcomes in these three patients, suggesting that patients who have anti-AAV5 antibodies may still be eligible for AAV5-based gene therapies. Since we only have been able to test a limited number of patients and have limited clinical and pre-clinical data, it is possible that future clinical studies may not confirm these results. This may limit the addressable market for Etranacogene dezaparvovec and any future revenues derived from the sale of the product, if approved.

***Any approved gene therapy we seek to offer may fail to achieve the degree of market acceptance by physicians, patients, third party payers and others in the medical community necessary for commercial success.***

Doctors may be reluctant to accept a gene therapy as a treatment option or, where available, choose to continue to rely on existing treatments. The degree of market acceptance of any of our product candidates that receive marketing approval in the future will depend on many factors, including:

- the efficacy and potential advantages of our therapies compared with alternative treatments;
- our ability to convince payers of the long-term cost-effectiveness of our therapies and, consequently, the availability of third-party coverage and adequate reimbursement;



- the cost of treatment with gene therapies, including ours, in comparison to traditional chemical and small-molecule treatments
- the limitations on use and label requirements imposed by regulators;
- the convenience and ease of administration of our gene therapies compared with alternative treatments;
- the willingness of the target patient population to try new therapies, especially a gene therapy, and of physicians to administer these therapies;
- the strength of marketing and distribution support;
- the prevalence and severity of any side effects;
- limited access to site of service that can perform the product preparation and administer the infusion; and
- any restrictions by regulators on the use of our products.

A failure to gain market acceptance for any of the above reasons, or any reasons at all, by a gene therapy for which we receive regulatory approval would likely hinder our ability to recapture our substantial investments in that and other gene therapies and could have a material adverse effect on our business, financial condition and results of operation.

***We face substantial competition, and others may discover, develop or commercialize competing products before or more successfully than we do.***

The development and commercialization of new biotechnology and biopharmaceutical products, including gene therapies, is highly competitive. We may face intense competition with respect to our product candidates, as well as with respect to any product candidates that we may seek to develop or commercialize in the future, from large and specialty pharmaceutical companies and biotechnology companies worldwide, who currently market and sell products or are pursuing the development of products for the treatment of many of the disease indications for which we are developing our product candidates. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. In recent years, there has been a significant increase in commercial and scientific interest and financial investment in gene therapy as a therapeutic approach, which has intensified the competition in this area.

We are aware of numerous companies focused on developing gene therapies in various indications, including Applied Genetic Technologies Corp, Abeona Therapeutics, Adverum Biotechnologies, Allergan, Ally Therapeutics, Asklepios BioPharmaceutical, Audentes Therapeutics, AVROBIO, Axovant Sciences, Bayer, Biogen, BioMarin, bluebird bio, CRISPR Therapeutics, Editas Medicine, Expression Therapeutics, Freeline Therapeutics, Generation Bio, Genethon, GlaxoSmithKline, Homology Medicines, Intellia Therapeutics, Johnson & Johnson, Krystal Biotech, LogicBio Therapeutics, Lysogene, MeiraGTx, Milo Biotechnology, Mustang Bio, Novartis, Orchard Therapeutics, Oxford Biomedica, Pfizer, REGENXBIO, Renova Therapeutics, Rocket Pharmaceuticals, Sangamo BioSciences, Sanofi, Selecta Biosciences, Sarepta, Shire, Solid Biosciences, Spark Therapeutics, Takeda, Ultragenyx, Vivet Therapeutics, and Voyager, as well as several companies addressing other methods for modifying genes and regulating gene expression. We may also face competition with respect to the treatment of some of the diseases that we are seeking to target with our gene therapies from protein, nucleic acid, antisense, RNAi and other pharmaceuticals under development or commercialized at pharmaceutical and biotechnology companies such as Alnylam, Amgen, Bayer, Biogen, BioMarin, CSL Behring, Dicerna, Ionis, Novartis, Novo Nordisk, Pfizer, Translate Bio, Roche, Sanofi, Shire, Sobi, Wave Biosciences and numerous other pharmaceutical and biotechnology firms.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than the products that we develop. Our competitors also may obtain FDA, EMA or other regulatory approval for their products more rapidly than we do, which could result in our competitors establishing a strong market position before we are able to enter the market. Because we expect that gene therapy patients may generally require only a single administration, we believe that the first gene therapy product to enter the market for a particular indication will likely enjoy a significant commercial advantage, and may also obtain market exclusivity under applicable orphan drug regimes.

Many of the companies with which we are competing or may compete in the future have significantly greater financial resources and expertise than we do in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

#### **Risks Related to Our Dependence on Third Parties**

***Our ongoing discussions with BMS to restructure the terms of our collaboration may not be successful or may result in material changes to these arrangements.***

The research term of our collaboration and license agreement with BMS expired in May 2019, and we are currently in discussions with BMS potentially to restructure that agreement and the other related agreements to eliminate, reduce or alter our obligations under the collaboration. Our discussions are ongoing and may or may not result in any restructuring or changes to our collaboration. If a restructuring of our collaboration with BMS were to be concluded, we expect it would result in a termination or amendment of existing agreements, or the execution of new agreements that collectively could include changes in the number of future collaboration targets that may be designated by BMS, the exclusivity provisions related to collaboration targets, our obligations to provide manufacturing services for collaboration targets, as well as changes in or the elimination of our economic rights on collaboration targets, milestone payments, and BMS's warrants to purchase our ordinary shares, among other potential matters. Any such restructuring, if concluded, may include additional or different provisions from those described above, and may include economic or other terms that are less advantageous for us.

Because the outcome of these discussions is unknown, we have not taken into account the impact of such restructuring, if any, on the timing of recognizing prepaid license revenue, or any other potential financial metrics, in our consolidated financial statements. We will account for any potential changes if and when the agreements are restructured.

***We rely on third parties for important aspects of our development programs. If these parties do not perform successfully or if we are unable to enter into or maintain key collaboration or other contractual arrangements, our business could be adversely affected.***

We have in the past entered into, and expect in the future to enter into, collaborations with other companies and academic research institutions with respect to important elements of our development programs.

Any collaboration, may pose several risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- we may have limited or no control over the design or conduct of clinical trials sponsored by collaborators;
- we may be hampered from entering into collaboration arrangements if we are unable to obtain consent from our licensors to enter into sublicensing arrangements of technology we have in-licensed;
- if any collaborator does not conduct the clinical trials they sponsor in accordance with regulatory requirements or stated protocols, we will not be able to rely on the data produced in such trials in our further development efforts;
- collaborators may not perform their obligations as expected;
- collaborators may also have relationships with other entities, some of which may be our competitors;
- collaborators may not pursue development and commercialization of any product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could develop, independently or with third parties, products that compete directly or indirectly with our products or product candidates, if, for instance, the collaborators believe that competitive products



are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- our collaboration arrangements may impose restrictions on our ability to undertake other development efforts that may appear to be attractive to us;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights that achieves regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including over proprietary rights, contract interpretation or the preferred course of development, could cause delays or termination of the research, development or commercialization of product candidates, lead to additional responsibilities for us, delay or impede reimbursement of certain expenses or result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our rights or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may in some cases be terminated for the convenience of the collaborator and, if terminated, we could be required to expend additional funds to pursue further development or commercialization of the applicable product or product candidates.

If any collaboration does not result in the successful development and commercialization of products or if a collaborator were to terminate an agreement with us, we may not receive future research funding or milestone or royalty payments under that collaboration, and we may lose access to important technologies and capabilities of the collaboration. All the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of any development collaborators.

#### **Risks Related to Our Intellectual Property**

***We rely on licenses of intellectual property from third parties, and such licenses may not provide adequate rights or may not be available in the future on commercially reasonable terms or at all, and our licensors may be unable to obtain and maintain patent protection for the technology or products that we license from them.***

We currently are heavily reliant upon licenses of proprietary technology from third parties that is important or necessary to the development of our technology and products, including technology related to our manufacturing process, our vector platform, our gene cassettes and the therapeutic genes of interest we are using. These and other licenses may not provide adequate rights to use such technology in all relevant fields of use. Licenses to additional third-party technology that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, which could have a material adverse effect on our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. In addition, some of our agreements with our licensors require us to obtain consent from the licensor before we can enforce patent rights, and our licensor may withhold such consent or may not provide it on a timely basis. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. In addition, if third parties who license patents to us fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

***Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.***

The agreements under which we license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business and financial condition.

***If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose rights that are important to our business.***

Our licensing arrangements with third parties may impose diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our counterparties may have the right to terminate these agreements either in part or in whole, in which case we might not be able to develop, manufacture or market any product that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or amended agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

***If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our ability to successfully commercialize our products may be impaired.***

We rely, in part, upon a combination of forms of intellectual property, including in-licensed and owned patents to protect our intellectual property. Our success depends in a large part on our ability to obtain and maintain this protection in the United States, the European Union and other countries, in part by filing patent applications related to our novel technologies and product candidates. Our patents may not provide us with any meaningful commercial protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. For example, patents we own currently are and may become subject to future patent opposition or similar proceedings, which may result in loss of scope of some claims or the entire patent. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

Successful challenges to our patents may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products.

The patent prosecution process is expensive, time-consuming and uncertain, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Additionally, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, EU patent law with respect to the patentability of methods of treatment of the human body is more limited than U.S. law. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after their priority date, or in some cases at all. Therefore, we cannot know with certainty whether we were the first to make the inventions or that we were the first to file for patent protection of the inventions claimed in our owned or licensed patents or pending patent applications. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the European Union, the United States or other countries may diminish the value of our patents or narrow the scope of our patent protection. Our inability to obtain and maintain appropriate patent protection for any one of our products could have a material adverse effect on our business, financial conditions and results of operations.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property or third parties may assert their intellectual property rights against us, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our owned or licensed patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, maintained in more narrowly amended form or interpreted narrowly.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, increase our operating losses, reduce available resources and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, which could have an adverse effect on the price of our ordinary shares.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business. For example, outside of the United States two of the patents we own are subject to patent opposition. If these or future oppositions are successful or if we are found to otherwise infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our products and technology. We may not be able to obtain the required license on commercially reasonable terms or at all. Even if we could obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product or otherwise to cease using the relevant intellectual property. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease or materially modify some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

For example, we are aware of patents owned by third parties that relate to some aspects of our programs that are still in development. In some cases, because we have not determined the final methods of manufacture, the method of administration or the therapeutic compositions for these programs, we cannot determine whether rights under such third party patents will be needed. In addition, in some cases, we believe that the claims of these patents are invalid or not infringed, or will expire before commercialization. However, if such patents are needed and found to be valid and infringed, we could be required to obtain licenses, which might not be available on commercially reasonable terms, or to cease or delay commercializing certain product candidates, or to change our programs to avoid infringement.

***If we are unable to protect the confidentiality of our proprietary information and know-how, the value of our technology and products could be adversely affected.***

In addition to seeking patent protection, we also rely on other proprietary rights, including protection of trade secrets, know-how and confidential and proprietary information. To maintain the confidentiality of our trade secrets and proprietary information, we enter into confidentiality agreements with our employees, consultants, collaborators and other third parties who have access to our trade secrets. Our agreements with employees also provide that any inventions conceived by the individual in the course of rendering services to us will be our exclusive property. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. In addition, in the event of unauthorized use or disclosure of our trade secrets or proprietary information, these agreements, even if obtained, may not provide meaningful protection, particularly for our trade secrets or other confidential information. To the extent that our employees, consultants or contractors use technology or know-how owned by third parties in their work for us, disputes may arise between us and those third parties as to the rights in related inventions.

Adequate remedies may not exist in the event of unauthorized use or disclosure of our confidential information including a breach of our confidentiality agreements. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time consuming, and the outcome is unpredictable. In addition, some courts in and outside of the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them from using that technology or information to compete with us. The disclosure of our trade secrets or the independent development of our trade secrets by a competitor or other third party would impair our competitive position and may materially harm our business, financial condition, results of operations, stock price and prospects.

***Our reliance on third parties may require us to share our trade secrets, which could increase the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

Because we collaborate from time to time with various organizations and academic research institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, materials transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, if we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements.

Some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us.

#### **Risks Related to Pricing and Reimbursement**

***We face uncertainty related to insurance coverage of, and pricing and reimbursement for product candidates for which we may receive marketing approval.***

We anticipate that the cost of treatment using our product candidates will be significant. We expect that most patients and their families will not be capable of paying for our products themselves. There will be no commercially viable market for our product candidates without reimbursement from third party payers, such as government health administration authorities, private health insurers and other organizations. Even if there is a commercially viable market, if the level of third-party reimbursement is below our expectations, most patients may not be able to afford treatment with our products and our revenues and gross margins will be adversely affected, and our business will be harmed.

Government authorities and other third-party payers, such as private health insurers and health maintenance organizations, decide for which medications they will pay and, subsequently, establish reimbursement levels. Reimbursement systems vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. Government authorities and third-party payers have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications and procedures. Increasingly, third party payers require drug companies to provide them with predetermined discounts from list prices, are exerting influence on decisions regarding the use of particular treatments and are limiting covered indications. Additionally, in the United States and some foreign jurisdictions, pending or potential legislative and regulatory changes regarding the healthcare system and insurance coverage, could result in more rigorous coverage criteria and downward pressure on drug prices, and may affect our ability to profitably sell any products for which we obtain marketing approval.

The pricing review period and pricing negotiations for new medicines take considerable time and have uncertain results. Pricing review and negotiation usually begins only after the receipt of regulatory marketing approval, and some authorities require approval of the sale price of a product before it can be marketed. In some markets, particularly the countries of the European Union, prescription pharmaceutical pricing remains subject to continuing direct governmental control and to drug reimbursement programs even after initial approval is granted and price reductions may be imposed. Prices of medical products may also be subject to varying price control mechanisms or limitations as part of national health systems if products are considered not cost-effective or where a drug company's profits are deemed excessive. In addition, pricing and reimbursement decisions in certain countries can lead to mandatory price reductions or additional reimbursement restrictions in other countries. Because of these restrictions, any product candidates for which we may obtain marketing approval may be subject to price regulations that delay or prohibit our or our partners' commercial launch of the product in a particular jurisdiction. In addition, we or any collaborator may elect to reduce the price of our products to increase the likelihood of obtaining reimbursement approvals. If countries impose prices, which are not sufficient to allow us or any collaborator to generate a profit, we or any collaborator may refuse to launch the product in such countries or withdraw the product from the market. If pricing is set at unsatisfactory levels, or if the price decreases, our business could be harmed, possibly materially. If we fail to obtain and sustain an adequate level of coverage and reimbursement for our products by third party payers, our ability to market and sell our products would be adversely affected and our business would be harmed.

***Due to the generally limited addressable market for our target orphan indications and the potential for our therapies to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates.***

The relatively small market size for orphan indications and the potential for long-term therapeutic benefit from a single administration present challenges to pricing review and negotiation of our product candidates for which we may obtain marketing authorization. Most of our product candidates target rare diseases with relatively small patient populations. If we are unable to obtain adequate levels of reimbursement relative to these small markets, our ability to support our development and commercial infrastructure and to successfully market and sell our product candidates for which we may obtain marketing approval will be adversely affected.

We also anticipate that many or all of our gene therapy product candidates may provide long-term, and potentially curative benefit, with a single administration. This is a different paradigm than that of other pharmaceutical therapies, which often require an extended course of treatment or frequent administration. As a result, governments and other payers may be reluctant to provide the significant level of reimbursement that we seek at the time of administration of our gene therapies or may seek to tie reimbursement to clinical evidence of continuing therapeutic benefit over time. Although it is possible that our product candidates will need to be administered only once, there may be situations in which re-administration is required, which may further complicate the pricing and reimbursement for these treatments. In addition, considering the anticipated cost of these therapies, governments and other payers may be particularly restrictive in making coverage decisions. These factors could limit our commercial success and materially harm our business.

#### **Risks Related to Our Financial Position and Need for Additional Capital**

***We have incurred significant losses to date, expect to incur losses over the next several years and may never achieve or maintain profitability.***

We had a net loss of \$82.8 million in the nine months ended September 30, 2019, \$83.3 million in the full year 2018 and \$79.3 million in the full year 2017. As of September 30, 2019, we had an accumulated deficit of \$618.3 million. To date, we have financed our operations primarily through the sale of equity securities and convertible debt, venture loans, through upfront payments from our collaboration partners and, to a lesser extent, subsidies and grants from governmental agencies and fees for services. We have devoted substantially all our financial resources and efforts to research and development, including preclinical studies and clinical trials. We expect to continue to incur significant expenses and losses over the next several years, and our net losses may fluctuate significantly from quarter to quarter and year to year.

We anticipate that our expenses will increase substantially as we:

- Build-out our commercial infrastructure and seek marketing approval for any product candidates (including Etranacogene dezaparvovec) that successfully complete clinical trials;
- Advance the clinical development of AMT-130 for our Huntington's disease gene therapy program;
- Advance multiple research programs related to gene therapy candidates targeting liver-directed and CNS diseases;
- Continue to build-out our clinical, medical and regulatory capabilities;

- Continue to expand, enhance and optimize our technology platform, including our manufacturing capabilities, next-generation viral vectors and promoters, and other enabling technologies;
- Acquire or in-license rights to new therapeutic targets or product candidates; and
- Maintain, expand and protect our intellectual property portfolio, including in-licensing additional intellectual property rights from third parties.

We may never succeed in these activities and, even if we do, may never generate revenues that are sufficient to achieve or sustain profitability. Our failure to become and remain profitable would depress the value of our company and could impair our ability to expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations.

***We will likely need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain capital when needed may force us to delay, limit or terminate our product development efforts or other operations which could have a material adverse effect on our business, financial conditions, results of operations and cash flows.***

We expect to incur significant expenses in connection with our on-going activities and that we will likely need to obtain substantial additional funding in connection with our continuing operations. In addition, we have based our estimate of our financing requirements on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Adequate capital may not be available to us when needed or may not be available on acceptable terms. Our ability to obtain debt financing may be limited by covenants we have made under our Second Amended and Restated Loan and Security Agreement (as amended, the “2018 Amended Facility”) with Hercules Technology Growth Capital, Inc. (“Hercules”) and our pledge to Hercules of substantially all our assets as collateral. If we raise additional capital through the sale of equity or convertible debt securities, our shareholders’ ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of holders of our ordinary shares.

If we raise additional funds through collaborations, strategic alliances, or marketing, distribution or licensing arrangements with third parties, we may have to issue additional equity, relinquish valuable rights to our technologies, future revenue streams, products or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts, which would have a negative impact on our financial condition, results of operations and cash flows.

***Our existing and any future indebtedness could adversely affect our ability to operate our business.***

As of September 30, 2019, we had \$35.0 million of outstanding principal of borrowings under the 2018 Amended Facility, which we are required to repay in monthly principal installments from January 2022 through June 2023. We could in the future incur additional debt obligations beyond our borrowings from Hercules. Our existing loan obligations, together with other similar obligations that we may incur in the future, could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, research and development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a disadvantage compared to our competitors that have less debt or better debt servicing options.

We may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing loan obligations. Failure to make payments or comply with other covenants under our existing debt could result in an event of default and acceleration of amounts due. Under the 2018 Amended Facility, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lender accelerates the amounts due, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all our assets.



### **Risks Related to Other Legal Compliance Matters**

***Our relationships with customers and third-party payers will be subject to applicable anti-kickback, anti-bribery, fraud and abuse and other laws and regulations, which, if we are found in violation thereof, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and third-party payers will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third party payers and customers may expose us to broadly applicable anti-bribery laws, including the Foreign Corrupt Practices Act, as well as fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we would be able to market, sell and distribute any products for which we obtain marketing approval.

Efforts to ensure that our business arrangements with third parties will comply with applicable laws and regulations will involve substantial costs. If our operations, or the activities of our collaborators, distributors or other third-party agents are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs and the curtailment or restructuring of our operations. The cost associated with any of these actions could be substantial and could cause irreparable harm to our reputation or otherwise have a material adverse effect on our business, financial condition and results of operations.

***We are subject to laws governing data protection in the different jurisdictions in which we operate. The implementation of such data protection regimes is complex, and should we fail to fully comply, we may be subject to penalties that they may have an adverse effect on our business, financial condition and results of operations.***

Many national and state laws govern the privacy and security of health information and other personal and private information. They often differ from each other in significant ways. For instance, the EU has adopted a comprehensive data protection law called the General Data Protection Regulation (“GDPR”) that took effect in May 2018. The GDPR, together with the national legislation of the EU member states governing the processing of personal data, impose strict obligations and restrictions on the ability to collect, analyze and transfer personal data, including health data from clinical trials and adverse event reporting. In particular, these obligations and restrictions concern the consent of the individuals to whom the personal data relates, the information provided to the individuals, the transfer of personal data out of the EU, security breach notifications, security and confidentiality of the personal data, and imposition of substantial potential fines for breaches of the data protection obligations. The GDPR imposes penalties for non-compliance of up to the greater of EUR 20 million or 4% of worldwide revenue. Data protection authorities from the different EU member states may interpret the GDPR and national laws differently and impose additional requirements, which add to the complexity of processing personal data in the EU. Guidance on implementation and compliance practices are often updated or otherwise revised. The significant costs of compliance with, risk of regulatory enforcement actions under, and other burdens imposed by the GDPR as well as under other regulatory schemes throughout the world related to privacy and security of health information and other personal and private data could have an adverse impact on our business, financial condition and results of operations.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain employer's liability insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions that could have a material adverse effect on our business, financial condition and results of operations.

***Product liability lawsuits could cause us to incur substantial liabilities and to limit commercialization of our therapies.***

We face an inherent risk of product liability related to the testing of our product candidates in human clinical trials and in connection with product sales. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we develop or sell;
- injury to our reputation and significant negative media attention;
- negative publicity or public opinion surrounding gene therapy;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to further develop or commercialize any products that we develop.

Dependent upon the country where the clinical trial is conducted, we currently hold a maximum of EUR 6,000,000 and minimum of EUR 2,000,000 in clinical trial insurance coverage in the aggregate, with a per incident limit of EUR 450,000 to EUR 1,000,000 with respect to the clinical studies we conduct. Such coverage may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials. In addition, insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. In the event insurance coverage is insufficient to cover liabilities that we may incur, it could have a material adverse effect on our business, financial condition and results of operations.

***Healthcare legislative and regulatory reform measures may have a material adverse effect on our financial operations.***

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA, is a sweeping measure intended to, among other things, expand healthcare coverage within the United States, primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program. Several provisions of the law may affect us and increase certain of our costs.

In addition, other legislative changes have been adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2018, will remain in effect through 2027 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that the PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Further, there have been, and there may continue to be, judicial and Congressional challenges to certain aspects of the PPACA. For example, the U.S. Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additional legislative and regulatory changes to the PPACA, its implementing regulations and guidance and its policies, remain possible in the 116th U.S. Congress and under the Trump Administration. However, it remains unclear how any new legislation or regulation might affect the prices we may obtain for any of our product candidates for which regulatory approval is obtained. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.



***Our internal computer systems, or those of our collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.***

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. The size and complexity of our information technology systems, and those of our collaborators, contractors and consultants, and the large amounts of confidential information stored on those systems, make such systems vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, third-party vendors and/or business partners, or from cyber-attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

While we have not experienced a system failure, accident, cyber-attack or security breach that has resulted in a material interruption in our operations to date, we have experienced and addressed recent system failures, cyber-attacks and security breaches. In the future, such events could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. Additionally, any such event that leads to unauthorized access, use or disclosure of personal information, including personal information regarding our patients or employees, could harm our reputation, cause us not to comply with federal and/or state breach notification laws and foreign law equivalents and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information. Security breaches and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. While we have implemented security measures to protect our information technology systems and infrastructure, there can be no assurance that such measures will prevent service interruptions or security breaches that could adversely affect our business and the further development and commercialization of our product and product candidates could be delayed.

#### **Risks Related to Employee Matters and Managing Growth**

***Our future success depends on our ability to retain key executives and technical staff and to attract, retain and motivate qualified personnel.***

We are highly dependent on hiring, training, retaining and motivating key personnel to lead our research and development, clinical operations and manufacturing efforts. Although we have entered into employment agreements with our key personnel, each of them may terminate their employment on short notice. We do not maintain key person insurance for any of our senior management or employees.

The loss of the services of our key employees could impede the achievement of our research and development objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing senior management and key employees may be difficult and may take an extended period because of the limited number of individuals in our industry with the breadth and depth of skills and experience required to successfully develop gene therapy products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms.

If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

## **Risks Related to Our Ordinary Shares**

***The price of our ordinary shares has been and may in the future be volatile and fluctuate substantially.***

Our share price has been and may in the future be volatile. From the start of trading of our ordinary shares on the NASDAQ Global Select Market on February 4, 2014 through October 23, 2019, the sale price of our ordinary shares ranged from a high of \$82.49 to a low of \$4.72. The closing price on October 23, 2019, was \$42.50 per ordinary share. The stock market in general and the market for smaller biopharmaceutical companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our ordinary shares may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of our product candidates or those of our competitors;
- public perception of gene therapy;
- regulatory delays and greater government regulation of potential products due to adverse events;
- regulatory or legal developments in the European Union, the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- mergers, acquisitions, licensing and collaboration activity among our peer companies in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

***An active trading market for our ordinary shares may not be sustained.***

Although our ordinary shares are listed on The NASDAQ Global Select Market, an active trading market for our shares may not be sustained. If an active market for our ordinary shares does not continue, it may be difficult for our shareholders to sell their shares without depressing the market price for the shares or sell their shares at all. Any inactive trading market for our ordinary shares may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

***Our directors, executive officers and major shareholders, if they choose to act together, will continue to have a significant degree of control with respect to matters submitted to shareholders for approval.***

Our directors, executive officers and major shareholders holding more than 5% of our outstanding ordinary shares, in the aggregate, beneficially own approximately 34.6% of our issued shares (including such shares to be issued in relation to exercisable options to purchase ordinary shares) as at September 30, 2019. As a result, if these shareholders were to choose to act together, they may be able, as a practical matter, to control many matters submitted to our shareholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could control the election of the board directors and the approval of any merger, consolidation or sale of all or substantially all our assets. These shareholders may have interests that differ from those of other of our shareholders and conflicts of interest may arise.

***Provisions of our articles of association or Dutch corporate law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace our board.***

Certain provisions of our articles of association may make it more difficult for a third party to acquire control of us or effect a change in our board. These provisions include:

- staggered terms of our directors;
- a provision that our directors may only be removed at a general meeting of shareholders by a two-thirds majority of votes cast representing more than half of the issued share capital of the Company; and
- a requirement that certain matters, including an amendment of our articles of association, may only be brought to our shareholders for a vote upon a proposal by our board.

***We do not expect to pay dividends in the foreseeable future.***

We have not paid any dividends since our incorporation. Even if future operations lead to significant levels of distributable profits, we currently intend that earnings, if any, will be reinvested in our business and that dividends will not be paid until we have an established revenue stream to support continuing dividends. Accordingly, shareholders cannot rely on dividend income from our ordinary shares and any returns on an investment in our ordinary shares will likely depend entirely upon any future appreciation in the price of our ordinary shares.

***We lost our status as an "emerging growth company" as of December 31, 2018 and are therefore subject to greater disclosure requirements.***

We were an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 ("JOBS Act") through 2018. As of December 31, 2018, we are considered a large accelerated filer and as a consequence lost our status as an emerging growth company. We therefore no longer are permitted to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions included:

- reduced disclosure obligations surrounding executive compensation in our periodic reports and proxy statements;
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved; and
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the independent auditor's report providing additional information about the audit and the financial statements.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. We are required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm.

Meeting these disclosure requirements as well as the auditor attestation of our internal control over financial reporting will require that our management and other personnel devote a substantial amount of time to these compliance incentives. Moreover, these rules and regulations have increased our legal and financial compliance costs and will make some activities costlier and more time-consuming. In order to meet these additional reporting requirements, we may be required to divert resources away from research and development efforts, which could have a material adverse effect on our business, financial condition and results of operations.

***If we fail to maintain an effective system of internal controls, we may be unable to accurately report our results of operations or prevent fraud or fail to meet our reporting obligations, and investor confidence and the market price of our ordinary shares may be materially and adversely affected.***

If we fail to maintain the adequacy of our internal control over financial reporting, we may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting. If we fail to maintain effective internal control over financial reporting, we could experience material misstatements in our financial statements and fail to meet our reporting obligations, which would likely cause investors to lose confidence in our reported financial information. This could in turn limit our access to capital markets, harm our results of operations, and lead to a decline in the trading price of our ordinary shares. Additionally, ineffective internal control over financial reporting could expose us to increased risk of fraud or misuse of corporate assets and subject us to potential delisting from The NASDAQ Global Select Market, regulatory investigations and civil or criminal sanctions. Our reporting and compliance obligations may place a significant strain on our management, operational and financial resources and systems for the foreseeable future.

***Unfavorable global economic conditions, including those caused by political instability in the United States or by the U.K.'s pending departure from the European Union ("Brexit"), could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. Political instability in the United States and surrounding Brexit has the potential to disrupt global economic conditions and supply changes. While we do not believe that our operations will be directly adversely affected materially by Brexit, we may not be able to anticipate the effects Brexit will have on our suppliers and any collaborators. The most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the most recent global financial crisis, could result in a

variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payers or our collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

#### **Risks for U.S. Holders**

***We have in the past qualified and in the future may qualify as a passive foreign investment company, which may result in adverse U.S. federal income tax consequence to U.S. holders.***

Based on our average value of our gross assets, our cash and cash equivalents as well as the price of our shares we qualified as a passive foreign investment company ("PFIC") for U.S. federal income tax for 2016 but not in 2017 or 2018. A corporation organized outside the United States generally will be classified as a PFIC for U.S. federal income tax purposes in any taxable year in which at least 75% of its gross income is passive income or on average at least 50% of the gross value of its assets is attributable to assets that produce passive income or are held to produce passive income. Passive income for this purpose generally includes dividends, interest, royalties, rents and gains from commodities and securities transactions. Our status in any taxable year will depend on our assets and activities in each year, and because this is a factual determination made annually after the end of each taxable year, there can be no assurance that we will continue to qualify as a PFIC in future taxable years. The market value of our assets may be determined in large part by reference to the market price of our ordinary shares, which is likely to fluctuate, and may fluctuate considerably given that market prices of biotechnology companies have been especially volatile. If we were considered a PFIC for the current taxable year or any future taxable year, a U.S. holder would be required to file annual information returns for such year, whether the U.S. holder disposed of any ordinary shares or received any distributions in respect of ordinary shares during such year. In certain circumstances a U.S. holder may be able to make certain tax elections that would lessen the adverse impact of PFIC status; however, in order to make such elections the U.S. holder will usually have to have been provided information about the company by us, and we do not intend to provide such information.

The U.S. federal income tax rules relating to PFICs are complex. U.S. holders are urged to consult their tax advisors with respect to the purchase, ownership and disposition of our shares, the possible implications to them of us being treated as a PFIC (including the availability of applicable election, whether making any such election would be advisable in their particular circumstances) as well as the federal, state, local and foreign tax considerations applicable to such holders in connection with the purchase, ownership and disposition of our shares.

***Any U.S. or other foreign judgments may be difficult to enforce against us in the Netherlands.***

Although we now report as a U.S. domestic filer for SEC reporting purposes, we are incorporated under the laws of the Netherlands. Some of the members of our board and senior management reside outside the United States. As a result, it may not be possible for shareholders to effect service of process within the United States upon such persons or to enforce judgments against them or us in U.S. courts, including judgments predicated upon the civil liability provisions of the federal securities laws of the United States. In addition, it is not clear whether a Dutch court would impose civil liability on us or any of our Board members in an original action based solely upon the federal securities laws of the United States brought in a court of competent jurisdiction in the Netherlands.

The United States and the Netherlands currently do not have a treaty providing for the reciprocal recognition and enforcement of judgments, other than arbitration awards, in civil and commercial matters. Consequently, a final judgment for payment given by a court in the United States, whether or not predicated solely upon U.S. securities laws, would not automatically be recognized or enforceable in the Netherlands. To obtain a judgment which is enforceable in the Netherlands, the party in whose favor a final and conclusive judgment of the U.S. court has been rendered will be required to file its claim with a court of competent jurisdiction in the Netherlands. Such party may submit to the Dutch court the final judgment rendered by the U.S. court. If and to the extent that the Dutch court finds that the jurisdiction of the U.S. court has been based on grounds which are internationally acceptable and that proper legal procedures have been observed, the Dutch court will, in principle, give binding effect to the judgment of the U.S. court, unless such judgment contravenes principles of public policy of the Netherlands. Dutch courts may deny the recognition and enforcement of punitive damages or other awards. Moreover, a Dutch court may reduce the amount of damages granted by a U.S. court and recognize damages only to the extent that they are necessary to compensate actual losses or damages. Enforcement and recognition of judgments of U.S. courts in the Netherlands are solely governed by the provisions of the Dutch Civil Procedure Code.

Therefore U.S. shareholders may not be able to enforce against us or our board members or senior management who are residents of the Netherlands or countries other than the United States any judgments obtained in U.S. courts in civil and commercial matters, including judgments under the U.S. federal securities laws.

***The rights and responsibilities of our shareholders and directors are governed by Dutch law and differ in some important respects from the rights and responsibilities of shareholders under U.S. law.***

Although we now report as a U.S. domestic filer for SEC purposes, our corporate affairs are governed by our articles of association and by the laws governing companies incorporated in the Netherlands. The rights of our shareholders and the responsibilities of members of our board under Dutch law are different than under the laws of some U.S. jurisdictions. In the performance of their duties, our board members are required by Dutch law to consider the interests of uniQure, its shareholders, its employees and other stakeholders and not only those of our shareholders (as would be required under the law of most U.S. jurisdictions). As a result of these considerations our directors may take action that would be different than those that would be taken by a company organized under the law of some U.S. jurisdictions.

**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds**

None.

**Item 3. Defaults Upon Senior Securities**

None.

**Item 4. Mine Safety Disclosures**

Not applicable.

**Item 5. Other Information**

None.

**Item 6. Exhibits**

See the Exhibit Index immediately preceding the signature page to this Quarterly Report on Form 10-Q for a list of exhibits filed or furnished with this report, which Exhibit Index is incorporated herein by reference.

## EXHIBIT INDEX

[3.1 Amended Articles of Association \(incorporated by reference to Exhibit 3.1 of the Company's annual report on Form 10-K for the year ended December 31, 2018 \(file no. 0001-36294\) filed with the Securities and Exchange Commission\).](#)

[10.1t Amended and Restated Employment Agreement, executed September 17, 2019, by and between the Company and Dr. Kuta \(incorporated by reference to Exhibit 10.1 of the Company's current report on form 8-K \(file no. 001-36294\) filed with the Securities and Exchange Commission\) filed on September 20, 2019.](#)

[10.2t Employment Agreement, executed September 17, 2019, by and between the Company and Dr. Sander van Deventer \(incorporated by reference to Exhibit 10.2 of the Company's current report on form 8-K \(file no. 001-36294\) filed with the Securities and Exchange Commission\) filed on September 20, 2019.](#)

[10.3t Separation Agreement, executed September 16, 2019, by and between the Company and Dr. Scott McMillan \(incorporated by reference to Exhibit 10.3 of the Company's current report on form 8-K \(file no. 001-36294\) filed with the Securities and Exchange Commission\) filed on September 20, 2019.](#)

[10.4†\\* Amended and Restated Collaboration Agreement by and between 4D Molecular Therapeutics, Inc and uniQure biopharma B.V., dated August 6, 2019.](#)

[10.5†\\* Collaboration and License Agreement by and between 4D Molecular Therapeutics, Inc and uniQure biopharma B.V., dated August 6, 2019.](#)

[31.1\\* Rule 13a-14\(a\)/15d-14\(a\) Certification of Chief Executive Officer](#)

[31.2\\* Rule 13a-14\(a\)/15d-14\(a\) Certification of Chief Financial Officer](#)

[32.1± Section 1350 Certification](#)

101\* The following financial information from our Quarterly Report on Form 10-Q for the period ended September 30, 2019, filed with the Securities and Exchange Commission on October 28, 2019, is formatted in Inline Extensible Business Reporting Language ("iXBRL"): (i) Consolidated Balance Sheets; (ii) Consolidated Statements of Operations and Comprehensive Loss; (iii) Consolidated Statements of Shareholders' Equity; (iv) Consolidated Statements of Cash Flows; and (v) Notes to Consolidated Financial Statements (tagged as blocks of text)

104\* The cover page from our Quarterly Report on Form 10-Q for the period ended September 30, 2019, filed with the Securities and Exchange Commission on October 28, 2019, is formatted in Inline Extensible Business Reporting Language ("iXBRL")

† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with the rules of the Securities and Exchange Commission.

\* Filed herewith.

± Furnished herewith.

t Indicates a management contract or compensatory plan or arrangement.

**SIGNATURE**

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

**UNIQUE, N.V.**

By: /s/ Matthew Kapusta

\_\_\_\_\_  
Matthew Kapusta

Chief Executive Officer

(Principal Executive and Financial Officer)

By: /s/ Christian Klemt

\_\_\_\_\_  
Christian Klemt

Chief Accounting Officer

Dated October 28, 2019



CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT HAVE BEEN OMITTED AND REPLACED WITH “[\*\*\*]”. SUCH IDENTIFIED INFORMATION HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS (I) NOT MATERIAL AND (II) WOULD LIKELY CAUSE COMPETITIVE HARM TO THE COMPANY IF DISCLOSED.

**AMENDED AND RESTATED**  
**COLLABORATION AND LICENSE AGREEMENT**  
**BY AND BETWEEN**  
**4D MOLECULAR THERAPEUTICS, INC**  
**AND**  
**UNIQUE BIOPHARMA B.V.**  
**August 6, 2019**

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## AMENDED AND RESTATED

### COLLABORATION AND LICENSE AGREEMENT

This Amended and Restated Collaboration and License Agreement (this “Agreement”) is entered into on and has an effective date of August 6, 2019, (the “Amended CLA Effective Date”) and amends and restates the original Collaboration and License Agreement (the “Original Agreement”), dated January 17, 2014 (the “Original CLA Effective Date” or “Effective Date”), by and between 4D Molecular Therapeutics, Inc, a corporation organized and existing under the laws of the State of Delaware and having a principal office located at 5858 Horton St, Emerystation North, Suite 460, Emeryville, CA 94608 (“4DMT”) (the original 4DMT party to the Agreement was 4D Molecular Therapeutics, LLC, a Delaware limited liability corporation that is now the entity defined as 4DMT in the foregoing), and uniQure biopharma B.V., a corporation organized and existing under the laws of The Netherlands and having a principal office located at Paasheuvelweg 25a, 1105 BP Amsterdam, The Netherlands (“uniQure”). The Original Agreement shall govern the rights between the parties for the period from the Original CLA Effective Date to, but excluding, the Amended CLA Effective Date, subject to any releases or other retrospective rights or obligations expressly provided in this Agreement.

### INTRODUCTION

1. 4DMT is a biopharmaceutical company focused on research, development, manufacturing and marketing of novel adeno-associated viral vectors for delivery of nucleic acids to target cells.
  2. uniQure is a biopharmaceutical company focused on the research, development, manufacturing and marketing of gene therapy based biopharmaceutical products.
  3. 4DMT and uniQure desire to conduct a research collaboration to identify improved AAV Capsid Variants (as defined below).
  4. 4DMT and uniQure now desire to amend, modify and restate the Original Agreement in its entirety via this Agreement, and 4DMT and uniQure are entering into a new Collaboration and License Agreement to be effective of even date herewith (the “New CLA”), pursuant to which, 4DMT and uniQure will pursue a new collaboration in which 4D will take the lead for the identification of novel AAV Capsid Variants for development and commercialization as therapeutic products in the Field and pursuant to the terms and conditions thereunder, and, through the execution of this Agreement and the New CLA, the Parties have resolved the matters that were referred to and described in correspondence between the Parties dated February 28, 2019 with respect to the Original Agreement.
  5. uniQure desires to receive from 4DMT exclusive rights under 4DMT’s intellectual property rights to research (subject to 4DMT’s retained rights to conduct research under the Research Program), Develop, manufacture and Commercialize Selected Capsid Variants, Royalty Bearing Compounds and Royalty Bearing Products in the Field (each as defined below) pursuant to this Agreement, subject to 4D’s non-exclusive rights with respect thereto as described next.
-

6. 4DMT desires to receive from uniQure non-exclusive rights under uniQure's intellectual property rights (including uniQure's rights in intellectual property generated by 4D under this Agreement) to research, Develop, manufacture and Commercialize 4DMT Proposed Products as Royalty Bearing Compounds and Royalty Bearing Products in the Field (each as defined below) pursuant to this Agreement.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and other good and valuable consideration, the receipt of which is hereby acknowledged, 4DMT and uniQure agree as follows effective as of the Effective Date:

## ARTICLE I

### DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, shall have the respective meanings set forth below:

1.1 "4DMT AAV Capsid Variant". 4DMT Capsid Variant means any AAV Capsid Variant that does not carry a Gene Therapy Construct contained in a Royalty Bearing Compound or Royalty Bearing Product.

1.2 "4DMT AAV Capsid Variant Library". 4DMT AAV Capsid Variant Library means any AAV Capsid Variant Library constructed by or licensed to 4DMT, including all AAV Capsid Variant Libraries provided to 4DMT pursuant to the UCB Agreements.

1.3 "4DMT Intellectual Property". 4DMT Intellectual Property means the 4DMT Know-How and the 4DMT Patent Rights.

1.4 "4DMT Know-How". 4DMT Know-How means Know-How that is (a) Controlled by 4DMT or its Affiliates as of the Effective Date or during the Research Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize the relevant Selected Capsid Variant, or a Royalty Bearing Compound or Royalty Bearing Product due to the presence of such Selected Capsid Variant therein. 4DMT Know-How includes Core 4DMT Know-How but does not include Joint Know-How.

1.5 "4DMT Patent Right". 4DMT Patent Right means any Patent Right Controlled by 4DMT or its Affiliates as of the Effective Date or during the Term that Covers 4DMT Know-How. Schedule 1.5 lists the 4DMT Patent Rights existing as of the Effective Date. 4DMT Patent Rights include Core 4DMT Patent Rights but do not include Joint Patent Rights.

1.6 "AAV". AAV means adeno-associated virus.

1.7 "AAV Capsid Variant". AAV Capsid Variant means an AAV capsid that is modified as compared to the wild type sequence.

1.8 "AAV Capsid Variant Library". AAV Capsid Variant Library means a collection of variant AAV capsid open reading frames inserted into an AAV genome in a manner

that renders such variants genome replication-competent with the appropriate helper virus functions and capable of being selected and evolved to optimize their ability to deliver nucleic acid sequences to human or animal cells.

1.9 “Accounting Standards”. Accounting Standards means, with respect to uniQure and its Affiliates, International Financial Reporting Standards (“IFRS”) or, to the extent applicable, generally accepted accounting principles as practiced in the United States (“GAAP”), and with respect to 4DMT and its Affiliates, GAAP, in each case as they exist from time to time, consistently applied.

1.10 “Affiliate”. Affiliate means, with respect to a Party, any entity that directly or indirectly controls, is controlled by, or is under common control with such Party. As used in this definition, the term “control” means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of an entity, whether through ownership of voting securities, by contract or otherwise. For purposes of this definition, “control” shall be presumed to exist if one of the following conditions are met: (a) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors, and (b) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities.

1.11 “Animal POC”. Animal POC means gene expression and/or gene function, in an animal model, of the transgene cassette that defines the relevant potential Product.

1.12 “Business Day”. Business Day means a day that is not a Saturday, Sunday or a day on which banking institutions in New York, New York, USA or Amsterdam, The Netherlands are authorized by Law to remain closed.

1.13 “Calendar Quarter”. Calendar Quarter means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31; provided, however, that the first Calendar Quarter hereunder shall commence on the Effective Date and the final Calendar Quarter hereunder shall end on the effective date of termination or expiration of this Agreement.

1.14 “Calendar Year”. Calendar Year means each successive period of twelve (12) months commencing on January 1 and ending on December 31; provided, however, that the first Calendar Year hereunder shall commence on the Effective Date and the final Calendar Quarter hereunder shall end on the effective date of termination or expiration of this Agreement.

1.15 “Candidate Success Criteria”. Candidate Success Criteria means the criteria that an AAV Capsid Variant identified through a Research Selection Process (or any Research Compound containing such AAV Capsid Variant) must meet before it progresses to the next stage of the Research Program, as determined and approved by the JRSC, and as further described in Section 3.3(a).

1.16 “CEO”. CEO means the Chief Executive Officer of a Party or, if there is no Chief Executive Officer of a Party, the Board Chairperson or senior-most executive officer or equivalent of such Party.

1.17 “Clinical Trial(s)”. Clinical Trial(s) means a Phase I Study, a Phase II clinical study, a Pivotal Study or a Phase III Study.

1.18 “Clinical POC”. Clinical POC means demonstration of safety and a Pre-agreed level of therapeutic efficacy, including a change in the levels of a Pre-agreed disease relevant biomarker in some cases as a substitute for therapeutic efficacy, in a Pre-agreed number of human patients.

1.19 “Commercially Reasonable Efforts”. Commercially Reasonable Efforts means, with respect to a Party, the efforts required in order to carry out a task in a diligent and sustained manner without undue interruption or delay, which level is at least commensurate with the level of effort that a similarly situated Third Party would devote to a product of similar market potential and having similar commercial and scientific advantages and disadvantages resulting from its own research efforts or to which it has rights, taking into account its safety and efficacy, regulatory status, the competitiveness of the marketplace, its proprietary position, pricing, reimbursement, launching strategy and other market-specific factors, and all other relevant factors.

1.20 “Commercialization” or “Commercialize”. Commercialization or Commercialize means any activity directed to obtaining pricing or reimbursement approvals, marketing, promoting, distributing, importing, exporting, offering to sell or selling a product, or to have any such activity performed. When used as a verb, “Commercialize” means to engage in Commercialization.

1.21 “Compound”. Compound means an AAV Capsid Variant carrying a Gene Therapy Construct.

1.22 “Confidential Information”. Confidential Information means any and all information and data, including all uniQure Know-How, 4DMT Know-How and Joint Know-How, and all other scientific, pre-clinical, clinical, regulatory, manufacturing, marketing, financial and commercial information or data, whether communicated in writing or orally or by any other method, which is provided by one Party to the other Party in connection with this Agreement or the Prior Confidentiality Agreement. All Core uniQure Know-How shall be considered the Confidential Information of uniQure, with respect to which: (a) uniQure shall be considered the disclosing Party, (b) 4DMT shall be considered the receiving Party, and (c) clauses (b) and (e) of Section 8.2 shall not apply. All Core 4DMT Know-How shall be considered the Confidential Information of 4DMT, with respect to which: (i) 4DMT shall be considered the disclosing Party, (ii) uniQure shall be considered the receiving Party, and (iii) clauses (b) and (e) of Section 8.2 shall not apply.

1.23 “Control”. Control means, with respect to any item of or right under Patent Rights or Know-How, the possession (whether by ownership or license, other than a license pursuant to this Agreement) of the ability of a Party or, as applicable, its Affiliate (subject to Section 12.7), to grant access to, or a license or sublicense of, such items or right as provided for herein without violating the terms of any agreement or other arrangement with any Third Party existing at the time such Party would be required hereunder to grant the other Party such access or license or sublicense.

1.24 “Core 4DMT Intellectual Property”. Core 4DMT Intellectual Property means Core 4DMT Know-How and Core 4DMT Patent Rights.

1.25 “Core 4DMT Know-How”. Core 4DMT Know-How means [\*\*\*].

1.26 “Core 4DMT Patent Right”. Core 4DMT Patent Right means any Patent Right that Covers the Core 4DMT Know-How.

1.27 “Core uniQure Intellectual Property”. Core uniQure Intellectual Property means Core uniQure Know-How and Core uniQure Patent Rights.

1.28 “Core uniQure Know-How”. Core uniQure Know-How means [\*\*\*].

1.29 “Core uniQure Patent Right”. Core uniQure Patent Right means any Patent Right that Covers the Core uniQure Know-How.

1.30 “Cover”, “Covering” or “Covered”. Cover, Covering or Covered means, with respect to a product, technology, process or method that, in the absence of ownership of or a license granted under a Valid Claim, the manufacture, use, offer for sale, sale or importation of such product or the practice of such technology, process or method would infringe such Valid Claim (or, in the case of a Valid Claim that has not yet issued, would infringe such Valid Claim if it were to issue).

1.31 “Default”. Default means with respect to a Party that (a) any representation or warranty of such Party set forth herein shall have been untrue in any material respect when made or (b) such Party shall have failed to perform any material obligation set forth in this Agreement.

1.32 “Delivery Success Criteria”. Delivery Success Criteria means the following criteria that determines whether an AAV Capsid Variant demonstrates improved delivery or function of a Gene Therapy Construct: [\*\*\*].

1.33 “Development” or “Develop”. Development or Develop means pre-clinical and clinical drug development activities, including: test method development and stability testing, toxicology, formulation, process development, manufacturing scale-up, development-stage manufacturing, quality assurance/quality control procedure development and performance with respect to clinical materials, statistical analysis and report writing and clinical studies, regulatory affairs, and all other pre-Regulatory Approval activities. When used as a verb, “Develop” means to engage in Development.

1.34 “EMA”. EMA means the European Medicines Agency, or any successor agency.

1.35 “European Union” or “EU”. European Union or EU means the countries that are members of the European Union, as redefined from time to time.

1.36 “FDA” or “Food and Drug Administration”. FDA or Food and Drug Administration means the United States Food and Drug Administration, or any successor agency.

1.37 “Field”. Field means the delivery of Gene Therapy Constructs to cells in (a) the central nervous system (“CNS”) or (b) the liver, in each case where such delivery is for the purpose of effecting expression of the applicable RNA or amino acid sequence in the targeted cells and is potentially useful for the diagnosis, treatment, palliation or prevention of a disease or medical condition in humans or animals, irrespective of the administration site or mode of administration (*e.g.*, intravenous, direct injection, subcutaneous or intrathecal) of the Compound used to effect delivery. For clarity, intravenous administration of any Compound targeted to cells in other organs (*i.e.*, not specifically targeted to liver or CNS tissues), including for treatment of neoplastic and eye disorders, is excluded from the Field.

1.38 “First Commercial Sale”. First Commercial Sale means, with respect to any Royalty Bearing Product and a country, the first sale for end use or consumption of such Royalty Bearing Product in such country after all required approvals, including Regulatory Approval, have been granted by the Regulatory Authority of such country. For clarity, sales for test marketing, sampling and promotional uses, clinical trials purposes or compassionate use shall not constitute a First Commercial Sale.

1.39 “FTE”. FTE means [\*\*\*] ([\*\*\*)] hours of work devoted to or in support directly of the Research Program that is carried out by one or more qualified scientific or technical employees of 4DMT or its Affiliates, measured in accordance with 4DMT’s normal time allocation practices from time to time. Overtime, and work on weekends, holidays and the like, shall not be counted with any multiplier (*e.g.*, time-and-a-half or double time) toward the number of hours that are used to calculate the FTE contribution. The portion of an FTE billable for one (1) individual during a Calendar Quarter shall be determined by dividing the number of hours worked directly by said individual on the Research Program during such Calendar Quarter by [\*\*\*] ([\*\*\*)] hours.

1.40 “FTE Costs”. FTE Costs means, for any Calendar Quarter, the number of FTEs multiplied by the FTE Rate.

1.41 “FTE Rate”. FTE Rate means the amount for each FTE as set forth in Schedule 1.41.

1.42 “Fully Burdened Manufacturing Cost”. Fully Burdened Manufacturing Cost means, as applicable to a Royalty Bearing Product, the cost of manufacturing such Royalty Bearing Product, which is equal to the sum of (a) for such Royalty Bearing Product (or components thereof), the costs of all direct material, direct labor and allocable manufacturing overhead consumed, provided, or procured by a Party, in each case for the manufacture of such Royalty Bearing Product, and (b) for such Royalty Bearing Product (or components thereof) made by a Third Party, the out-of-pocket costs paid to such Third Party by a Party; in each case (a) and (b) to the extent such costs are incurred by a Party or its Affiliates and to the extent such costs are reasonably allocable to the manufacture of such Royalty Bearing Product. For clarity, Fully Burdened Manufacturing Cost excludes costs of excess capacity. Fully Burdened Manufacturing Cost shall be calculated in a manner consistent with Accounting Standards.

1.43 “Gene Therapy Construct”. Gene Therapy Construct means any nucleic acid sequence that encodes an RNA or an amino acid sequence that is intended to be delivered to a targeted tissue to treat, prevent or ameliorate a disease or condition.

1.44 “GLP Tox Compound”. GLP Tox Compound means a Research Compound that uniQure, in its sole discretion, elects to progress to GLP Tox Studies to be conducted by or on behalf of uniQure in accordance with Section 3.3(a).

1.45 “GLP Tox Study”. GLP Tox Study means a formal toxicology study of a Research Compound conducted under Good Laboratory Practices that is required to obtain approval from a regulatory authority, whether the FDA or otherwise, to begin conducting Clinical Trials.

1.46 “Good Laboratory Practices”. Good Laboratory Practices means the then-current good laboratory practice standards promulgated or endorsed by the FDA, as defined in 21 C.F.R. Part 58 (or such other comparable regulatory standards in jurisdictions outside the U.S. to the extent applicable to the relevant study, as they may be updated from time to time).

1.47 “Governmental Authority”. Governmental Authority means any United States federal, state or local or any foreign government, or political subdivision thereof, or any multinational organization or authority or any authority, agency or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, any court or tribunal (or any department, bureau or division thereof), or any governmental arbitrator or arbitral body.

1.48 “Grant Letter”. Grant Letter means each of the Option Agreements, dated as of even date herewith, by and between uniQure’s Affiliate, uniQure B.V., and (a) in the first case, Dr. David Schaffer and (b) in the second case, Dr. David Kirn.

1.49 “IGT”. IGT means Integrative Gene Therapeutics, Inc., a California corporation, which jointly owns with UC certain of the UC Patent Rights.

1.50 “Indication”. Indication means any disease, condition or syndrome.

1.51 “Initial Research Term”. Initial Research Term means the period commencing on the Effective Date and ending on [\*\*\*].

1.52 “Initiation”. Initiation means, with respect to a Clinical Trial, the first dosing of a participant in such Clinical Trial.

1.53 “Invention”. Invention means any new and useful process, article of manufacture, compound, composition of matter, formulation or apparatus, or any improvement thereof, discovery or finding, which is patentable.

1.54 “Invoice”. Invoice means an original invoice sent by 4DMT to uniQure with respect to any payment due hereunder substantially in the form attached hereto as Schedule 1.54.

1.55 “Know-How”. Know-How means (a) any scientific or technical information, results and data of any type whatsoever, in any tangible or intangible form whatsoever, that is not in the public domain, including databases, practices, methods, techniques, specifications, formulations, formulae, protein sequences, nucleic acid sequences, AAV Capsid



Variants, AAV Capsid Variant Libraries, Gene Therapy Constructs, Compounds, knowledge, know-how, trade secrets, skill, experience, test data including pharmacological, medicinal chemistry, biological, chemical, biochemical, toxicological and clinical test data, analytical and quality control data, stability data, studies and procedures, and manufacturing process and development information, results and data, and (b) any biological, chemical, or physical material or composition of matter that is not in the public domain or otherwise generally available to the public.

1.56 “Law”. Law means all laws, statutes, rules, codes, regulations, orders, judgments or ordinances applicable to a Party, this Agreement or the activities contemplated hereunder.

1.57 “Lead Optimization”. Lead Optimization means the discovery phase dedicated to the evaluation of new AAV Capsid Variants derived from an AAV Capsid Variant Library following a Research Selection Process to identify one or more Research Compounds that meet Delivery Success Criteria.

1.58 “Licensed IP”. Licensed IP means the 4DMT Intellectual Property, Core uniQure Intellectual Property, and Joint Intellectual Property.

1.59 “Materials”. Materials means any tangible chemical or biological research materials that are provided or otherwise made available by one Party to the other Party under the terms of Section 3.4 for use in performance of the Research Program; provided, however, that Materials will not include any AAV Capsid Variants or AAV Capsid Variant Libraries.

1.60 “NDA”. NDA means a New Drug Application or Biologics License Application filed with the FDA or any other application required for the purpose of marketing or selling or commercially using a therapeutic or prophylactic product to be filed with a Regulatory Authority in a non-U.S. country or group of countries, including a Product License Application or Marketing Authorization Application (“MAA”) in the European Union or Japan.

1.61 “Net Sales”. Net Sales means, with respect to a Royalty Bearing Product, the gross amount of sales of such Royalty Bearing Product invoiced by uniQure or its Affiliates to Third Parties, less the following to the extent related to such Royalty Bearing Product and incurred by such uniQure or its Affiliates and invoiced to the Third Party:

(a) sales returns and allowances actually paid, granted or accrued, including trade, quantity and cash discounts and any other adjustments, including those granted on account of price adjustments or billing errors;

(b) rejected goods, damaged or defective goods, recalls, returns;

(c) rebates, chargeback rebates, compulsory rebates, reimbursements or similar payments granted or given to wholesalers or other distributors, buying groups or health care insurance carriers;

(d) non-collectable receivables;

(e) customs or excise duties, sales tax, consumption tax, value added tax, and other taxes (except income taxes); or

(f) charges for packing, freight, shipping and insurance.

Each of the foregoing deductions shall be determined as incurred in the ordinary course of business in type and amount consistent with good industry practice and in accordance with Accounting Standards on a basis consistent with uniQure's audited consolidated financial statements. For clarity, sales by uniQure or its Affiliates of a Royalty Bearing Product to a Third Party Distributor of such Royalty Bearing Product in a given country shall be considered a sale to a Third Party customer. All such discounts, allowances, credits, rebates, and other deductions shall be fairly and equitably allocated to the Royalty Bearing Products and other products of uniQure and its Affiliates such that the Royalty Bearing Product does not bear a disproportionate portion of such deductions.

In the event any Royalty Bearing Product is sold for consideration other than cash, Net Sales for such sale shall be the average price of such Royalty Bearing Product sold for cash during the relevant period in the relevant country.

In the event that any discount, reduction, payment or rebate is offered for a Royalty Bearing Product where such Royalty Bearing Product is sold to a Third Party customer as part of a grouped set of products, the applicable discount, reduction, payment or rebate for such Royalty Bearing Product in such arrangement shall be based on the weighted average discount, reduction, payment or rebate of such grouped set of products.

Any Royalty Bearing Products used for promotional or advertising purposes (in reasonable and customary amounts) or used for Clinical Trials or other research purposes shall not be included in Net Sales. Donations for charity reasons or compassionate use shall also not be included in Net Sales.

1.62 "Net Sales by 4D" has the same meaning as given in the definition of "Net Sales," but substituting "4DMT" for "uniQure" in each instance where "uniQure" appears in such definition.

1.63 "Party" and "Parties". Party means uniQure or 4DMT individually, and Parties means uniQure and 4DMT collectively.

1.64 "Patent Rights". Patent Rights means patents, patent applications or provisional patent applications, utility models and utility model applications, petty patents, innovation patents, patents of addition, divisionals, continuations, continuation-in-part applications, continued prosecution applications, requests for continued examinations, reissues, renewals, reexaminations and extensions and supplementary protection certificates granted in relation thereto, in any country of the world. For clarity, Patent Rights shall include any Patent Right that claims priority to or common priority with such Patent Rights.

1.65 "Phase I Study". A Phase I Study is a human clinical trial conducted in any country that meets the requirements of 21 CFR §312.21(a). By way of example and not limitation, a Phase I Study is usually performed as a single or multiple dose clinical study in healthy

volunteers or patients to assess specific administration, distribution, metabolism, excretion (ADME), safety and tolerability, bioavailability/bioequivalence or exploratory efficacy (in the sense of demonstrating “proof-of-principle”) of an investigational drug, and the emphasis in Phase I is usually on safety and tolerability and it is typically used to plan patient dosing in Phase II clinical studies. For clarity, a Phase I Study may also represent the initial phase of a combined Phase Ib/II clinical study.

1.66 “Phase III Study”. A Phase III Study is a human clinical trial conducted in any country that meets the requirements of 21 CFR §312.21(c). By way of example and not limitation, a Phase III Study is a large scale clinical study (usually several hundreds of patients) performed after preliminary evidence suggesting effectiveness of the drug has been obtained in Phase II clinical studies, and it is intended to gather the pivotal information about effectiveness and safety that is needed to evaluate the overall benefit-risk relationship of the drug and, along with earlier Clinical Trials, to provide an adequate basis for Regulatory Approval. For clarity, a Phase III Study may also represent the second part of a combined Phase II/III clinical study.

1.67 “Pivotal Study”. A Pivotal Study is a human clinical trial conducted in any country, the principal purpose of which is to establish safety and efficacy of a Royalty Bearing Product in patients with the applicable Indication and to gather the pivotal information about such safety and effectiveness that is needed to evaluate the overall benefit-risk relationship of the drug and, along with earlier Clinical Trials, to provide an adequate basis for Regulatory Approval. A Pivotal Study includes any human clinical trial intended as a pivotal study of such Royalty Bearing Product regarding such Indication, such as a phase II/III or phase IIb clinical trial, whether or not such study is a traditional Phase III Study.

1.68 “Pre-agreed”. Pre-agreed means on terms that are determined by the JRSC in accordance with Section 2.5.

1.69 “Prior Confidentiality Agreement”. Prior Confidentiality Agreement means the Two Way Confidentiality Disclosure Agreement between uniQure and 4DMT, dated August 26, 2013.

1.70 “Product”. Product means any preparation in final form, either for sale by prescription, over-the-counter or any other method, or for administration to human patients in Clinical Trials, for any and all uses, and in any and all formulations and combinations, which preparation contains a Compound.

1.71 “Project Team”. Project Team means the 4DMT and uniQure personnel involved in the Research Program, including the Project Leaders.

1.72 “Prosecution and Maintenance”. Prosecution and Maintenance means, with respect to a Patent Right, the preparation, filing, prosecution and maintenance of such Patent Right, as well as reexaminations, reissues and the like with respect to such Patent Right, together with the conduct of interferences, the defense of oppositions and other similar proceedings with respect to the particular Patent Right; and “Prosecute and Maintain” shall have the correlative meaning.

1.73 “Regulatory Approval”. Regulatory Approval means the technical, medical and scientific licenses, registrations, authorizations and approvals (including approvals of NDAs

and labeling approvals) of any Regulatory Authority necessary for the distribution, marketing, promotion, offer for sale, use, import, export or sale of a Royalty Bearing Product in a regulatory jurisdiction.

1.74 “Regulatory Authority”. Regulatory Authority means any applicable Governmental Authority involved in granting approvals for the manufacturing, marketing, reimbursement or pricing of a Royalty Bearing Product in the Territory or any portion thereof, including the FDA and EMA (as applicable), and any successor Governmental Authority having substantially the same function.

1.75 “Research Compound”. Research Compound means a Compound containing a Designated Capsid Variant that is the subject of activities under the Research Program.

1.76 “Research Plan”. Research Plan means the research plan developed by the Parties that sets forth the activities to be undertaken during the Research Term with respect to the Research Program and the budget for such activities. The initial outline of the Research Plan is attached as Schedule 1.76.

1.77 “Research Program”. Research Program means a program of collaborative research to be undertaken by the Parties pursuant to the Research Plan to identify optimized AAV Capsid Variants for use in the Field that demonstrate improved expression of the delivered Gene Therapy Construct in the targeted tissue as compared to currently available AAV Capsid Variants.

1.78 “Research Selection Process”. Research Selection Process means the iterative evolution or isolation of lead AAV Capsid Variants from one or more 4DMT AAV Capsid Variant Libraries in cells (cultured or primary) *in vitro* or in animals *in vivo* intended to result in the identification of AAV Capsid Variants demonstrating Pre-agreed properties suitable to proceed into Lead Optimization using a Pre-agreed evaluation methodology and that are targeted to a specified target tissue. A given Research Selection Process is different from another Research Selection Process if such Research Selection Process was conducted to identify AAV Capsid Variants that specifically target a different tissue or are delivered by means of a different mode of administration (e.g., such process was conducted to identify AAV Capsid Variants useful for intravenous, direct injection, subcutaneous or intrathecal delivery means).

1.79 “Research Term”. Research Term means the Initial Research Term and, if applicable, the Extended Research Term.

1.80 “Research Year”. Research Year means a twelve (12) month period beginning on the Effective Date or on any anniversary thereof.

1.81 “Royalty Bearing Compound”. Royalty Bearing Compound means a Compound containing a Selected Capsid Variant.

1.82 “Royalty Bearing Product”. Royalty Bearing Product means a Product containing a Royalty Bearing Compound.

1.83 “Royalty Term”. Royalty Term means, with respect to a Royalty Bearing Product, on a Royalty Bearing Product-by-Royalty Bearing Product and a country-by-country basis, the period beginning on the First Commercial Sale of such Royalty Bearing Product in such country by uniQure or any of its Affiliates or Sublicensees, and ending on latest of: (a) the expiration of the last Valid Claim within the Licensed IP Covering such Royalty Bearing Product in such country, (b) the expiration of any applicable exclusivity, including orphan drug status or data exclusivity, and any extension thereto, granted by a Regulatory Authority in such country with respect to such Royalty Bearing Product, or (c) the tenth (10<sup>th</sup>) anniversary of the date of the First Commercial Sale by uniQure or any of its Affiliates or Sublicensees of such Royalty Bearing Product in such country.

1.84 “Selected Capsid Variant”. Selected Capsid Variant means (a) an AAV Capsid Variant selected by uniQure in accordance with Section 3.4 (as provided in Schedule 1.83), (b) an AAV Capsid Variant resulting from a modification by uniQure or by 4DMT (or by any Third Party licensed pursuant to this Agreement) to an AAV Capsid Variant described in subsection (a), or (c) an AAV Capsid Variant resulting from a modification by uniQure or by 4DMT (or by any Third Party licensed pursuant to this Agreement) to any AAV capsid to contain a sequence conferring the properties that were the subject of the Research Selection Process for an AAV Capsid Variant described in subsection (a); provided that the resulting Know-How with respect to the modified AAV Capsid Variants shall be Core uniQure Know-How. Notwithstanding anything express or implied in this Agreement: (i) uniQure and those deriving rights from uniQure shall have no right under this Agreement (but shall have the right under the New CLA) to modify a Selected Capsid Variant of clause (a) or (b) with or to include any motif, mutation, or substitution identified under the New CLA, (ii) any such modified AAV Capsid Variant -- other than an AAV Capsid Variant of clause (a) (i.e., any of the precise AAV Capsid Variants set forth in Schedule 1.83 with no further modifications) -- that includes any such motif, mutation, or substitution shall be deemed *not* to be a Selected Capsid Variant under this Agreement but rather to be a New Capsid Variant under the New CLA, (iii) the activities to so modify a Selected Capsid Variant shall be deemed to have occurred under the New CLA, and (iv) the Know-How and Patent Rights related to such modifications and resulting New Capsid Variants shall be deemed to arise under the New CLA and be owned by 4DMT as New Variant Patents and the Know-How that is the subject matter of New Variant Patents. For clarity, except as stated in the preceding sentence, all AAV Capsid Variants described in clauses (b) and (c) are Selected Capsid Variants for purposes of this Agreement, are subject to being potentially included in Proposed Products under Section 4.4, and are subject to Vector Characterization Data sharing under Section 4.3.

1.85 “Selection Process”. Selection Process means the iterative evolution or isolation of lead AAV Capsid Variants from one or more AAV Capsid Variant Libraries in cells (cultured or primary) *in vitro* or in animals *in vivo* intended to result in the identification of AAV Capsid Variants demonstrating properties suitable to a specified target tissue. For clarity, a Selection Process can be one that is performed by 4DMT or its Affiliate either for itself or for, with or by any Third Party under rights granted by 4DMT to such Third Party, and need not be one that is conducted under the Research Program of this Agreement or designed for the same type of tissue in order to qualify under this definition.

1.86 “Sublicensee”. Sublicensee means, with respect to uniQure, a Third Party to whom uniQure (or its Affiliate or another of its Sublicensees) has granted a license or sublicense

under the Licensed IP to Develop, make and have made, use or Commercialize a Royalty Bearing Product; provided, however, that a Sublicensee shall not include any Third Party Distributor.

1.87 “Territory”. Territory means all countries and territories in the world.

1.88 “Third Party”. Third Party means an entity other than uniQure, 4DMT and their respective Affiliates.

1.89 “Third Party Distributor”. Third Party Distributor means any Third Party that provides (but does not Develop) Royalty Bearing Products directly to customers under agreement with uniQure, its Affiliates or Sublicensees.

1.90 “UC AAV Capsid Variant”. UC AAV Capsid Variant means any AAV Capsid Variant provided to 4DMT pursuant to the UCB Agreements.

1.91 “UC Patent Right”. UC Patent Right means any Patent Right licensed to 4DMT pursuant to the UCB Agreements.

1.92 “UC Product”. UC Product means a Royalty Bearing Product that is Covered by a UC Patent Right.

1.93 “UCB Agreements”. UCB Agreements means (a) the Exclusive License and Bailment Agreement between 4DMT and the Regents of the University of California (“UC”), Agreement Control No. 2014-03-0089, dated December 19, 2013; (b) the Exclusive License and Bailment Agreement between 4DMT and UC, Agreement Control No. 2014-03-0090, dated December 19, 2013; and (c) the Agreement for Use of Certain Biological Materials between 4DMT and UC, Agreement Control No. 2014-30-0088, dated December 19, 2013, in each case in the form provided to uniQure by 4DMT as of the Effective Date.

1.94 “uniQure Intellectual Property”. uniQure Intellectual Property means uniQure Know-How and uniQure Patent Rights.

1.95 “uniQure Know-How”. uniQure Know-How means Know-How that is (a) Controlled by uniQure or its Affiliates as of the Effective Date or during the Research Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize the relevant Selected Capsid Variant, or a Royalty Bearing Compound or Royalty Bearing Product due to the presence of such Selected Capsid Variant therein. uniQure Know-How includes Core uniQure Know-How but does not include Joint Know-How.

1.96 “uniQure Patent Right”. uniQure Patent Right means any Patent Right Controlled by uniQure or its Affiliates as of the Effective Date or during the Term that Covers uniQure Know-How. uniQure Patent Rights include Core uniQure Patent Rights but do not include Joint Patent Rights.

1.97 “Valid Claim”. Valid Claim means (a) a claim of an issued patent that has not expired or been abandoned, or been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable

judgment (or judgment from which no appeal was taken within the allowable time period), or (b) a claim within a patent application which application has not been pending for more than [\*\*\*] ([\*\*\*)] years from the date of its priority filing date and which claim has not been irretrievably revoked, irretrievably cancelled, irretrievably withdrawn, held invalid or abandoned by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period), or finally determined to be unallowable in a decision from which an appeal cannot or can no longer be taken; provided, however, that with respect to [\*\*\*].

1.98 “Vector Characterization Data” means any and all data, results and other Know-How that is generated either by or on behalf of a Party or its Affiliate, whether alone or together with, by or for any of its Third Party licensees, contractors or collaborators either under this Agreement or outside of this Agreement, with respect to any Selected Capsid Variant, in regards to any of the following with respect to such Selected Capsid Variant: [\*\*\*]

1.99 Additional Definitions. Each of the following definitions is set forth in the section of this Agreement indicated below:

<b><u>Definition:</u></b>	<b><u>Section:</u></b>
4DMT	Preamble
4DMT Indemnities	9.5
Acquiring/Acquired Party	5.6(c)
Additional Cure Period	10.2(a)
Agreement	Preamble
Audited Party	6.7
Auditing Party	6.7
Bankruptcy Code	5.5
CNS	1.37
CREATE Act	7.10
Damages	9.5
Defaulting Party	10.2(a)
Designated Capsid Variant	3.4(a)
Dispute	11.1
Effective Date	Preamble
Equipment Payment	6.2(c)
Excluded Claim	11.2
Executives	2.5(b)
Extended Research Term	3.1(c)
Failure to Amend	4.4(d)
Fair Market Value	6.5(b)(iii)
GAAP	1.9
GLP Tox Candidate Review Period	3.3(a)
IFRS	1.9
Initiating Party	7.6(d)
Joint Counsel	7.5
Joint Intellectual Property	7.2(a)

**Definition:****Section:**

Joint Know-How	7.2(a)
Joint Patent Rights	7.2(a)
JRSC	2.2(a)
M&A Event	12.7
MAA	1.60
Non-Defaulting Party	10.2(a)
Orange Book	7.9(a)
Paragraph IV Certification	7.9(b)
Paragraph IV Proceeding	7.9(b)(ii)
Project Leader	2.1
Records	3.7(a)(i)
SEC Filing	8.5(c)
Sublicense Consideration	6.5(b)
Sublicense Income Sharing Percentages	6.5(a)
Term	10.1
Third Party Claim	9.5
Third Party Competitive Product	4.4(a)
Third Party Proposal	4.4(a)
Third Party Proposed Products	4.4(a)
Third Party Proposer	4.4(a)
Trade Secret Election	7.3(b)
USPTO	7.10
UC	1.89
uniQure	Preamble
uniQure Indemnitees	9.6

**ARTICLE II****GOVERNANCE**

2.1 **Project Leaders.** Within [\*\*\*] ([\*\*\*)] Business Days after the Effective Date, each Party will appoint (and provide written notice to the other Party of the identity of) a senior representative having a general understanding of pharmaceutical discovery and development issues to act as its project leader under this Agreement (each, a “**Project Leader**”). The Project Leaders will serve as the contact point between the Parties with respect to the Research Program, and will be primarily responsible for: (a) facilitating the flow of information and otherwise promoting communication, coordination of the day-to-day work and collaboration between the Parties; (b) providing single point communication for seeking consensus internally within the respective Party’s organization; and (c) raising cross-Party or cross-functional disputes in a timely manner. The Project Leaders shall conduct regular telephone conferences as deemed necessary or appropriate, to exchange informal information regarding the progress of the Research Program. Each Party may change its designated Project Leaders from time to time upon prior written notice to the other Party. Each Project Leader may designate a substitute to temporarily perform the functions of that Project Leader by prior written notice to the other Party.



## 2.2 Joint Research Steering Committee.

(a) Composition. Promptly after the Effective Date, the Parties shall establish a joint research steering committee (the “JRSC”). The JRSC shall be comprised of at least [\*\*\*] ([\*\*\*)] named representatives of uniQure and at least [\*\*\*] ([\*\*\*)] named representatives of 4DMT, one of whom shall be [\*\*\*] (unless due to his death, illness or disability), or such other numbers as the Parties may agree in writing. As soon as practicable after the Effective Date (but in no event more than [\*\*\*] ([\*\*\*)] Business Days after the Effective Date), each Party shall designate by written notice to the other Party its initial representatives on the JRSC. Each Party may replace one or more of its non-mandatory representatives, in its sole discretion, effective upon written notice to the other Party of such change. These representatives shall have appropriate technical credentials, experience and knowledge, and ongoing familiarity with the Research Program. The JRSC shall be disbanded upon expiration of the Research Term.

(b) Function and Powers of the JRSC. During the Research Term, the JRSC’s responsibilities shall include: (i) approving the initial Research Plan and any amendment thereto, including allocation of tasks and resources; (ii) developing and approving the Candidate Success Criteria; (iii) developing and approving parameters for Animal POC; (iv) developing and approving parameters for Clinical POC; (v) determining the frequency of meetings of the Project Team, or subgroups of the Project Team, and the members of the Project Team to attend such meetings, which meetings are expected to occur at least [\*\*\*], with such meetings expected to occur in person at least [\*\*\*]; (vi) reviewing, approving procedures, and making recommendations regarding Lead Optimization; (vii) determining whether a Research Compound achieves the relevant Delivery Success Criteria; (viii) proposing Research Compounds that have achieved the Delivery Success Criteria for uniQure’s acceptance as GLP Tox Compounds; (ix) providing a forum for discussion of the Research Plan, the status of the Research Program, and relevant data; (x) serving as a forum for informal resolution of disagreements that may arise in the relation to the Parties’ activities under the Research Program, including any disagreement within any subcommittee; (xi) determining and approving the overall strategy for publications and presentations pursuant to Section 8.4; and (xii) considering and acting upon such other matters as may be specified in this Agreement. Any decision made by the JRSC under this Section 2.2(b) shall be deemed a decision of the JRSC, as applicable, for purposes of this Agreement.

2.3 Subcommittees. The JRSC may establish and disband such subcommittees as deemed necessary by the JRSC. Each such subcommittee shall consist of the same number of representatives designated by each Party, which number shall be mutually agreed by the Parties. Each Party shall be free to change its representatives on written notice to the other Party or to send a substitute representative to any subcommittee meeting. Each Party’s representatives and any substitute for a representative shall be bound by a written agreement with confidentiality obligations substantially the same as those set forth in ARTICLE VIII. The rules for the conduct of each subcommittee, and the scope of its responsibilities, shall be determined by the JRSC, provided that no subcommittee shall have the authority to bind the Parties hereunder, and each subcommittee shall report to the JRSC.

2.4 Meetings. The JRSC shall each hold at least [\*\*\*] per Calendar Quarter. Upon necessity, either Party shall be entitled to request additional meetings of the JRSC. Meetings of the JRSC shall be effective only if at least [\*\*\*] ([\*\*\*)] representatives of each Party are present

or participating. The location of meetings shall be as agreed by the Parties, and may be held in person, alternating locations between the Parties, or by telephone conference call or by videoconference; provided, however, that at least [\*\*\*] ([\*\*\*)] meetings of the JRSC each Calendar Year are held in person. 4DMT's costs and expenses incurred in connection with preparing for and participating in all such meetings shall be paid for by uniQure in accordance with the budget for the Research Plan. Either Party may, from time to time, invite additional representatives or consultants to attend JRSC meetings; provided that at least [\*\*\*] ([\*\*\*)] Business Days' prior written notice is given of a Party's intention to invite such other representatives or consultants and providing full details about the name, employer and professional background of such other representatives or consultants. Each representative and consultant participating in or attending a JRSC meeting shall be bound by a written agreement with confidentiality obligations substantially the same as those set forth in ARTICLE VIII. The JRSC shall be co-chaired by a representative from each Party. The chairpersons shall set the agendas for the JRSC meeting in advance. Within [\*\*\*] ([\*\*\*)] Business Days prior to each scheduled meeting, each Party shall, in accordance with Section 3.7(b), provide a report to the JRSC detailing its progress with respect to the Research Program. The Parties will rotate the responsibility for recording, preparing and issuing minutes for each JRSC meeting, to be circulated within [\*\*\*] ([\*\*\*)] Business Days after each meeting.

## 2.5 Decision-making.

(a) Initial Dispute Resolution Procedures. Subject to the provisions of this Section 2.5, actions to be taken by the JRSC shall be taken only following a unanimous vote, with each Party, through its representatives, having one (1) vote. If any subcommittee fails to reach unanimous agreement (with each Party, through its representatives, having one (1) vote) for a period in excess of [\*\*\*] ([\*\*\*)] Business Days, the matter shall be referred to the JRSC.

(b) Referral of Unresolved Matters to Executives. If, in accordance with Section 2.5(a), the JRSC does not resolve any matter considered by it within [\*\*\*] ([\*\*\*)] Business Days after the matter is first considered by it, the matter may be referred by either Party to the CEO of 4DMT and CEO of uniQure (the "Executives") to be resolved by negotiation in good faith as soon as practicable, but in no event later than [\*\*\*] ([\*\*\*)] Business Days after referral. Such resolution, if any, of a referred issue by the Executives shall be final and binding on the Parties. Any decision made by the Executives under this Section 2.5(b) shall be deemed a decision of the JRSC for purposes of this Agreement.

(c) Final Decision-Making. If a dispute referred to the Executives pursuant to Section 2.5(b) has not been resolved in accordance with Section 2.5(b), then, subject to Section 2.5(d), uniQure shall have the final decision-making authority. Any decision made by uniQure pursuant to this Section 2.5(c) shall be deemed a decision of the JRSC for purposes of this Agreement.

(d) Exceptions. Notwithstanding Section 2.5(c), uniQure shall not have the right to exercise such decision-making authority (i) in a manner that excuses uniQure from any of its obligations specifically enumerated under this Agreement; (ii) in a manner that negates any consent rights or other rights specifically allocated to 4DMT under this Agreement; (iii) [intentionally omitted]; (iv) in a manner that would require 4DMT to perform activities (A) for

which uniQure will not reimburse 4DMT's costs (except as expressly set forth in this Agreement), (B) that 4DMT has not agreed to perform as set forth in this Agreement or the Research Plan, or as otherwise agreed in writing by 4DMT, or (C) that require 4DMT to use any Know-How or other technology not contemplated in the Research Plan and that is not developed internally by 4DMT and with respect to the use of which 4DMT would owe a royalty or other payment; (v) in a manner that would change the total number of 4DMT FTEs or the allocation among the various technical disciplines as set forth in the Research Plan; (vi) in a manner that would reduce payments committed to 4DMT pursuant to this Agreement or take away 4DMT's right to perform activities that 4DMT has previously agreed to perform as set forth in the Research Plan; (vii) in a manner that would require 4DMT to perform any act that it reasonably believes to be inconsistent with any Law or any approval, order, policy, guidelines of a Regulatory Authority or ethical requirements or ethical guidelines; (viii) to determine that uniQure has fulfilled any obligation under this Agreement or that 4DMT has breached any obligation under this Agreement; or (ix) to amend the relevant Delivery Success Criteria. In the event that any matter set forth in the preceding clauses (i)-(ix) is unresolved through the JRSC and subsequently such dispute cannot be resolved by the Executives in accordance with Section 2.5(b), then either (A) for all such matters set forth in the preceding clauses (iv)-(vi), there shall be no change in the Research Plan or associated budget unless the Parties otherwise mutually agree in writing, (B) for all such matters set forth in the preceding clauses (i), (ii), (vii) and (viii), either Party may require the specific issue to be referred to binding arbitration pursuant to Section 11.2, or (C) for all such matters set forth in the preceding clauses (iii) and (ix), either Party may require the specific issue to be submitted to a panel of external scientific experts to review the dispute pursuant to the remainder of this Section 2.5(d). Each Party shall select, upon either Party's request, one (1) external scientific expert within [\*\*\*] ([\*\*\*)] Business Days after such request, and the two (2) so selected shall choose a third (3rd) external scientific expert within an additional [\*\*\*] ([\*\*\*)] Business Days to resolve the dispute, and all three (3) shall serve as neutrals. Each expert must be free of any conflict of interest with respect to either or both Parties and their Affiliates and shall have expertise in the matters concerning the unresolved dispute. The decision of the external scientific expert panel shall be issued within [\*\*\*] ([\*\*\*)] Business Days after nomination of the third external expert and shall be final and binding on the Parties. The Parties agree to share equally the cost of the proceedings, including fees of the panel members; provided, that each Party shall bear its own attorneys' fees and associated costs and expenses.

2.6 Limitations on JRSC Authority. The JRSC and any subcommittee shall have only the powers assigned expressly to it in this ARTICLE II and elsewhere in this Agreement, and shall not have any power to amend, modify or waive compliance with this Agreement. In furtherance thereof, 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 JRSC or any subcommittee unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

## ARTICLE III

### **RESEARCH PROGRAM**

#### 3.1 General.

(a) Objectives. The objectives of the Research Program are to (i) identify and characterize AAV Capsid Variants and Research Compounds, (ii) optimize such AAV Capsid Variants and Research Compounds and (iii) conduct other research activities with respect to Research Compounds containing Gene Therapy Constructs of interest in place of marker or other proof-of-principle genes with which screening and AAV Capsid Variant optimization may have been performed, in each case to identify Research Compounds that meet the Delivery Success Criteria, with the objective of having such Research Compounds accepted by uniQure for Animal POC and subsequently as GLP Tox Compounds, consistent with the Candidate Success Criteria.

(b) Research Plan. The Parties shall agree to the Research Plan and shall conduct the Research Program in accordance with the Research Plan. The JRSC shall endeavor to approve the initial Research Plan (including its associated budget) within [\*\*\*] ([\*\*\*)] days after the Effective Date, which initial Research Plan shall set forth the tasks to be undertaken by the Parties (including relevant technology to be used and Materials to be provided) under the Research Program.

(c) Extended Research Term. In the event that uniQure reasonably believes that the Parties will not complete the activities under the Research Plan during the Initial Research Term, then uniQure, at its sole discretion, may extend the Research Term to complete the goals of such Research Plan as then in effect for an additional [\*\*\*] ([\*\*\*)] month period from the expiration of the Initial Research Term (the “Extended Research Term”). uniQure may so extend the Research Term by giving written notice to 4DMT at least [\*\*\*] ([\*\*\*)] months prior to the expiration of the Initial Research Term. The Parties shall mutually agree upon the number of FTEs at 4DMT needed to perform the research during the Extended Research Term, as well as out-of-pocket costs, and uniQure shall provide funding for such FTEs and out-of-pocket costs in accordance with Section 6.2(a) and, if the Parties are unable to agree on such matters prior to the expiration of the Initial Research Term, then the Research Term shall expire at the end of the Initial Research Term. The Parties may further extend the Extended Research Term by mutual written agreement.

#### 3.2 Conduct of the Research Program.

(a) 4DMT and uniQure shall each use Commercially Reasonable Efforts to conduct the Research Program in accordance with the Research Plan. In addition, uniQure shall use Commercially Reasonable Efforts to assess reasonably promptly whether each Designated Capsid Variant provided to uniQure in connection with assessing the Delivery Success Criteria can be manufactured in insect cells.

(b) Either Party shall have the right to utilize the services of any Third Party to perform its obligations under the Research Plan to the extent that such Third Party is specifically approved in the Research Plan or otherwise approved by the JRSC, provided that any

permitted Third Party must have entered into a written agreement with such Party that includes terms and conditions (i) protecting and limiting use and disclosure of Confidential Information at least to the same extent as under ARTICLE VIII, and (ii) requiring the Third Party and its personnel to assign to such Party all right, title and interest in and to any intellectual property (and intellectual property rights) created or conceived in connection with performance of subcontracted activities. Each Party shall remain at all times fully liable for its responsibilities under this Agreement.

(c) 4DMT and uniQure shall conduct the Research Program in accordance with all applicable Laws, including, if and as applicable, Good Laboratory Practices. Each Party hereby certifies that it will not employ or otherwise use in any capacity in performing any activity hereunder the services of any person or entity known to it to be debarred under 21 USC §335a.

(d) If the JRSC determines that it is desirable to transfer the AAV Capsid Variant Libraries into baculovirus, then prior to such transfer, the Parties will negotiate in good faith an amendment to this Agreement specifying the allocation of ownership of Materials, Know-How, and Patent Rights. Except as otherwise agreed by the Parties in writing, in no event shall 4DMT transfer the 4DMT AAV Capsid Variant Libraries to uniQure, and in no event shall uniQure transfer its baculovirus insect cell manufacturing Know-How to 4DMT.

### 3.3 Candidate Success Criteria.

(a) Within [\*\*\*] ([\*\*\*)] days following the date on which the Research Plan is approved by the JRSC, the JRSC shall determine and approve the minimum Candidate Success Criteria applicable to each class or series of Research Compounds. For clarity, the Candidate Success Criteria shall include [\*\*\*]. The objectives of the Research Program will always be to identify the best possible AAV Capsid Variants for delivery of Gene Therapy Constructs to target cells, rather than to identify AAV Capsid Variants that merely meet the minimum Candidate Success Criteria specified in the Research Plan. Subsequently in the Research Program (*i.e.*, when AAV Capsid Variants have been accepted by uniQure as being ready for Animal POC testing or in parallel with the identification with lead AAV Capsid Variants for Lead Optimization), the JRSC will (i) agree on disease models for testing Gene Therapy Constructs of interest for efficacy against particular target diseases, (ii) agree on procedures for testing in these animal disease models and the Candidate Success Criteria in these models intending to result in data sufficient for submission to regulatory authorities, and (iii) recommend that Research Compounds meeting these criteria should proceed to GLP Tox Studies. The Candidate Success Criteria shall in all of cases (i)-(iii) be expected to be able to be met only using Research Compound stocks that have been prepared by uniQure in insect cells using standard uniQure SOPs in comparison to reference vectors also prepared by uniQure in the same way. Notwithstanding the foregoing, the Candidate Success Criteria shall be deemed to have been met for any Research Compound that uniQure advances into GLP Tox Studies.

(b) The JRSC may, from time to time during the Research Term, nominate a Research Compound that has achieved the Candidate Success Criteria for Animal POC (provided, however, that the JRSC may, as appropriate, nominate a Research Compound that has not achieved all the Candidate Success Criteria) for consideration as a GLP Tox Compound. uniQure will consider all data relating to the nominated Research Compound for designation as a

GLP Tox Compound, including data generated by either uniQure or 4DMT pursuant to this Agreement. Such data shall include the results from all tests and other measures included in the Candidate Success Criteria and such other information and results as uniQure reasonably requests from 4DMT. Within [\*\*\*] ([\*\*\*)] days after delivery to uniQure of such data (the applicable “GLP Tox Candidate Review Period”), uniQure shall provide 4DMT written notice whether uniQure accepts such nominated Research Compound as a GLP Tox Compound and intends to Develop and Commercialize such nominated Research Compound in accordance with the terms of this Agreement. Notwithstanding the foregoing, uniQure shall be deemed to have accepted as a GLP Tox Compound any Research Compound that it advances into pre-clinical Development conducted under Good Laboratory Practices.

#### 3.4 Selection of AAV Capsid Variants.

(a) Within [\*\*\*] ([\*\*\*)] days after 4DMT provides uniQure with the list of AAV Capsid Variant sequences arising from each Research Selection Process and all other data arising from or relating to such Research Selection Process, uniQure shall submit by written notice to 4DMT a list specifying up to [\*\*\*] ([\*\*\*)] AAV Capsid Variants from each such Research Selection Process (the “Designated Capsid Variants”). If uniQure has not provided such written notice to 4DMT within [\*\*\*] ([\*\*\*)] days, 4DMT shall provide written notice to uniQure of the date that the foregoing [\*\*\*] ([\*\*\*)] day period will expire, and the Parties will have the option to agree an extension by mutual consent, not to be unreasonably withheld.

(b) Prior to the [\*\*\*] of the expiration of the Research Term, uniQure shall submit by written notice to 4DMT a list specifying up to [\*\*\*] ([\*\*\*)] AAV Capsid Variants from the list of Designated Capsid Variants for each Research Selection Process. All AAV Capsid Variants included in such list shall be included as “Selected Capsid Variants,” subject to the terms and conditions of this Agreement. For clarity, all modifications by uniQure to the Selected Capsid Variants and other modifications set forth in Section 1.84 shall also be deemed “Selected Capsid Variants” for purposes of the payment obligations under this Agreement. 4DMT shall provide written notice to uniQure if uniQure has not provided such list to 4DMT by the date that is [\*\*\*] ([\*\*\*)] days prior to the [\*\*\*] of the expiration of the Research Term.

(c) For clarity, the subset of Designated Capsid Variants not subsequently selected as Selected Capsid Variants may be used and licensed by 4DMT to Third Parties outside the Field, but only if they also arise from a Selection Process conducted outside the Field. Unless such subset of Designated Capsid Variants also arise from a Selection Process conducted outside the Field, 4DMT may not conduct any research using such subset of Designated Capsid Variants unless otherwise agreed under the Research Plan. For further clarity, Selected Capsid Variants may not be used, or licensed to Third Parties, by 4DMT or its Affiliates outside the Field.

#### 3.5 Materials and Know-How Transfer/Use of Compounds.

(a) In order to facilitate the Research Program, each Party shall, as set forth in the Research Plan, provide to the other Party certain Materials and, subject to Section 3.6, Know-How Controlled by the supplying Party for use by the other Party in furtherance of the Research Program. In addition, 4DMT shall transfer to uniQure such quantities of Designated

Capsid Variants as the JRSC may reasonably request from time to time during the Research Term to exercise its rights hereunder. All Materials and Know-How provided by one Party to the other Party remain the sole property of the supplying Party.

(b) All Materials transferred pursuant to the Research Program shall be used (i) only for the specific purpose provided for in the Research Plan, and (ii) solely under the control of the receiving Party. The Materials may not be used or delivered to or for the benefit of any Third Party without the prior written consent of the supplying Party, and shall not be used in research or testing involving human subjects, except as expressly contemplated in the Research Plan or in accordance with this Agreement. All Materials shall be returned to the supplying Party or destroyed (at the election of the supplying Party) promptly after completion of the use permitted under this Agreement.

(c) THE MATERIALS ARE PROVIDED "AS IS" AND WITHOUT ANY REPRESENTATION OR WARRANTY, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTY OF MERCHANTABILITY OR OF FITNESS FOR ANY PARTICULAR PURPOSE OR ANY WARRANTY THAT THE USE OF THE MATERIALS WILL NOT INFRINGE OR VIOLATE ANY PATENT OR OTHER PROPRIETARY RIGHT OF ANY THIRD PARTY.

(d) At the end of the Research Term, upon request by uniQure, 4DMT shall promptly provide to uniQure all quantities of the Royalty Bearing Compounds in 4DMT's possession and shall promptly destroy other Research Compounds.

3.6 Third Party Intellectual Property. The conduct of activities under the Research Plan will use Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, subject to the terms and conditions of the UCB Agreements. 4DMT shall be solely responsible for all obligations under the UCB Agreements, including any and all payments and royalties due thereunder. In developing the Research Plan, the Parties shall discuss whether any Third Party Patent Rights or Know-How, other than Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, will be utilized in the conduct of activities under the Research Plan. 4DMT shall disclose to uniQure the details of any restrictions on use or payment obligations of which it is aware that would be triggered by such use of Third Party Patent Rights or Know-How in the Research Program. If the Parties mutually agree to use any inventions claimed in any Patent Right or use any Know-How that is licensed to or has been acquired by 4DMT other than pursuant to the UCB Agreements, and if such use would require the payment of additional consideration to the Third Party from which the Patent Rights or Know-How was licensed or acquired, then such Patent Right or Know-How shall be deemed under the Control of 4DMT, provided that uniQure expressly agrees in writing to bear any such additional consideration actually to be paid by 4DMT to the Third Party (which amounts uniQure may offset pursuant to Section 6.4(c)(ii)) with respect to the Development, manufacture or Commercialization of Royalty Bearing Compounds or Royalty Bearing Products. For clarity, nothing in this Section 3.6 shall limit uniQure's rights to obtain from a Third Party, independent of 4DMT, a license or other right with respect to such Third Party's Patent Rights or Know-How.

### 3.7 Records and Reports.

#### (a) Records.

(i) 4DMT shall maintain records, in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes, which shall fully and properly reflect all work done and results achieved in the performance of the Research Program by or on behalf of 4DMT (the "Records"), including the procedures, techniques and methodologies used, the progress made, and any Invention conceived or reduced to practice or otherwise made within the scope of or in connection with the Research Program. As part of keeping the Records, 4DMT shall ensure that all of its personnel, and all of its agents that are involved in the Research Program, will keep accurate laboratory notebooks, which laboratory notebooks: (A) shall be duly signed, dated and witnessed; and (B) shall be created and maintained in accordance with its standard operating procedures that would be sufficient to allow for said laboratory notebooks to be used in any proceeding before the United States Patent and Trademark Office or United States courts, in order to establish the date of invention for any Invention in accordance with the United States patent laws. During the Term, 4DMT shall, upon written request by uniQure, which shall not be unreasonably made: (1) make all Records available for inspection and review by uniQure during normal business hours in a timely manner; and (2) provide copies of the Records or any part thereof to uniQure, as reasonably requested by uniQure.

(ii) After a Research Compound has been accepted by uniQure as a GLP Tox Compound, uniQure shall have the right to request that a copy of the relevant portions of the laboratory notebooks relating to all stages of the generation of such GLP Tox Compound be provided by 4DMT to uniQure. After such request by uniQure, 4DMT shall provide such copies of the laboratory notebooks promptly to uniQure, which shall be maintained by uniQure as 4DMT's Confidential Information.

(b) Reports to the JRSC. Between [\*\*\*] ([\*\*\*)] and [\*\*\*] ([\*\*\*)] Business Days prior to each scheduled JRSC meeting, the Parties shall provide to the JRSC a written report on the progress of the Research Program, summarizing the work performed under the Research Program and evaluating the work performed in relation to the goals of the Research Program. Each Party shall provide such other information required by the Research Program or reasonably requested by the other Party and reasonably available, relating to the progress of the goals or performance of the Research Program.

## ARTICLE IV

### **DEVELOPMENT AND COMMERCIALIZATION OF PRODUCTS; DILIGENCE**

4.1 Responsibility. uniQure shall have full responsibility, [\*\*\*], for the worldwide research, Development, manufacturing and Commercialization of Compounds and Products in the Field, subject to the payment obligations and other relevant terms and conditions of this Agreement.

4.2 Diligence. The Parties will have no rights or obligations pursuant to this Section of the Original Agreement.



#### 4.3 Obligation to Share Vector Characterization Data for Selected Capsid Variants.

(a) Commencing on the Amended CLA Effective Date and continuing until the termination or expiration of this Agreement, uniQure shall provide, within [\*\*\*] ([\*\*\*)] days after each January 31<sup>st</sup> and July 31<sup>st</sup> of each Calendar Year, a written report to 4DMT that summarizes the Vector Characterization Data generated by or on behalf of uniQure or its Affiliate or Sublicensee with respect to each Selected Capsid Variant for which any research or Development activities were conducted by or on behalf of uniQure or its Affiliate or Sublicensee during the [\*\*\*] ([\*\*\*)] months that ended on the immediately prior [\*\*\*] as applicable.

(b) Commencing on the Amended CLA Effective Date and continuing until the termination or expiration of this Agreement, 4DMT shall provide, within [\*\*\*] ([\*\*\*)] days after each January 31<sup>st</sup> and July 31<sup>st</sup> of each Calendar Year, a written report to uniQure that summarizes the Vector Characterization Data generated by or on behalf of uniQure or its Affiliate or Sublicensee with respect to each Selected Capsid Variant for which any research or Development activities were conducted by or on behalf of 4DMT or its Affiliate or Sublicensee during the [\*\*\*] ([\*\*\*)] months that ended on the immediately prior [\*\*\*] as applicable.

(c) Either Party may terminate its obligation to provide written reports pursuant to this Section 4.3 of the Agreement, if it ceases all research, development, commercialization or other activities that would result in the generation of any further unreported Vector Characterization Data with respect to Selected Capsid Variants, and the Party provides written notice to the other Party so stating and also certifying that all Vector Characterization Data that is required to be reported with respect to Selected Capsid Variants has been so reported and that the party provides notice that it has given up all of its rights associated with any such Selected Capsid Variants.

#### 4.4 Proposed Products in the Field.

(a) If, at any time after the Amended CLA Effective Date, a Third Party makes a bona fide proposal to 4DMT for Developing and Commercializing a Product in the Field based on a Selected Capsid Variant (a “Third Party Proposed Product”) using 4DMT Know-How or Joint Know-How, or the making, using or selling of which in the absence of an appropriate license would infringe a Valid Claim under the 4DMT Patent Rights or Joint Patent Rights, then 4DMT promptly shall notify uniQure of the proposal of such Third Party (“Third Party Proposer”) and shall provide uniQure with such information regarding such Third Party proposal, including a development plan and a plan to finance such activities (“Third Party Proposal”) as uniQure may reasonably request to evaluate such Third Party Proposal and its potential conflict with the ongoing efforts and future plans of uniQure. At any time after the Research Term, 4DMT may make a bona fide proposal to uniQure for Developing and Commercializing a Product in the Field based on a Selected Capsid Variant (a “4DMT Proposed Product”), including a development plan and a plan to finance such activities. Within [\*\*\*] ([\*\*\*)] days after receipt of a notice from 4DMT of a Third Party Proposal or 4DMT Proposed Product, uniQure shall notify 4DMT whether uniQure is conducting or is interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product, or a Product that uniQure believes in good faith is or would be competitive with such Third Party Proposed Product or 4DMT Proposed Product (a “Competitive”).

Product”). 4DMT shall have the right to make a maximum total of [\*\*\*] ([\*\*\*)] proposals per calendar year on a non-exclusive basis for Developing and Commercializing a Collaboration Proposed Product (as defined below) in the Field under this Section 4.4 and under Section 4.4 of the New CLA, such calendar year total to be determined in the aggregate under this Agreement and the New CLA, taken collectively. 4DMT shall have no other right to make a proposal for Developing or Commercializing a Product, or to otherwise develop or commercialize any product, in the Field using a Selected Capsid Variant, except as is expressly provided herein. “Collaboration Proposed Products” means, collectively or separately, Third Party Proposed Products, 4DMT Proposed Products and New CLA Proposed Products (as that term is defined in the New CLA). An “SCV Proposed Product” means, collectively or separately, 4DMT Proposed Products and Third Party Proposed Products.

(b) If uniQure notifies 4DMT that uniQure is conducting or is interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product or Competitive Product, uniQure shall, within [\*\*\*] ([\*\*\*)] months after such notice, deliver to 4DMT a plan (including projected timelines) for the research and Development thereof on a timeline consistent with the application of Commercially Reasonable Efforts, and, thereafter, shall use Commercially Reasonable Efforts to research, Develop, manufacture and Commercialize such Third Party Proposed Product, 4DMT Proposed Product or Competitive Product in accordance with such plan. uniQure shall provide progress reports to 4DMT in conjunction with the reports of Vector Characterization Data under Section 4.3 from and after the date of uniQure’s notice under this Section 4.4(b), and such reports shall contain a summary of the activities undertaken and the status of uniQure’s research and Development efforts with respect to such Third Party Proposed Product, 4DMT Proposed Product, or Competitive Product during the [\*\*\*] ([\*\*\*)] months that ended on the immediately prior [\*\*\*] as applicable.

(c) If uniQure notifies 4DMT that uniQure is not conducting and is not interested in conducting research or Development of such Third Party Proposed Product, 4DMT Proposed Product, or Competitive Product:

(i) and the applicable Proposed Product was a Third Party Proposed Product, then the Parties shall meet to discuss the grant of an appropriate license by uniQure to the Third Party Proposer. If 4DMT determines after such meeting and due consideration that the grant of a license to such Third Party Proposer is necessary or appropriate, uniQure shall have [\*\*\*] ([\*\*\*)] months after the date of receipt of written notice of such determination (or such longer time as shall be agreed to by the Parties in writing) to negotiate and enter into a non-exclusive sublicense under any relevant 4DMT Patent Rights and any relevant Patent Rights of uniQure (including uniQure Core Patent Rights generated under this Agreement) that are relevant due to the presence of the applicable Selected Capsid Variant therein, to provide such Third Party Proposer with sufficient rights under such 4DMT Patent Rights and uniQure Core Patent Rights (and no other intellectual property rights of any kind or Controlled by any person or entity), to research, Develop, manufacture and Commercialize the Third Party Proposed Product in the Field on commercially reasonable terms to be agreed by uniQure and such Third Party Proposer (such financial terms shall be equal to or greater than the amounts as set forth in Sections 6.3(b), 6.4 and 6.5). uniQure and such Third Party Proposer shall define and agree on the uniQure Know-How and uniQure Patent Rights that are relevant due to the presence of the applicable

Selected Capsid Variant therein, to the extent necessary to Develop or Commercialize AAV Capsid Variants to be licensed in such non-exclusive sublicense or amendment, as applicable.

(ii) and the applicable Proposed Product was a 4DMT Proposed Product, then [\*\*\*] uniQure hereby grants to 4DMT (who accepts such license) a non-exclusive sublicense under 4DMT Patent Rights and a non-exclusive license under the uniQure Intellectual Property that is necessary or useful due to the presence of the applicable Selected Capsid Variant therein, and all Vector Characterization Data reported by uniQure to 4DMT under this Agreement, to research, Develop, manufacture and Commercialize the 4DMT Proposed Product in the Field on the financial terms and conditions provided for in this Agreement. Such license shall be sublicensable through one (1) or more tiers or layers of sublicensees without the need to obtain consent from uniQure.

(d) In the case of a Third Party Proposer, if uniQure fails to enter into such a non-exclusive sublicense and license agreement within such [\*\*\*] ([\*\*\*)] month period, uniQure shall promptly (but in any event within [\*\*\*] ([\*\*\*)] days after the end of such period) provide 4DMT in writing an explanation for such failure along with the proposed terms offered by uniQure to such Third Party Proposer. If 4DMT determines in its good faith judgment based on reasonable inquiry that the terms offered by uniQure to such Third Party Proposer were not commercially reasonable, 4DMT shall notify uniQure of such determination and provide uniQure with an additional [\*\*\*] ([\*\*\*)] days to enter into a sublicense with such Third Party Proposer. If uniQure fails to enter into an agreement with such Third Party Proposer [\*\*\*], then 4DMT shall be free to dispute pursuant to ARTICLE XI whether uniQure has complied with its obligations under this Section 4.4.

4.5 Pharmacovigilance. Within [\*\*\*] ([\*\*\*)] months after the Amended CLA Effective Date, the Parties shall enter into an agreement governing the exchange of adverse event safety data (including post-marketing spontaneous reports) received by a Party and its Affiliates, including such data received from, in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, relating to any AAV Capsid Variant provided to uniQure by 4DMT hereunder in order to monitor the safety of all Compounds and Products and to meet reporting requirements with any applicable Regulatory Authority. Such data sharing agreement shall not require the sharing of data that would disclose confidential know-how or trade secrets of a Party or its Affiliates, or in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, if such data may be cross-referenced, such as through a Drug Master File, to satisfy the requirements of Law and any applicable Regulatory Authority.

4.6 Marking. Prior to the issuance in the United States of Patent Rights included in the UC Patent Rights, uniQure agrees to mark Royalty Bearing Product(s) Covered by any UC Patent Right (or their containers or labels) sold in the United States under the licenses granted in this Agreement with the words “Patent Pending,” and following the issuance in the United States of one or more Patent Rights included in the UC Patent Rights, with the patent numbers of the UC Patent Right(s) Covering such Royalty Bearing Product. All Royalty Bearing Products Covered by any UC Patent Right sold in other countries will be marked in such manner as to conform with the patent Laws and practice of such countries.

## ARTICLE V

### **GRANTS OF RIGHTS**

#### 5.1 Licenses to uniQure.

(a) Research License to uniQure. Subject to the terms and conditions of this Agreement, 4DMT hereby grants to uniQure, and uniQure hereby accepts, during the Research Term and any applicable GLP Tox Candidate Review Period in effect as of the end of the Research Term, an exclusive (but not as to 4DMT), worldwide, royalty-free, non-sublicenseable license under the 4DMT Intellectual Property and 4DMT's interest in the Joint Intellectual Property, solely to (i) conduct activities assigned to uniQure under the Research Plan, (ii) evaluate Research Compounds, or (iii) evaluate the data developed in the conduct of activities under the Research Plan during the Research Term.

(b) Development and Commercialization License to uniQure. Subject to the terms and conditions of this Agreement, and subject to any non-exclusive license granted to 4DMT under Section 5.2(c) with respect to any SCV Proposed Products, 4DMT hereby grants to uniQure, and uniQure hereby accepts, an exclusive (even as to 4DMT), worldwide, milestone- and royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the 4DMT Intellectual Property and 4DMT's interest in the Joint Intellectual Property, and any Vector Characterization Data reported by 4DMT to uniQure under this Agreement, to research (subject to 4DMT's retained rights to conduct research under the Research Program), Develop, make and have made, use and Commercialize Selected Capsid Variants, Royalty Bearing Compounds, and Royalty Bearing Products in the Field.

(c) Recordation. Following the Effective Date or at any time during the Term, 4DMT at the request and expense of uniQure shall promptly register or record the licenses granted to uniQure under this Agreement with the appropriate patent offices in all applicable countries of the Territory; provided that such registration or recordation specifies the applicable limitations of such license, and provided further that such registration shall have no effect on the allocation of Prosecution and Maintenance rights and obligations set forth in ARTICLE VII. In the event any of the licenses granted to uniQure under this Agreement are terminated in accordance with the terms of this Agreement, uniQure shall promptly take such actions and execute such documents as are reasonably requested by 4DMT to cancel such registration(s) or recordation(s) in the applicable countries with respect to the terminated license grants.

(d) Grant-Back License to uniQure. 4DMT hereby grants to uniQure, and uniQure hereby accepts, a non-exclusive, worldwide, royalty-free license, including the right to grant sublicenses through multiple tiers, under the 4DMT Patent Rights and 4DMT Know-How that (i) arise from activities that are conducted under this Agreement in connection with Royalty Bearing Compounds and Royalty Bearing Products in the course of making modifications to Selected Capsid Variants and (ii) claim or cover compositions of matter or general methods of use of Selected Capsid Variants (for clarity, including such Patent Rights and Know-How claiming or covering compositions combining Gene Therapy Constructs in general and AAV Capsid Variants in general or general methods of making or using such combinations of Gene Therapy Constructs

and AAV Capsid Variants), to research, Develop, make and have made, use and Commercialize Selected Capsid Variants, and Products containing Selected Capsid Variants.

## 5.2 Licenses to 4DMT.

(a) Research License to 4DMT. Subject to the terms and conditions of this Agreement, uniQure hereby grants to 4DMT, and 4DMT hereby accepts, during the Research Term and any applicable GLP Tox Candidate Review Period in effect as of the end of the Research Term, a non-exclusive, worldwide, royalty-free, non-sublicenseable license under the uniQure Intellectual Property, solely to the extent necessary to conduct activities assigned to 4DMT under the Research Plan.

(b) Grant-Back License to 4DMT Outside the Field. uniQure hereby grants to 4DMT, and 4DMT hereby accepts, a non-exclusive, worldwide, royalty-free license, including the right to grant sublicenses through multiple tiers, under all Vector Characterization Data reported from uniQure to 4DMT under this Agreement and the Patent Rights and Know-How Controlled by uniQure that is relevant due to the presence of the applicable Selected Capsid Variant therein, that (i) arise from activities that are conducted under this Agreement in connection with Royalty Bearing Compounds and Royalty Bearing Products in the course of making modifications to Selected Capsid Variants and (ii) claim or cover compositions of matter or general methods of use of Selected Capsid Variants that are applicable outside the Field (for clarity, excluding Patent Rights and Know-How claiming or covering (A) insect cell manufacturing technology, including technology or sequence modifications for adapting AAV Capsid Variants to insect cells or insect cell expression vectors and systems, or (B) compositions, methods of manufacture, or methods of use of Gene Therapy Constructs, but for further clarity, including such Patent Rights and Know-How that is necessary or useful due to the presence of the applicable Selected Capsid Variant therein, claiming or covering compositions combining Gene Therapy Constructs in general and AAV Capsid Variants in general or general methods of making or using such combinations of Gene Therapy Constructs and AAV Capsid Variants), to research, Develop, make and have made, use and Commercialize 4DMT AAV Capsid Variants (excluding Selected Capsid Variants), and Products containing such 4DMT AAV Capsid Variants, in all cases outside the Field. For the avoidance of doubt, 4DMT's practice of the foregoing license shall be subject to its obligations set forth in Section 5.6. If any Patent Rights or Know-how subject to the foregoing license are subject to agreements between uniQure and a Third Party that require payments to be made to the Third Party by reason of the practice of the rights granted to 4DMT under this Section 5.2(b), such Patent Rights and Know-How shall only be deemed Controlled by uniQure if 4DMT agrees in writing to pay to uniQure the portion of the amounts due to such Third Party that is reasonably attributable to the practice of such rights.

(c) Non-Exclusive License for SCV Proposed Products under Section 4.4. uniQure grants 4DMT the sublicenses and licenses provided for in Section 4.4(c)(ii) effective upon the time set forth therein, and 4DMT accepts such sublicense and license effective as of such time. In association with any license agreement pursuant to Section 4.4(c) with a Third Party related to a Third Party Proposed Product and subject to the terms and conditions of this Agreement, uniQure shall grant to the Third Party Proposer as applicable, and the Third Party Proposer shall accept, a non-exclusive, worldwide, milestone- and royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the relevant uniQure

Intellectual Property and uniQure's interest in the relevant Joint Intellectual Property, in each case that is necessary or useful due to the presence of the applicable Selected Capsid Variant therein, to research, Develop, make and have made, use and Commercialize that Third Party's Third Party Proposed Products in the Field.

(d) Any licenses granted to 4DMT under the uniQure Intellectual Property (including any subset or aspect of the uniQure Intellectual Property) pursuant to this Agreement, including, without limitation, pursuant to Sections 4.4 and 5.2, are limited to only uniQure Intellectual Property that specifically relates to Selected Capsid Variants (including patent claims specifying a Selected Capsid Variant or specifically claiming any methods of use or making any Selected Capsid Variants, and excluding all other uniQure Intellectual Property (e.g., without limitation, compositions of matter or methods of making compositions of matter and methods of manufacturing Products (but not the Selected Capsid Variant therein) pursuant to this Agreement).

5.3 Sublicenses. uniQure shall have the right to grant sublicenses under the license granted to it under Section 5.1(a) to Affiliates of uniQure and Third Parties; provided that any sublicense granted to a Third Party under this Agreement shall be pursuant to a written agreement that subjects such Sublicensee to all relevant restrictions and limitations set forth in this Agreement. uniQure shall provide 4DMT with the name and address of each Sublicensee of its rights under this ARTICLE V, the date of the grant of the sublicense and a description of the rights granted promptly after the execution and delivery of the sublicense agreement. uniQure shall remain responsible for the performance of its Sublicensees, and shall ensure that each Sublicensee complies with the applicable terms and conditions of this Agreement.

5.4 Rights Retained by the Parties. Except as expressly set forth in this Agreement, neither Party shall acquire any license or other intellectual property interest, by implication or otherwise, in any Confidential Information of the other Party or under any Patent Right or Know-How in which such other Party or its Affiliates has rights. Without limiting the generality of the foregoing, any of 4DMT's rights to 4DMT Intellectual Property not specifically licensed to uniQure shall be retained by 4DMT, and any of uniQure's rights to uniQure Intellectual Property not specifically licensed to 4DMT shall be retained by uniQure.

5.5 Section 365(n) of the Bankruptcy Code. All rights and licenses granted under or pursuant to any section of this Agreement are and will otherwise be deemed to be for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended or any comparable Law outside the United States (the "Bankruptcy Code"), licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code. Each Party agrees that the other Party, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code or any other provisions of applicable Law outside the United States that provide similar protection for "intellectual property." The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) the intellectual property licensed to such other Party and all embodiments of such intellectual property, to the extent necessary for such other Party to practice the licenses granted to it pursuant to this Agreement under such

intellectual property, which, if not already in such other Party's possession, will be promptly delivered to it upon such other Party's written request thereof. Any agreement supplemental hereto will be deemed to be "agreements supplementary to" this Agreement for purposes of Section 365(n) of the Bankruptcy Code.

5.6 Exclusivity.

(a) Original Exclusivity Removed. The Parties will have no rights or obligations pursuant to Sections 5.6(a) and (b) of the Original Agreement. As of the Amended CLA Effective Date and with respect to the entire Agreement, the parties have only those rights expressly provided in this Agreement.

(b) Of 4DMT. The Parties acknowledge that as of the Amended CLA Effective Date, without otherwise detracting from the license and intellectual property ownership rights expressly granted to uniQure hereunder (including, without limitation, with respect to uniQure's exclusive rights to any Selected Capsid Variants in the Field (recognizing however that uniQure's rights to Selected Capsid Variants may be partially non-exclusive due to any non-exclusive rights granted 4DMT under Section 4.4)), 4DMT or its Affiliates or licensees or sublicensees shall have the right to conduct pre-clinical research activities in the Field using Selected Capsid Variants and such activities shall not be deemed to violate the terms of this Section 5.6(b). For any and all such pre-clinical research activities in and outside of the Field using or related to any Selected Capsid Variants, Royalty Bearing Compounds or Royalty Bearing Products, 4DMT shall be obligated to provide to uniQure the Vector Characterization Data in accordance with the provisions of Section 4.3. 4DMT, its Affiliates, licensees and sublicensees shall have no right to conduct any other activities, including any development, manufacturing, commercialization or other use of Selected Capsid Variants, except pursuant to any rights pursuant to Section 4.4 of this Agreement or as otherwise expressly provided in this Agreement. Moreover, apart from any obligations of 4DMT related to the Selected Capsid Variants explicitly set forth in this Agreement, neither Party (including its Affiliates, licensees and sublicensees) shall have any exclusivity obligations to the other Party or its Affiliates whatsoever under this Agreement with respect to other AAV Capsid Variants (i.e., other than Selected Capsid Variants) for the Field.

(c) uniQure Independent Activities. The Parties acknowledge and agree that uniQure will conduct research, Development, manufacturing and Commercialization activities independently of this Agreement, inside and outside of the Field, including with respect to AAV Capsid Variants, AAV Capsid Variant Libraries, Gene Therapy Constructs, Compounds and Products, and no provision of this Agreement shall apply to any such activity.

5.7 UCB Agreement Pass-Through Provisions. uniQure acknowledges that 4DMT has provided it with a copy of the executed UCB Agreements, and agrees that this Agreement is subject in all respects to the terms and conditions of the UCB Agreements. Notwithstanding the generality of the foregoing:

(a) uniQure acknowledges that UC (and, to the extent applicable, IGT) may publish any and all technical data resulting from any research performed by UC (and, to the extent applicable, IGT) relating to the inventions disclosed in the UC Patent Rights, and UC (and, to the extent applicable, IGT) expressly reserves the right to use such inventions, UC AAV Capsid

Variants and related technology for its educational and research purposes, to disseminate the UC AAV Capsid Variants and other tangible materials associated with, or required to practice such inventions or the UC Patent Rights to researchers at nonprofit institutions for their educational and research purposes, and to permit other nonprofit institutions to use the UC AAV Capsid Variants to practice the UC Patent Rights for education and research purposes.

(b) uniQure shall keep 4DMT informed of its large/small entity status, as defined in 15 U.S.C. 632.

(c) uniQure acknowledges that certain of the inventions disclosed in the UC Patent Rights were funded in part by the U.S. Government, and agrees that in accordance with 35 U.S.C. 204, to the extent required by Law, any products covered by the UC Patent Rights and sold in the United States will be substantially manufactured in the United States.

(d) uniQure acknowledges that 4DMT's exclusive rights, privileges, and licenses under the UCB Agreements will expire on the date of the last-to-expire Valid Claim under the UC Patent Rights covered in each agreement, respectively, unless earlier terminated.

(e) For any sublicense under the UC Patent Rights that uniQure grants under Section 5.3, uniQure shall ensure that (i) such further sublicense is subject to a written sublicense agreement and is bound by all of the applicable terms, conditions, obligations, restrictions and other covenants of the UCB Agreements that protect or benefit UC's (and, if applicable, the U.S. Government's) rights and interests to the same extent that this Agreement does, and (ii) it or the Sublicensee shall, within [\*\*\*] ([\*\*\*)] days after executing such sublicense agreement, furnish to 4DMT for delivery to UC, subject to any confidentiality provisions, all material terms of such sublicense pertaining to UC's interests, including the Sublicensee's name and address, and indemnification of UC as provided in this Agreement.

(f) The Parties acknowledge and agree that upon termination of the UCB Agreements for any reason, uniQure's sublicenses under the UC Patent Rights under this Agreement will remain in effect and will be assigned to UC, except that UC will not be bound to perform any duties or obligations set forth herein that extend beyond the duties and obligations of UC set forth in the UCB Agreements.

(g) uniQure acknowledges that nothing contained in this Agreement will be construed as conferring any right to use in advertising, publicity or other promotional activities any name, trademark, trade name, or other designation of UC (including any contraction, abbreviation, or simulation of any of the foregoing), and that unless required by Law, regulation, or rules of a securities exchange, or consented to in writing by UC, the use by uniQure of the name "The Regents of the University of California" or the name of any University of California campus in advertising, publicity or other promotional activities is expressly prohibited.

## ARTICLE VI

### **PAYMENTS; ROYALTIES AND REPORTS**

6.1 Initial License Payment. In consideration of the rights to 4DMT Intellectual Property granted herein, uniQure shall pay to 4DMT non-creditable and non-refundable sums of:



(a) One Hundred Thousand Dollars (\$100,000) within [\*\*\*] ([\*\*\*)] Business Days after the later of (i) the Effective Date and (ii) receipt of an Invoice for such amount and a duly signed original of this Agreement and, thereafter, (b) One Hundred Thousand Dollars (\$100,000) within [\*\*\*] ([\*\*\*)] Business Days after the later of (i) the JRSC's approval of the initial Research Plan (including its associated budget) and (ii) receipt of an Invoice for such amount.

## 6.2 Research Program Funding.

(a) Out-of-Pocket Costs. Following approval of the Research Plan (including its associated budget), uniQure shall fund all out-of-pocket costs to be incurred by 4DMT as specifically contemplated in the Research Plan, in accordance with the agreed-upon budget for such costs set forth in the Research Plan or as otherwise agreed to by uniQure. On or before the first date of each Calendar Quarter during the Research Term, uniQure shall pay 4DMT for such out-of-pocket costs to be incurred by 4DMT during such Calendar Quarter. Within [\*\*\*] ([\*\*\*)] days after the end of each Calendar Quarter during the Research Term, 4DMT shall provide uniQure with a statement identifying such out-of-pocket costs incurred by 4DMT and paid to Third Parties in connection with the Research Program during such Calendar Quarter, in reasonable detail and with appropriate supporting documentation. If the supporting documentation shows that uniQure has overpaid or underpaid the out-of-pocket costs for such Calendar Quarter, 4DMT will, together with the supporting documentation, (i) send uniQure a credit note for the amount overpaid, upon which uniQure may credit the amount overpaid against any other payment due by uniQure under this Agreement, or if no other payment is due under this Agreement, 4DMT shall within [\*\*\*] ([\*\*\*)] days refund the amount overpaid to uniQure, or (ii) send uniQure an Invoice for the amount underpaid, which uniQure shall pay within [\*\*\*] ([\*\*\*)] days after uniQure's receipt of such Invoice. For clarity, no out-of-pocket costs will be paid by uniQure unless covered by an agreed-upon budget for such expenses set forth in the Research Plan or as otherwise agreed to by uniQure.

(b) 4DMT Committed FTEs. It is the Parties' intent that the Research Program will support the number of 4DMT FTEs in the performance of the activities under the Research Plan during the Research Term, as specified in the Research Plan and approved by the JRSC. Following approval of the Research Plan (including its associated budget), on or before the first day of each Calendar Quarter during the Research Term, uniQure shall pay 4DMT the FTE Costs for FTEs in the then-current Research Plan for such Calendar Quarter; provided that such payment may be prorated in the first and last Calendar Quarters of the Research Term. Within [\*\*\*] ([\*\*\*)] days after the end of each Calendar Quarter during the Research Term, 4DMT shall provide supporting documentation for the purpose of verifying the calculation of the FTE charges paid by uniQure for such Calendar Quarter. If the supporting documentation shows that uniQure has overpaid or underpaid the FTE payments for such Calendar Quarter, 4DMT will, together with the supporting documentation, (i) send uniQure a credit note for the amount overpaid, upon which uniQure may credit the amount overpaid against any FTE or other payment due by uniQure under this Agreement, or if no other payment is due under this Agreement, 4DMT shall within [\*\*\*] ([\*\*\*)] days refund the amount overpaid to uniQure, or (ii) send uniQure an Invoice for the amount underpaid, which uniQure shall pay within [\*\*\*] ([\*\*\*)] days after uniQure's receipt of such Invoice. For clarity, no FTE Costs will be paid by uniQure unless covered by an agreed-upon budget for such FTEs set forth in the Research Plan or as otherwise agreed to by uniQure.

(c) Equipment Payment Reimbursement. Any amount paid by uniQure pursuant to Section 6.2(a) for the purchase of equipment ("Equipment Payment") shall be subject to partial reimbursement by 4DMT in accordance with this Section 6.2(c). For each of the first [\*\*\*] ([\*\*\*]) Third Party collaborations 4DMT enters into after the Effective Date, 4DMT shall reimburse uniQure for a *pro rata* portion of the Equipment Payment based on the following formula: [\*\*\*]. For example, if 4DMT conducts [\*\*\*] ([\*\*\*]) Research Selection Processes hereunder and [\*\*\*] ([\*\*\*]) Selection Processes for the first such Third Party collaboration in which such equipment was actually used, 4DMT shall reimburse uniQure for [\*\*\*] percent ([\*\*\*]%) of the Equipment Payments. If 4DMT subsequently conducts another [\*\*\*] ([\*\*\*]) Selection Processes for the second Third Party collaboration in which such equipment was actually used, 4DMT shall reimburse uniQure for a further [\*\*\*] percent ([\*\*\*]%) of Equipment Payments, since the [\*\*\*] ([\*\*\*]) Research Selection Processes it conducted for uniQure represents [\*\*\*] of the aggregate Selection Processes conducted by 4DMT for uniQure and for the first [\*\*\*] ([\*\*\*]) Third Party collaborations 4DMT entered into after the Effective Date. 4DMT shall pay uniQure any such amount payable under this Section 6.2(c) within [\*\*\*] ([\*\*\*]) days after the end of the Calendar Quarter during which 4DMT conducted any Selection Process for either of the first [\*\*\*] ([\*\*\*]) Third Party collaborations 4DMT enters into after the Effective Date in which such equipment was actually used, and shall contemporaneously provide uniQure with a written report detailing the calculation of such amount.

6.3 DELETED.

6.4 Royalties.

On a Royalty Bearing Product-by-Royalty Bearing Product basis, uniQure shall pay to 4DMT royalties on worldwide Net Sales as provided in this Section 6.4:

(a) Royalty Rate. uniQure shall pay to 4DMT royalties on Net Sales of each Royalty Bearing Product by uniQure and its Affiliates equal to [\*\*\*] percent ([\*\*\*]%) of all such Net Sales of such Royalty Bearing Product achieved during the applicable Calendar Year.

(b) Royalty Term. uniQure's royalty obligations to 4DMT under this Section 6.4 shall be in effect on a country-by-country and Royalty Bearing Product-by-Royalty Bearing Product basis during the relevant Royalty Term. Upon expiration of the Royalty Term for a Royalty Bearing Product in a country, the license under Section 5.1(a) shall be fully paid-up, irrevocable, perpetual and exclusive under the relevant Licensed IP for such Royalty Bearing Product in such country.

(c) Royalty Adjustments.

(i) Non-Patented Product. If a Royalty Bearing Product is sold in a country and the composition of matter, formulation, or method of use of such Royalty Bearing Product is not Covered by a Valid Claim within the Licensed IP in such country at the time of sale, then the royalty rate for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the applicable rate determined pursuant to Section 6.4(a), unless such Royalty Bearing Product embodies an Invention

with respect to which uniQure made a Trade Secret Election, in which case no such reduction shall apply.

(ii) Third Party Offset. If uniQure is required, in order to avoid infringement of any Patent Right not licensed hereunder that Covers the composition of matter, formulation, or method of use of a Royalty Bearing Product, to obtain a license from a Third Party in order to Develop, make, have made, use or Commercialize such Royalty Bearing Product in a country in the Territory and to pay a royalty or other consideration under such license (including in connection with the settlement of a patent infringement claim), then the royalty payments due under Section 6.4(a) with respect to Net Sales for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the amounts payable by uniQure to such Third Party for such license that are reasonably and appropriately allocable to such Royalty Bearing Product in such country, provided that in no event shall the foregoing reduce the amount of royalties payable to 4DMT in any [\*\*\*] by more than [\*\*\*] percent ([\*\*\*]%) of the amount determined pursuant to Section 6.4(a), as adjusted by application of the terms of Section 6.4(c)(i).

(iii) Limits on Deductions. Except as expressly provided in this Section 6.4, there shall not be any offset to or deduction from the royalties payable pursuant to this Section 6.4. Notwithstanding Sections 6.4(c)(i) and (ii) to the contrary, in no event shall the cumulative effect of the deductions in Sections 6.4(c)(i) and (ii) reduce the royalties to less than [\*\*\*] percent ([\*\*\*]%) of the amounts determined pursuant to Section 6.4(a).

On a Royalty Bearing Product-by-Royalty Bearing Product basis, for each 4DMT Proposed Product commercialized by 4DMT and its Affiliates pursuant to Section 4.4, 4DMT shall pay to uniQure royalties on worldwide 4DMT Net Sales as provided in this Section 6.4:

(d) Royalty Rate. 4DMT shall pay to uniQure royalties on 4DMT Net Sales of each Royalty Bearing Product by 4DMT and its Affiliates equal to [\*\*\*] percent ([\*\*\*]%) of all such 4DMT Net Sales of such Royalty Bearing Product achieved during the applicable Calendar Year.

(e) Royalty Term. 4DMT's royalty obligations to uniQure under this Section 6.4 shall be in effect on a country-by-country and Royalty Bearing Product-by-Royalty Bearing Product basis during the relevant Royalty Term. Upon expiration of the Royalty Term for a Royalty Bearing Product in a country, the license under Section 4.4(c) shall be fully paid-up, irrevocable, perpetual and non-exclusive under the relevant Licensed IP for such Royalty Bearing Product in such country.

(f) Royalty Adjustments.

(i) Non-Patented Product. If a Royalty Bearing Product is sold in a country and the composition of matter, formulation, or method of use of such Royalty Bearing Product is not Covered by a Valid Claim within the Patent Rights sublicensed and licensed from

uniQure to 4DMT in such country at the time of sale, then the royalty rate for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the applicable rate determined pursuant to Section 6.4(a), unless such Royalty Bearing Product embodies an Invention with respect to which 4DMT made a Trade Secret Election, in which case no such reduction shall apply.

(ii) Third Party Offset. If 4DMT is required, in order to avoid infringement of any Patent Right not licensed hereunder that Covers the composition of matter, formulation, or method of use of a Royalty Bearing Product, to obtain a license from a Third Party in order to Develop, make, have made, use or Commercialize such Royalty Bearing Product in a country in the Territory and to pay a royalty or other consideration under such license (including in connection with the settlement of a patent infringement claim), then the royalty payments due under Section 6.4(a) with respect to 4DMT Net Sales for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the amounts payable by 4DMT to such Third Party for such license that are reasonably and appropriately allocable to such Royalty Bearing Product in such country, provided that in no event shall the foregoing reduce the amount of royalties payable to uniQure in any Calendar Quarter by more than [\*\*\*] percent ([\*\*\*]%) of the amount determined pursuant to Section 6.4(a), as adjusted by application of the terms of Section 6.4(c)(i).

(iii) Limits on Deductions. Except as expressly provided in this Section 6.4, there shall not be any offset to or deduction from the royalties payable pursuant to this Section 6.4. Notwithstanding Sections 6.4(c)(i) and (ii) to the contrary, in no event shall the cumulative effect of the deductions in Sections 6.4(c)(i) and (ii) reduce the royalties to less than [\*\*\*] percent ([\*\*\*]%) of the amounts determined pursuant to Section 6.4(a).

## 6.5 Sublicense Consideration.

(a) uniQure shall pay to 4DMT the following percentages (“Sublicense Income Sharing Percentages”) of Sublicense Consideration received by uniQure for sublicenses under the Licensed IP under this Agreement:

(i) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) does not require uniQure to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(ii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) requires uniQure to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(iii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that does not meet the criteria set forth in Section 6.5(a)(i) or Section 6.5(a)(ii) above;

provided, however, that none of subsections (i), (ii) or (iii) shall result in uniQure paying to 4DMT under this Section 6.5 a percentage of any Sublicense Consideration consisting of

royalties from Sublicensees on sales of UC Products during the applicable Royalty Term that is less than [\*\*\*] percent ([\*\*\*]%) of 4DMT Net Sales by such Sublicensee of such UC Products.

(b) The term “Sublicense Consideration” shall mean consideration of any kind received by uniQure from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees, royalties or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as defined below) for stock of uniQure in consideration for such sublicense; provided, however, the following are not included in Sublicense Consideration:

(i) Support for activities of uniQure relating to the research, Development, manufacturing or Commercialization of Royalty Bearing Products, which shall not exceed the fully burdened cost (and in the case of manufacturing costs, the Fully Burdened Manufacturing Cost) for undertaking such activities performed by or for uniQure (including Third Parties on uniQure’s behalf) by more than [\*\*\*] percent ([\*\*\*]%)

(ii) Proceeds derived from debt financing and any loans to uniQure by the Sublicensee;

(iii) Consideration received for the purchase of stock in uniQure or its Affiliate to the extent that the price per share for such equity does not exceed the Fair Market Value of such stock. The term “Fair Market Value” shall mean the average price at which the stock in question is publicly trading at for [\*\*\*] ([\*\*\*]) days prior to the earlier of (A) the date of the announcement of its purchase by the Sublicensee or (B) the date of its purchase by the Sublicensee, or if the stock is not publicly traded, the value of such stock as determined in good faith by the Board of Directors of uniQure or its applicable Affiliate as of the time of receipt of payment; and

(iv) Reimbursement of uniQure’s patent costs related to Patent Rights.

(c) 4DMT shall pay to uniQure the following percentages (“4D Sublicense Income Sharing Percentages”) of 4D Sublicense Consideration received by 4DMT for sublicenses under the Licensed IP under this Agreement:

(i) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) does not require 4DMT to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(ii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Compound or Product that is subject of the sublicense and (B) requires 4DMT to manufacture any such Compound or Product for Clinical Trial or commercial purposes;

(iii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that does not meet the criteria set forth in Section 6.5(a)(i) or Section 6.5(a)(ii) above;

provided, however, that none of subsections (i), (ii) or (iii) shall result in 4DMT paying to uniQure under this Section 6.5 a percentage of any 4D Sublicense Consideration consisting of royalties from Sublicensees on sales of UC Products during the applicable Royalty Term that is less than [\*\*\*] percent ([\*\*\*]%) of Net Sales by such Sublicensee of such UC Products.

(d) The term “4D Sublicense Consideration” shall mean consideration of any kind received by 4DMT from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees, royalties or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as defined below) for stock of 4DMT in consideration for such sublicense; provided, however, the following are not included in 4D Sublicense Consideration:

(i) Support for activities of 4DMT relating to the research, Development, manufacturing or Commercialization of Royalty Bearing Products, which shall not exceed the fully burdened cost (and in the case of manufacturing costs, the Fully Burdened Manufacturing Cost) for undertaking such activities performed by or for 4DMT (including Third Parties on 4DMT’s behalf) by more than [\*\*\*] percent ([\*\*\*]%)

(ii) Proceeds derived from debt financing and any loans to 4DMT by the Sublicensee;

(iii) Consideration received for the purchase of stock in 4DMT or its Affiliate to the extent that the price per share for such equity does not exceed the Fair Market Value of such stock. The term “Fair Market Value” shall mean the average price at which the stock in question is publicly trading at for [\*\*\*] ([\*\*\*]) days prior to the earlier of (A) the date of the announcement of its purchase by the Sublicensee or (B) the date of its purchase by the Sublicensee, or if the stock is not publicly traded, the value of such stock as determined in good faith by the Board of Directors of 4DMT or its applicable Affiliate as of the time of receipt of payment; and

(iv) Reimbursement of 4DMT’s patent costs related to Patent Rights.

(e) For purposes of this Article 6, “Sublicense Consideration received by uniQure” shall include Sublicense Consideration received by uniQure’s Affiliates (applying the definition of Sublicense Consideration *mutatis mutandis* to such Affiliates) and “4D Sublicense Consideration received by 4D” shall include 4D Sublicense Consideration received by 4DMT’s Affiliates (applying the definition of Sublicense Consideration *mutatis mutandis* to such Affiliates).

6.6 Reports; Payments. Within [\*\*\*] ([\*\*\*]) days after the end of each Calendar Quarter during which there are Net Sales or 4DMT Net Sales giving rise to a payment obligation under Section 6.4 or uniQure or 4DMT (as applicable) received Sublicense Consideration or 4D Sublicense Consideration giving rise to a payment obligation under Section 6.5, (a) uniQure or 4DMT (as applicable) shall submit to 4DMT or uniQure (as applicable) a report (i) identifying for each Royalty Bearing Product the Net Sales or 4DMT Net Sales for such Royalty Bearing Product for each country for such Calendar Quarter, the calculation of royalties (including

gross sales and all deductions taken from gross sales and all reductions pursuant to Section 6.4(c)), and the royalties payable to 4DMT or uniQure (as applicable) and (ii) identifying the Sublicense Consideration or 4D Sublicense Consideration received by uniQure or 4DMT (as applicable) in such Calendar Quarter and the one or more Sublicense Income Sharing Percentages or 4D Sublicense Income Sharing Percentages applicable to such Sublicense Consideration, and (b) uniQure or 4DMT (as applicable) shall pay to 4DMT or uniQure (as applicable) all royalties payable under Section 6.4 and portions of Sublicense Consideration or 4D Sublicense Consideration payable under Section 6.5.

6.7 Books and Records; Audit Rights. Each Party (the “Audited Party”) shall keep (and shall cause its Affiliates and Sublicensees to keep) complete, true and accurate books and records in accordance with its Accounting Standards in sufficient detail for the other Party (the “Auditing Party”) to determine the payments due and costs incurred under this Agreement. Each Auditing Party shall have the right, [\*\*\*] at its own expense, to have an independent, certified public accounting firm of nationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, review any such records of the Audited Party in the location(s) where such records are maintained by the Audited Party upon reasonable notice (which shall be no less than [\*\*\*] ([\*\*\*)] days prior notice) and during regular business hours and under obligations of strict confidence, for the sole purpose of verifying the accuracy of the amounts paid under this Agreement within a [\*\*\*] Calendar Year period preceding the date of the request for review. The report of such accounting firm shall be limited to a certificate stating whether any report made or invoice or payment submitted by the Audited Party during such period is accurate or inaccurate, the actual amounts of 4DMT or uniQure (as applicable) out-of-pocket expenses under Section 6.2(a), FTE Costs under Section 6.2(b), Equipment Payment reimbursements under Section 6.2(c), and any payments under Section 3.6, and the amount of any Net Sales, milestone, royalty or other payment discrepancy. No other information shall be provided to the Auditing Party. The Audited Party shall receive a copy of each such report concurrently with receipt by the Auditing Party. Should such inspection lead to the discovery of a discrepancy to the Auditing Party’s detriment, the Audited Party shall pay the amount of the discrepancy within [\*\*\*] ([\*\*\*)] days after its receipt from the accounting firm of the certificate showing the amount of the discrepancy. The Auditing Party shall pay the full cost of the review unless (a) uniQure or 4DMT (as applicable) was the Audited Party and the audit determined an underpayment of milestones or royalties which is greater than [\*\*\*] percent ([\*\*\*)% of the amount due for the applicable period, in which case uniQure or 4DMT (as applicable) shall pay the reasonable costs charged by such accounting firm for such review, or (b) 4DMT or uniQure (as applicable) was the Audited Party and the audit determined an overpayment of 4DMT or uniQure (as applicable) out-of-pocket expenses under Section 6.2(a) or FTE Costs under Section 6.2(b), or underpayment of Equipment Payment reimbursements under Section 6.2(c), which is greater than [\*\*\*] percent ([\*\*\*)% of the amount due for the applicable period, in which case 4DMT or uniQure (as applicable) shall pay the reasonable costs charged by such accounting firm for such review. Any overpayment of royalties by uniQure (or 4DMT, as applicable) revealed by an inspection shall be fully creditable against future royalty payments under Section 6.4. As of the Amended CLA Effective Date, notwithstanding anything express or implied, the Parties agree that there shall be no audits under this Section 6.7 as to accounting records for any time period prior to [\*\*\*] before the Amended CLA Effective Date.

6.8 Withholding Taxes. (a) Subject to the provisions of Section 12.7, if Laws require withholding by uniQure of taxes imposed upon 4DMT on account of any royalty or other payment paid under this Agreement, such taxes shall be deducted by uniQure as required by Law from such remittable royalty or other payment and shall be paid by uniQure to the proper tax authorities; provided that before making any such deduction or withholding, uniQure shall give 4DMT notice of the intention to make such deduction or withholding, which notice shall include the authority, basis and method of calculation for the proposed deduction or withholding, and shall be provided to the extent practicable at least a reasonable period of time before such deduction or withholding is required, in order for 4DMT to obtain reduction of or relief from such deduction or withholding. Official receipts of payment of withholding taxes shall be secured and sent to 4DMT as evidence of such payment. The Parties shall exercise their best efforts to ensure that any withholding tax imposed is reduced as far as possible under the provisions of any relevant tax treaty.

(b) Subject to the provisions of Section 12.7, if Laws require withholding by 4DMT of taxes imposed upon uniQure on account of any royalty or other payment paid under this Agreement, such taxes shall be deducted by 4DMT as required by Law from such remittable royalty or other payment and shall be paid by 4DMT to the proper tax authorities; provided that before making any such deduction or withholding, 4DMT shall give uniQure notice of the intention to make such deduction or withholding, which notice shall include the authority, basis and method of calculation for the proposed deduction or withholding, and shall be provided to the extent practicable at least a reasonable period of time before such deduction or withholding is required, in order for uniQure to obtain reduction of or relief from such deduction or withholding. Official receipts of payment of withholding taxes shall be secured and sent to uniQure as evidence of such payment. The Parties shall exercise their best efforts to ensure that any withholding tax imposed is reduced as far as possible under the provisions of any relevant tax treaty.

6.9 United States Dollars. All dollar (\$) amounts specified in this Agreement are United States dollar amounts.

6.10 Payment Method and Currency Conversion. Except as otherwise provided herein, all payments due to a Party hereunder shall be due and payable within [\*\*\*] ([\*\*\*)] days after receipt of an invoice from the other Party and shall be paid via a bank wire transfer to such bank account as such Party shall designate. For the purposes of determining the amount of any payment due hereunder for the relevant Calendar Quarter under Section 6.4 or Section 6.5, amounts received by a Party in any foreign currency shall be converted into United States dollars in accordance with the normal business practice of such Party, as applied consistently across its business.

6.11 Blocked Payments.

(a) If, by reason of applicable Laws in any country in the Territory, it becomes impossible or illegal for uniQure or any of its Affiliates or Sublicensees to transfer, or have transferred on its behalf, royalties or other payments to 4DMT, uniQure shall promptly notify 4DMT of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of 4DMT in a recognized banking institution with a good creditworthiness, such banking institution to be designated by 4DMT or, if none is designated



by 4DMT within [\*\*\*] ([\*\*\*) days, in a recognized banking institution selected by uniQure or its Affiliate or Sublicensee, as the case may be, and identified in a written notice given to 4DMT. If so deposited in a foreign country, uniQure shall provide, or cause its Affiliate or Sublicensee to provide, reasonable cooperation to 4DMT so as to allow 4DMT to assume control over such deposit as promptly as practicable.

(b) If, by reason of applicable Laws in any country in the Territory, it becomes impossible or illegal for 4DMT or any of its Affiliates or Sublicensees to transfer, or have transferred on its behalf, royalties or other payments to uniQure, 4DMT shall promptly notify uniQure of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of uniQure in a recognized banking institution with a good creditworthiness, such banking institution to be designated by uniQure or, if none is designated by uniQure within [\*\*\*] ([\*\*\*) days, in a recognized banking institution selected by 4DMT or its Affiliate or Sublicensee, as the case may be, and identified in a written notice given to uniQure. If so deposited in a foreign country, 4DMT shall provide, or cause its Affiliate or Sublicensee to provide, reasonable cooperation to uniQure so as to allow uniQure to assume control over such deposit as promptly as practicable.

6.12 Late Payments. Any payment not made within [\*\*\*] ([\*\*\*) Business Days after the due date for such payment pursuant to the terms of this Agreement shall bear interest at a rate of the thirty-day U.S. dollar LIBOR rate effective for the date that payment was due (as published in The Wall Street Journal, Eastern Edition) plus [\*\*\*]. Calculation of interest will be made for the exact number of days the payment was past due based on a year of 360 days (actual days/360).

## ARTICLE VII

### PATENTS

7.1 Disclosure. Each Party shall promptly disclose to the other Party any Inventions that it or its Affiliates or Sublicensees or their employees, independent contractors, or agents solely or jointly make, conceive, reduce to practice, or otherwise discover under this Agreement, and each Party shall maintain and make available to the other Party records regarding any Inventions that it has an obligation to assign under Section 7.2(a).

#### 7.2 Ownership.

(a) uniQure shall solely own all Core uniQure Intellectual Property, and 4DMT shall solely own all Core 4DMT Intellectual Property. Without additional consideration, each Party shall assign and hereby does assign to the other Party such of its right, title, and interest in and to such Patent Rights (and shall require its Affiliates and Sublicensees, and all employees, independent contractors and their employees, and agents of such Party and its Affiliates and Sublicensees to so assign to the other Party such of their right, title, and interest) as is necessary to effectuate the allocation of right, title, and interest as set forth in this Section 7.2(a).

(b) Except as set forth in Section 7.2(a), as between the Parties, (i) each Party shall solely own all Know-How and Inventions invented solely by employees, agents and

consultants of such Party or its Affiliates, and any Patent Right related thereto, subject to the licenses granted under ARTICLE V, and (ii) Know-How and Inventions invented jointly by employees, agents, or consultants of the Parties or their Affiliates (“Joint Intellectual Property”, which includes any Patent Right Covering such Know-How and Inventions (“Joint Patent Rights”) and any Know-How included in such Joint Intellectual Property (“Joint Know-How”)) shall be jointly owned, subject to the licenses granted under ARTICLE V. Inventorship shall be determined in accordance with U.S. patent Laws for purposes of determining ownership in accordance with the foregoing.

(c) Except as expressly provided in this Agreement, and subject to any restriction herein (including the licenses and exclusivity granted under ARTICLE V), (i) each joint owner may engage in research, Development, manufacturing and Commercialization activities relating to Joint Intellectual Property, and (ii) each may assign, license, sell or otherwise encumber or transfer any such interest without the prior written approval of the other Party and without obligation to account or provide compensation to the other Party.

### 7.3 uniQure Prosecution and Maintenance of Patent Rights.

(a) uniQure shall be solely responsible for the Prosecution and Maintenance of the uniQure Patent Rights, including the Core uniQure Patent Rights, at its sole expense and its sole discretion. uniQure shall give 4DMT an opportunity to review the text of each application, office action response or other substantive document for a Core uniQure Patent Right specifically relating to [\*\*\*] (but not any other uniQure Patent Right) before filing with any patent office in the Territory, shall consider 4DMT’s reasonable comments with respect thereto, and shall supply 4DMT with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number.

(b) uniQure shall have the sole right to determine whether any patent application is filed with respect to any Core uniQure Know-How and whether to maintain any Invention included in the Core uniQure Know-How as a trade secret. uniQure shall provide 4DMT with written notice if uniQure elects not to file a patent application claiming any particular Invention included in the Core uniQure Know-How specifically relating to compositions of matter of, methods of use of, or methods of making any Selected Capsid Variant because uniQure prefers to maintain such Invention as a trade secret (each, a “Trade Secret Election”).

(c) uniQure shall notify 4DMT at least [\*\*\*] ([\*\*\*]) days in advance of any applicable deadline if (i) uniQure decides that it does not wish to continue the Prosecution and Maintenance of a [\*\*\*] for which no substitute has been filed, or (ii) uniQure decides that it intends to abandon claim scope in a [\*\*\*], which claim scope is intended to be maintained by 4DMT, in which case, with respect to this clause (ii), 4DMT may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (i) or (ii), uniQure shall allow 4DMT to assume responsibility for Prosecution and Maintenance of such Core uniQure Patent Right or divisional application at 4DMT’s expense. If 4DMT assumes such responsibility, then 4DMT may designate any counsel of its choice reasonably acceptable to uniQure to handle the Prosecution and Maintenance of such Core uniQure Patent Right or divisional application (which shall otherwise continue to be part of the Core uniQure Patent Rights).

7.4 4DMT Prosecution and Maintenance of Patent Rights. 4DMT shall be solely responsible for the Prosecution and Maintenance of the 4DMT Patent Rights, including the Core 4DMT Patent Rights, at its sole expense and its sole discretion. 4DMT will reasonably inform uniQure regarding the Prosecution and Maintenance of 4DMT Patent Rights (including in any case, an update at least [\*\*\*]). Notwithstanding the foregoing, the Parties acknowledge that UC will handle the Prosecution and Maintenance of the UC Patent Rights in accordance with the terms of the UCB Agreements.

7.5 Prosecution and Maintenance of Joint Patent Rights. The Prosecution and Maintenance of any Joint Patent Right shall be through a mutually selected patent counsel. Within [\*\*\*] ([\*\*\*)] days following the Effective Date, the Parties shall agree on a patent counsel ("Joint Counsel") who shall be engaged by both Parties for the Prosecution and Maintenance of all such Joint Patent Rights. The following terms shall apply to each Joint Patent Right:

(a) The Parties shall instruct Joint Counsel to conduct its activities as follows: The Joint Counsel shall give uniQure and 4DMT (or each Party's designee) an opportunity to review the text of each application, office action response or other substantive document for a Joint Patent Right before filing with any patent office in the Territory, shall incorporate uniQure's and 4DMT's (or each Party's designee) reasonable comments with respect thereto, and shall supply uniQure and 4DMT (or each Party's designee) with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number. In the event that 4DMT and uniQure provide Joint Counsel with conflicting instructions regarding the Prosecution and Maintenance of a Joint Patent Right, Joint Counsel shall make the Parties aware of such conflicting instructions and, if the Parties are not able to resolve such conflict within a reasonable time prior to the applicable filing deadline, the Joint Counsel shall take such action as would reasonably be expected to maximize the scope, extent and coverage of such Joint Patent Right.

(b) Both Parties shall cooperate with Joint Counsel in Prosecution and Maintenance of patent applications for Joint Patent Rights, including providing Joint Counsel with data and other information as appropriate with respect thereto.

(c) Joint Counsel shall keep uniQure and 4DMT advised of the status of the Prosecution and Maintenance of Joint Patent Rights, including actual and prospective patent filings for Joint Patent Rights, and shall provide each Party with advance copies of any and all papers related thereto. Joint Counsel shall promptly give notice to uniQure and 4DMT of the grant, lapse, revocation, surrender, invalidation or abandonment of any Joint Patent Right.

(d) The Parties shall equally share all fees and costs charged by Joint Counsel with respect to the Prosecution and Maintenance of Joint Patent Rights and all other mutually agreed and approved out-of-pocket costs and expenses incurred by either Party in connection with such Prosecution and Maintenance of Joint Patent Rights.

(e) uniQure shall notify 4DMT and Joint Counsel at least [\*\*\*] ([\*\*\*)] days in advance of the next deadline if (A) uniQure decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) uniQure decides that it intends to abandon claim scope in a Joint Patent Right

which claim scope is intended to be maintained by 4DMT, in which case, with respect to this clause (B), 4DMT may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (A) or (B), uniQure shall allow 4DMT to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including payments incurred after [\*\*\*] ([\*\*\*)] days after receipt of uniQure's notice. If 4DMT assumes such responsibility, then: (i) 4DMT may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights; (ii) uniQure shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a 4DMT Patent Right; and (iii) uniQure shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to 4DMT as the sole owner. If 4DMT decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

(f) 4DMT shall notify uniQure and Joint Counsel at least [\*\*\*] ([\*\*\*)] days in advance of the next deadline if (A) 4DMT decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) 4DMT decides that it intends to abandon claim scope in a Joint Patent Right which claim scope is intended to be maintained by uniQure, in which case, with respect to this clause (B), uniQure may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (A) or (B), 4DMT shall allow uniQure to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including payments incurred after [\*\*\*] ([\*\*\*)] days after receipt of 4DMT's notice. If uniQure assumes such responsibility, then: (i) uniQure may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights and no further uniQure royalty obligations shall exist under this Agreement with respect thereto; (ii) 4DMT shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a uniQure Patent Right; and (iii) 4DMT shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to uniQure as the sole owner. If uniQure decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

#### 7.6 Third Party Infringement.

(a) Notice. Each Party shall promptly report in writing to the other Party any known or suspected (i) infringement of any of the 4DMT Patent Rights, uniQure Patent Rights or Joint Patent Rights, or (ii) unauthorized use or misappropriation of any of the 4DMT Know-How, uniQure Know-How or Joint Know-How, of which such Party becomes aware and shall provide the other Party with all available evidence regarding such known or suspected infringement or unauthorized use.

(b) Enforcement of Solely Owned Patent Rights. uniQure shall have the sole right to enforce the uniQure Patent Rights, including the Core uniQure Patent Rights. Subject to UC's rights under the UCB Agreements with respect to any UC Patent Right included

in the 4DMT Patent Rights, 4DMT shall have the sole right to enforce any 4DMT Patent Right, including the Core 4DMT Patent Rights. Each Party shall cooperate in the prosecution of any such suit brought by the enforcing Party as may be reasonably requested by the enforcing Party; provided that the enforcing Party shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by the non-enforcing Party in connection with such cooperation.

(c) Enforcement of Joint Patent Rights.

(i) In the Field. uniQure shall have the first right, but not the obligation, to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement in the Field. 4DMT shall cooperate in the prosecution of any such suit as may be reasonably requested by uniQure, including joining any action as party-plaintiff at uniQure's sole discretion; provided that uniQure shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by 4DMT in connection with such cooperation.

(ii) Outside the Field. 4DMT shall retain any and all rights to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement outside the Field. uniQure shall cooperate in the prosecution of any such suit as may be reasonably requested by 4DMT, including joining any action as party-plaintiff at 4DMT's sole discretion; provided that 4DMT shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by uniQure in connection with such cooperation.

(iii) Step-In Right. If either Party does not initiate a lawsuit or take other reasonable action pursuant to this Section 7.6(c) (the "Non-Enforcing Party"), then the other Party (the "Enforcing Party") shall have the right, but not the obligation, to initiate such lawsuit or take such other action, after providing [\*\*\*] ([\*\*\*)] days' notice to the Non-Enforcing Party and giving good faith consideration to the Non-Enforcing Party's reason(s) for not initiating a lawsuit or taking other action. For this purpose, the Non-Enforcing Party shall cooperate in the prosecution of any such suit as may be reasonably requested by the Enforcing Party, including joining any action as party-plaintiff at the Non-Enforcing Party's sole discretion; provided, that the Enforcing Party shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by the Non-Enforcing Party in connection with such cooperation.

(d) Conduct of Certain Actions; Costs. The Party initiating legal action shall have the sole and exclusive right to select counsel for any suit initiated by it pursuant to Section 7.6(b) or 7.6(c) (the "Initiating Party"). The Initiating Party shall bear its own out-of-pocket costs incurred in any such legal action, including the fees and expenses of the counsel selected by it. The other Party shall have the right to participate and be represented in any such legal action (in cases where such other Party has standing) by its own counsel at its own expense. The Initiating Party shall have the final say about the strategy and decisions in the suit and any settlement.

(e) Recoveries. Any amount recovered in any action or settlement of any such action shall be allocated first to equally reimburse each Party's actual out-of-pocket costs (including reasonable attorneys' fees and expenses) incurred in such action and any amount remaining shall be allocated to the Initiating Party; provided that if uniQure is the Initiating Party with respect to any such suit to enforce any Patent Right included in the Licensed IP in the Field, then, with respect to any remaining portion of such recovery, (i) any amount that reflects punitive or exemplary damages shall be allocated [\*\*\*] percent ([\*\*\*]%) to uniQure and [\*\*\*] ([\*\*\*]%) to 4DMT, and (ii) any other amounts shall be treated as Net Sales and subject to payment of royalties under Section 6.4(a); and provided further that if uniQure is the Initiating Party with respect to any such suit to enforce any Joint Patent Right outside the Field, or if 4DMT is the Initiating Party with respect to any such suit to enforce any Joint Patent Right in the Field, any amount remaining shall be allocated [\*\*\*].

7.7 Patent Invalidity Claim. Each Party shall promptly notify the other in the event of any legal or administrative action by any Third Party against a 4DMT Patent Right, uniQure Patent Right or Joint Patent Right of which it becomes aware, including any nullity, revocation, reexamination or compulsory license proceeding. To the extent such action is in connection with an enforcement of such Patent Right under Section 7.6, the Parties' rights with respect to defending any such Patent Right in any such proceeding shall correspond to those set forth in Section 7.6.

7.8 Patent Term Extensions.

(a) uniQure shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any uniQure Patent Right, including any Core uniQure Patent Right, and all costs and expenses relating thereto shall be paid by uniQure.

(b) 4DMT shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any 4DMT Patent Right, including any Core 4DMT Patent Right, and all costs and expenses relating thereto shall be paid by 4DMT.

(c) The Parties shall jointly determine how to defend any such action relating to any Joint Patent Right.

(d) The Parties shall reasonably cooperate with each other in obtaining patent term extensions or supplemental protection certificates or their equivalents in any country in the Territory.

7.9 Orange Book; Paragraph IV Certification.

(a) uniQure shall have the right, but not the obligation, to list any uniQure Patent Rights in the then-current edition of the FDA publication "Approved Drug Products With Therapeutic Equivalence Evaluations" (the "Orange Book"), or equivalent patent listings in other countries.

(b) With respect to any notification provided by a Third Party to uniQure or 4DMT under 21 U.S.C. § 355(j)(2)(B) making a certification described in 21 U.S.C. § 355(j)(2)(A)(vii)(IV) with respect to any uniQure Patent Right that is listed for a Royalty Bearing Product in the Orange Book, or equivalent actions in other countries, (each a “Paragraph IV Certification”), the following shall apply notwithstanding Sections 7.6 and 7.7:

(i) Without any avoidable delay, however at the latest within [\*\*\*] ([\*\*\*)] Business Days after receipt of any notification of a Paragraph IV Certification, such Party shall notify the other Party in writing and attach a copy of such notification. uniQure and 4DMT shall thereafter consult and cooperate fully to determine a course of action with respect to any such proceeding, including the negotiation of the offer of confidential access.

(ii) With respect to any uniQure Patent Right, uniQure shall have the sole right to initiate any infringement proceeding as a result of such Paragraph IV Certification (a “Paragraph IV Proceeding”) with respect to a Royalty Bearing Product, including by commencing a patent infringement action under 35 U.S.C. § 271(e)(2)(A), and shall bear the expense of any such Paragraph IV Proceeding and, if legally required, may commence such action in 4DMT’s or the relevant 4DMT Affiliate’s name and on 4DMT’s or the relevant 4DMT Affiliate’s behalf.

(iii) Section 7.6(e) shall apply if any amount is recovered in any Paragraph IV Proceeding or settlement of any Paragraph IV Proceeding under this Section 7.9(b).

7.10 CREATE Act. Each Party acknowledges and agrees that this Agreement is a “joint research agreement” as contemplated by 35 U.S.C. § 102(c), and that all Inventions are intended to have the benefit of the rights and protections conferred by the Cooperative Research and Enhancement Act of 2004 (the “CREATE Act”). In the event that a Party seeks to rely on the foregoing and to invoke the CREATE Act with respect to any Invention, such Party will give prior written notice to the other Party of its intent to invoke the CREATE Act and of each submission or disclosure such Party intends to make to the United States Patent and Trademark Office (the “USPTO”) pursuant to the CREATE Act, including: (a) any disclosure of the existence or contents of this Agreement to the USPTO, (b) the disclosure of any “subject matter developed by the other Party” (as such term is used in the CREATE Act) in an information disclosure statement or otherwise, or (c) the filing of any terminal disclaimer over the intellectual property of the other Party, it being agreed that no such submission, disclosure or filing shall be made by such Party without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned or delayed, except that no such consent shall be required to disclose to the USPTO, through an information disclosure statement or otherwise, any “subject matter developed by the other Party” that was previously published or included in a published patent application by the other Party. The other Party will provide reasonable cooperation to such Party in connection with such Party’s efforts to invoke and rely on the CREATE Act.

## ARTICLE VIII

### **CONFIDENTIALITY AND PUBLICATION**

8.1 Confidentiality Obligations. Each Party shall (a) maintain in confidence the Confidential Information of the other Party to the same extent such Party maintains its own confidential information, (b) not disclose such Confidential Information to any Third Party without the prior written consent of the other Party, and (c) not use such Confidential Information for any purpose except those permitted by this Agreement. Such obligations shall survive for a period of [\*\*\*] ([\*\*\*)] years after termination or expiration of this Agreement, except that such obligations shall survive with respect to any Confidential Information identified by the disclosing Party as a trade secret for so long as such Confidential Information remains a trade secret.

8.2 Exceptions to Confidentiality. Notwithstanding the foregoing, the obligations of confidentiality set forth in Section 8.1 shall not apply to information that, in each case as demonstrated by competent written documentation:

(a) is publicly disclosed or made generally available to the public by the disclosing Party, either before or after it becomes known to the receiving Party;

(b) was known to the receiving Party, without any obligation to keep it confidential, prior to the date of first disclosure by the disclosing Party to the receiving Party, as shown by the receiving Party's files and records;

(c) is subsequently disclosed to the receiving Party by a Third Party lawfully in possession thereof without obligation to keep it confidential and without a breach of such Third Party's obligations of confidentiality;

(d) has been publicly disclosed or made generally available to the public other than through any act or omission of the receiving Party or its Affiliates in breach of this Agreement; or

(e) has been independently developed by the receiving Party without the aid, application or use of the disclosing Party's Confidential Information (the competent written proof of which must be contemporaneous with such independent development).

8.3 Authorized Disclosure. Notwithstanding Section 8.1, a Party may disclose Confidential Information of the other Party to the extent such disclosure is reasonably necessary in the following instances:

(a) Prosecuting and Maintaining Patent Rights in accordance with this Agreement;

(b) making filings with Regulatory Authorities in accordance with this Agreement;

(c) complying with applicable Laws or submitting information to tax or other Governmental Authorities; provided that if a Party is required by Law to make any public



disclosure of Confidential Information of the other Party, to the extent it may legally do so, it will give reasonable advance notice to the other Party of such disclosure and will use its reasonable efforts to secure confidential treatment of such Confidential Information prior to its disclosure (whether through protective orders or otherwise);

(d) to its Affiliates, and to prospective and actual acquirers, licensees, sublicensees, employees, consultants, agents, accountants, lawyers, advisors, investors and underwriters, on a need to know basis, each of whom prior to disclosure must be bound by written or professional ethical obligations of confidentiality and non-use equivalent in scope to those set forth in this ARTICLE VIII and that are of reasonable duration in view of the circumstances of the disclosure; or

(e) to the extent mutually agreed to in writing by the Parties.

8.4 Scientific Publications. During the Research Term, neither Party shall first publish or first present in a public forum the scientific or technical results of any activity performed pursuant to this Agreement without the opportunity for prior review and comment by the other Party. Each Party agrees to provide the other Party with the opportunity to review any proposed abstract, manuscript or scientific presentation (including any verbal presentation) that relates to its activities performed pursuant to this Agreement during the Research Term, at least [\*\*\*] ([\*\*\*)] days prior to its intended submission for publication and agrees, upon request, not to submit any such abstract or manuscript for publication until the other Party is given a reasonable period of time up to [\*\*\*] ([\*\*\*)] months to secure patent protection for any material in such publication that it believes to be patentable. Both Parties understand that a reasonable commercial strategy may require delay of publication of information or filing of patent applications first with respect to activities performed or results obtained pursuant to this Agreement during the Research Term, or not to publish at all if necessary to preserve trade secrets. The Parties agree to review and decide whether to delay publication of such information to permit filing of patent applications. Neither Party shall have the right to publish or present any Confidential Information of the other Party, except as provided in Section 8.3. After the Research Term, each Party and its Affiliates may publish or present results, data or scientific findings of any of their activities performed after the Research Term without the prior review of the other Party, provided that such publication or presentation does not disclose any of the other Party's Confidential Information. After the Research Term, neither Party nor its Affiliates may publish or present any of the results, data or scientific findings of any activity performed by the other Party or its Affiliates pursuant to this Agreement without prior review and prior written consent of such other Party. Nothing contained in this Section 8.4 shall prohibit the inclusion of information necessary for a patent application; provided that the non-filing Party is given a reasonable opportunity to review the information to be included prior to submission of such patent application. For clarity, any publication under this Section 8.4 shall be consistent with uniQure's internal publication strategy, which shall be made available to 4DMT upon request. Nothing contained in this Section 8.4 shall prohibit either Party from disclosing the results, data or scientific findings of any activity performed by the other Party or its Affiliates pursuant to this Agreement without prior review and prior written consent of the other Party, where required, as reasonably determined by the disclosing Party's legal counsel, by applicable Law; provided that if a Party is required by Law to make any such disclosure, to the extent it may legally do so, it will give reasonable advance notice to the other Party of such

disclosure and will use its reasonable efforts to secure confidential treatment of such information prior to its disclosure (whether through protective orders or otherwise).

#### 8.5 Press Releases and Other Permitted Disclosures.

(a) 4DMT and uniQure each agree not to disclose any of the terms and conditions of this Agreement to any Third Party, except as described below in this Section 8.5. The Parties will cooperate in the release of a mutually agreed upon press release announcing the collaboration contemplated by this Agreement as soon as practicable after the Effective Date. Subject to the other provisions of this Agreement, no other press release, public statement or public disclosure concerning the existence or terms of this Agreement shall be made, either directly or indirectly, by either Party, without first obtaining the written approval of the other Party, which such approval shall not be unreasonably withheld or delayed beyond [\*\*\*] ([\*\*\*)] Business Days (or [\*\*\*] ([\*\*\*)] Business Days if the Party wishing to make such disclosure or any of its controlling Affiliates is then a public company) following submission to the approving Party of a draft of the respective press release, public statement or public disclosure. In no event shall any such subsequent press release, public statement or public disclosure by 4DMT disclose, if previously undisclosed, the identity of any Compound or Product or the stage of development of any Compound or Product that uniQure is researching, Developing, manufacturing, or Commercializing; provided that for clarity, uniQure may disclose, without the written approval of 4DMT, the identity of any Compound or Product or the stage of development of any Compound or Product that uniQure is researching, Developing, manufacturing, or Commercializing. In no event shall any such subsequent press release, public statement or public disclosure by a Party disclose, if previously undisclosed, the financial terms of this Agreement; provided that 4DMT may disclose the receipt of, and uniQure may disclose the payment of, any milestone payment but not the amount of such milestone payment; provided, further, however, that if disclosure of the amount of a milestone payment is required by applicable Law, by applicable stock exchange regulation, or by order or other ruling of a competent court, as set forth in Section 8.5(c), then 4DMT or uniQure, as the case may be, may also disclose such amount in a public statement or disclosure. Once any public statement or public disclosure has been approved in accordance with this Section 8.5, then either Party may appropriately communicate information contained in such permitted statement or disclosure.

(b) Either Party may disclose the existence and terms of this Agreement in confidence to its attorneys, to UC, and to each of the following, under an agreement with terms of confidentiality and non-use no less rigorous than the terms contained in this Agreement and, as applicable, to use such information solely for the purpose permitted pursuant to the applicable subsection of this Section 8.5(b):

- (i) professional accountants, consultants, or auditors;
- (ii) bankers or other financial advisors, in connection with an initial public offering, private financing or other strategic transaction, or corporate valuation for internal purposes;
- (iii) potential acquirers (and their respective attorneys and professional advisors), in connection with a potential merger, acquisition or reorganization;

provided that the Party making the disclosure has a *bona fide* offer (e.g., a signed term sheet or letter of intent, even if non-binding) from such Third Party for such a transaction;

(iv) to actual or potential investors, lenders or permitted assignees of such Party (and their respective attorneys and professional advisors); or

(v) to actual or potential licensees or sublicensees of such Party (and their respective attorneys and professional advisors); provided that such disclosure in the case of 4DMT shall not include any financial terms, the Candidate Success Criteria, the Delivery Success Criteria, or Schedule 1.76.

(c) Notwithstanding the foregoing provisions of this ARTICLE VIII, a Party may disclose the existence and terms of this Agreement, however excluding, as far as legally possible, Schedule 1.76, or the Parties' activities under this Agreement, where required, as reasonably determined by the legal counsel of the disclosing Party, by applicable Law, by applicable stock exchange regulation or by order or other ruling of a competent court, although, to the extent practicable, the other Party shall be given [\*\*\*] ([\*\*\*) Business Days advance notice of any such legally required disclosure to comment and reasonably consider such comments provided by such other Party on the proposed disclosure. In case either Party is obliged to publish this Agreement as a "material agreement" in accordance with the U.S. stock exchange regulations ("SEC Filing"), this Agreement shall be redacted by the filing Party as far as legally possible, and the filing Party shall cooperate with the other Party reasonably in advance to such SEC Filing to enable the other Party to review and comment on the scope of such redaction.

## ARTICLE IX

### REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION

9.1 Representations and Warranties of the Parties. uniQure and 4DMT each represent, warrant and covenant to the other that:

(a) as of the Effective Date, it has the authority and right to enter into and perform this Agreement and grant the rights embodied herein, and it is not aware of any legal impediment that could inhibit its ability to perform its obligations under this Agreement;

(b) as of the Effective Date, its execution, delivery and performance of this Agreement does not conflict with, or constitute a breach of, any order, judgment, agreement or instrument to which it is a party or is otherwise bound;

(c) it shall comply in all material respects with all Laws applicable to its actions under this Agreement; and

(d) as of the Effective Date, no consent of any Third Party is required for such Party to grant the licenses and rights granted to the other Party under this Agreement or to perform its obligations hereunder.

9.2 Representations and Warranties of 4DMT. 4DMT represents, warrants and covenants to uniQure that:

(a) as of the Effective Date, Schedule 1.5 is compiled accurately and, to the extent set forth in Section 1.5, is complete regarding the subject matter set forth therein;

(b) as of the Effective Date, 4DMT has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in 4DMT Intellectual Property in a manner inconsistent with the terms hereof;

(c) as of the Effective Date, 4DMT has valid and existing licenses, free and clear of all liens, charges and encumbrances, to the 4DMT Patent Rights not owned by 4DMT;

(d) as of the Effective Date, to 4DMT's knowledge, the conception, development and reduction to practice of the 4DMT Intellectual Property has not constituted or involved the misappropriation of trade secrets of any Third Party or the infringement of issued Patent Rights of any Third Party;

(e) as of the Effective Date, 4DMT has not received any written notice of any unauthorized use, infringement, or misappropriation by any person or entity, including any current or former employee or consultant of 4DMT, of any 4DMT Intellectual Property;

(f) as of the Effective Date, to 4DMT's knowledge, there are no claims, judgments, settlements pending or any action with respect to the 4DMT Intellectual Property;

(g) as of the Effective Date, to 4DMT's knowledge, uniQure's use of the 4DMT Intellectual Property, as reasonably anticipated to be used in the conduct of the Research Program, will not infringe any valid Patent Right existing as of the Effective Date and owned by any Third Party;

(h) all of 4DMT's personnel and employees, and Third Parties, including agents and consultants, hired by 4DMT and involved in the Research Program are, or when hired will be, under a written obligation to assign to 4DMT any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(i) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that arise on its part out of this Agreement or that would deprive uniQure of the benefits of or rights granted under this Agreement;

(j) as of the Effective Date, each of the UCB Agreements is in full force and effect, and 4DMT will not, after the Effective Date, terminate, amend or otherwise modify any of the terms thereof without prior written consent from uniQure, or take any action or refrain from taking any action that would permit UC to terminate any UCB Agreement (it being recognized that if the Selected Capsid Variants are not UC AAV Capsid Variants, and UC terminates any UCB Agreement, 4DMT shall not be deemed to be in breach of the foregoing), and 4DMT shall promptly provide uniQure with a copy of each notice it receives from UC under any UCB Agreement; and

(k) if, during the Term, 4DMT has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then 4DMT shall immediately notify uniQure in writing.

For purposes of this Section 9.2, “knowledge” shall mean the actual knowledge of 4DMT, including [\*\*\*].

9.3 Representations and Warranties of uniQure. uniQure represents, warrants and covenants to 4DMT that:

(a) all of uniQure’s personnel and employees, and Third Parties, including agents and consultants, hired by uniQure and involved in the Research Program are, or when hired will be, under a written obligation to assign to uniQure any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(b) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that arise on its part out of this Agreement or that would deprive 4DMT of the benefits of or rights granted under this Agreement;

(c) if, during the Term, uniQure has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then uniQure shall immediately notify 4DMT in writing.

9.4 No Other Warranties.

(a) EXCEPT AS OTHERWISE EXPRESSLY SET FORTH HEREIN, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, AND PARTICULARLY THAT PRODUCT(S) WILL BE SUCCESSFULLY DEVELOPED HEREUNDER, AND IF PRODUCT(S) ARE DEVELOPED, WITH RESPECT TO SUCH PRODUCT(S), THE PARTIES DISCLAIM ALL IMPLIED WARRANTIES OF TITLE, NON-INFRINGEMENT, MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE.

(b) uniQure acknowledges that UC has not warranted to 4DMT under the UCB Agreements as to the validity of any Patent Rights or that practice under such Patent Rights shall be free of infringement. UNIQURE, ITS AFFILIATES AND ITS SUBLICENSEE(S) AGREE THAT (I) THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS, THE UC AAV CAPSID VARIANTS, AND THE ASSOCIATED INVENTIONS ARE PROVIDED WITHOUT WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE OR ANY OTHER WARRANTY, EXPRESSED OR IMPLIED; (II) UC MAKES NO REPRESENTATION OR WARRANTY THAT ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS WILL NOT INFRINGE ANY PATENT OR OTHER PROPRIETARY RIGHT; AND (III) IN NO EVENT WILL UC BE LIABLE FOR ANY

INCIDENTAL, SPECIAL OR CONSEQUENTIAL DAMAGES RESULTING FROM EXERCISE OF THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS OR THE USE OF ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS.

9.5 Indemnification by uniQure. uniQure shall indemnify, hold harmless and defend 4DMT, its Affiliates and all of their respective officers, directors, employees, agents and shareholders (collectively, the “4DMT Indemnitees”) from and against any and all losses, damages, liabilities, judgments, fines, amounts paid in settlement, expenses and costs of defense (including reasonable attorneys’ fees and witness fees) (collectively, “Damages”) resulting from any demand, claim, action or proceeding brought or initiated by a Third Party (each a “Third Party Claim”) against any 4DMT Indemnatee to the extent arising out of: (a) a Default by uniQure; (b) the negligence or willful misconduct of a uniQure Indemnatee; or (c) the use, Development, Commercialization, storage or other exploitation of any Compound or Product by uniQure, its Affiliates, Sublicensees, Third Party Distributors, or Third Party independent contractors; provided that (i) the 4DMT Indemnitees shall comply with the procedures set forth in Section 9.7(a); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by 4DMT under Section 9.6.

9.6 Indemnification by 4DMT. 4DMT shall indemnify, hold harmless and defend uniQure, its Affiliates and all of their respective officers, directors, employees, agents, and shareholders (collectively, the “uniQure Indemnitees”) from and against any and all Damages resulting from any Third Party Claim against any uniQure Indemnatee to the extent arising out of: (a) a Default by 4DMT; (b) the negligence or willful misconduct of a 4DMT Indemnatee; or (c) the use, Development, Commercialization, storage or other exploitation of any 4DMT AAV Capsid Vector, Compound, Product (other than a Royalty Bearing Compound or Royalty Bearing Product), or 4DMT Product with which 4DMT proceeds under Section 4.4, in each case by 4DMT, its Affiliates, sublicensees or Third Party independent contractors; provided that (i) the uniQure Indemnitees shall comply with the procedures set forth in Section 9.7(b); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by uniQure under Section 9.5.

9.7 Procedure.

(a) To be eligible for the 4DMT Indemnitees to be indemnified hereunder, 4DMT shall provide uniQure with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.5 and the exclusive ability to defend or settle any such claim; provided however that uniQure shall not enter into any settlement for damages, or that imposes upon 4DMT any obligation or liability, without 4DMT’s prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. 4DMT shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by uniQure.

(b) To be eligible for the uniQure Indemnitees to be indemnified hereunder, uniQure shall provide 4DMT with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.6 and the exclusive ability to defend or settle any such claim; provided however that 4DMT shall not enter into any settlement for damages, or that

imposes upon uniQure any obligation or liability, without uniQure's prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. uniQure shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by 4DMT.

9.8 uniQure Indemnity to UC. uniQure shall, and shall require its Sublicensees to, indemnify, defend, and hold harmless UC and IGT, and their officers, employees, and agents; sponsor(s) of the research that led to the inventions disclosed in the UC Patent Rights and the UC AAV Capsid Variants; and the inventors of any UC Patent Rights and their employers against any and all losses, damages, costs, fees, and expenses resulting from Third Party claims and suits arising out of uniQure's activities under this Agreement or of any Sublicensee activities under any sublicense agreement granting rights under the UC Patent Rights or the UC AAV Capsid Variants, or any use or possession of the UC AAV Capsid Variants resulting from uniQure's exploitation of its rights thereto. This indemnification will include any product liability claims. uniQure will keep UC informed of its defense of any claims pursuant to this Section 9.8, and UC will cooperate reasonably in any such suit. If UC invokes the provisions of this Section 9.8, UC will not make any admissions or take any actions in such claim or suit that may prejudice or impair uniQure's ability to defend such claim or suit without uniQure's prior written consent, and uniQure will not admit liability or wrongdoing on behalf of UC without UC's prior written consent.

9.9 Insurance. Each Party shall procure and maintain insurance or self-insurance, including general liability insurance and product liability insurance, adequate to cover its obligations hereunder and that are consistent with normal business practices of prudent companies similarly situated, at all times during which any Research Compound, Royalty Bearing Compound, or Royalty Bearing Product is being Developed, clinically tested in human subjects or Commercialized by or on behalf of such Party, its Affiliates or sublicensees, including, in the case of uniQure, its Sublicensees. It is understood that any such insurance or self-insurance shall not be construed to create a limit of a Party's liability with respect to its indemnification obligations under this ARTICLE IX. Each Party shall provide the other Party with written evidence of such insurance or self-insurance upon request. Each Party shall provide the other Party with written notice at least [\*\*\*] ([\*\*\*)] days prior to the cancellation, non-renewal or material change in such insurance or self-insurance which could adversely affect rights hereunder. Without limiting the generality of the foregoing:

(a) uniQure, at its sole cost and expense, will ensure that the applicable entity performing activities in connection with any work performed hereunder, whether uniQure, an Affiliate, or a Sublicensee, will obtain, keep in force, and maintain the following insurance:

(i) prior to the start of Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included) with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

(ii) Upon the start of any Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

(iii) upon the First Commercial Sale of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

If the above insurance is written on a claims-made form, it shall continue for [\*\*\*] ([\*\*\*)] years following termination or expiration of this Agreement.

(iv) worker's compensation as legally required in the jurisdiction in which uniQure, an Affiliate, or a Sublicensee, as applicable, is doing business.

uniQure will promptly notify UC of any material reduction in the insurance coverages below the amounts required hereunder.

(b) Within [\*\*\*] ([\*\*\*)] days after the Effective Date, uniQure will furnish 4DMT with certificates of insurance evidencing compliance with all requirements. Such certificates will:

(i) where possible, provide for [\*\*\*] ([\*\*\*)] days' ([\*\*\*] ([\*\*\*)] days for non-payment of premium) advance written notice to 4DMT and UC of any cancellation of insurance coverages described above in Section 9.9(a);

(ii) indicate that 4DMT and UC have been endorsed as additional insureds under the coverage described above in Section 9.9(a); and

(iii) include a provision that the coverages described above in Section 9.9(a) will be primary and will not participate with, nor will be excess over, any valid and collectable insurance or program of self-insurance maintained by 4DMT or UC.

9.10 No Consequential or Punitive Damages. EXCEPT WITH RESPECT TO (a) THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER THIS AGREEMENT WITH RESPECT TO THIRD PARTY CLAIMS, (b) A BREACH OF THE



CONFIDENTIALITY OBLIGATIONS OF ARTICLE VIII, (c) A BREACH OF SECTION 5.6, OR (d) A PARTY'S WILLFUL MISCONDUCT, NEITHER PARTY HERETO WILL BE LIABLE FOR INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL, EXEMPLARY OR PUNITIVE DAMAGES, INCLUDING LOST PROFITS, ARISING FROM OR RELATING TO THIS AGREEMENT, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES.

## ARTICLE X

### TERM AND TERMINATION

10.1 Term and Expiration. This Agreement shall be effective as of the Effective Date and unless terminated earlier pursuant to Section 10.2, this Agreement shall continue in effect until the expiration of all of uniQure's and 4DMT's payment obligations hereunder (the "Term"). Upon expiration, all licenses granted hereunder shall be fully paid-up, perpetual and irrevocable.

#### 10.2 Termination.

##### (a) Termination of Agreement for Cause.

(i) This Agreement may be terminated at any time during the Term upon written notice by either Party (the "Non-Defaulting Party") upon Default of the other Party (the "Defaulting Party"), which Default remains uncured for ninety (90) days after written notice requesting cure of such Default. The Non-Defaulting Party shall provide written notice to the Defaulting Party, which notice shall identify the Default, the intent to so terminate and the actions or conduct that it considers would be an acceptable cure of such Default. If the Defaulting Party disputes the Default under this Section 10.2(a), then the issue of whether the Non-Defaulting Party may properly terminate this Agreement on expiration of the applicable cure period shall be resolved in accordance with ARTICLE XI. If, as a result of such dispute resolution process, it is determined that the alleged Defaulting Party committed a Default and the Defaulting Party does not cure such Default within sixty (60) days after the date of such dispute resolution award (the "Additional Cure Period"), then such termination shall be effective as of the expiration of the Additional Cure Period. If the Parties dispute whether such Default was so cured, either Party alone may request the same tribunal to determine whether it was so cured, and the Parties shall cooperate to allow such determination to be made within thirty (30) days after such request by either Party. Any such dispute resolution proceeding does not suspend any obligation of either Party hereunder, and each Party shall use reasonable efforts to mitigate any damage. If, as a result of any such dispute resolution proceeding, it is determined that the alleged Defaulting Party did not commit such Default (or such Default was cured in accordance with this Section 10.2(a)), then no termination shall be effective, and this Agreement shall continue in full force and effect. Notwithstanding the foregoing, if 4DMT is the Non-Defaulting Party and the claimed Default by uniQure as the Defaulting Party relates to one or more Compounds or Products, and not this entire Agreement, then this Agreement shall be terminated only with respect to the Indication for which such Compound(s) or Product(s) were intended to treat and such Indication shall be removed from the Field.

(ii) Notwithstanding Section 10.2(a)(i), uniQure shall have the right to terminate this Agreement during the Research Term immediately upon written notice to

4DMT if David Schaffer ceases to be a representative of 4DMT on the JRSC or is otherwise unavailable to direct 4DMT's Research Program activities during any consecutive fifteen (15) Business Day period, in each case for any reason other than his death, illness or disability, which shall be deemed a Default by 4DMT.

(b) Termination for Bankruptcy. To the extent allowed under applicable Law, either Party shall have the right to terminate this Agreement in the event of the commencement of any proceeding in or for bankruptcy, insolvency, dissolution or winding up by or against the other Party (other than pursuant to a corporate restructuring) that is not dismissed or otherwise disposed of within sixty (60) days thereafter.

(c) Termination for Futility. uniQure shall have the right terminate this Agreement immediately upon written notice to 4DMT summarizing the basis for such termination if, at any point prior to the first (1<sup>st</sup>) anniversary of the Effective Date, the JRSC determines that (i) it would be futile to continue the Research Program, including if the JRSC determines that any Candidate Success Criteria or Delivery Success Criteria cannot be met through use of the 4DMT Intellectual Property following the reasonable efforts of 4DMT to achieve such Candidate Success Criteria or Delivery Success Criteria or (ii) 4DMT is not making *bona fide* efforts to achieve the timelines set forth in the Research Plan.

(d) Termination for Convenience. uniQure shall have the right terminate this Agreement at any time after the Research Term, for any reason or for no reason, by giving 4DMT ninety (90) days' prior written notice thereof.

(e) Special Termination Right of 4DMT. In the event that (i) uniQure B.V. does not complete an underwritten public offering of its ordinary shares pursuant to an effective registration statement under the U.S. Securities Act of 1933 and the listing of its ordinary shares on the Nasdaq Global Market by September 1, 2014, December 31, 2014, or December 31, 2015, as the case may be, and (ii) uniQure B.V. has not agreed in writing to pay the applicable "Cash-Out Amount" provided for in Article 4c of each of the Grant Letters in respect of options that will vest on the first vesting date following such applicable date, 4DMT shall have the right to terminate this Agreement by providing written notice thereof to uniQure within thirty (30) days following such applicable date, and any such termination shall be effective as of the thirtieth (30<sup>th</sup>) day following such applicable date.

### 10.3 Effect of Termination

(a) If uniQure terminates this Agreement under Section 10.2(a) or Section 10.2(b):

(i) uniQure's licenses pursuant to this Agreement shall continue; provided however that uniQure shall continue to fulfill uniQure's payment obligations with respect to milestones and royalties under ARTICLE VI; and provided further that uniQure may reduce such payment obligations by the amount of monetary damage suffered by uniQure as a direct result of 4DMT's Default, as determined (A) in a final decision of the arbitrators in accordance with Section 11.2 or, with respect to an Excluded Claim, a court of competent

jurisdiction, which decision is not appealable or has not been appealed within the time allowed for appeal, or (B) by the Parties in a settlement agreement;

(ii) 4DMT shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination of this Agreement;

(iii) For clarity, uniQure shall be released of its ongoing diligence obligations under Section 4.2 and uniQure and 4DMT shall be released of their disclosure and information exchange obligations under ARTICLE III and ARTICLE IV;

(iv) For clarity, the JRSC and its subcommittees shall not meet anymore;

(v) No further options under each Grant Letter shall vest from and after the effective date of such termination; and

(vi) If this Agreement is terminated pursuant to Section 10.2(a)(ii), uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [\*\*\*] ([\*\*\*)] months immediately following the effective date of such termination.

(b) Upon termination of this Agreement by uniQure under Section 10.2(c) or Section 10.2(d), or by 4DMT under Section 10.2(a), Section 10.2(b), or Section 10.2(e):

(i) For clarity, uniQure's licenses pursuant to Section 5.1 and 4DMT's exclusivity obligations pursuant to Section 5.6 shall terminate as of the effective date of such termination;

(ii) Effective as of the effective date of such termination, the license granted to 4DMT under Section 5.2(b) shall be automatically expanded to include the Selected Capsid Variants and all fields of use;

(iii) uniQure shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to 4DMT, copies of all 4DMT's Confidential Information and 4DMT Intellectual Property and all Materials provided by 4DMT; except that uniQure may retain one copy of the 4DMT Confidential Information solely for legal archive purposes;

(iv) 4DMT shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination or are granted upon termination of this Agreement;

(v) For a period of [\*\*\*] ([\*\*\*) months, if termination occurs after Regulatory Approval of Royalty Bearing Products, uniQure and its Affiliates shall be entitled to finish work in progress and to sell any of the Royalty Bearing Products remaining in inventory in accordance with the terms of this Agreement to the extent such Royalty Bearing Products were being sold in the Territory at the time of termination, provided that such sales shall be subject to the royalty and milestone provisions of this Agreement;

(vi) If this Agreement is terminated pursuant to Section 10.2(c), (A) uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [\*\*\*] ([\*\*\*) months immediately following the effective date of such termination, but in no event for less than [\*\*\*] after the Effective Date, and (B) no further options under each Grant Letter shall vest from and after the date that [\*\*\*] percent ([\*\*\*)% of all options under such Grant Letter have vested; and

(vii) If this Agreement is terminated pursuant to Section 10.2(e), uniQure shall continue to fund the FTEs included in the Research Plan pursuant to Section 6.2(b) for the [\*\*\*] ([\*\*\*) months immediately following the effective date of such termination, but in no event for less than [\*\*\*] after the Effective Date.

Notwithstanding the foregoing, if such termination is under Section 10.2(a) solely with respect to one or more given Indication(s), then uniQure's licenses pursuant to Section 5.1 do not terminate but the Field is automatically narrowed to exclude the relevant Indication(s), and 4DMT's exclusivity obligations pursuant to Section 5.6 terminate solely with respect to the relevant Indication(s); subsection (ii) shall not apply; the license granted to 4DMT under Section 5.2(b) shall be automatically expanded to include the relevant Indication(s) rather than all fields of use; and uniQure's obligations under subsection (iii) shall be limited to copies of 4DMT's Confidential Information and 4DMT Intellectual Property and Materials that relate solely to the relevant Indication(s).

#### 10.4 Effect of Expiration or Termination; Survival.

(a) Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination. Any expiration or termination of this Agreement shall be without prejudice to the rights of either Party against the other accrued or accruing under this Agreement prior to expiration or termination, including the obligation to pay royalties for Royalty Bearing Product(s) sold prior to such expiration or termination. Termination of this Agreement shall be in addition to, and shall not prejudice, the Parties' remedies at law or in equity, including the Parties' ability to receive legal damages or equitable relief with respect to any breach of this Agreement, regardless of whether or not such breach was the reason for the termination.

(b) The provisions of ARTICLE I, ARTICLE VII, ARTICLE VIII, ARTICLE XI, ARTICLE XII, and Sections 4.5, 5.2(b), 5.4, 5.5, 6.2(c), 9.4, 9.5, 9.6, 9.7, 9.8, 9.9, 9.10, 10.3 and 10.4 shall survive any expiration or termination of this Agreement, and with respect to those Royalty Bearing Products in such countries for which uniQure retains a Development and Commercialization license after the expiration or termination of this Agreement, the provisions of ARTICLE VI shall also survive.

## ARTICLE XI

### **DISPUTE RESOLUTION**

11.1 Seeking Consensus. If any dispute arises out of, in connection with or related to this Agreement, including disputes over the interpretation, performance, enforcement or breach of this Agreement, including any dispute that is not within the jurisdiction of the JRSC, (a “Dispute”), excluding any dispute resolved in accordance with Section 2.5(c) (subject to Section 2.5(d)), then upon the written request of either Party, the matter shall be referred to the Executives, who shall meet in a good faith effort to resolve the dispute within [\*\*\*] ([\*\*\*]) days. If the Parties’ Executives cannot agree on a resolution of the Dispute within such [\*\*\*] ([\*\*\*]) day period, then it shall be resolved pursuant to the remaining provisions of this ARTICLE XI.

11.2 Arbitration. If the Parties do not fully settle a Dispute pursuant to Section 2.5 (only as to those matters that may be referred to arbitration) or 11.1, as applicable, and a Party wishes to pursue the matter, each such Dispute that is not an Excluded Claim (as defined below) shall be finally resolved by binding arbitration in accordance with the Rules of Arbitration of the ICC (International Chamber of Commerce) and judgment on the arbitration award may be entered in any court having jurisdiction thereof.

(a) The arbitration shall be conducted by a panel of three (3) persons. Within [\*\*\*] ([\*\*\*]) days after initiation of arbitration, each Party shall select one person to act as arbitrator and the two Party-selected arbitrators shall select a third arbitrator within [\*\*\*] ([\*\*\*]) days after their appointment. If the arbitrators selected by the Parties are unable or fail to agree upon the third arbitrator, the third arbitrator shall be appointed by the ICC. The place of arbitration shall be New York City, New York, and all proceedings and communications shall be in English.

(b) Either Party may apply to the arbitrators for interim injunctive relief until the arbitration award is rendered or the Dispute is otherwise resolved. Either Party also may, without waiving any remedy under this Agreement, seek from any court having jurisdiction any injunctive or provisional relief necessary to protect the rights or property of that Party pending the arbitration award. The scope of the authority of the arbitrators shall be limited to the strict application of law. The arbitrators shall have no authority to award punitive or any other type of damages not measured by a Party’s compensatory damages, except as permitted by Section 9.10. Each Party participating in an arbitration pursuant to the terms of this Agreement shall, [\*\*\*]. The arbitrators shall have the power to award recovery of all costs (including reasonable attorney’s fees, administrative fees, arbitrators’ fees and court costs) to the prevailing Party.

(c) Neither Party shall be required to give general discovery of documents, but may be required to produce documents or testimony that are relevant or considered relevant by the arbitrators to the Dispute. It is the objective and intent of the Parties that any arbitration proceeding be conducted in such a manner that a decision will be rendered by the arbitrators within [\*\*\*] ([\*\*\*]) days after the third arbitrator is appointed to the panel, and the Parties and the panel selected in the manner provided above will adopt rules and procedures intended to implement such objective and intent.

(d) Except to the extent necessary to confirm or vacate an award or as may be required by Law (including applicable securities laws or the rules of any stock exchange on which a Party's securities may then be listed), neither a Party nor an arbitrator may disclose the existence, content, or results of arbitration without the prior written consent of both Parties. In no event shall arbitration be initiated after the date when commencement of a legal or equitable proceeding based on the dispute, controversy or claim would be barred by the applicable New York statute of limitations.

(e) The Parties agree that any payment made pursuant to this Agreement pending resolution of the Dispute shall be refunded or credited if the arbitrators or court determines that such payments are not due.

As used in this Section 11.2, the term "Excluded Claim" shall mean a Dispute that concerns (a) the validity, enforceability, scope or infringement of a patent, trademark or copyright; or (b) any antitrust, anti-monopoly or competition law or regulation, whether or not statutory.

## ARTICLE XII

### MISCELLANEOUS

12.1 Governing Law. This Agreement shall be governed by and construed in accordance with the Laws of the State of New York, other than any principle of conflict or choice of laws that would cause the application of the Laws of any other jurisdiction.

12.2 Waiver. Waiver by a Party of a breach hereunder by the other Party shall not be construed as a waiver of any succeeding breach of the same or any other provision. No delay or omission by a Party to exercise or avail itself of any right, power or privilege that it has or may have hereunder shall operate as a waiver of any right, power or privilege by such Party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the Party granting the waiver.

12.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address specified in this Section 12.3 and shall be: (a) delivered personally; (b) transmitted by facsimile; (c) sent by registered or certified mail, return receipt requested, postage prepaid; or (d) sent via a reputable international overnight delivery service. Any such notice, instruction or communication shall be deemed to have been delivered (i) upon receipt if delivered by hand, (ii) when transmitted with electronic confirmation of receipt, if transmitted by facsimile (if such transmission is on a Business Day; otherwise, on the next Business Day following such transmission), provided that an original document is sent via an internationally recognized overnight delivery service (receipt requested), (iii) three (3) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, or (iv) one (1) Business Day after it is sent via a reputable international overnight delivery service.

If to 4DMT, to: 4D Molecular Therapeutics, Inc.  
5858 Horton St. Emerystation North, Suite 460,  
Emeryville, CA 94608  
Facsimile: (650) 463-2600

with a copy to: Latham & Watkins LLP  
140 Scott Drive  
Menlo Park, CA 94025  
Attention: Alan Mendelson and Judith Hasko  
Facsimile: (650) 463-2600

And

[\*\*\*]

If to uniQure, to: uniQure biopharma B.V.  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Attention: CEO  
Facsimile: +31 20 566 9272

with a copy to: [\*\*\*]

or to such other address as the Party to whom notice is to be given may have furnished to the other Party in writing in accordance herewith.

12.4 Entire Agreement; Amendment. This Agreement (including its Exhibits and Schedules) contains the complete understanding of the Parties with respect to the subject matter hereof and supersedes all prior understandings and writings relating to such subject matter. In particular, it supersedes and replaces the Prior Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties or their Affiliates prior to the Effective Date. No amendment, change or addition to this Agreement will be effective or binding on either Party unless reduced to writing and duly executed on behalf of both Parties.

12.5 Headings. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement.

12.6 Severability. If any provision or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement shall be construed as if such clause of portion thereof had never been contained in this Agreement, and there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by applicable Law.

12.7 Assignment. Neither this Agreement nor any right or obligation hereunder may be assigned or otherwise transferred by any Party without the consent of the other Party; provided, however, that any Party may, without such consent, assign this Agreement, in whole or in part: (a) to any of its respective Affiliates; provided that the assigning Party shall remain jointly and severally liable with such Affiliate in respect of all obligations so assigned, or (b) to any successor in interest by way of merger, acquisition or sale of all or substantially all of its assets to which this Agreement relates (an “M&A Event”). Any assignment not in accordance with this Section 12.7 shall be void. Each Party agrees that, notwithstanding any provision of this Agreement to the contrary, neither the assignment of this Agreement by a Party in connection with an M&A Event, nor the occurrence of such M&A Event (whether or not a formal assignment of this Agreement occurs), shall provide the non-assigning Party with rights or access to any intellectual property or technology of the acquirer of the assigning Party or its Affiliates that were not Affiliates of the assigning Party prior to such M&A Event. If uniQure assigns its rights and obligations hereunder to an Affiliate or Third Party outside the United States or The Netherlands pursuant to this Section 12.7, and if such Affiliate or Third Party shall be required by applicable Law to withhold additional taxes from or in respect of any amount payable under this Agreement as a result of such assignment, then any such amount payable under this Agreement shall be increased to take into account the additional taxes withheld as may be necessary so that, after making all required withholdings, 4DMT receives an amount equal to the sum it would have received had no such assignment been made.

12.8 Counterparts. This Agreement may be executed in any number of counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument. Signatures provided by facsimile transmission or in Adobe Portable Document Format (PDF) sent by electronic mail shall be deemed to be original signatures.

12.9 Force Majeure. No Party shall be liable for failure of or delay in performing obligations (other than payment obligations) set forth in this Agreement, and no Party shall be deemed in breach of its obligations, if such failure or delay is due to a natural disaster, explosion, fire, flood, tornado, thunderstorm, hurricane, earthquake, war, terrorism, riot, embargo, loss or shortage of power, labor stoppage, substance or material shortage, events caused by reason of laws of any Governmental Authority, events caused by acts or omissions of a Third Party or any other cause reasonably beyond the control of such Party, if the Party affected gives prompt notice of any such cause to the other Party. The Party giving such notice shall thereupon be excused from such of its obligations hereunder as it is thereby disabled from performing for so long as it is so disabled, provided, however, that such affected Party commences and continues to use its Commercially Reasonable Efforts to cure such cause.

12.10 Third Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, other than a 4DMT Indemnitee under Section 9.5 or uniQure Indemnitee under Section 9.6. No such Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against either Party.

12.11 Relationship of the Parties. Each Party shall bear its own costs incurred in the performance of its obligations hereunder without charge or expense to the other, except as expressly provided in this Agreement. Neither Party shall have any responsibility for the hiring,



termination or compensation of the other Party's employees or for any employee compensation or benefits of the other Party's employees. No employee or representative of a Party shall have any authority to bind or obligate the other Party for any sum or in any manner whatsoever, or to create or impose any contractual or other liability on the other Party without said other Party's approval. For all purposes, and notwithstanding any other provision of this Agreement to the contrary, the legal relationship under this Agreement of each Party to the other Party shall be that of independent contractor. Nothing in this Agreement shall be construed to establish a relationship of partners or joint ventures between the Parties.

12.12 Performance by Affiliates. To the extent that this Agreement imposes obligations on Affiliates of a Party or permits a Party to exercise its rights or perform its obligations through its Affiliates, such Party agrees to cause its Affiliates to perform such obligations and shall guarantee performance of this Agreement by its Affiliates. If any disagreement arises out of the performance of this Agreement by an Affiliate of a Party, or the alleged failure of an Affiliate to comply with the conditions and obligations of this Agreement, the Party seeking to resolve such dispute shall have the right do so directly with the other Party, without any obligation to first pursue an action against, or recovery from, the Affiliate which is alleged to have caused a breach of this Agreement.

12.13 Construction. Each Party acknowledges that it has been advised by counsel during the course of negotiation of this Agreement, and, therefore, that this Agreement shall be interpreted without regard to any presumption or rule requiring construction against the Party causing this Agreement to be drafted. Any reference in this Agreement to an ARTICLE, Section, subsection, paragraph, clause, or Schedule shall be deemed to be a reference to any article, section, subsection, paragraph, clause, schedule or exhibit, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (a) wherever used, the use of any gender will be applicable to all genders; (b) the word "or" is used in the inclusive sense (and/or); (c) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restriction on such amendments, supplements or modifications set forth herein or therein); (d) any reference to any Law refers to such Law as from time to time enacted, repealed or amended; (e) the words "herein", "hereof" and hereunder", and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof; and (f) the words "include", "includes" and "including" shall be deemed to be followed by the phrase "but not limited to", "without limitation" or words of similar import.

*[Signature page follows]*

**IN WITNESS WHEREOF**, the Parties have executed this Amended and Restated Collaboration and License Agreement as of the Amended CLA Effective Date.

**UNIQUE BIOPHARMA B.V.**

**4D MOLECULAR THERAPEUTICS, INC.**

BY: s/s Lilly Burggraaf  
NAME: Lilly Burggraaf  
TITLE: Vice President, Global Human Resources

BY: s/s David Kirn  
NAME: David Kirn, MD  
TITLE: Chief Executive Officer

List of Schedules

Exhibit A	Commitment Letter from uniQure B.V.
Schedule 1.5	4DMT Patent Rights
Schedule 1.41	Outline of Budget for Research Plan
Schedule 1.54	Draft Invoice
Schedule 1.76	Outline of Research Plan

**Exhibit A**

**COMMITMENT LETTER FROM UNIQUE B.V.**

**[\*\*\*]**

EXHIBIT A - Page 1

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**Schedule 1.5**

**4DMT PATENT RIGHTS**

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SCHEDULE 1.5 - Page 1

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**Schedule 1.41**

**OUTLINE OF BUDGET FOR RESEARCH PLAN**

[\*\*\*]

SCHEDULE 1.41 - Page 1

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**Schedule 1.54**

**DRAFT INVOICE**

[\*\*\*]

SCHEDULE 1.54 - Page 1

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**Schedule 1.76**

**OUTLINE OF RESEARCH PLAN**

**[\*\*\*]**

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**SCHEDULE 1.83**

**SELECTED CAPSID VARIANTS**

[\*\*\*]

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CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT HAVE BEEN OMITTED AND REPLACED WITH “[\*\*\*]”. SUCH IDENTIFIED INFORMATION HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS (I) NOT MATERIAL AND (II) WOULD LIKELY CAUSE COMPETITIVE HARM TO THE COMPANY IF DISCLOSED.

**COLLABORATION AND LICENSE AGREEMENT**

**BY AND BETWEEN**

**4D MOLECULAR THERAPEUTICS, INC**

**AND**

**UNIQUE BIOPHARMA B.V.**

**August 6, 2019**

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## COLLABORATION AND LICENSE AGREEMENT

This Collaboration and License Agreement (this “Agreement” or “New CLA”) is entered into and made effective on August 6, 2019 (the “New CLA Effective Date”), by and between 4D Molecular Therapeutics, Inc., a corporation organized and existing under the laws of the State of Delaware and having a principal office located at 5858 Horton St, Emerystation North, Suite 460, Emeryville, CA 94608 (“4DMT”), and uniQure biopharma B.V., a corporation organized and existing under the laws of The Netherlands and having a principal office located at Paasheuvelweg 25a, 1105 BP Amsterdam, The Netherlands (“uniQure”).

### INTRODUCTION

1. 4DMT is a biopharmaceutical company focused on research, development, manufacturing and marketing of novel adeno-associated viral vectors for delivery of nucleic acids to target cells and gene therapy biopharmaceutical products based thereon.
  2. uniQure is a biopharmaceutical company focused on the research, development, manufacturing and marketing of gene therapy based biopharmaceutical products.
  3. 4DMT and uniQure desire for 4DMT to conduct a new research program to identify improved AAV Capsid Variants (as defined below) for delivery to liver (in the case of some such AAV Capsid Variants) or the central nervous system (in the case of others).
  4. 4DMT and uniQure have previously entered into a Collaboration and License Agreement effective on January 17th, 2014, (the “Original CLA”) pursuant to which, 4DMT and uniQure had a collaboration for the identification of novel AAV Capsid Variants for development and commercialization as therapeutic products in the Field (defined below), and the Parties are, concurrent with the execution of this Agreement, amending and restating the Original CLA in its entirety by entering into an Amended and Restated Collaboration and License Agreement, effective of even date herewith (the “Amended and Restated CLA”), and, through the execution of this Agreement and the Amended and Restated CLA, the Parties have resolved the matters that were referred to and described in correspondence between the Parties dated February 28, 2019 with respect to the Original CLA.
  5. uniQure desires to receive from 4DMT exclusive rights under 4DMT’s intellectual property rights to research (subject to 4DMT’s retained rights to conduct research), develop, manufacture and commercialize certain gene therapy Products based on use of New Capsid Variants (defined below) to deliver Transgenes (defined below) based on Restricted Targets (defined below) in the Field pursuant to this Agreement, and subject to 4DMT’s Step-In Rights (defined below).
  6. 4DMT desires to retain non-exclusive rights to, exclude from the exclusive grant described above the non-exclusive rights to, and/or receive from uniQure non-exclusive rights under uniQure’s intellectual property rights to, research, develop, manufacture and commercialize certain gene therapy products based on use of New Capsid Variants to deliver Transgenes based on Restricted Targets within and outside of the Field, in accordance with 4DMT’s Step-In Rights pursuant to this Agreement.
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7. uniQure also desires to receive from 4DMT non-exclusive rights under 4DMT's intellectual property rights to research, develop, manufacture and commercialize certain gene therapy products based on use of New Capsid Variants to deliver Transgenes based on targets other than Restricted Targets (Non-Restricted Targets, more particularly defined below), in accordance with uniQure's Step-In Rights pursuant to this Agreement.

8. 4DMT would retain all other rights to New Capsid Variants (e.g., rights to New Capsid Variants outside the Field and related to Non-Restricted Targets), subject to uniQure's Step-In Rights for Non-Restricted Targets and information rights as described herein.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and other good and valuable consideration, the receipt of which is hereby acknowledged, 4DMT and uniQure agree as follows effective as of the Effective Date:

## ARTICLE I

### DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, shall have the respective meanings set forth below:

1.1 "4DMT AAV Capsid Variant". 4DMT AAV Capsid Variant means any AAV Capsid Variant that does not carry a Gene Therapy Construct contained in a Royalty Bearing Construct or Royalty Bearing Product.

1.2 "4DMT AAV Capsid Variant Library". 4DMT AAV Capsid Variant Library means any AAV Capsid Variant Library constructed by or licensed to 4DMT, including all AAV Capsid Variant Libraries provided to 4DMT pursuant to the UCB Agreements.

1.3 "4DMT Intellectual Property". 4DMT Intellectual Property means the 4DMT Know-How and the 4DMT Patent Rights.

1.4 "4DMT Know-How". 4DMT Know-How means Know-How that is (a) Controlled by 4DMT or its Affiliates as of the Effective Date or during the Research Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize any New Capsid Variant, or a Royalty Bearing Construct or Royalty Bearing Product due to the presence of such New Capsid Variant therein. 4DMT Know-How includes Core 4DMT Know-How but does not include Joint Know-How.

1.5 "4DMT Patent Right". 4DMT Patent Right means any Patent Right Controlled by 4DMT or its Affiliates as of the Effective Date or during the Term that Covers 4DMT Know-How. 4DMT Patent Rights include Core 4DMT Patent Rights but do not include Joint Patent Rights.

1.6 "4DMT Product". 4DMT Product means a Royalty Bearing Product that (a) delivers a Transgene that Affects a Non-Restricted Target (or variant of a Non-Restricted Target), or (b) that delivers a Transgene that Affects a Restricted Target (or variant of a Restricted Target), and, in the case of (b) (but to avoid doubt, this is not required for (a)), 4DMT has obtained

non-exclusive rights to Develop and Commercialize such Royalty-Bearing Product pursuant to the exercise of its Step-In Rights in Section 4.4. Notwithstanding anything express or implied, no 4DMT Product shall deliver a Transgene that relates to any Restricted Target for which 4DMT has not obtained rights to deliver as part of a Royalty-Bearing Product pursuant to the exercise of its Step-In Rights under Section 4.4.

1.7 “4D Product Patent”. 4D Product Patent means any Product Patent upon which 4DMT’s or its Affiliates’ personnel are properly named inventors (as determined under U.S. patent law) and uniQure’s and its Affiliates’ are not.

1.8 “AAV”. AAV means adeno-associated virus.

1.9 “AAV Capsid Variant”. AAV Capsid Variant means an AAV capsid that is modified as compared to the wild type sequence.

1.10 “AAV Capsid Variant Library”. AAV Capsid Variant Library means a collection of variant AAV capsid open reading frames inserted into an AAV genome in a manner that renders such variants genome replication-competent with the appropriate helper virus functions and capable of being selected and evolved to optimize their ability to deliver nucleic acid sequences to human or animal cells.

1.11 “Accounting Standards”. Accounting Standards means, with respect to uniQure and its Affiliates, International Financial Reporting Standards (“IFRS”) or, to the extent applicable, generally accepted accounting principles as practiced in the United States (“GAAP”), and with respect to 4DMT and its Affiliates, GAAP, in each case as they exist from time to time, consistently applied.

1.12 “Affects”. Affects, with respect to a Transgene and a Target, means that the Transgene (a) encodes such Target or a variant of such Target, (b) knocks down the mRNA corresponding to such Target or a variant of such Target, (c) encodes an antibody or other protein that specifically binds the protein encoded by such Target, or (d) otherwise directly affects such Target (e.g., via gene editing) or the protein that such Target encodes.

1.13 “Affiliate”. Affiliate means, with respect to a Party, any entity that directly or indirectly controls, is controlled by, or is under common control with such Party. As used in this definition, the term “control” means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of an entity, whether through ownership of voting securities, by contract or otherwise. For purposes of this definition, “control” shall be presumed to exist if one of the following conditions are met: (a) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors, and (b) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities.

1.14 “Animal POC”. Animal POC means demonstration of gene expression and/or gene function of a Transgene cassette for a Target, in an animal model or patient-derived cells; provided that such demonstration shall be in an established, relevant animal model of a disease, if available.

1.15 “Business Day”. Business Day means a day that is not a Saturday, Sunday or a day on which banking institutions in New York, New York, USA or Amsterdam, The Netherlands are authorized by Law to remain closed.

1.16 “Calendar Quarter”. Calendar Quarter means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31; provided, however, that the first Calendar Quarter hereunder shall commence on the Effective Date and the final Calendar Quarter hereunder shall end on the effective date of termination or expiration of this Agreement.

1.17 “Calendar Year”. Calendar Year means each successive period of twelve (12) months commencing on January 1 and ending on December 31; provided, however, that the first Calendar Year hereunder shall commence on the Effective Date and the final Calendar Year hereunder shall end on the effective date of termination or expiration of this Agreement

1.18 “CEO”. CEO means the Chief Executive Officer of a Party or, if there is no Chief Executive Officer of a Party, the Board Chairperson or senior-most executive officer or equivalent of such Party.

1.19 “CNS”. CNS means the central nervous system. [\*\*\*]

1.20 “CNS Term”. The CNS Term means the period beginning on the New CLA Effective Date and ending on the later of (a) [\*\*\*] ([\*\*\*)] years from the date of initiation of work under the Research Plan for CNS, and (b) the date on which 4DMT delivers [\*\*\*] ([\*\*\*)] (i.e., when it has delivered [\*\*\*)] New CNS Variants to uniQure with the Vector Characterization Data with respect to each of such [\*\*\*] ([\*\*\*)] New CNS Variants that the Research Plan for CNS requires 4DMT to provide. It is understood that the Selection Processes giving rise to such New CNS Variants shall be the Selection Processes directed at identifying CNS-targeted variants as defined and called for in the Research Plan, or, if such Selection Processes do not yield [\*\*\*] ([\*\*\*)] New CNS Variants meeting the Delivery Success Criteria (that can then proceed into the studies that generate Vector Characterization Data in accordance with the Research Plan for CNS), then Selection Processes for CNS-targeted AAV Capsid Variants that 4DMT conducts in addition or as follow-ups to (but in each case not in replacement or in lieu of) such CNS-directed Selection Processes called for in the Research Plan, and in any event meeting the requirements of Section 3.1. If 4DMT conducts such in-addition-to and/or follow-up CNS-directed Selection Processes, then the Vector Characterization Data that 4DMT would have to report to uniQure in order for the applicable New CNS Variants to count as one of the [\*\*\*] ([\*\*\*)] New CNS Variants of clause (b) of the first sentence of this definition, shall be equivalent to the Vector Characterization Data that 4DMT is required to report to uniQure under the Research Plan with respect to the New CNS Variants that it delivers thereunder. uniQure’s information and input rights as to any such additional Selection Processes that 4DMT may choose to conduct are as provided for in Section 3.1.

1.21 “Commercially Reasonable Efforts”. Commercially Reasonable Efforts means, with respect to a Party, the efforts required in order to carry out a task in a diligent and sustained manner without undue interruption or delay, which level is at least commensurate with the level of effort that a similarly situated Third Party would devote to a product of similar market

potential and having similar commercial and scientific advantages and disadvantages resulting from its own research efforts or to which it has rights, taking into account its safety and efficacy, regulatory status, the competitiveness of the marketplace, its proprietary position, pricing, reimbursement, launching strategy and other market-specific factors, and all other relevant factors.

1.22 “Commercialization” or “Commercialize”. Commercialization or Commercialize means any activity directed to obtaining pricing or reimbursement approvals, marketing, promoting, distributing, importing, exporting, offering to sell or selling a product, or to have any such activity performed. When used as a verb, “Commercialize” means to engage in Commercialization.

1.23 “Construct”. Construct means an AAV Capsid Variant carrying and comprising a Gene Therapy Construct.

1.24 “Confidential Information”. Confidential Information means any and all information and data, including all uniQure Know-How, 4DMT Know-How and Joint Know-How, and all other scientific, pre-clinical, clinical, regulatory, manufacturing, marketing, financial and commercial information or data, whether communicated in writing or orally or by any other method, which is provided by one Party to the other Party in connection with this Agreement or the Prior Confidentiality Agreement. All Core uniQure Know-How shall be considered the Confidential Information of uniQure, with respect to which: (a) uniQure shall be considered the disclosing Party, (b) 4DMT shall be considered the receiving Party, and (c) clauses (b) and (e) of Section 8.2 shall not apply. All Core 4DMT Know-How shall be considered the Confidential Information of 4DMT, with respect to which: (i) 4DMT shall be considered the disclosing Party, (ii) uniQure shall be considered the receiving Party, and (iii) clauses (b) and (e) of Section 8.2 shall not apply.

1.25 “Control”. Control means, with respect to any item of or right under Patent Rights or Know-How, the possession (whether by ownership or license, other than a license pursuant to this Agreement) of the ability of a Party or, as applicable, its Affiliate (subject to Section 12.7), to grant access to, or a license or sublicense of, such items or right as provided for herein without violating the terms of any agreement or other arrangement with any Third Party existing at the time such Party would be required hereunder to grant the other Party such access or license or sublicense.

1.26 “Core 4DMT Intellectual Property”. Core 4DMT Intellectual Property means Core 4DMT Know-How and Core 4DMT Patent Rights.

1.27 “Core 4DMT Know-How”. Core 4DMT Know-How means [\*\*\*].

1.28 “Core 4DMT Patent Right”. Core 4DMT Patent Right means any Patent Right that Covers the Core 4DMT Know-How, including New Variant Patents.

1.29 “Core uniQure Intellectual Property”. Core uniQure Intellectual Property means Core uniQure Know-How and Core uniQure Patent Rights.

1.30 “Core uniQure Know-How”. Core uniQure Know-How means [\*\*\*].

1.31 “Core uniQure Patent Right”. Core uniQure Patent Right means any Patent Right that Covers the Core uniQure Know-How. For clarity, Core uniQure Patent Rights excludes New Variant Patents.

1.32 “Cover”, “Covering” or “Covered”. Cover, Covering or Covered means, with respect to a product, technology, process or method that, in the absence of ownership of or a license granted under a Valid Claim, the manufacture, use, offer for sale, sale or importation of such product or the practice of such technology, process or method would infringe such Valid Claim (or, in the case of a Valid Claim that has not yet issued, would infringe such Valid Claim if it were to issue). With respect to a composition, “Coverage” exists if the applicable Valid Claim claims such composition, its method of manufacture, or its methods of use.

1.33 “Default”. Default means with respect to a Party that (a) any representation or warranty of such Party set forth herein shall have been untrue in any material respect when made or (b) such Party shall have failed to perform any material obligation set forth in this Agreement.

1.34 “Delivery Success Criteria”. Delivery Success Criteria means the following criteria that determines whether a given AAV Capsid Variant demonstrates superior delivery to the CNS or liver for introduction into those additional validation and characterization studies that are defined in the applicable Research Plan: [\*\*\*].

1.35 “Development” or “Develop”. Development or Develop means pre-clinical and clinical drug development activities, including: test method development and stability testing, toxicology, formulation, process development, manufacturing scale-up, development-stage manufacturing, quality assurance/quality control procedure development and performance with respect to clinical materials, statistical analysis and report writing and clinical studies, regulatory affairs, and all other pre-Regulatory Approval activities. When used as a verb, “Develop” means to engage in Development.

1.36 “EMA”. EMA means the European Medicines Agency, or any successor agency.

1.37 “European Union” or “EU”. European Union or EU means the countries that are members of the European Union, as redefined from time to time.

1.38 “FDA” or “Food and Drug Administration”. FDA or Food and Drug Administration means the United States Food and Drug Administration, or any successor agency.

1.39 “Field”. Field means the delivery of Gene Therapy Constructs to cells in (a) the CNS or (b) the liver, in each case where such delivery is for the purpose of effecting expression of the applicable RNA or amino acid sequence in the targeted cells and is potentially useful for the diagnosis, treatment, cure, palliation or prevention of a disease or medical condition in humans or animals, irrespective of the administration site or mode of administration (*e.g.*, intravenous, direct injection, subcutaneous or intrathecal) of the Construct used to effect delivery. For clarity, intravenous or intrathecal administration of any Construct targeted to cells in other organs (*i.e.*, not specifically targeted to liver or CNS tissues), including for treatment of neoplastic and eye disorders, are excluded from the Field.

1.40 “First Commercial Sale”. First Commercial Sale means, with respect to any Royalty Bearing Product and a country, the first sale for end use or consumption of such Royalty Bearing Product in such country after all required approvals, including Regulatory Approval, have been granted by the Regulatory Authority of such country. For clarity, sales for test marketing, sampling and promotional uses, clinical trials purposes or compassionate use shall not constitute a First Commercial Sale.

1.41 “Gene Therapy Construct”. Gene Therapy Construct means any Transgene that is packaged into an AAV Capsid Variant to form a Construct, and is intended to be delivered to a targeted tissue to treat, cure, prevent or ameliorate a disease or condition of the CNS or liver by any gene therapy application or modality.

1.42 “Good Laboratory Practices”. Good Laboratory Practices means the then-current good laboratory practice standards promulgated or endorsed by the FDA, as defined in 21 C.F.R. Part 58 (or such other comparable regulatory standards in jurisdictions outside the U.S. to the extent applicable to the relevant study, as they may be updated from time to time).

1.43 “Governmental Authority”. Governmental Authority means any United States federal, state or local or any foreign government, or political subdivision thereof, or any multinational organization or authority or any authority, agency or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, any court or tribunal (or any department, bureau or division thereof), or any governmental arbitrator or arbitral body.

1.44 “IGT”. IGT means Integrative Gene Therapeutics, Inc., a California corporation, which jointly owns with UC certain of the UC Patent Rights.

1.45 “Indication”. Indication means any disease, condition or syndrome.

1.46 “Invention”. Invention means any new and useful process, article of manufacture, Construct, composition of matter, formulation or apparatus, or any improvement thereof, discovery or finding, which is patentable.

1.47 “Invoice”. Invoice means an original invoice sent by 4DMT to uniQure with respect to any payment due hereunder substantially in the form attached hereto as Schedule 1.

1.48 “Know-How”. Know-How means (a) any scientific or technical information, results and data of any type whatsoever, in any tangible or intangible form whatsoever, that is not in the public domain, including databases, practices, methods, techniques, specifications, formulations, formulae, protein sequences, nucleic acid sequences, AAV Capsid Variants, AAV Capsid Variant Libraries, Gene Therapy Constructs, Constructs, knowledge, know-how, trade secrets, skill, experience, test data including pharmacological, medicinal chemistry, biological, chemical, biochemical, toxicological and clinical test data, analytical and quality control data, stability data, studies and procedures, and manufacturing process and development information, results and data, and (b) any biological, chemical, or physical material or composition of matter that is not in the public domain or otherwise generally available to the public.



1.49 “Law”. Law means all laws, statutes, rules, codes, regulations, orders, judgments or ordinances applicable to a Party, this Agreement or the activities contemplated hereunder.

1.50 “Licensed IP”. Licensed IP means the 4DMT Intellectual Property, the Joint Intellectual Property, and the Intellectual Property licensed by uniQure to 4DMT pursuant to Section 5.2.

1.51 “Liver Term”. The Liver Term means the period beginning on the New CLA Effective Date and ending on the later of (a) [\*\*\*] ([\*\*\*)] years from the date of initiation of work under the Research Plan for Liver, and (b) the date on which 4DMT delivers [\*\*\*] ([\*\*\*)] (i.e., when it has delivered [\*\*\*]) New Liver Variants to uniQure with the Vector Characterization Data with respect to each of such [\*\*\*] ([\*\*\*)] New Liver Variants that the Research Plan for Liver requires 4DMT to provide. It is understood that the Selection Processes giving rise to such New Liver Variants shall be the Selection Processes directed at identifying liver -targeted variants as defined and called for in the Research Plan, or, if such Selection Processes do not yield [\*\*\*] ([\*\*\*)] New Liver Variants meeting the Delivery Success Criteria (that can then proceed into the studies that generate Vector Characterization Data in accordance with the Research Plan for Liver), then Selection Processes for liver-targeted AAV Capsid Variants that 4DMT conducts in addition or as follow-ups to (but in each case not in replacement or in lieu of) such liver-directed Selection Processes called for in the Research Plan, and in any event meeting the requirements of Section 3.1. If 4DMT conducts such in-addition-to and/or follow-up liver -directed Selection Processes, then the Vector Characterization Data that 4DMT would have to report to uniQure in order for the applicable New Liver Variants to count as one of the [\*\*\*] ([\*\*\*)] New Liver Variants of clause (b) of the first sentence of this definition, shall be equivalent to the Vector Characterization Data that 4DMT is required to report to uniQure under the Research Plan with respect to the New Liver Variants that it delivers thereunder. uniQure’s information and input rights as to any such additional Selection Processes that 4DMT may choose to conduct are as provided for in Section 3.1.

1.52 “Materials”. Materials means any tangible chemical or biological research materials that are provided or otherwise made available by one Party to the other Party under the terms of Section 3.3 for use in performance of the Research Program; provided, however, that Materials will not include any AAV Capsid Variant Libraries.

1.53 “NDA”. NDA means a New Drug Application or Biologics License Application filed with the FDA or any other application required for the purpose of marketing or selling or commercially using a therapeutic or prophylactic product to be filed with a Regulatory Authority in a non-U.S. country or group of countries, including a Product License Application or Marketing Authorization Application (“MAA”) in the European Union or Japan.

1.54 “Net Sales”. Net Sales means, with respect to a Royalty Bearing Product (either a uniQure Product or a 4DMT Product, as applicable), the gross amount of sales of such Royalty Bearing Product invoiced by a Party or its Affiliates to Third Parties, less the following to the extent related to such Royalty Bearing Product and incurred by such Party or its Affiliates and invoiced to the Third Party:

(a) sales returns and allowances actually paid, granted or accrued, including trade, quantity and cash discounts and any other adjustments, including those granted on account of price adjustments or billing errors;

(b) rejected goods, damaged or defective goods, recalls, returns;

(c) rebates, chargeback rebates, compulsory rebates, reimbursements or similar payments granted or given to wholesalers or other distributors, buying groups or health care insurance carriers;

(d) non-collectable receivables;

(e) customs or excise duties, sales tax, consumption tax, value added tax, and other taxes (except income taxes); or

(f) charges for packing, freight, shipping and insurance.

Each of the foregoing deductions shall be determined as incurred in the ordinary course of business in type and amount consistent with good industry practice and in accordance with Accounting Standards on a basis consistent with such Party's audited consolidated financial statements. For clarity, sales by uniQure or its Affiliates of a Royalty Bearing Product to a Third Party Distributor of such Royalty Bearing Product in a given country shall be considered a sale to a Third Party customer. All such discounts, allowances, credits, rebates, and other deductions shall be fairly and equitably allocated to the Royalty Bearing Products and other products of uniQure and its Affiliates such that the Royalty Bearing Product does not bear a disproportionate portion of such deductions.

In the event any Royalty Bearing Product is sold for consideration other than cash, Net Sales for such sale shall be the average price of such Royalty Bearing Product sold for cash during the relevant period in the relevant country.

In the event that any discount, reduction, payment or rebate is offered for a Royalty Bearing Product where such Royalty Bearing Product is sold to a Third Party customer as part of a grouped set of products, the applicable discount, reduction, payment or rebate for such Royalty Bearing Product in such arrangement shall be based on the weighted average discount, reduction, payment or rebate of such grouped set of products.

Any Royalty Bearing Products used for promotional or advertising purposes (in reasonable and customary amounts) or used for Clinical Trials or other research purposes shall not be included in Net Sales. Donations for charity reasons or compassionate use shall also not be included in Net Sales.

1.55 "New Capsid Variant(s)". New Capsid Variant means any New CNS Variant or New Liver Variant.

1.56 "New CNS Variant(s)". New CNS Variant means any AAV Capsid Variant identified by 4DMT or its Affiliate, or any Third Party pursuant to rights granted by 4DMT or its Affiliate, during the CNS Term in a Selection Process designed to identify AAV Capsid Variants

specifically targeting cells of the CNS, but excluding any such AAV Capsid Variants that are Selected Capsid Variants. An AAV Capsid Variant is “identified” in a Selection Process when it is sequenced. An AAV Capsid Variant does not need to satisfy (or necessarily have been tested against) the applicable Delivery Success Criteria to qualify as a New CNS Variant.

If data of the types listed in the definition of Vector Characterization Data that is generated by, for or under right from 4DMT demonstrate that an AAV Capsid Variant from any other Selection Process (i.e., one not under the Research Plan of this Agreement or one that was not designed to identify AAV Capsid Variants specifically targeting cells of the CNS) conducted by, for or under right from 4DMT during the CNS Term is improved or superior to the top [\*\*\*] ([\*\*\*)] New CNS Variants arising under the Research Program in the following ways, then 4DMT will share the Vector Characterization Data with uniQure:[\*\*\*]

In that case, any such AAV Capsid Variant with respect to which 4DMT is required to share such data of the types listed in Vector Characterization Data demonstrating such improvement or superiority ((a) or (b) or (c)) shall be deemed a New CNS Variant, and such data shall be deemed Vector Characterization Data. It is understood that 4DMT is not under any obligation to generate such data (nor to have it generated for or under right from 4DMT), nor to perform any research or other activities not set forth in the Research Plan, but that, pursuant to the terms of this Agreement, uniQure may further investigate any such deemed New CNS Variants.

1.57 “New Liver Variant(s)”. New Liver Variant means any AAV Capsid Variant identified by 4DMT or its Affiliate, or any Third Party pursuant to rights granted by 4DMT or its Affiliate, during the Liver Term in a Selection Process designed to identify AAV Capsid Variants specifically targeting cells of the liver, but excluding any such AAV Capsid Variants that are Selected Capsid Variants. An AAV Capsid Variant is “identified” in a Selection Process when it is sequenced. An AAV Capsid Variant does not need to satisfy (or necessarily have been tested against) the applicable Delivery Success Criteria to qualify as a New Liver Variant.

If data of the types listed in the definition of Vector Characterization Data that is generated by, for or under right from 4DMT demonstrate that an AAV Capsid Variant from any other Selection Process (i.e., one not under the Research Plan of this Agreement or one that was not designed to identify AAV Capsid Variants specifically targeting cells of the liver) conducted by, for or under right from 4DMT during the Liver Term is improved or superior to the top [\*\*\*] ([\*\*\*)] New Liver Variants arising under the Research Program in the following ways, then 4DMT will share the Vector Characterization Data with uniQure:[\*\*\*]

In that case, any such AAV Capsid Variant with respect to which 4DMT is required to share such data of the types listed in Vector Characterization Data demonstrating such improvement or superiority ((a) or (b) or (c)) shall be deemed a New Liver Variant, and such data shall be deemed Vector Characterization Data. It is understood that 4DMT is not under any obligation to generate such data (nor to have it generated for or under right from 4DMT), nor to perform any research or other activities not set forth in the Research Plan, but that, pursuant to the terms of this Agreement, uniQure may further investigate any such deemed New Liver Variants.

1.58 “New Variant Patent”. New Variant Patent means any Patent Right Covering one or more New Capsid Variants (for clarity, whether by their composition, method of use, or method of manufacture). It is understood that New Variant Patents (1) may include dependent claims directed to the combination of a New Capsid Variant and a Transgene and (2) do not include any Patent Rights claiming inventions that are conceived and reduced to practice solely by employees, agents and/or consultants of uniQure or its Affiliate independently and outside of the Research Program, without the use of information pertaining to a Selected Capsid Variant or New Capsid Variant sequence disclosed to uniQure under this Agreement or the Amended and Restated CLA.

1.59 “Non-Restricted Targets” means all Targets within the Field that are not Restricted Targets.

1.60 “Party” and “Parties”. Party means uniQure or 4DMT individually, and Parties means uniQure and 4DMT collectively.

1.61 “Patent Rights”. Patent Rights means patents, patent applications or provisional patent applications, utility models and utility model applications, petty patents, innovation patents, patents of addition, divisionals, continuations, continuation-in-part applications, continued prosecution applications, requests for continued examinations, reissues, renewals, reexaminations and extensions and supplementary protection certificates granted in relation thereto, in any country of the world. For clarity, Patent Rights shall include any Patent Right that claims priority to or common priority with such Patent Rights.

1.62 “Product”. Product means any preparation in final form, either for sale by prescription, over-the-counter or any other method, or for administration to human patients in clinical trials, for any and all uses, and in any and all formulations and combinations, which preparation contains a Construct.

1.63 “Product Patent”. Any Patent Right Covering an invention invented pursuant to the activities conducted under this Agreement, the independent claims of which Patent Right are specifically drawn to a Construct combining (i) a New Capsid Variant and (ii) the Gene Therapy Construct of a given Product, and that does not claim priority to any New Variant Patent.

1.64 “Prosecution and Maintenance”. Prosecution and Maintenance means, with respect to a Patent Right, the preparation, filing, prosecution and maintenance of such Patent Right, as well as reexaminations, reissues and the like with respect to such Patent Right, together with the conduct of interferences, the defense of oppositions and other similar proceedings with respect to the particular Patent Right; and “Prosecute and Maintain” shall have the correlative meaning.

1.65 “Regulatory Authority”. Regulatory Authority means any applicable Governmental Authority involved in granting approvals for the manufacturing, marketing, reimbursement or pricing of a Royalty Bearing Product in the Territory or any portion thereof, including the FDA and EMA (as applicable), and any successor Governmental Authority having substantially the same function.

1.66 “Research Plan for CNS”. Research Plan for CNS means the research plan providing for one (1) or more Selection Processes intended to identify AAV Capsid Variants for delivery to the CNS, that is attached as Schedule 2.

1.67 “Research Plan for Liver”. Research Plan for Liver means the research plan providing for one (1) or more Selection Processes intended to identify AAV Capsid Variants for delivery to the liver, that is attached as Schedule 2.

1.68 “Research Program”. Research Program means, collectively, the program of research to be undertaken by 4DMT as described in the Research Plan for CNS and the Research Plan for Liver.

1.69 “Research Term”. Research Term means the period commencing on the New CLA Effective Date and ending on the date of expiration of the Liver Term or the CNS Term, whichever expires later.

1.70 “Research Year”. Research Year means a twelve (12) month period during the Research Term beginning on the Effective Date or on any anniversary thereof.

1.71 “Restricted Target(s)”, Restricted Target(s) means, the [\*\*\*] ([\*\*\*)] Targets to be selected by uniQure from within the Field, each of such [\*\*\*] ([\*\*\*)] Targets is to be selected by uniQure within the [\*\*\*] ([\*\*\*)] day period commencing on the New CLA Effective Date, and each of which must relate to a disease or condition of the CNS or the liver.

1.72 “Royalty Bearing Construct”. Royalty Bearing Construct means a Construct containing a New Capsid Variant and a Gene Therapy Construct that Affects a Restricted Target or a Non-Restricted Target (but not any Target that is not a Restricted Target or a Non-Restricted Target).

1.73 “Royalty Bearing Product”. Royalty Bearing Product means a Product containing a Royalty Bearing Construct.

1.74 “Royalty Term”. Royalty Term means, with respect to a Royalty Bearing Product, on a Royalty Bearing Product-by-Royalty Bearing Product and a country-by-country basis, the period beginning on the First Commercial Sale of such Royalty Bearing Product in such country by a Party or any of its Affiliates or Sublicensees, and ending on latest of: (a) the expiration of the last Valid Claim within the Licensed IP Covering such Royalty Bearing Product in such country, (b) the expiration of any applicable exclusivity, including orphan drug status or data exclusivity, and any extension thereto, granted by a Regulatory Authority in such country with respect to such Royalty Bearing Product, or (c) the tenth (10<sup>th</sup>) anniversary of the date of the First Commercial Sale by a Party or any of its Affiliates or Sublicensees of such Royalty Bearing Product in such country.

1.75 “Selected Capsid Variant”. Selected Capsid Variant means the AAV Capsid Variants listed in Schedule 3 to this Agreement.

1.76 “Selection Process”. Selection Process means the iterative evolution or isolation of lead AAV Capsid Variants from one or more AAV Capsid Variant Libraries in cells

(cultured or primary) *in vitro* or in animals *in vivo* intended to result in the identification of AAV Capsid Variants demonstrating properties suitable to a specified target tissue. For clarity, a Selection Process can be one that is performed by 4DMT or its Affiliate either for itself or for, with or by any Third Party under rights granted by 4DMT to such Third Party, and need not be one that is conducted under the Research Program of this Agreement or designed for the same type of tissue in order to qualify under this definition. .

1.77 “Step-In Rights”. Step-In Rights means, on a AAV Capsid Variant-by-AAV Capsid Variant and Target-by-Target basis, the rights of uniQure or of 4DMT, respectively, to step-in and obtain non-exclusive license rights with respect to a New Capsid Variant for delivery of a Transgene that Affects a Non-Restricted Target (in the case of uniQure) or a Restricted Target (in the case of 4DMT), in each case pursuant to the provisions of Section 4.4.

1.78 “Sublicensee”. Sublicensee means, with respect to a Party, a Third Party to whom such Party (or its Affiliate or another of its Sublicensees) has granted a license or sublicense under the Licensed IP to Develop, make and have made, use or Commercialize a Royalty Bearing Product; provided, however, that a Sublicensee shall not include any Third Party Distributor.

1.79 “Target”. Target means the biological gene or genetic material of interest to affect a disease or condition of the CNS or liver.

1.80 “Territory”. Territory means all countries and territories in the world.

1.81 “Third Party”. Third Party means an entity other than uniQure, 4DMT and their respective Affiliates.

1.82 “Third Party Distributor”. Third Party Distributor means any Third Party that provides (but does not Develop) Royalty Bearing Products directly to customers under agreement with uniQure, its Affiliates or Sublicensees.

1.83 “Transgene”. Transgene means (a) a given nucleic acid sequence that encodes an RNA sequence, and (b) any functionally equivalent sequence variants of such given nucleic acid sequence, [\*\*\*].

1.84 “UC AAV Capsid Variant”. UC AAV Capsid Variant means any AAV Capsid Variant provided to 4DMT pursuant to the UCB Agreements.

1.85 “UC Patent Right”. UC Patent Right means any Patent Right licensed to 4DMT pursuant to the UCB Agreements.

1.86 “UC Product”. UC Product means a Royalty Bearing Product that is Covered by a UC Patent Right.

1.87 “UCB Agreements”. UCB Agreements means (a) the Exclusive License and Bailment Agreement between 4DMT and the Regents of the University of California (“UC”), Agreement Control No. 2014-03-0089, dated December 19, 2013; (b) the Exclusive License and Bailment Agreement between 4DMT and UC, Agreement Control No. 2014-03-0090, dated December 19, 2013; and (c) the Agreement for Use of Certain Biological Materials between

4DMT and UC, Agreement Control No. 2014-30-0088, dated December 19, 2013, in each case in the form provided to uniQure by 4DMT as of the Effective Date.

1.88 “uniQure Intellectual Property”. uniQure Intellectual Property means uniQure Know-How and uniQure Patent Rights.

1.89 “uniQure Know-How”. uniQure Know-How means Know-How that is (a) Controlled by uniQure or its Affiliates as of the Effective Date or during the Research Term, and (b) necessary or useful to conduct the Research Program or to research, Develop, make and have made, use or Commercialize the relevant New Capsid Variant, or a Royalty Bearing Compound or Royalty Bearing Product due to the presence of such New Capsid Variant therein. uniQure Know-How includes Core uniQure Know-How but does not include Joint Know-How.

1.90 “uniQure Patent Right”. uniQure Patent Right means any Patent Right Controlled by uniQure or its Affiliates as of the Effective Date or during the Term that Covers uniQure Know-How. uniQure Patent Rights include Core uniQure Patent Rights but do not include Joint Patent Rights.

1.91 “uniQure Product”. uniQure Product means a Royalty Bearing Product that (a) delivers a Transgene that Affects a Restricted Target (or variant of a Restricted Target), or (b) that delivers a Transgene that Affects a Non-Restricted Target (or variant of a Non-Restricted Target), and in the case of (b) uniQure has obtained non-exclusive rights to Develop and Commercialize such Royalty-Bearing Product pursuant to the exercise of its Step-In Rights in Section 4.4. Notwithstanding anything express or implied, no uniQure Product shall deliver any Transgene that relates to any Non-Restricted Target for which uniQure has *not* obtained rights to deliver as part of a Royalty-Bearing Product pursuant to the exercise of its Step-In Rights under Section 4.4.

1.92 “uniQure Product Patent”. uniQure Product Patent means any Product Patent upon which uniQure’s or its Affiliates’ personnel are properly named inventors (as determined under U.S. patent law) and 4DMT and its Affiliates’ are not.

1.93 “Valid Claim”. Valid Claim means (a) a claim of an issued patent that has not expired or been abandoned, or been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period), or (b) a claim within a patent application which application has not been pending for more than [\*\*\*] ([\*\*\*)] years from the date of its priority filing date and which claim has not been irretrievably revoked, irretrievably cancelled, irretrievably withdrawn, held invalid or abandoned by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period), or finally determined to be unallowable in a decision from which an appeal cannot or can no longer be taken; provided, however, that with respect to the UC Patent Rights licensed under the Exclusive License and Bailment Agreement between 4DMT and UC, Agreement Control No. 2014-03-0089, the foregoing [\*\*\*] ([\*\*\*)] year limitation shall be extended to [\*\*\*] ([\*\*\*)] years.

“Vector Characterization Data” means any and all data, results and other Know-How that is generated either by or on behalf of a Party or its Affiliate, whether alone or together with, by or for any of its Third Party licensees, contractors or collaborators, with respect to any New Capsid Variant, in regards to any of the following with respect to such New Capsid Variant:[\*\*\*]

1.94 Additional Definitions. Each of the following definitions is set forth in the section of this Agreement indicated below:

<b><u>Definition:</u></b>	<b><u>Section:</u></b>
4DMT	Preamble
4DMT Indemnities	9.5
Additional Cure Period	10.2(a)
Agreement	Preamble
Audited Party	6.5
Auditing Party	6.5
Bankruptcy Code	5.4
CREATE Act	7.10
Damages	9.5
Defaulting Party	10.2(a)
Dispute	11.1
Effective Date	Preamble
Excluded Claim	11.2
Executives	2.3(b)
Fair Market Value	6.3(b)(iii)
GAAP	1.11
IFRS	1.11
Initiating Party	7.6(d)
Joint Counsel	7.5
Joint Intellectual Property	7.2(a)
Joint Know-How	7.2(a)
Joint Patent Rights	7.2(a)
JRSC	2.1(a)
M&A Event	12.7
MAA	1.53
Non-Defaulting Party	10.2(a)
Orange Book	7.9(a)
Paragraph IV Certification	7.9(b)
Paragraph IV Proceeding	7.9(b)(ii)
Records	3.5(a)(i)
SEC Filing	8.5(c)
Sublicense Consideration	6.3(b)
Sublicense Income Sharing Percentages	6.3(a)
Term	10.1
Third Party Claim	9.5
Trade Secret Election	7.3(b)



**Definition:**

USPTO  
UC  
uniQure  
uniQure Indemnitees

**Section:**

7.10  
1.87  
Preamble  
9.6

**ARTICLE II****GOVERNANCE****2.1 Joint Research Steering Committee.**

(a) **Composition.** Promptly after the Effective Date, the Parties shall establish a joint research steering committee (the “**JRSC**”). The JRSC shall be comprised of at least [\*\*\*] ([\*\*\*)] named representatives of uniQure and at least [\*\*\*] ([\*\*\*)] named representatives of 4DMT, one of whom shall be David Schaffer (unless due to his death, illness or disability), or such other numbers as the Parties may agree in writing. As soon as practicable after the Effective Date (but in no event more than [\*\*\*] ([\*\*\*)] Business Days after the Effective Date), each Party shall designate by written notice to the other Party its initial representatives on the JRSC. Each Party may replace one or more of its non-mandatory representatives, in its sole discretion, effective upon written notice to the other Party of such change. These representatives shall have appropriate technical credentials, experience and knowledge, and ongoing familiarity with the Research Program. The JRSC shall be disbanded upon expiration of the Research Term.

(b) **Function and Powers of the JRSC.** During the Research Term, the JRSC’s responsibilities shall include: (i) providing a forum for discussion of the Research Plan for Liver and the Research Plan for CNS, the status of the Research Program, and relevant data (but not making any decisions with respect thereto, other than as provided in clause (iii) of this sentence or as provided in Section 2.6); (ii) serving as a forum for informal resolution of disagreements that may arise in the relation to the Parties’ activities under the Research Program (but not deciding any such disagreement); and (iii) amending the Research Plan for Liver and/or the Research Plan for CNS, solely in the circumstances described in and under the terms and conditions of Section 2.6.

**2.2 Meetings.** The JRSC shall each hold at least [\*\*\*] per Calendar Quarter during the Research Term. Upon necessity, either Party shall be entitled to request additional meetings of the JRSC. Meetings of the JRSC shall be effective only if at least [\*\*\*] ([\*\*\*)] representatives of each Party are present or participating. The location of meetings shall be as agreed by the Parties, and may be held in person, alternating locations between the Parties, or by telephone conference call or by videoconference; provided, however, that at least [\*\*\*] ([\*\*\*)] meetings of the JRSC each Calendar Year are held in person. 4DMT’s costs and expenses incurred in connection with preparing for and participating in all such meetings shall be paid for by uniQure in accordance with the budget for the Research Plan for Liver or Research Plan for CNS, as applicable. Either Party may, from time to time, invite additional representatives or consultants to attend JRSC meetings; provided that at least [\*\*\*] ([\*\*\*)] Business Days’ prior

written notice is given of a Party's intention to invite such other representatives or consultants and providing full details about the name, employer and professional background of such other representatives or consultants. Each representative and consultant participating in or attending a JRSC meeting shall be bound by a written agreement with confidentiality obligations substantially the same as those set forth in ARTICLE VIII. The JRSC shall be co-chaired by a representative from each Party. The chairpersons shall set the agendas for the JRSC meeting in advance. Within ten (10) Business Days prior to each scheduled meeting, each Party shall, in accordance with Section 3.5(b), provide a report to the JRSC detailing its progress with respect to the Research Program. The Parties will rotate the responsibility for recording, preparing and issuing minutes for each JRSC meeting, to be circulated within [\*\*\*] ([\*\*\*)] Business Days after each meeting.

### 2.3 Decision-making.

(a) Initial Dispute Resolution Procedures. Subject to the provisions of this Section 2.3, actions to be taken by the JRSC shall be taken only following a unanimous vote, with each Party, through its representatives, having one (1) vote. Notwithstanding the foregoing, and subject to Section 2.6, in the circumstances described in such Section, [\*\*\*] shall have the final say and final decision-making authority on any and all disputes pertaining to any amendments to the Research Plan for CNS or amendments to the Research Plan for Liver, and any such final decision by [\*\*\*] on such matters shall not be subject to further review by referral to Executives or otherwise under this Section 2.3 or under any of the dispute resolution provisions of this Agreement.

(b) Referral of Unresolved Matters to Executives. If, in accordance with Section 2.3(a), the JRSC does not resolve any matter considered by it within [\*\*\*] ([\*\*\*)] Business Days after the matter is first considered by it, the matter may be referred by either Party to the CEO of 4DMT and CEO of uniQure (the "Executives") to be resolved by negotiation in good faith as soon as practicable, but in no event later than [\*\*\*] ([\*\*\*)] Business Days after referral. Such resolution, if any, of a referred issue by the Executives shall be final and binding on the Parties. Any decision made by the Executives under this Section 2.3(b) shall be deemed a decision of the JRSC for purposes of this Agreement.

(c) Final Decision-Making. If a dispute referred to the Executives pursuant to Section 2.3(b) has not been resolved in accordance with Section 2.3(b), then, subject to Section 2.3(d), [\*\*\*] shall have the final decision-making authority. Any decision made by [\*\*\*] pursuant to this Section 2.3(c) shall be deemed a decision of the JRSC for purposes of this Agreement.

(d) Exceptions. Notwithstanding Section 2.3(c), [\*\*\*] shall not have the right to exercise such decision-making authority (i) in a manner that excuses [\*\*\*] from any of its obligations specifically enumerated under this Agreement; (ii) in a manner that negates any consent rights or other rights specifically allocated to [\*\*\*] under this Agreement; (iii) in a manner that would require [\*\*\*] to perform activities (A) for which [\*\*\*] (except as expressly set forth in this Agreement), (B) that [\*\*\*], or (C) that [\*\*\*]; (iv) in a manner that would take away [\*\*\*]'s right to perform activities that [\*\*\*] has previously agreed to perform as set forth in the Research Plan; (v) in a manner that would require [\*\*\*] to perform any act that it reasonably believes to be

inconsistent with any Law or any approval, order, policy, guidelines of a Regulatory Authority or ethical requirements or ethical guidelines; (vi) to determine that [\*\*\*] has fulfilled any obligation under this Agreement or that [\*\*\*] has breached any obligation under this Agreement; or (vii) to amend the relevant Delivery Success Criteria.

(e) This Section 2.3 (and each of its subsections) shall not be used to imply any greater decision-making authority on the part of the JRSC than is set forth in Section 2.1(b) (i.e., the JRSC's sole decision-making authority is to decide upon amendments to the Research Plan for CNS and the Research Plan For Liver, subject always and solely in the circumstance and manner stated in Section 2.6).

2.4 Limitations on JRSC Authority. The JRSC shall have only the powers assigned expressly to it in this ARTICLE II and elsewhere in this Agreement, and shall not have any power to amend, modify or waive compliance with this Agreement. In furtherance thereof, 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 JRSC unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

2.5 Sharing of Vector Characterization and Other Data at the JRSC. Each Party shall share with and disclose to the other Party the Vector Characterization Data obtained by such Party with respect to New Capsid Variants, pursuant to the requirements of Section 4.3. Each Party shall do so during the Research Term and thereafter, and whether such Vector Characterization Data is generated within or outside of the Research Program. During the Research Term, 4DMT will keep the JRSC informed of: (a) any and all reasonably relevant data and information generated under the Research Program (including Vector Characterization Data; and (b) all New CNS Variants and New Liver Variants that have been identified by 4DMT, [\*\*\*], in connection with the next JRSC meeting after their identification.

2.6 Amendments to Research Plans. Notwithstanding anything express or implied in this Agreement, the JRSC shall only have the power to amend the Research Plan for CNS and the Research Plan for Liver in the following circumstances: (a) the then-current version of the applicable research plan (i.e., the Research Plan for CNS or the Research Plan for Liver) cannot be carried out as written; or (b) the JRSC achieves unanimous consensus (with no exercise of any final say) that a change needs to be made and as to the change.

### **ARTICLE III**

#### **RESEARCH PROGRAM**

3.1 Objectives of the Research Program. The Research Program under this Agreement shall be defined, collectively, by the activities as described in the Research Plan for CNS and the Research Plan for Liver, each as appended to this Agreement as of the Effective Date (or as they may be amended in accordance with this Agreement). The objective of the Research Plan for CNS is for 4DMT to identify [\*\*\*] ([\*\*\*) New CNS Variants that meet the applicable Delivery Success Criteria for entry into validation studies, the Vector Characterization Data from which validation studies will be the package of data that 4DMT is required to provide

to uniQure in order to satisfy its obligation to present [\*\*\*] ([\*\*\*)] New CNS Variants with the required Vector Characterization Data. The objective for the Research Plan for Liver is to identify the New Liver Variants that meet the applicable Delivery Success Criteria, the Vector Characterization Data from which validation studies will be the package of data that 4DMT is required to provide to uniQure in order to satisfy its obligation to present [\*\*\*] ([\*\*\*)] New Liver Variants with the required Vector Characterization Data. As and to the extent provided for in the Research Plan, 4DMT would provide quantities of such New Capsid Variants to uniQure for testing. If the CNS Selection Processes or the liver Selection Processes of the Research Program do not yield at least [\*\*\*] ([\*\*\*)] New Capsid Variants meeting the applicable Delivery Success Criteria for entry into validation studies to generate the required Vector Characterization Data packages, then (a) if requested by 4DMT, uniQure may (but is not required to) [\*\*\*]; and/or (b) [\*\*\*].

### 3.2 Conduct of the Research Program.

(a) 4DMT shall initiate work on the Research Plan for Liver and the Research Plan for CNS within [\*\*\*] ([\*\*\*)] weeks after the New CLA Effective Date. Any amendments to the Research Plan for CNS or the Research Plan for Liver will be only as agreed by the JRSC.

(b) 4DMT shall use Commercially Reasonable Efforts to conduct the Research Program in accordance with the Research Plan for CNS and the Research Plan for Liver. 4DMT shall have an affirmative obligation during the term the Research Program and thereafter during the term of this Agreement to disclose all data and other information related to the Research Plan on a timely basis, including, without limitation, all Vector Characterization Data created pursuant to the Research Program, the Research Plan for Liver, and the Research Plan for CNS, as well as the existence and status of all New Capsid Variants generated outside the Research Program during the applicable CNS Term or Liver Term (as applicable based on whether the variant at issue is a New CNS Variant or a New Liver Variant), and all Vector Characterization Data associated with such New Capsid Variants. For clarity, New CNS Variants only arise during the CNS Term, and New Liver Variants only arise during the Liver Term, even though they, or the Vector Characterization Data with respect thereto, may be later reported between the Parties.

(c) Either Party shall have the right to utilize the services of any Third Party to perform its obligations under the Research Plan to the extent that such Third Party is specifically approved in the Research Plan or otherwise approved by the JRSC, provided that any permitted Third Party must have entered into a written agreement with such Party that includes terms and conditions (i) protecting and limiting use and disclosure of Confidential Information at least to the same extent as under ARTICLE VIII, and (ii) requiring the Third Party and its personnel to assign to such Party all right, title and interest in and to any intellectual property (and intellectual property rights) created or conceived in connection with performance of subcontracted activities. Each Party shall remain at all times fully liable for its responsibilities under this Agreement.

(d) 4DMT and uniQure shall conduct the Research Program in accordance with all applicable Laws, including, if and as applicable, Good Laboratory Practices. Each Party hereby certifies that it will not employ or otherwise use in any capacity in performing any activity hereunder the services of any person or entity known to it to be debarred under 21 USC

§335a. For clarity, each of the New CNS Variants and each of the New Liver Variants to be entered into validation studies under the Research Program shall meet the applicable Delivery Success Criteria as defined in the Research Plans unless the Parties in their discretions agree otherwise in writing.

### 3.3 Materials and Know-How Transfer/Use of Constructs.

(a) In order to facilitate the Research Program, each Party shall, to the extent set forth in the Research Plan, provide to the other Party certain Materials and, subject to Section 3.4, Know-How Controlled by the supplying Party for use by the other Party in furtherance of the Research Program. All Materials and Know-How provided by one Party to the other Party remain the sole property of the supplying Party.

(b) All Materials transferred pursuant to the Research Program shall be used (i) only for the specific purpose provided for in the Research Plan, and (ii) solely under the control of the receiving Party. The Materials may not be used or delivered to or for the benefit of any Third Party without the prior written consent of the supplying Party, and shall not be used in research or testing involving human subjects, except as expressly contemplated in the Research Plan or in accordance with this Agreement. All Materials shall be returned to the supplying Party or destroyed (at the election of the supplying Party) promptly after completion of the use permitted under this Agreement.

(c) THE MATERIALS ARE PROVIDED "AS IS" AND WITHOUT ANY REPRESENTATION OR WARRANTY, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTY OF MERCHANTABILITY OR OF FITNESS FOR ANY PARTICULAR PURPOSE OR ANY WARRANTY THAT THE USE OF THE MATERIALS WILL NOT INFRINGE OR VIOLATE ANY PATENT OR OTHER PROPRIETARY RIGHT OF ANY THIRD PARTY.

(d) At the end of the Research Term, to the extent that samples have not already been provided to uniQure under the Research Program, 4DMT shall promptly provide to uniQure samples of New Capsid Variants that are at that time in 4DMT's possession. For clarity, this means the quantities specified in the Research Plan, not all quantities of New Capsid Variants at that time in 4DMT's possession. Other than as may be provided in the Research Plan, 4DMT shall not be required to transfer any Royalty Bearing Constructs to uniQure, unless the Parties mutually otherwise agree in writing in their discretions at a later date.

3.4 Third Party Intellectual Property. The conduct of activities under the Research Plan may use Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, subject to the terms and conditions of the UCB Agreements. 4DMT shall be solely responsible for all obligations under the UCB Agreements, including any and all payments and royalties due thereunder. In developing the Research Plan, the Parties shall discuss whether any Third Party Patent Rights or Know-How, other than Patent Rights or Know-How licensed by 4DMT pursuant to the UCB Agreements, will be utilized in the conduct of activities under the Research Plan. 4DMT shall disclose to uniQure the details of any restrictions on use or payment obligations of which it is aware that would be triggered by such use of Third Party Patent Rights or Know-How in the Research Program. If the Parties mutually agree to use any inventions

claimed in any Patent Right or use any Know-How that is licensed to or has been acquired by 4DMT other than pursuant to the UCB Agreements, and if such use would require the payment of additional consideration to the Third Party from which the Patent Rights or Know-How was licensed or acquired, then such Patent Right or Know-How shall be deemed under the Control of 4DMT, provided that uniQure expressly agrees in writing to bear any such additional consideration actually to be paid by 4DMT to the Third Party (which amounts uniQure may offset pursuant to Section 6.2(c)(ii)) with respect to the Development, manufacture or Commercialization of Royalty Bearing Constructs or Royalty Bearing Products. For clarity, nothing in this Section 3.4 shall limit uniQure's rights to obtain from a Third Party, independent of 4DMT, a license or other right with respect to such Third Party's Patent Rights or Know-How.

### 3.5 Records and Reports.

#### (a) Records.

(i) 4DMT shall maintain records, in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes, which shall fully and properly reflect all work done and results achieved in the performance of the Research Program by or on behalf of 4DMT (the "Records"), including the procedures, techniques and methodologies used, the progress made, and any Invention conceived or reduced to practice or otherwise made within the scope of or in connection with the Research Program. As part of keeping the Records, 4DMT shall ensure that all of its personnel, and all of its agents that are involved in the Research Program, will keep accurate laboratory notebooks, which laboratory notebooks: (A) shall be duly signed, dated and witnessed; and (B) shall be created and maintained in accordance with its standard operating procedures that would be sufficient to allow for said laboratory notebooks to be used in any proceeding before the United States Patent and Trademark Office or United States courts, in order to establish the date of invention for any Invention in accordance with the United States patent laws. During the Term, 4DMT shall, upon written request by uniQure, which shall not be unreasonably made: (1) make all Records available for inspection and review by uniQure during normal business hours in a timely manner; and (2) provide copies of the Records or any part thereof to uniQure, as reasonably requested by uniQure.

(ii) In connection with uniQure's exercise of its back-up right for patent filing as relates to the New Variant Patents (if applicable), uniQure shall have the right to request that a copy of the relevant portions of the laboratory notebooks relating to all stages of the generation of the applicable New Capsid Variants be provided by 4DMT to uniQure. After such request by uniQure, 4DMT shall provide such copies of the laboratory notebooks promptly to uniQure, which shall be maintained by uniQure as 4DMT's Confidential Information.

(b) Reports to the JRSC. Between [\*\*\*] ([\*\*\*)] and [\*\*\*] ([\*\*\*)] Business Days prior to each scheduled JRSC meeting, the Parties shall provide to the JRSC a written report on the progress of the Research Program, summarizing the work performed under the Research Program and evaluating the work performed in relation to the goals of the Research Program. Each Party shall provide such other information required by the Research Program or reasonably requested by the other Party and reasonably available, relating to the progress of the goals or performance of the Research Program.

## ARTICLE IV

### **DEVELOPMENT AND COMMERCIALIZATION OF PRODUCTS; DILIGENCE**

#### **4.1 Responsibility.**

(a) uniQure shall have the full right, but not the obligation, at its sole expense, for the worldwide research, Development, manufacturing and Commercialization of uniQure Products pursuant to the exercise by uniQure of its rights under this Agreement (including its ownership rights and/or its exercise of any of the licenses granted to uniQure under Section 5.1(b)) in accordance with the terms and conditions and limitations of such license rights, and, subject to the payment obligations under Article VI and all other relevant terms and conditions of this Agreement. For clarity, this does not apply to those 4D Products, if any, addressing Restricted Targets pursuant to 4DMT's non-exclusive rights after an exercise of 4DMT's Step-In Rights that results in 4DMT obtaining non-exclusive rights to such 4DMT Products addressing Restricted Targets. Moreover, it does not apply to any 4DMT Products directed to a Non-Restricted Target, even after an exercise by uniQure of its Step-In Rights related to a uniQure Product to such Non-Restricted Target.

(b) 4DMT shall have the full right, but not the obligation, at its sole expense, for the worldwide research, Development, manufacturing and Commercialization of 4DMT Products pursuant to the exercise by 4DMT of its rights under this Agreement (including its ownership rights and/or its exercise of any of the licenses granted to 4DMT under Section 5.2(c)) in accordance with the terms and conditions and limitations of such license rights, and subject to the payment obligations under Article VI and all other relevant terms and conditions of this Agreement. For clarity, this does not apply to those uniQure Products, if any, addressing Non-Restricted Targets pursuant to uniQure's non-exclusive rights after an exercise of uniQure's Step-In Rights that results in uniQure obtaining non-exclusive rights to such uniQure Products addressing Non-Restricted Targets.

4.2 Diligence. No Party will have any diligence obligations with respect to either 4DMT Products or uniQure Products, except as provided in Section 4.4 with respect to Proposed Products, and the circumstance in which either a not-stepping-in Party chooses to pursue a Proposed Product in lieu of allowing the other Party to obtain rights thereto pursuant to such other Party's Step-In Rights, or in the circumstance in which the stepping-in Party obtains non-exclusive rights to such Proposed Product.

#### **4.3 Obligation to Share Vector Characterization Data for AAV Capsid Variants.**

(a) Commencing on the New CLA Effective Date and continuing throughout the Term, uniQure shall provide, within [\*\*\*] ([\*\*\*)] days after each January 31<sup>st</sup> and July 31<sup>st</sup> of each Calendar Year, a written report to 4DMT that summarizes the Vector Characterization Data generated by or on behalf of uniQure or its Affiliate or Sublicensee with respect to each New Capsid Variant for which any research, Development, Commercialization or other vector characterization activities were conducted by or on behalf of uniQure or its Affiliate or Sublicensee during the [\*\*\*].

(b) Commencing on the New CLA Effective Date and continuing throughout the Term, 4DMT shall provide, within [\*\*\*] ([\*\*\*)] days after each January 31<sup>st</sup> and July 31<sup>st</sup> of each Calendar Year, a written report to uniQure that summarizes the Vector Characterization Data generated by or on behalf of uniQure or its Affiliate or Sublicensee with respect to each AAV Capsid Variant or New Capsid Variant for which any research, Development, Commercialization or other vector characterization activities were conducted by or on behalf of 4DMT or its Affiliate or Sublicensee during the [\*\*\*].

(c) Either Party may terminate its obligation to provide written reports pursuant to this Section 4.3 after the Research Term, if it ceases all research, development, commercialization or other activities that would result in the generation of any further unreported Vector Characterization Data with respect to New Capsid Variants, and the Party provides written notice to the other Party so stating and also certifying that all Vector Characterization Data that is required to be reported with respect to New Capsid Variants has been so reported.

#### 4.4 Step-In Rights of each Party for Proposed Products.

##### (a) Step-In Rights of 4DMT.

(i) At any time after the expiration of the CNS Term (for products based on New CNS Variants) or the Liver Term (for products based on New Liver Variants), 4DMT may make a bona fide proposal to uniQure for Developing and Commercializing a Product using a New Capsid Variant in the Field to deliver a Transgene that Affects any Restricted Target (each, a “4DMT Proposed Product”), including a development plan and a plan to finance such activities. Within [\*\*\*] ([\*\*\*)] days after receipt of a notice from 4DMT of a 4DMT Proposed Product, uniQure shall notify 4DMT whether uniQure is conducting or is interested in conducting research or Development of such 4DMT Proposed Product, or a Product that uniQure believes in good faith is or would be competitive with such 4DMT Proposed Product (a “Competitive Product”). 4DMT shall have the right to select a maximum total of [\*\*\*] ([\*\*\*)] Proposed Products as 4DMT Proposed Products per calendar year under this Section 4.4 and under Section 4.4 of the Amended and Restated CLA, such total to be determined in the aggregate under this Agreement and the Amended and Restated CLA, taken collectively.

(ii) If uniQure notifies 4DMT in good faith that uniQure is conducting or is interested in conducting research or Development of such 4DMT Proposed Product or Competitive Product, uniQure shall within [\*\*\*] ([\*\*\*)] months after such notice, deliver to 4DMT a plan (including projected timelines) for the research and Development thereof and, thereafter, shall use Commercially Reasonable Efforts to research, Develop, manufacture and Commercialize such 4DMT Proposed Product or Competitive Product in accordance with such plan. Each progress report provided to 4DMT under Section 4.3 from and after the date of uniQure’s notice under this Section shall contain a summary of the activities undertaken and the status of uniQure’s research and Development efforts with respect to such Third Party Proposed Product, 4DMT Proposed Product, uniQure Proposed Product or Competitive Product during the [\*\*\*].

(iii) If uniQure notifies 4DMT that uniQure is not conducting and is not interested in conducting research or Development of such 4DMT Proposed Product, or



Competitive Product, then the date of uniQure's such written notice (or the deadline therefor, if uniQure is required to provide such notice and fails to provide notice by such date whether clause (ii) above or this clause (iii) would otherwise), then this shall be the "Effective Time" for such 4DMT Proposed Product and the applicable Restricted Target, and the license to 4DMT in Section 5.2(c) shall become effective as of the Effective Time.

(b) Step-In Rights of uniQure.

(i) At any time after the expiration of the CNS Term (for products based on New CNS Variants) or the Liver Term (for products based on New Liver Variants), uniQure may make a bona fide proposal to 4DMT for Developing and Commercializing a Product using a New Capsid Variant in the Field to deliver a Transgene that Affect any Non-Restricted Target (each, a "uniQure Proposed Product"), including a development plan and a plan to finance such activities. Within [\*\*\*] ([\*\*\*)] days after receipt of a notice from uniQure of a uniQure Proposed Product, 4DMT shall notify uniQure whether 4DMT is conducting or is interested in conducting research or Development of such uniQure Proposed Product, or a Product that 4DMT believes in good faith is or would be competitive with such uniQure Proposed Product (a "4D Competitive Product"). uniQure shall have the right to select a maximum total of [\*\*\*] ([\*\*\*)] Proposed Products as uniQure Proposed Products per year under this Section 4.4.

(ii) If 4DMT notifies uniQure in good faith that 4DMT is conducting or is interested in conducting research or Development of such uniQure Proposed Product or Competitive Product, 4DMT shall within [\*\*\*] ([\*\*\*)] months after such notice, deliver to uniQure a plan (including projected timelines) for the research and Development thereof and, thereafter, shall use Commercially Reasonable Efforts to research, Develop, manufacture and Commercialize such uniQure Proposed Product or Competitive Product in accordance with such plan. Each progress report provided to uniQure under Section 4.3 from and after the date of 4DMT's notice under this Section shall contain a summary of the activities undertaken and the status of 4DMT's research and Development efforts with respect to such Third Party Proposed Product, uniQure Proposed Product, 4DMT Proposed Product or Competitive Product during the [\*\*\*].

(iii) If 4DMT notifies uniQure that 4DMT is not conducting and is not interested in conducting research or Development of such uniQure Proposed Product, or Competitive Product, then the date of 4DMT's such written notice (or the deadline therefor, if 4DMT is required to provide such notice and fails to provide notice by such date whether clause (ii) above or this clause (iii) would otherwise apply), then this shall be the "UQ Effective Time" for such uniQure Proposed Product and the applicable Non-Restricted Target, and the license to uniQure in Section 5.1(b)(ii) shall become effective as of the UQ Effective Time.

(c) General Rights of 4DMT Related to Non-Restricted Targets. For clarity, 4DMT owns the New Variant Patents and has the right to pursue 4DMT Products delivering Transgenes that Affect Non-Restricted Targets, without the need to obtain any rights under this Section 4.4 (i.e., as a default matter 4DMT has the right to pursue 4DMT Products delivering Transgenes that Affect Non-Restricted Targets, with no need to "step in" to obtain such rights, and for that reason, 4DMT's Step-In Rights under this Section 4.4 do not apply to 4DMT Products delivering Transgenes that Affect Non-Restricted Targets).

4.5 Pharmacovigilance. Within [\*\*\*] ([\*\*\*)] months after the Effective Date, the Parties shall enter into an agreement governing the exchange of adverse event safety data (including post-marketing spontaneous reports) received by a Party and its Affiliates, including such data received from, in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, relating to any AAV Capsid Variant provided to uniQure by 4DMT hereunder in order to monitor the safety of all Constructs and Products and to meet reporting requirements with any applicable Regulatory Authority. Such data sharing agreement shall not require the sharing of data that would disclose confidential know-how or trade secrets of a Party or its Affiliates, or in the case of uniQure, its Sublicensees or, in the case of 4DMT, its licensees, if such data may be cross-referenced, such as through a Drug Master File, to satisfy the requirements of Law and any applicable Regulatory Authority.

4.6 Marking. Prior to the issuance in the United States of Patent Rights included in the UC Patent Rights, uniQure agrees to mark Royalty Bearing Product(s) Covered by any UC Patent Right (or their containers or labels) sold in the United States under the licenses granted in this Agreement with the words “Patent Pending,” and following the issuance in the United States of one or more Patent Rights included in the UC Patent Rights, with the patent numbers of the UC Patent Right(s) Covering such Royalty Bearing Product. All Royalty Bearing Products Covered by any UC Patent Right sold in other countries will be marked in such manner as to conform with the patent Laws and practice of such countries.

## ARTICLE V

### GRANTS OF RIGHTS

#### 5.1 Licenses to uniQure.

(a) Research License to uniQure. Subject to the terms and conditions of this Agreement, 4DMT hereby grants to uniQure, and uniQure hereby accepts, during the Research Term, an exclusive (but not as to 4DMT), worldwide, royalty-free, non-sublicenseable license under the 4DMT Intellectual Property and 4DMT’s interest in the Joint Intellectual Property, solely to (i) conduct activities assigned to uniQure under the Research Plan for Liver or the Research Plan for CNS, (ii) evaluate Constructs, or (iii) evaluate the data developed in the conduct of activities under the Research Program or during the Research Term. This license is intended to include the right for uniQure to make sequence modifications to New Capsid Variants solely for the purpose of (1) adapting New Capsid Variants to insect cells or insect cell expression vectors and systems, and/or (2) modifying any “Selected Capsid Variants” as defined in the Amended and Restated CLA with or to include any motif, mutation, or substitution identified under this New CLA; *provided* that (x) uniQure shall promptly disclose to 4DMT all AAV Capsid Variants resulting from such activities, (y) such resulting AAV Capsid Variants shall be deemed New Capsid Variants for all purposes under this Agreement, and (z) the Patent Rights that may be filed with respect to such resulting deemed New Capsid Variants shall be deemed New Variant Patents for all purposes under this Agreement, and the Know-How with respect thereto shall be deemed the subject matter of New Variant Patents (whether or not Patent Rights are ever filed with respect to such Know-How) and therefore Core 4DMT Know-How, for all purposes under this Agreement. For clarity, the obligations of uniQure under the foregoing clauses (x), (y) and (z) with respect to any uniQure-modified New Capsid Variants or any uniQure-modified Selected

Capsid Variants as described in the foregoing sentence shall not apply to any AAV Capsid Variant (or any modification or improvement thereof) that is identified or generated by uniQure or any of its Affiliates or Sublicensees independently and outside of the Research Program, without the use of any information disclosed to uniQure pursuant to this Agreement or the Amended and Restated CLA as to the sequence of any New Capsid Variant or Selected Capsid Variant.

(b) Development and Commercialization Licenses to uniQure.

Exclusive License for use of New Capsid Variants in connection with the Restricted Targets. Subject to the terms and conditions of this Agreement, and on a New Capsid Variant-by-New Capsid Variant basis, 4DMT hereby grants to uniQure, and uniQure hereby accepts, an exclusive (even as to 4DMT, except solely to the extent that 4DMT obtains non-exclusive rights within the scope of this license pursuant to an exercise of 4DMT's Step-In Rights), worldwide, royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the 4DMT Intellectual Property (including all Vector Characterization Data reported by 4DMT to uniQure under this Agreement) and 4DMT's interest in the Joint Intellectual Property, to research (subject to 4DMT's retained rights to conduct research under the Research Program and to research Constructs related to Restricted Targets for potential exercise of 4DMT's Step-In Rights in relation thereto), Develop, make and have made, use, import, sell and Commercialize the New Capsid Variants, and any modifications or improvements thereto, as and into Royalty Bearing Constructs and Royalty Bearing Products in the Field. For clarity, the license granted to uniQure under this paragraph shall expressly include the right to create improvements or modifications to the sequence or composition of matter of any New Capsid Variant, *provided* that such improved or modified sequence or composition of matter is used solely in connection with the applicable Restricted Target in the Field, and (x) uniQure shall promptly disclose to 4DMT all resulting AAV Capsid Variants made, (y) such resulting AAV Capsid Variants shall be deemed New Capsid Variants for all purposes under this Agreement, and (z) the Patent Rights that may be filed with respect to such resulting deemed New Capsid Variants shall be deemed New Variant Patents for all purposes under this Agreement, and the Know-How with respect thereto shall be deemed the subject matter of New Variant Patents (whether or not Patent Rights are ever filed with respect to such Know-How) and therefore Core 4DMT Know-How, for all purposes under this Agreement. For clarity, the obligations of uniQure under the foregoing clauses (x), (y) and (z) with respect to any uniQure-modified New Capsid Variants described in the foregoing sentence shall not apply to any AAV Capsid Variant (or any modification or improvement thereof) that is identified or generated by uniQure or any of its Affiliates or Sublicensees independently and outside of the Research Program, without the use of any information disclosed to uniQure pursuant to this Agreement or the Amended and Restated CLA as to the sequence of any New Capsid Variant or Selected Capsid Variant.

(c) Non-Exclusive License for Proposed Products elected by uniQure pursuant to its Step-In Rights under Section 4.4.

(i) Subject to the terms and conditions of this Agreement (including 4DMT's retained rights related to Products delivering Transgenes related to the applicable Non-Restricted Target), and on a New Capsid Variant by New Capsid Variant basis, effective upon the UQ Effective Time for the applicable Non-Restricted Target and New Capsid Variant under Section 4.4(b), 4DMT hereby grants to uniQure, and uniQure hereby accepts, a non-

exclusive, worldwide, royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the 4DMT Intellectual Property (including all Vector Characterization Data reported by 4DMT to uniQure under this Agreement) and 4DMT's interest in the Joint Intellectual Property, to research, Develop, make and have made, use and Commercialize the uniQure Proposed Product to the applicable Non-Restricted Target as Royalty Bearing Constructs and Royalty Bearing Products within the Field. Such license may become effective one (1) or more times, in connection with one (1) or more elections by uniQure under Section 4.4 that result in the UQ Effective Time occurring under Section 4.4(b) for the applicable uniQure Proposed Product, Non-Restricted Target, and New Capsid Variant. For clarity, the license granted under this paragraph to uniQure shall expressly include the right to create improvements or modifications to the sequence or composition of matter of any New Capsid Variant, provided that such improved or modified sequence or composition of matter is used solely in connection with the applicable Non-Restricted Target in the Field.

(ii) In order to enable uniQure to research Constructs related to Non-Restricted Targets for the potential exercise of uniQure's Step-In Rights pursuant to Section 4.4, uniQure shall have, and 4DMT hereby grants to uniQure, a non-exclusive research-use-only license to use any and all Vector Characterization Data reported to uniQure by 4DMT and any other necessary 4DMT Intellectual Property, on a New Capsid Variant by New Capsid Variant basis, regardless of whether such New Capsid Variant was generated or identified under this Agreement or outside of this Agreement, to the extent necessary for uniQure to evaluate whether to exercise its Step-In Rights pursuant to Section 4.4.

(d) Recordation. Following the Effective Date or at any time during the Term, 4DMT at the request and expense of uniQure shall promptly register or record the licenses granted to uniQure under this Agreement with the appropriate patent offices in all applicable countries of the Territory; provided that such registration or recordation specifies the applicable limitations of such license, and provided further that such registration shall have no effect on the allocation of Prosecution and Maintenance rights and obligations set forth in ARTICLE VII. In the event any of the licenses granted to uniQure under this Agreement are terminated in accordance with the terms of this Agreement, uniQure shall promptly take such actions and execute such documents as are reasonably requested by 4DMT to cancel such registration(s) or recordation(s) in the applicable countries with respect to the terminated license grants.

(e) Grant-Back License to uniQure. 4DMT hereby grants to uniQure, and uniQure hereby accepts, a non-exclusive, worldwide, royalty-free license, including the right to grant sublicenses through multiple tiers, under the 4DMT Patent Rights and 4DMT Know-How that (i) arise from activities that are conducted under this Agreement in connection with Royalty Bearing Constructs and Royalty Bearing Products in the course of making modifications to New Capsid Variants and (ii) claim or cover compositions of matter or general methods of use of New Capsid Variants (for clarity, including such Patent Rights and Know-How claiming or covering compositions combining Gene Therapy Constructs in general and AAV Capsid Variants in general or general methods of making or using such combinations of Gene Therapy Constructs and AAV Capsid Variants), to research, Develop, make and have made, use, import, sell and Commercialize New Capsid Variants, and Products containing New Capsid Variants in connection solely with the Restricted Targets and any Non-Restricted Targets licensed to uniQure pursuant to the Step-In Rights under Section 4.4.

## 5.2 Licenses to 4DMT.

(a) Research License to 4DMT. Subject to the terms and conditions of this Agreement, uniQure hereby grants to 4DMT, and 4DMT hereby accepts, during the Research Term, a non-exclusive, worldwide, royalty-free, non-sublicenseable license under the uniQure Intellectual Property, solely to the extent necessary to conduct activities assigned to 4DMT under the Research Program during the Research Term.

(b) Grant-Back License to 4DMT. uniQure hereby grants to 4DMT, and 4DMT hereby accepts, a non-exclusive, worldwide, royalty-free license, including the right to grant sublicenses through multiple tiers, under the Patent Rights and Know-How Controlled by uniQure pursuant to the licenses granted by 4DMT to uniQure in Section 5.1 (including such Patent Rights and Know-How licensed to uniQure pursuant to Section 5.1 claiming or covering compositions combining Gene Therapy Constructs in general and AAV Capsid Variants in general or general methods of making or using such combinations of Gene Therapy Constructs and AAV Capsid Variants), and to use the Vector Characterization Data reported by uniQure to 4DMT under this Agreement, to research, Develop, make and have made, use and Commercialize New Capsid Variants, and Products containing New Capsid Variants, in all cases outside the Field or within 4D Products within the scope of rights within which 4DMT is entitled to research, Develop, and Commercialize 4D Products under this Agreement. For the avoidance of doubt, 4DMT's practice of the foregoing license shall be subject to the license rights of uniQure under Section 5.1 and its right to grant sublicenses under Section 5.3.

(c) Non-Exclusive License for Proposed Products elected by 4DMT pursuant to its Step-In Rights under Section 4.4. Subject to the terms and conditions of this Agreement (including uniQure's retained rights related to Products delivering Transgenes that Affect the applicable Restricted Target), and on a New Capsid Variant-by-New Capsid Variant basis, effective upon the Effective Time with respect to the given 4DMT Proposed Product and Restricted Target pursuant to Section 4.4(a), uniQure hereby grants to 4DMT, and 4DMT hereby accepts, a non-exclusive, worldwide, royalty-bearing license, including the right to grant sublicenses in accordance with Section 5.3, under the uniQure Intellectual Property that is necessary or useful due to the presence of the applicable New Capsid Variant (including all Vector Characterization Data reported by uniQure to 4DMT under this Agreement) and uniQure's interest in the Joint Intellectual Property, to research, Develop, make and have made, use and Commercialize such 4DMT Proposed Products as Royalty Bearing Constructs and Royalty Bearing Products within the Field. Any licenses granted pursuant to this Section are limited to only uniQure Intellectual Property that specifically relates to New Capsid Variants, including patent claims specifying a New Capsid Variant (if any) or specifically claiming any methods of use or making any New Capsid Variants (if any), and excluding all other uniQure Intellectual Property, including compositions of matter or methods of making compositions of matter and methods of manufacturing Products (but not the New Capsid Variants therein) pursuant to this Agreement. Such license may become effective one (1) or more times, in connection with one (1) or more elections by 4DMT under Section 4.4 that result in the Effective Time occurring under Section 4.4(a)(iii) for the applicable Proposed Product and Restricted Target.

5.3 Sublicenses. Each Party shall have the right to grant sublicenses (through multiple tiers) under the license granted to it under Section 5.1(b) (in the case of uniQure) or

Section 5.2(c) (in the case of 4DMT) to its Affiliates and Third Parties; provided that any sublicense granted to a Third Party under this Agreement shall be pursuant to a written agreement that subjects such Sublicensee to all relevant restrictions and limitations set forth in this Agreement. Each Party granting a sublicense shall provide the other Party with the name and address of each Sublicensee of its rights under this ARTICLE V, the date of the grant of the sublicense and a description of the rights granted promptly after the execution and delivery of the sublicense agreement. The Party granting the sublicense shall remain responsible for the performance of its Sublicensees, and shall ensure that each Sublicensee complies with the applicable terms and conditions of this Agreement.

5.4 Rights Retained by the Parties. Except as expressly set forth in this Agreement, neither Party shall acquire any license or other intellectual property interest, by implication or otherwise, in any Confidential Information of the other Party or under any Patent Right or Know-How in which such other Party or its Affiliates has rights. Without limiting the generality of the foregoing, any of 4DMT's rights to 4DMT Intellectual Property not specifically licensed to uniQure shall be retained by 4DMT, and any of uniQure's rights to uniQure Intellectual Property not specifically licensed to 4DMT shall be retained by uniQure.

5.5 Section 365(n) of the Bankruptcy Code. All rights and licenses granted under or pursuant to any section of this Agreement are and will otherwise be deemed to be for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended or any comparable Law outside the United States (the "Bankruptcy Code"), licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code. Each Party agrees that the other Party, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code or any other provisions of applicable Law outside the United States that provide similar protection for "intellectual property." The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) the intellectual property licensed to such other Party and all embodiments of such intellectual property, to the extent necessary for such other Party to practice the licenses granted to it pursuant to this Agreement under such intellectual property, which, if not already in such other Party's possession, will be promptly delivered to it upon such other Party's written request thereof. Any agreement supplemental hereto will be deemed to be "agreements supplementary to" this Agreement for purposes of Section 365(n) of the Bankruptcy Code

5.6 UCB Agreement Pass-Through Provisions. uniQure acknowledges that 4DMT has provided it with a copy of the executed UCB Agreements, and agrees that this Agreement is subject in all respects to the terms and conditions of the UCB Agreements. Notwithstanding the generality of the foregoing:

(a) uniQure acknowledges that UC (and, to the extent applicable, IGT) may publish any and all technical data resulting from any research performed by UC (and, to the extent applicable, IGT) relating to the inventions disclosed in the UC Patent Rights, and UC (and, to the extent applicable, IGT) expressly reserves the right to use such inventions, UC AAV Capsid

Variants and related technology for its educational and research purposes, to disseminate the UC AAV Capsid Variants and other tangible materials associated with, or required to practice such inventions or the UC Patent Rights to researchers at nonprofit institutions for their educational and research purposes, and to permit other nonprofit institutions to use the UC AAV Capsid Variants to practice the UC Patent Rights for education and research purposes.

(b) uniQure shall keep 4DMT informed of its large/small entity status, as defined in 15 U.S.C. 632.

(c) uniQure acknowledges that certain of the inventions disclosed in the UC Patent Rights were funded in part by the U.S. Government, and agrees that in accordance with 35 U.S.C. 204, to the extent required by Law, any products covered by the UC Patent Rights and sold in the United States will be substantially manufactured in the United States.

(d) uniQure acknowledges that 4DMT's exclusive rights, privileges, and licenses under the UCB Agreements will expire on the date of the last-to-expire Valid Claim under the UC Patent Rights covered in each agreement, respectively, unless earlier terminated.

(e) For any sublicense under the UC Patent Rights that uniQure grants under Section 5.3, uniQure shall ensure that (i) such further sublicense is subject to a written sublicense agreement and is bound by all of the applicable terms, conditions, obligations, restrictions and other covenants of the UCB Agreements that protect or benefit UC's (and, if applicable, the U.S. Government's) rights and interests to the same extent that this Agreement does, and (ii) it or the Sublicensee shall, within [\*\*\*] ([\*\*\*)] days after executing such sublicense agreement, furnish to 4DMT for delivery to UC, subject to any confidentiality provisions, all material terms of such sublicense pertaining to UC's interests, including the Sublicensee's name and address, and indemnification of UC as provided in this Agreement.

(f) The Parties acknowledge and agree that upon termination of the UCB Agreements for any reason, uniQure's sublicenses under the UC Patent Rights under this Agreement will remain in effect and will be assigned to UC, except that UC will not be bound to perform any duties or obligations set forth herein that extend beyond the duties and obligations of UC set forth in the UCB Agreements.

(g) uniQure acknowledges that nothing contained in this Agreement will be construed as conferring any right to use in advertising, publicity or other promotional activities any name, trademark, trade name, or other designation of UC (including any contraction, abbreviation, or simulation of any of the foregoing), and that unless required by Law, regulation, or rules of a securities exchange, or consented to in writing by UC, the use by uniQure of the name "The Regents of the University of California" or the name of any University of California campus in advertising, publicity or other promotional activities is expressly prohibited.

## ARTICLE VI

### **PAYMENTS; ROYALTIES AND REPORTS**

6.1 [Intentionally omitted].

## 6.2 Royalties.

### (I) Royalties Payable by uniQure for uniQure Products.

On a Royalty Bearing Product-by-Royalty Bearing Product basis, uniQure shall pay to 4DMT royalties on worldwide Net Sales of uniQure Products as provided in this Section 6.2:

(a) Royalty Rate. uniQure shall pay to 4DMT royalties on Net Sales of each Royalty Bearing Product Commercialized by uniQure and its Affiliates equal to [\*\*\*] percent ([\*\*\*]%) of all such Net Sales of such Royalty Bearing Product achieved during the applicable Calendar Year.

(b) Royalty Term. uniQure's royalty obligations to 4DMT under this Section 6.2 for uniQure Products shall be in effect on a country-by-country and Royalty Bearing Product-by-Royalty Bearing Product basis during the relevant Royalty Term. Upon expiration of the Royalty Term for a Royalty Bearing Product in a country, the license under Section 5.1(b) shall be fully paid-up, irrevocable, perpetual and exclusive under the relevant Licensed IP for such Royalty Bearing Product in such country.

### (c) Royalty Adjustments.

(i) Non-Patented Product. If a Royalty Bearing Product is sold in a country and the composition of matter, formulation, or method of use of such Royalty Bearing Product is not Covered by a Valid Claim within the Licensed IP in such country at the time of sale, then the royalty rate for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the applicable rate determined pursuant to Section 6.2(I)(a), unless such Royalty Bearing Product embodies an Invention with respect to which uniQure made a Trade Secret Election, in which case no such reduction shall apply.

(ii) Third Party Offset. If uniQure is required, in order to avoid infringement of any Patent Right not licensed hereunder that Covers the composition of matter, formulation, or method of use of a Royalty Bearing Product, to obtain a license from a Third Party in order to Develop, make, have made, use or Commercialize such Royalty Bearing Product in a country in the Territory and to pay a royalty or other consideration under such license (including in connection with the settlement of a patent infringement claim), then the royalty payments due under Section 6.2(I)(a) with respect to Net Sales for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the amounts payable by uniQure to such Third Party for such license that are reasonably and appropriately allocable to such Royalty Bearing Product in such country, provided that in no event shall the foregoing reduce the amount of royalties payable to 4DMT in any [\*\*\*] by more than [\*\*\*] percent ([\*\*\*]%) of the amount determined pursuant to Section 6.2(I)(a), as adjusted by application of the terms of Section 6.2(I)(c)(i).

(iii) Limits on Deductions. Except as expressly provided in this Section 6.2, there shall not be any offset to or deduction from the royalties payable pursuant to this Section 6.2. Notwithstanding Sections 6.2(c)(i) and (ii) to the contrary, in no event shall the cumulative effect of the deductions in Sections 6.2(I)(c) 6.2(c)(i) and (ii) reduce the royalties to less than [\*\*\*] percent ([\*\*\*]%) of the amounts determined pursuant to Section 6.2(I)(a).



(II) Royalties Payable by 4DMT for 4DMT Products.

On a Royalty Bearing Product-by-Royalty Bearing Product basis, for each 4DMT Product Commercialized by 4DMT and its Affiliates, 4DMT shall pay to uniQure royalties on annual worldwide Net Sales of such 4DMT Product as provided in this Section 6.2:

(a) Royalty Rate. 4DMT shall pay to uniQure royalties on Net Sales of each Royalty Bearing Product Commercialized by 4DMT and its Affiliates equal to [\*\*\*] percent ([\*\*\*]%) of all such Net Sales of such Royalty Bearing Product achieved during the applicable Calendar Year.

(b) Royalty Term. 4DMT's royalty obligations to uniQure under this Section 6.2 shall be in effect on a country-by-country and Royalty Bearing Product-by-Royalty Bearing Product basis during the relevant Royalty Term. Upon expiration of the Royalty Term for a Royalty Bearing Product in a country, the license under Section 5.2(c) shall be fully paid-up, irrevocable, perpetual and non-exclusive under the relevant Licensed IP for such Royalty Bearing Product in such country.

(c) Royalty Adjustments.

(i) Non-Patented Product. If a Royalty Bearing Product is sold in a country and the composition of matter, formulation, or method of use of such Royalty Bearing Product is not Covered by a Valid Claim within the Licensed IP in such country at the time of sale, then the royalty rate for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the applicable rate determined pursuant to Section 6.2(a), unless such Royalty Bearing Product embodies an Invention with respect to which 4DMT made a Trade Secret Election, in which case no such reduction shall apply.

(ii) Third Party Offset. If 4DMT is required, in order to avoid infringement of any Patent Right not licensed hereunder that Covers the composition of matter, formulation, or method of use of a Royalty Bearing Product, to obtain a license from a Third Party in order to Develop, make, have made, use or Commercialize such Royalty Bearing Product in a country in the Territory and to pay a royalty or other consideration under such license (including in connection with the settlement of a patent infringement claim), then the royalty payments due under Section 6.2(a) with respect to Net Sales for such Royalty Bearing Product in such country shall be reduced by [\*\*\*] percent ([\*\*\*]%) of the amounts payable by 4DMT to such Third Party for such license that are reasonably and appropriately allocable to such Royalty Bearing Product in such country, provided that in no event shall the foregoing reduce the amount of royalties payable to uniQure in any [\*\*\*] by more than [\*\*\*] percent ([\*\*\*]%) of the amount determined pursuant to Section 6.2(a), as adjusted by application of the terms of Section 6.2(c)(i).

(iii) Limits on Deductions. Except as expressly provided in this Section 6.2, there shall not be any offset to or deduction from the royalties payable pursuant to this Section 6.2. Notwithstanding Sections 6.2(II)(c)(i) and (ii) to the contrary, in no event shall the cumulative effect of the deductions in Sections 6.2(c)(i) and (ii) reduce the royalties to less than [\*\*\*] percent ([\*\*\*]%) of the amounts determined pursuant to Section 6.2(II)(a).

6.3 Sublicense Consideration.

(a) uniQure shall pay to 4DMT the following percentages (“Sublicense Income Sharing Percentages”) of Sublicense Consideration received by uniQure for sublicenses under the Licensed IP under this Agreement:

(i) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Construct or Product that is subject of the sublicense and (B) does not require uniQure to manufacture any such Construct or Product for Clinical Trial or commercial purposes;

(ii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Construct or Product that is subject of the sublicense and (B) requires uniQure to manufacture any such Construct or Product for Clinical Trial or commercial purposes;

(iii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that does not meet the criteria set forth in Section 6.3(a)(i) or Section 6.3(a)(ii) above;

provided, however, that none of subsections (i), (ii) or (iii) shall result in uniQure paying to 4DMT under this Section 6.3 a percentage of any Sublicense Consideration consisting of royalties from Sublicensees on sales of UC Products during the applicable Royalty Term that is less than [\*\*\*] percent ([\*\*\*]%) of Net Sales by such Sublicensee of such UC Products.

(b) The term “Sublicense Consideration” shall mean consideration of any kind received by uniQure from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees, royalties or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as defined below) for stock of uniQure in consideration for such sublicense; provided, however, the following are not included in Sublicense Consideration:

(i) Support for activities of uniQure relating to the research, Development, manufacturing or Commercialization of Royalty Bearing Products, which shall not exceed the fully burdened cost (and in the case of manufacturing costs, the Fully Burdened Manufacturing Cost) for undertaking such activities performed by or for uniQure (including Third Parties on uniQure’s behalf) by more than [\*\*\*] percent ([\*\*\*]%)

(ii) Proceeds derived from debt financing and any loans to uniQure by the Sublicensee;

(iii) Consideration received for the purchase of stock in uniQure or its Affiliate to the extent that the price per share for such equity does not exceed the Fair Market Value of such stock. The term “Fair Market Value” shall mean the average price at which the stock in question is publicly trading at for [\*\*\*] ([\*\*\*]) days prior to the earlier of (A) the date of the announcement of its purchase by the Sublicensee or (B) the date of its purchase by the Sublicensee, or if the stock is not publicly traded, the value of such stock as determined in good faith by the Board of Directors of uniQure or its applicable Affiliate as of the time of receipt of payment; and

(iv) Reimbursement of uniQure's patent costs related to Patent Rights.

(c) 4DMT shall pay to uniQure the following percentages ("4D Sublicense Income Sharing Percentages") of 4D Sublicense Consideration received by 4DMT for sublicenses under the Licensed IP under this Agreement:

(i) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Construct or Product that is subject of the sublicense and (B) does not require 4DMT to manufacture any such Construct or Product for Clinical Trial or commercial purposes;

(ii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that (A) is granted prior to initiating Animal POC for any Construct or Product that is subject of the sublicense and (B) requires 4DMT to manufacture any such Construct or Product for Clinical Trial or commercial purposes;

(iii) [\*\*\*] percent ([\*\*\*]%) for any sublicense that does not meet the criteria set forth in Section 6.3(a)(i) or Section 6.3(a)(ii) above;

provided, however, that none of subsections (i), (ii) or (iii) shall result in 4DMT paying to uniQure under this Section 6.3 a percentage of any 4D Sublicense Consideration consisting of royalties from Sublicensees on sales of UC Products during the applicable Royalty Term that is less than [\*\*\*] percent ([\*\*\*]%) of Net Sales by such Sublicensee of such UC Products.

(d) The term "4D Sublicense Consideration" shall mean consideration of any kind received by 4DMT from a Sublicensee for the grant of a sublicense under this Agreement, such as upfront fees, royalties or milestone fees and including any premium paid by the Sublicensee over the Fair Market Value (as defined below) for stock of 4DMT in consideration for such sublicense; provided, however, the following are not included in Sublicense Consideration:

(i) Support for activities of 4DMT relating to the research, Development, manufacturing or Commercialization of Royalty Bearing Products, which shall not exceed the fully burdened cost (and in the case of manufacturing costs, the Fully Burdened Manufacturing Cost) for undertaking such activities performed by or for 4DMT (including Third Parties on 4DMT's behalf) by more than [\*\*\*] percent ([\*\*\*]%)

(ii) Proceeds derived from debt financing and any loans to 4DMT by the Sublicensee;

(iii) Consideration received for the purchase of stock in 4DMT or its Affiliate to the extent that the price per share for such equity does not exceed the Fair Market Value of such stock. The term "Fair Market Value" shall mean the average price at which the stock in question is publicly trading at for [\*\*\*] ([\*\*\*]) days prior to the earlier of (A) the date of the announcement of its purchase by the Sublicensee or (B) the date of its purchase by the

Sublicensee, or if the stock is not publicly traded, the value of such stock as determined in good faith by the Board of Directors of 4DMT or its applicable Affiliate as of the time of receipt of payment; and

(iv) Reimbursement of 4DMT's patent costs related to Patent Rights.

(e) For purposes of this Article 6, "Sublicense Consideration received by uniQure" shall include Sublicense Consideration received by uniQure's Affiliates (applying the definition of Sublicense Consideration *mutatis mutandis* to such Affiliates) and "4D Sublicense Consideration received by 4D" shall include 4D Sublicense Consideration received by 4DMT's Affiliates (applying the definition of Sublicense Consideration *mutatis mutandis* to such Affiliates).

6.4 Reports; Payments. Within [\*\*\*] ([\*\*\*)] days after the end of each Calendar Quarter during which there are Net Sales giving rise to a payment obligation under Section 6.2 or uniQure (or 4DMT, as applicable) received Sublicense Consideration or 4D Sublicense Consideration giving rise to a payment obligation under Section 6.3, (a) uniQure (or 4DMT as applicable) shall submit to 4DMT (or uniQure as applicable) a report (i) identifying for each Royalty Bearing Product the Net Sales for such Royalty Bearing Product for each country for such Calendar Quarter, the calculation of royalties (including gross sales and all deductions taken from gross sales and all reductions pursuant to Section 6.2(c)), and the royalties payable to 4DMT (or uniQure as applicable) and (ii) identifying the Sublicense Consideration received by uniQure (or 4DMT as applicable) in such Calendar Quarter and the one or more Sublicense Income Sharing Percentages applicable to such Sublicense Consideration, or 4D Sublicense Income Sharing Percentages applicable to such 4D Sublicense Consideration and (b) uniQure (or 4DMT as applicable) shall pay to 4DMT (or uniQure as applicable) all royalties payable under Section 6.2 and portions of Sublicense Consideration or 4D Sublicense Consideration payable under Section 6.3.

6.5 Books and Records; Audit Rights. Each Party (the "Audited Party") shall keep (and shall cause its Affiliates and Sublicensees to keep) complete, true and accurate books and records in accordance with its Accounting Standards in sufficient detail for the other Party (the "Auditing Party") to determine the payments due under this Agreement. Each Auditing Party shall have the right, once annually at its own expense, to have an independent, certified public accounting firm of nationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, review any such records of the Audited Party in the location(s) where such records are maintained by the Audited Party upon reasonable notice (which shall be no less than [\*\*\*] ([\*\*\*)] days prior notice) and during regular business hours and under obligations of strict confidence, for the sole purpose of verifying the accuracy of the amounts paid under this Agreement within a [\*\*\*] period preceding the date of the request for review. The report of such accounting firm shall be limited to a certificate stating whether any report made or invoice or payment submitted by the Audited Party during such period is accurate or inaccurate, and the amount of any Net Sales, Sublicense Consideration, 4D Sublicense Consideration, royalty or other payment discrepancy. No other information shall be provided to the Auditing Party. The Audited Party shall receive a copy of each such report concurrently with receipt by the Auditing Party. Should such inspection lead to the discovery of a discrepancy to the Auditing Party's

detriment, the Audited Party shall pay the amount of the discrepancy within [\*\*\*] ([\*\*\*)] days after its receipt from the accounting firm of the certificate showing the amount of the discrepancy. The Auditing Party shall pay the full cost of the review unless the audit determined an underpayment of royalties by the Audited Party which is greater than [\*\*\*] percent ([\*\*\*)% of the amount due for the applicable period, in which case the Audited Party shall pay the reasonable costs charged by such accounting firm for such review. Any overpayment of royalties by uniQure (or 4DMT, as applicable) revealed by an inspection shall be fully creditable against future royalty payments by such Audited Party under Section 6.2.

#### 6.6 Withholding Taxes.

(a) Subject to the provisions of Section 12.7, if Laws require withholding by uniQure of taxes imposed upon 4DMT on account of any royalty or other payment paid under this Agreement, such taxes shall be deducted by uniQure as required by Law from such remittable royalty or other payment and shall be paid by uniQure to the proper tax authorities; provided that before making any such deduction or withholding, uniQure shall give 4DMT notice of the intention to make such deduction or withholding, which notice shall include the authority, basis and method of calculation for the proposed deduction or withholding, and shall be provided to the extent practicable at least a reasonable period of time before such deduction or withholding is required, in order for 4DMT to obtain reduction of or relief from such deduction or withholding. Official receipts of payment of withholding taxes shall be secured and sent to 4DMT as evidence of such payment. The Parties shall exercise their best efforts to ensure that any withholding tax imposed is reduced as far as possible under the provisions of any relevant tax treaty.

(b) Subject to the provisions of Section 12.7, if Laws require withholding by 4DMT of taxes imposed upon uniQure on account of any royalty or other payment paid under this Agreement, such taxes shall be deducted by 4DMT as required by Law from such remittable royalty or other payment and shall be paid by 4DMT to the proper tax authorities; provided that before making any such deduction or withholding, 4DMT shall give uniQure notice of the intention to make such deduction or withholding, which notice shall include the authority, basis and method of calculation for the proposed deduction or withholding, and shall be provided to the extent practicable at least a reasonable period of time before such deduction or withholding is required, in order for uniQure to obtain reduction of or relief from such deduction or withholding. Official receipts of payment of withholding taxes shall be secured and sent to uniQure as evidence of such payment. The Parties shall exercise their best efforts to ensure that any withholding tax imposed is reduced as far as possible under the provisions of any relevant tax treaty.

6.7 United States Dollars. All dollar (\$) amounts specified in this Agreement are United States dollar amounts.

6.8 Payment Method and Currency Conversion. Except as otherwise provided herein, all payments due to a Party hereunder shall be due and payable within [\*\*\*] ([\*\*\*)] days after receipt of an invoice from the other Party and shall be paid via a bank wire transfer to such bank account as such Party shall designate. For the purposes of determining the amount of any payment due hereunder for the relevant Calendar Quarter under Section 6.2 or Section 6.3, amounts received by a Party in any foreign currency shall be converted into United States dollars

in accordance with the normal business practice of such Party , as applied consistently across its business.

6.9 Blocked Payments. (a) If, by reason of applicable Laws in any country in the Territory, it becomes impossible or illegal for uniQure or any of its Affiliates or Sublicensees to transfer, or have transferred on its behalf, royalties or other payments to 4DMT, uniQure shall promptly notify 4DMT of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of 4DMT in a recognized banking institution with a good creditworthiness, such banking institution to be designated by 4DMT or, if none is designated by 4DMT within [\*\*\*] ([\*\*\*)] days, in a recognized banking institution selected by uniQure or its Affiliate or Sublicensee, as the case may be, and identified in a written notice given to 4DMT. If so deposited in a foreign country, uniQure shall provide, or cause its Affiliate or Sublicensee to provide, reasonable cooperation to 4DMT so as to allow 4DMT to assume control over such deposit as promptly as practicable.

(b) If, by reason of applicable Laws in any country in the Territory, it becomes impossible or illegal for 4DMT or any of its Affiliates or Sublicensees to transfer, or have transferred on its behalf, royalties or other payments to uniQure, 4DMT shall promptly notify uniQure of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of uniQure in a recognized banking institution with a good creditworthiness, such banking institution to be designated by uniQure or, if none is designated by uniQure within [\*\*\*] ([\*\*\*)] days, in a recognized banking institution selected by 4DMT or its Affiliate or Sublicensee, as the case may be, and identified in a written notice given to uniQure. If so deposited in a foreign country, 4DMT shall provide, or cause its Affiliate or Sublicensee to provide, reasonable cooperation to uniQure so as to allow uniQure to assume control over such deposit as promptly as practicable.

6.10 Late Payments. Any payment not made within [\*\*\*] ([\*\*\*)] Business Days after the due date for such payment pursuant to the terms of this Agreement shall bear interest at a rate of the thirty-day U.S. dollar LIBOR rate effective for the date that payment was due (as published in The Wall Street Journal, Eastern Edition) plus [\*\*\*] per annum. Calculation of interest will be made for the exact number of days the payment was past due based on a year of 360 days (actual days/360).

## ARTICLE VII

### PATENTS

7.1 Disclosure. Each Party shall promptly disclose to the other Party any Inventions that it or its Affiliates or Sublicensees or their employees, independent contractors, or agents solely or jointly make, conceive, reduce to practice, or otherwise discover under this Agreement, and each Party shall maintain and make available to the other Party records regarding any Inventions that it has an obligation to assign under Section 7.2(a).

## 7.2 Ownership.

(a) uniQure shall solely own all Core uniQure Intellectual Property, and 4DMT shall solely own all Core 4DMT Intellectual Property. Without limiting the generality of the foregoing, this means that 4DMT shall own the New Variant Patents, and uniQure shall own the uniQure Product Patents. All other Inventions arising under this Agreement or the Parties' activities hereunder, shall be owned by inventorship. Without additional consideration, each Party shall assign and hereby does assign to the other Party such of its right, title, and interest in and to such Inventions, Know-How and Patent Rights (and shall require its Affiliates and Sublicensees, and all employees, independent contractors and their employees, and agents of such Party and its Affiliates and Sublicensees to so assign to the other Party such of their right, title, and interest) and agrees to take all necessary actions and execute any documents as is necessary to effectuate the allocation of right, title, and interest as set forth in this Section 7.2(a).

(b) Except as otherwise expressly set forth in Section 7.2(a), as between the Parties, (i) each Party shall solely own all Know-How and Inventions invented solely by employees, agents and consultants of such Party or its Affiliates, and any Patent Right related thereto, subject to the licenses granted under ARTICLE V, and (ii) Know-How and Inventions invented jointly by employees, agents, or consultants of the Parties or their Affiliates ("Joint Intellectual Property", which includes any Patent Right Covering such Know-How and Inventions ("Joint Patent Rights") and any Know-How included in such Joint Intellectual Property ("Joint Know-How")) shall be jointly owned, subject to the licenses granted under ARTICLE V. Inventorship shall be determined in accordance with U.S. patent Laws for purposes of determining ownership in accordance with the foregoing. Except as explicitly provided for herein, the nature of the ownership rights in Joint Patent Rights shall be equivalent to the rights of co-inventors under U.S. patent law in the absence of a written agreement.

(c) Except as expressly provided in this Agreement, and subject to any restriction herein (including the licenses granted under ARTICLE V), (i) each joint owner may engage in research, Development, manufacturing and Commercialization activities relating to Joint Intellectual Property, and (ii) each may assign, license, sell or otherwise encumber or transfer any such interest without the prior written approval of the other Party and without obligation to account or provide compensation to the other Party.

## 7.3 uniQure Prosecution and Maintenance of Patent Rights.

(a) uniQure shall be solely responsible for the Prosecution and Maintenance of the uniQure Patent Rights, including the Core uniQure Patent Rights, at its sole expense and its sole discretion. uniQure shall give 4DMT an opportunity to review the text of each application, office action response or other substantive document for a Core uniQure Patent Right specifically relating to [\*\*\*] (but not any other uniQure Patent Right) before filing with any patent office in the Territory, shall consider 4DMT's reasonable comments with respect thereto, and shall supply 4DMT with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number.

(b) uniQure shall have the sole right to determine whether any patent application is filed with respect to any Core uniQure Know-How and whether to maintain any

Invention included in the Core uniQure Know-How as a trade secret. uniQure shall provide 4DMT with written notice if uniQure elects not to file a patent application claiming any particular Invention included in the Core uniQure Know-How (each, a “Trade Secret Election”).

(c) Notwithstanding anything express or implied and provided that a Patent Right has not been filed hereunder with respect to a New Capsid Variant, uniQure shall give reasonable notice to 4DMT (no less than [\*\*\*] ([\*\*\*)] days) prior to filing a uniQure Product Patent disclosing a New Capsid Variant, and the Parties shall cooperate reasonably in the filing of such uniQure Product Patent, including coordinating the timely filing of a Patent Right with respect to a New Capsid Variant and, where appropriate, the simultaneous filing of such patents by each Party.

#### 7.4 4DMT Prosecution and Maintenance of Patent Rights.

(a) 4DMT shall be solely responsible for the Prosecution and Maintenance of the 4DMT Patent Rights, including the Core 4DMT Patent Rights, at its sole expense and its sole discretion. 4DMT will reasonably inform uniQure regarding the Prosecution and Maintenance of 4DMT Patent Rights ([\*\*\*]). Notwithstanding the foregoing, the Parties acknowledge that UC will handle the Prosecution and Maintenance of the UC Patent Rights in accordance with the terms of the UCB Agreements.

(b) 4DMT shall have the sole right to determine whether any patent application is filed with respect to any Core 4DMT Know-How and whether to maintain any Invention included in the Core 4DMT Know-How as a trade secret. 4DMT shall provide uniQure with written notice if 4DMT elects not to file a patent application claiming any particular Invention included in the Core 4DMT Know-How specifically relating to compositions of matter of, methods of use of, or methods of making any New Capsid Variant because 4DMT prefers to maintain such Invention as a trade secret (each, a “Trade Secret Election”).

(c) 4DMT shall notify uniQure at least [\*\*\*] ([\*\*\*)] days in advance of any applicable deadline if (i) 4DMT decides that it does not wish to continue the Prosecution and Maintenance of a published Core 4DMT Patent Right specifically relating to [\*\*\*] for which no substitute has been filed, or (ii) 4DMT decides that it intends to abandon claim scope in a [\*\*\*], which claim scope is intended to be maintained by uniQure, in which case, with respect to this clause (ii), uniQure may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (i) or (ii), 4DMT shall allow uniQure to assume responsibility for Prosecution and Maintenance of such Core 4DMT Patent Right or divisional application [\*\*\*]. If uniQure assumes such responsibility, then uniQure may designate any counsel of its choice reasonably acceptable to 4DMT to handle the Prosecution and Maintenance of such Core 4DMT Patent Right or divisional application (which shall otherwise continue to be part of the Core 4DMT Patent Rights).

7.5 Prosecution and Maintenance of Joint Patent Rights. The Prosecution and Maintenance of any Joint Patent Right shall be through a mutually selected patent counsel. Within [\*\*\*] ([\*\*\*)] days following the Effective Date, the Parties shall agree on a patent counsel (“Joint Counsel”) who shall be engaged by both Parties for the Prosecution and Maintenance of all such Joint Patent Rights. The following terms shall apply to each Joint Patent Right:



(a) The Parties shall instruct Joint Counsel to conduct its activities as follows: The Joint Counsel shall give uniQure and 4DMT (or each Party's designee) an opportunity to review the text of each application, office action response or other substantive document for a Joint Patent Right before filing with any patent office in the Territory, shall incorporate uniQure's and 4DMT's (or each Party's designee) reasonable comments with respect thereto, and shall supply uniQure and 4DMT (or each Party's designee) with a copy of each such application, office action response or other substantive document as filed, together with notice of its filing date and serial number. In the event that 4DMT and uniQure provide Joint Counsel with conflicting instructions regarding the Prosecution and Maintenance of a Joint Patent Right, Joint Counsel shall make the Parties aware of such conflicting instructions and, if the Parties are not able to resolve such conflict within a reasonable time prior to the applicable filing deadline, the Joint Counsel shall take such action as would reasonably be expected to maximize the scope, extent and coverage of such Joint Patent Right.

(b) Both Parties shall cooperate with Joint Counsel in Prosecution and Maintenance of patent applications for Joint Patent Rights, including providing Joint Counsel with data and other information as appropriate with respect thereto.

(c) Joint Counsel shall keep uniQure and 4DMT advised of the status of the Prosecution and Maintenance of Joint Patent Rights, including actual and prospective patent filings for Joint Patent Rights, and shall provide each Party with advance copies of any and all papers related thereto. Joint Counsel shall promptly give notice to uniQure and 4DMT of the grant, lapse, revocation, surrender, invalidation or abandonment of any Joint Patent Right.

(d) The Parties shall equally share all fees and costs charged by Joint Counsel with respect to the Prosecution and Maintenance of Joint Patent Rights and all other mutually agreed and approved out-of-pocket costs and expenses incurred by either Party in connection with such Prosecution and Maintenance of Joint Patent Rights.

(e) uniQure shall notify 4DMT and Joint Counsel at least [\*\*\*] ([\*\*\*)] days in advance of the next deadline if (A) uniQure decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) uniQure decides that it intends to abandon claim scope in a Joint Patent Right which claim scope is intended to be maintained by 4DMT, in which case, with respect to this clause (B), 4DMT may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (A) or (B), uniQure shall allow 4DMT to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including [\*\*\*]. If 4DMT assumes such responsibility, then: (i) 4DMT may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights; (ii) uniQure shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a 4DMT Patent Right; and (iii) uniQure shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to 4DMT as the sole owner. If 4DMT decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

(f) 4DMT shall notify uniQure and Joint Counsel at least [\*\*\*] ([\*\*\*)] days in advance of the next deadline if (A) 4DMT decides that it does not wish to continue paying for the Prosecution and Maintenance of a particular Joint Patent Right for which no substitute has been filed, or (B) 4DMT decides that it intends to abandon claim scope in a Joint Patent Right which claim scope is intended to be maintained by uniQure, in which case, with respect to this clause (B), uniQure may assume responsibility for such claim scope by filing a divisional application restricted to such claim scope. In such cases (A) or (B), 4DMT shall allow uniQure to assume responsibility for Prosecution and Maintenance of the respective Patent Rights, including [\*\*\*]. If uniQure assumes such responsibility, then: (i) uniQure may designate any counsel of its choice to handle the Prosecution and Maintenance of such Joint Patent Right or of the divisional application and it shall cease to be a part of the Joint Patent Rights and no further uniQure royalty obligations shall exist under this Agreement with respect thereto; (ii) 4DMT shall lose its licenses to such former Joint Patent Right or divisional application under ARTICLE V and such former Joint Patent Right or divisional application shall be deemed a uniQure Patent Right; and (iii) 4DMT shall and hereby does transfer and assign all right, title and interest in said former Joint Patent Right or of the divisional application to uniQure as the sole owner. If uniQure decides not to assume such responsibility, then it shall instruct Joint Counsel to abandon the Prosecution and Maintenance of such Joint Patent Right or not to file such divisional application.

#### 7.6 Third Party Infringement.

(a) Notice. Each Party shall promptly report in writing to the other Party any known or suspected (i) infringement of any of the 4DMT Patent Rights, uniQure Patent Rights or Joint Patent Rights, or (ii) unauthorized use or misappropriation of any of the 4DMT Know-How, uniQure Know-How or Joint Know-How, of which such Party becomes aware and shall provide the other Party with all available evidence regarding such known or suspected infringement or unauthorized use.

(b) Enforcement of Solely Owned Patent Rights. uniQure shall have the sole right to enforce the uniQure Patent Rights, including the Core uniQure Patent Rights. Subject to UC's rights under the UCB Agreements with respect to any UC Patent Right included in the 4DMT Patent Rights, 4DMT shall have the sole right to enforce any 4DMT Patent Right, including the Core 4DMT Patent Rights. Each Party shall cooperate in the prosecution of any such suit brought by the enforcing Party as may be reasonably requested by the enforcing Party; provided that the enforcing Party shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by the non-enforcing Party in connection with such cooperation.

#### (c) Enforcement of Joint Patent Rights.

(i) Restricted Target Products. uniQure shall have the first right, but not the obligation, to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement in the Field by a Product that delivers a Transgene related to a Restricted Target. 4DMT shall cooperate in the prosecution of any such suit as may be reasonably requested by uniQure, including joining any action as party-plaintiff at uniQure's sole discretion; provided that uniQure shall promptly reimburse all out-of-pocket expenses (including

reasonable counsel fees and expenses) actually incurred by 4DMT in connection with such cooperation.

(ii) Non-Restricted Target Products. 4DMT shall retain any and all rights to initiate a lawsuit or take other reasonable action to enforce the Joint Patent Rights against any infringement that is (A) outside the Field, and/or (B) by Products related to Non-Restricted Targets. uniQure shall cooperate in the prosecution of any such suit as may be reasonably requested by 4DMT, including joining any action as party-plaintiff at 4DMT's sole discretion; provided that 4DMT shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by uniQure in connection with such cooperation.

(iii) Step-In Right of 4D. If uniQure does not initiate a lawsuit or take other reasonable action pursuant to this Section 7.6(c) regarding infringement or alleged infringement of a New Variant Patent, then 4DMT shall have the right, but not the obligation, to initiate such lawsuit or take such other action, after providing [\*\*\*] ([\*\*\*)] days' notice to uniQure and giving good faith consideration to uniQure's reason(s) for not initiating a lawsuit or taking other action. For this purpose, uniQure shall cooperate in the prosecution of any such suit as may be reasonably requested by 4DMT, including joining any action as party-plaintiff if needed for standing purposes; provided, that 4DMT shall promptly reimburse all out-of-pocket expenses (including reasonable counsel fees and expenses) actually incurred by uniQure in connection with such cooperation.

(d) Conduct of Certain Actions; Costs. The Party initiating legal action shall have the sole and exclusive right to select counsel for any suit initiated by it pursuant to Section 7.6(b) or 7.6(c) (the "Initiating Party"). The Initiating Party shall bear its own out-of-pocket costs incurred in any such legal action, including the fees and expenses of the counsel selected by it. The other Party shall have the right to participate and be represented in any such legal action (in cases where such other Party has standing) by its own counsel at its own expense. The Initiating Party shall have the final say about the strategy and decisions in the suit and any settlement.

(e) Recoveries. Any amount recovered in any action or settlement of any such action shall be allocated first to equally reimburse each Party's actual out-of-pocket costs (including reasonable attorneys' fees and expenses) incurred in such action and any amount remaining shall be allocated to the Initiating Party; provided that for recoveries for infringement within the Field, the amount of remaining recovery received by the Initiating Party or its Affiliate will be [\*\*\*].

7.7 Patent Invalidity Claim. Each Party shall promptly notify the other in the event of any legal or administrative action by any Third Party against a 4DMT Patent Right, uniQure Patent Right or Joint Patent Right of which it becomes aware, including any nullity, revocation, reexamination or compulsory license proceeding. To the extent such action is in connection with an enforcement of such Patent Right under Section 7.6, the Parties' rights with

respect to defending any such Patent Right in any such proceeding shall correspond to those set forth in Section 7.6.

#### 7.8 Patent Term Extensions.

(a) uniQure shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any uniQure Patent Right, including any Core uniQure Patent Right, and all costs and expenses relating thereto shall be paid by uniQure.

(b) 4DMT shall have full and exclusive right to determine and control all filings of requests for any patent term extension or supplemental patent certificate or their equivalents in any country in the Territory for any 4DMT Patent Right, including any Core 4DMT Patent Right, and all costs and expenses relating thereto shall be paid by 4DMT.

(c) The Parties shall jointly determine how to defend any such action relating to any Joint Patent Right.

(d) The Parties shall reasonably cooperate with each other in obtaining patent term extensions or supplemental protection certificates or their equivalents in any country in the Territory.

#### 7.9 Orange Book; Paragraph IV Certification.

(a) uniQure shall have the right, but not the obligation, to list any uniQure Patent Rights in the then-current edition of the FDA publication “Approved Drug Products With Therapeutic Equivalence Evaluations” (the “Orange Book”), or equivalent patent listings in other countries. 4DMT shall have the right, but not the obligation, to list any 4DMT Patent Rights in the then-current edition of the Orange Book, or equivalent patent listings in other countries.

(b) With respect to any notification provided by a Third Party to uniQure or 4DMT under 21 U.S.C. § 355(j)(2)(B) making a certification described in 21 U.S.C. § 355(j)(2)(A)(vii)(IV) with respect to any uniQure Patent Right that is listed for a Royalty Bearing Product in the Orange Book, or equivalent actions in other countries, (each a “Paragraph IV Certification”), the following shall apply notwithstanding Sections 7.6 and 7.7:

(i) Without any avoidable delay, however at the latest within [\*\*\*] ([\*\*\*)] Business Days after receipt of any notification of a Paragraph IV Certification, such Party shall notify the other Party in writing and attach of copy of such notification. uniQure and 4DMT shall thereafter consult and cooperate fully to determine a course of action with respect to any such proceeding, including the negotiation of the offer of confidential access.

(ii) With respect to any uniQure Patent Right, uniQure shall have the sole right to initiate any infringement proceeding as a result of such Paragraph IV Certification (a “Paragraph IV Proceeding”) with respect to a Royalty Bearing Product, including by commencing a patent infringement action under 35 U.S.C. § 271(e)(2)(A), and shall bear the expense of any such Paragraph IV Proceeding and, if legally required, may commence such action

in 4DMT's or the relevant 4DMT Affiliate's name and on 4DMT's or the relevant 4DMT Affiliate's behalf.

(iii) Section 7.6(e) shall apply if any amount is recovered in any Paragraph IV Proceeding or settlement of any Paragraph IV Proceeding under this Section 7.9(b).

7.10 CREATE Act. Each Party acknowledges and agrees that this Agreement is a "joint research agreement" as contemplated by 35 U.S.C. § 102(c), and that all Inventions are intended to have the benefit of the rights and protections conferred by the Cooperative Research and Enhancement Act of 2004 (the "CREATE Act"). In the event that a Party seeks to rely on the foregoing and to invoke the CREATE Act with respect to any Invention, such Party will give prior written notice to the other Party of its intent to invoke the CREATE Act and of each submission or disclosure such Party intends to make to the United States Patent and Trademark Office (the "USPTO") pursuant to the CREATE Act, including: (a) any disclosure of the existence or contents of this Agreement to the USPTO, (b) the disclosure of any "subject matter developed by the other Party" (as such term is used in the CREATE Act) in an information disclosure statement or otherwise, or (c) the filing of any terminal disclaimer over the intellectual property of the other Party, it being agreed that no such submission, disclosure or filing shall be made by such Party without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned or delayed, except that no such consent shall be required to disclose to the USPTO, through an information disclosure statement or otherwise, any "subject matter developed by the other Party" that was previously published or included in a published patent application by the other Party. The other Party will provide reasonable cooperation to such Party in connection with such Party's efforts to invoke and rely on the CREATE Act.

## ARTICLE VIII

### CONFIDENTIALITY AND PUBLICATION

8.1 Confidentiality Obligations. Each Party shall (a) maintain in confidence the Confidential Information of the other Party to the same extent such Party maintains its own confidential information, (b) not disclose such Confidential Information to any Third Party without the prior written consent of the other Party, and (c) not use such Confidential Information for any purpose except those permitted by this Agreement. Such obligations shall survive for a period of [\*\*\*] ([\*\*\*)] years after termination or expiration of this Agreement, except that such obligations shall survive with respect to any Confidential Information identified by the disclosing Party as a trade secret for so long as such Confidential Information remains a trade secret.

8.2 Exceptions to Confidentiality. Notwithstanding the foregoing, the obligations of confidentiality set forth in Section 8.1 shall not apply to information that, in each case as demonstrated by competent written documentation:

(a) is publicly disclosed or made generally available to the public by the disclosing Party, either before or after it becomes known to the receiving Party;

(b) was known to the receiving Party, without any obligation to keep it confidential, prior to the date of first disclosure by the disclosing Party to the receiving Party, as shown by the receiving Party's files and records;

(c) is subsequently disclosed to the receiving Party by a Third Party lawfully in possession thereof without obligation to keep it confidential and without a breach of such Third Party's obligations of confidentiality;

(d) has been publicly disclosed or made generally available to the public other than through any act or omission of the receiving Party or its Affiliates in breach of this Agreement; or

(e) has been independently developed by the receiving Party without the aid, application or use of the disclosing Party's Confidential Information (the competent written proof of which must be contemporaneous with such independent development).

8.3 Authorized Disclosure. Notwithstanding Section 8.1, a Party may disclose Confidential Information of the other Party to the extent such disclosure is reasonably necessary in the following instances:

(a) Prosecuting and Maintaining Patent Rights in accordance with this Agreement;

(b) making filings with Regulatory Authorities in accordance with this Agreement;

(c) complying with applicable Laws or submitting information to tax or other Governmental Authorities; provided that if a Party is required by Law to make any public disclosure of Confidential Information of the other Party, to the extent it may legally do so, it will give reasonable advance notice to the other Party of such disclosure and will use its reasonable efforts to secure confidential treatment of such Confidential Information prior to its disclosure (whether through protective orders or otherwise);

(d) to its Affiliates, and to prospective and actual acquirers, licensees, sublicensees, employees, consultants, agents, accountants, lawyers, advisors, investors and underwriters, on a need to know basis, each of whom prior to disclosure must be bound by written or professional ethical obligations of confidentiality and non-use equivalent in scope to those set forth in this ARTICLE VIII and that are of reasonable duration in view of the circumstances of the disclosure; or

(e) to the extent mutually agreed to in writing by the Parties.

8.4 Scientific Publications. During the Research Term, neither Party shall first publish or first present in a public forum the scientific or technical results of any activity performed pursuant to this Agreement without the opportunity for prior review and comment by and obtaining the permission of the other Party, except that either Party may publish or first present in a public forum any information related to the Research Program and/or any pre-clinical or clinical results obtained by such Party pertaining to any New Capsid Variants, without the need

to obtain the other Party's review or permission, provided that the publication or presentation does not disclose the sequence of any New Capsid Variant for which a Patent Right has not yet been filed under this Agreement, and provided in the case of uniQure that the publication must only present data and information as to a Royalty-Bearing Construct or Royalty-Bearing Product for which uniQure at that time holds a commercialization license under this Agreement, not a New Capsid Variant outside the context of a Royalty-Bearing Construct or Royalty-Bearing Product for which uniQure at that time holds a commercialization license under this Agreement.

#### 8.5 Press Releases and Other Permitted Disclosures.

(a) 4DMT and uniQure each agree not to disclose any of the terms and conditions of this Agreement to any Third Party, except as described below in this Section 8.5. The Parties will cooperate in the release of a mutually agreed upon press release announcing the collaboration contemplated by this Agreement as soon as practicable after the Effective Date. Subject to the other provisions of this Agreement, no other press release, public statement or public disclosure concerning the existence or terms of this Agreement shall be made, either directly or indirectly, by either Party, without first obtaining the written approval of the other Party, which such approval shall not be unreasonably withheld or delayed beyond [\*\*\*] ([\*\*\*) Business Days (or [\*\*\*] ([\*\*\*) Business Days if the Party wishing to make such disclosure or any of its controlling Affiliates is then a public company) following submission to the approving Party of a draft of the respective press release, public statement or public disclosure. In no event shall any such subsequent press release, public statement or public disclosure by 4DMT disclose, if previously undisclosed, the identity of any Construct or Product or the stage of development of any Construct or Product that uniQure is researching, Developing, manufacturing, or Commercializing; provided that for clarity, uniQure may disclose, without the written approval of 4DMT, the identity of any Construct or Product or the stage of development of any Construct or Product that uniQure is researching, Developing, manufacturing, or Commercializing. In no event shall any such subsequent press release, public statement or public disclosure by a Party disclose, if previously undisclosed, the financial terms of this Agreement; provided that 4DMT may disclose the receipt of, and uniQure may disclose the payment of, any payment but not the amount of such payment; provided, further, however, that if disclosure of the amount of a payment is required by applicable Law, by applicable stock exchange regulation, or by order or other ruling of a competent court, as set forth in Section 8.5(c), then 4DMT or uniQure, as the case may be, may also disclose such amount in a public statement or disclosure. Once any public statement or public disclosure has been approved in accordance with this Section 8.5, then either Party may appropriately communicate information contained in such permitted statement or disclosure.

(b) Either Party may disclose the existence and terms of this Agreement in confidence to its attorneys, to UC, and to each of the following, under an agreement with terms of confidentiality and non-use no less rigorous than the terms contained in this Agreement and, as applicable, to use such information solely for the purpose permitted pursuant to the applicable subsection of this Section 8.5(b):

- (i) professional accountants, consultants, or auditors;

(ii) bankers or other financial advisors, in connection with an initial public offering, private financing or other strategic transaction, or corporate valuation for internal purposes;

(iii) potential acquirers (and their respective attorneys and professional advisors), in connection with a potential merger, acquisition or reorganization; provided that the Party making the disclosure has a *bona fide* offer (e.g., a signed term sheet or letter of intent, even if non-binding) from such Third Party for such a transaction;

(iv) to actual or potential investors, lenders or permitted assignees of such Party (and their respective attorneys and professional advisors); or

(v) to actual or potential licensees or sublicensees of such Party (and their respective attorneys and professional advisors); provided that such disclosure in the case of 4DMT shall not include any financial terms, the Delivery Success Criteria, nor any other contents of the Research Plan for Liver nor the Research Plan for CNS.

(c) Notwithstanding the foregoing provisions of this ARTICLE VIII, a Party may disclose the existence and terms of this Agreement, however excluding, as far as legally possible, Schedule 2, or the Parties' activities under this Agreement, where required, as reasonably determined by the legal counsel of the disclosing Party, by applicable Law, by applicable stock exchange regulation or by order or other ruling of a competent court, although, to the extent practicable, the other Party shall be given [\*\*\*] ([\*\*\*) Business Days advance notice of any such legally required disclosure to comment and reasonably consider such comments provided by such other Party on the proposed disclosure. In case either Party is obliged to publish this Agreement as a "material agreement" in accordance with the U.S. stock exchange regulations ("SEC Filing"), this Agreement shall be redacted by the filing Party as far as legally possible, and the filing Party shall cooperate with the other Party reasonably in advance to such SEC Filing to enable the other Party to review and comment on the scope of such redaction.

## ARTICLE IX

### **REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION**

9.1 Representations and Warranties of the Parties. uniQure and 4DMT each represent, warrant and covenant to the other that:

(a) as of the Effective Date, it has the authority and right to enter into and perform this Agreement and grant the rights embodied herein, and it is not aware of any legal impediment that could inhibit its ability to perform its obligations under this Agreement;

(b) as of the Effective Date, its execution, delivery and performance of this Agreement does not conflict with, or constitute a breach of, any order, judgment, agreement or instrument to which it is a party or is otherwise bound;

(c) it shall comply in all material respects with all Laws applicable to its actions under this Agreement; and



(d) as of the Effective Date, no consent of any Third Party is required for such Party to grant the licenses and rights granted to the other Party under this Agreement or to perform its obligations hereunder.

9.2 Representations and Warranties of 4DMT. 4DMT represents, warrants and covenants to uniQure that:

(a) [Intentionally omitted.]

(b) as of the Effective Date, 4DMT has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in 4DMT Intellectual Property in a manner inconsistent with the terms hereof;

(c) as of the Effective Date, 4DMT has valid and existing licenses, free and clear of all liens, charges and encumbrances, to the 4DMT Patent Rights not owned by 4DMT;

(d) as of the Effective Date, to 4DMT's knowledge, the conception, development and reduction to practice of the 4DMT Intellectual Property has not constituted or involved the misappropriation of trade secrets of any Third Party or the infringement of issued Patent Rights of any Third Party;

(e) as of the Effective Date, 4DMT has not received any written notice of any unauthorized use, infringement, or misappropriation by any person or entity, including any current or former employee or consultant of 4DMT, of any 4DMT Intellectual Property;

(f) as of the Effective Date, to 4DMT's knowledge, there are no claims, judgments, settlements pending or any action with respect to the 4DMT Intellectual Property;

(g) as of the Effective Date, to 4DMT's knowledge, uniQure's use of the 4DMT Intellectual Property, as reasonably anticipated to be used in the conduct of the Research Program, will not infringe any valid Patent Right existing as of the Effective Date and owned by any Third Party;

(h) all of 4DMT's personnel and employees, and Third Parties, including agents and consultants, hired by 4DMT and involved in the Research Program are, or when hired will be, under a written obligation to assign to 4DMT any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(i) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that arise on its part out of this Agreement or that would deprive uniQure of the benefits of or rights granted under this Agreement;

(j) as of the Effective Date, each of the UCB Agreements is in full force and effect, and 4DMT will not, after the Effective Date, terminate, amend or otherwise modify any of the terms thereof without prior written consent from uniQure, or take any action or refrain from taking any action that would permit UC to terminate any UCB Agreement (it being recognized

that if the New Capsid Variants are not UC AAV Capsid Variants, and UC terminates any UCB Agreement, 4DMT shall not be deemed to be in breach of the foregoing), and 4DMT shall promptly provide uniQure with a copy of each notice it receives from UC under any UCB Agreement; and

(k) if, during the Term, 4DMT has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then 4DMT shall immediately notify uniQure in writing.

For purposes of this Section 9.2, “knowledge” shall mean the actual knowledge of 4DMT, including [\*\*\*].

9.3 Representations and Warranties of uniQure. uniQure represents, warrants and covenants to 4DMT that:

(a) all of uniQure’s personnel and employees, and Third Parties, including agents and consultants, hired by uniQure and involved in the Research Program are, or when hired will be, under a written obligation to assign to uniQure any right they may have in any Invention first invented, discovered, made, conceived or reduced to practice in the conduct of activities pursuant to the Research Program, and all intellectual property rights therein;

(b) it will not, after the Effective Date, enter into any written or oral contractual obligation with any Third Party that would be inconsistent with the obligations that arise on its part out of this Agreement or that would deprive 4DMT of the benefits of or rights granted under this Agreement;

(c) if, during the Term, uniQure has reason to believe that it or any of its employees, officers, subcontractors, or consultants rendering services hereunder (i) is or shall be debarred or convicted of a crime under 21 U.S.C. Section 335a, or (ii) is or shall be under indictment under said Section 335a, then uniQure shall immediately notify 4DMT in writing.

9.4 No Other Warranties.

(a) EXCEPT AS OTHERWISE EXPRESSLY SET FORTH HEREIN, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, AND PARTICULARLY THAT PRODUCT(S) WILL BE SUCCESSFULLY DEVELOPED HEREUNDER, AND IF PRODUCT(S) ARE DEVELOPED, WITH RESPECT TO SUCH PRODUCT(S), THE PARTIES DISCLAIM ALL IMPLIED WARRANTIES OF TITLE, NON-INFRINGEMENT, MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE.

(b) uniQure acknowledges that UC has not warranted to 4DMT under the UCB Agreements as to the validity of any Patent Rights or that practice under such Patent Rights shall be free of infringement. UNIQURE, ITS AFFILIATES AND ITS SUBLICENSEE(S) AGREE THAT (I) THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS, THE UC AAV CAPSID VARIANTS, AND THE ASSOCIATED INVENTIONS ARE PROVIDED WITHOUT WARRANTY OF MERCHANTABILITY OR FITNESS FOR A

PARTICULAR PURPOSE OR ANY OTHER WARRANTY, EXPRESSED OR IMPLIED; (II) UC MAKES NO REPRESENTATION OR WARRANTY THAT ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS WILL NOT INFRINGE ANY PATENT OR OTHER PROPRIETARY RIGHT; AND (III) IN NO EVENT WILL UC BE LIABLE FOR ANY INCIDENTAL, SPECIAL OR CONSEQUENTIAL DAMAGES RESULTING FROM EXERCISE OF THE LICENSES GRANTED PURSUANT TO THE UCB AGREEMENTS OR THE USE OF ANY INVENTION CLAIMED BY THE UC PATENT RIGHTS, THE UC AAV CAPSID VARIANTS, THE UC PATENT RIGHTS, OR THE UC PRODUCTS.

9.5 Indemnification by uniQure. uniQure shall indemnify, hold harmless and defend 4DMT, its Affiliates and all of their respective officers, directors, employees, agents and shareholders (collectively, the “4DMT Indemnitees”) from and against any and all losses, damages, liabilities, judgments, fines, amounts paid in settlement, expenses and costs of defense (including reasonable attorneys’ fees and witness fees) (collectively, “Damages”) resulting from any demand, claim, action or proceeding brought or initiated by a Third Party (each a “Third Party Claim”) against any 4DMT Indemnatee to the extent arising out of: (a) a Default by uniQure; (b) the negligence or willful misconduct of a uniQure Indemnatee; or (c) the use, Development, Commercialization, storage or other exploitation of any Construct or Product by uniQure, its Affiliates, Sublicensees, Third Party Distributors, or Third Party independent contractors; provided that (i) the 4DMT Indemnitees shall comply with the procedures set forth in Section 9.7(a); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by 4DMT under Section 9.6.

9.6 Indemnification by 4DMT. 4DMT shall indemnify, hold harmless and defend uniQure, its Affiliates and all of their respective officers, directors, employees, agents, and shareholders (collectively, the “uniQure Indemnitees”) from and against any and all Damages resulting from any Third Party Claim against any uniQure Indemnatee to the extent arising out of: (a) a Default by 4DMT; (b) the negligence or willful misconduct of a 4DMT Indemnatee; or (c) the use, Development, Commercialization, storage or other exploitation of any 4DMT AAV Capsid Variant, Construct or Product (other than a uniQure Product Developed, or Commercialized by uniQure, its Affiliate, or Sublicensee) by 4DMT, its Affiliates, Sublicensees or Third Party independent contractors; provided that (i) the uniQure Indemnitees shall comply with the procedures set forth in Section 9.7(b); and (ii) such indemnity shall not apply to the extent such Third Party Claim is subject to indemnification by uniQure under Section 9.5.

9.7 Procedure.

(a) To be eligible for the 4DMT Indemnitees to be indemnified hereunder, 4DMT shall provide uniQure with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.5 and the exclusive ability to defend or settle any such claim; provided however that uniQure shall not enter into any settlement for damages, or that imposes upon 4DMT any obligation or liability, without 4DMT’s prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. 4DMT shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by uniQure.

(b) To be eligible for the uniQure Indemnities to be indemnified hereunder, uniQure shall provide 4DMT with prompt notice of the Third Party Claim giving rise to the indemnification obligation under Section 9.6 and the exclusive ability to defend or settle any such claim; provided however that 4DMT shall not enter into any settlement for damages, or that imposes upon uniQure any obligation or liability, without uniQure's prior written consent, such consent not to be unreasonably withheld, delayed or conditioned. uniQure shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by 4DMT.

9.8 uniQure Indemnity to UC. uniQure shall, and shall require its Sublicensees to, indemnify, defend, and hold harmless UC and IGT, and their officers, employees, and agents; sponsor(s) of the research that led to the inventions disclosed in the UC Patent Rights and the UC AAV Capsid Variants; and the inventors of any UC Patent Rights and their employers against any and all losses, damages, costs, fees, and expenses resulting from Third Party claims and suits arising out of uniQure's activities under this Agreement or of any Sublicensee activities under any sublicense agreement granting rights under the UC Patent Rights or the UC AAV Capsid Variants, or any use or possession of the UC AAV Capsid Variants resulting from uniQure's exploitation of its rights thereto. This indemnification will include any product liability claims. uniQure will keep UC informed of its defense of any claims pursuant to this Section 9.8, and UC will cooperate reasonably in any such suit. If UC invokes the provisions of this Section 9.8, UC will not make any admissions or take any actions in such claim or suit that may prejudice or impair uniQure's ability to defend such claim or suit without uniQure's prior written consent, and uniQure will not admit liability or wrongdoing on behalf of UC without UC's prior written consent.

9.9 Insurance. Each Party shall procure and maintain insurance or self-insurance, including general liability insurance and product liability insurance, adequate to cover its obligations hereunder and that are consistent with normal business practices of prudent companies similarly situated, at all times during which any Research Construct, Royalty Bearing Construct, or Royalty Bearing Product is being Developed, clinically tested in human subjects or Commercialized by or on behalf of such Party, its Affiliates or sublicensees, including, in the case of uniQure, its Sublicensees. It is understood that any such insurance or self-insurance shall not be construed to create a limit of a Party's liability with respect to its indemnification obligations under this ARTICLE IX. Each Party shall provide the other Party with written evidence of such insurance or self-insurance upon request. Each Party shall provide the other Party with written notice at least [\*\*\*] ([\*\*\*)] days prior to the cancellation, non-renewal or material change in such insurance or self-insurance which could adversely affect rights hereunder. Without limiting the generality of the foregoing:

(a) uniQure, at its sole cost and expense, will ensure that the applicable entity performing activities in connection with any work performed hereunder, whether uniQure, an Affiliate, or a Sublicensee, will obtain, keep in force, and maintain the following insurance:

(i) prior to the start of Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included) with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

(ii) Upon the start of any Clinical Trials of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

(iii) Upon the First Commercial Sale of a UC Product, commercial form general liability insurance (contractual liability included), and product liability insurance if not otherwise included, with limits as follows:

Each Occurrence	\$[***]
Products/Completed Operations Aggregate	\$[***]
Personal and Advertising Injury	\$[***]
General Aggregate	\$[***]

If the above insurance is written on a claims-made form, it shall continue for [\*\*\*] ([\*\*\*)] years following termination or expiration of this Agreement.

(iv) worker's compensation as legally required in the jurisdiction in which uniQure, an Affiliate, or a Sublicensee, as applicable, is doing business.

uniQure will promptly notify UC of any material reduction in the insurance coverages below the amounts required hereunder.

(b) Within [\*\*\*] ([\*\*\*)] days after the Effective Date, uniQure will furnish 4DMT with certificates of insurance evidencing compliance with all requirements. Such certificates will:

(i) where possible, provide for [\*\*\*] ([\*\*\*)] days' ([\*\*\*] ([\*\*\*)] days for non-payment of premium) advance written notice to 4DMT and UC of any cancellation of insurance coverages described above in Section 9.9(a);

(ii) indicate that 4DMT and UC have been endorsed as additional insureds under the coverage described above in Section 9.9(a); and

(iii) include a provision that the coverages described above in Section 9.9(a) will be primary and will not participate with, nor will be excess over, any valid and collectable insurance or program of self-insurance maintained by 4DMT or UC.

9.10 No Consequential or Punitive Damages. EXCEPT WITH RESPECT TO (a) THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER THIS AGREEMENT WITH RESPECT TO THIRD PARTY CLAIMS, (b) A BREACH OF THE CONFIDENTIALITY OBLIGATIONS OF ARTICLE VIII, (c) A BREACH OF SECTION 5.6, OR (d) A PARTY'S WILLFUL MISCONDUCT, NEITHER PARTY HERETO WILL BE LIABLE FOR INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL, EXEMPLARY OR PUNITIVE DAMAGES, INCLUDING LOST PROFITS, ARISING FROM OR RELATING TO THIS AGREEMENT, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES.

## ARTICLE X

### TERM AND TERMINATION

10.1 Term and Expiration. This Agreement shall be effective as of the Effective Date and unless terminated earlier pursuant to Section 10.2, this Agreement shall continue in effect until the expiration of all of uniQure's and 4DMT's payment obligations hereunder (the "Term"). Upon expiration, all licenses granted hereunder shall be fully paid-up, perpetual and irrevocable.

#### 10.2 Termination.

##### (a) Termination of Agreement for Cause.

(i) This Agreement may be terminated at any time during the Term upon written notice by either Party (the "Non-Defaulting Party") upon Default of the other Party (the "Defaulting Party"), which Default remains uncured for ninety (90) days after written notice requesting cure of such Default. The Non-Defaulting Party shall provide written notice to the Defaulting Party, which notice shall identify the Default, the intent to so terminate and the actions or conduct that it considers would be an acceptable cure of such Default. If the Defaulting Party disputes the Default under this Section 10.2(a), then the issue of whether the Non-Defaulting Party may properly terminate this Agreement on expiration of the applicable cure period shall be resolved in accordance with ARTICLE XI. If, as a result of such dispute resolution process, it is determined that the alleged Defaulting Party committed a Default and the Defaulting Party does not cure such Default within sixty (60) days after the date of such dispute resolution award (the "Additional Cure Period"), then such termination shall be effective as of the expiration of the Additional Cure Period. If the Parties dispute whether such Default was so cured, either Party alone may request the same tribunal to determine whether it was so cured, and the Parties shall cooperate to allow such determination to be made within thirty (30) days after such request by either Party. Any such dispute resolution proceeding does not suspend any obligation of either Party hereunder, and each Party shall use reasonable efforts to mitigate any damage. If, as a result of any such dispute resolution proceeding, it is determined that the alleged Defaulting Party did not commit such Default (or such Default was cured in accordance with this Section 10.2(a)), then no termination shall be effective, and this Agreement shall continue in full force and effect.

Notwithstanding the foregoing, if the claimed Default relates to one or more Royalty-Bearing Constructs or Royalty-Bearing Products, and not this entire Agreement, then this Agreement shall be terminated only with respect to the Indication for which such Royalty-Bearing Construct(s) or Royalty-Bearing Product(s) were intended to treat and if uniQure was the Defaulting Party then additionally such Indication shall be removed from the Field.

(b) Termination for Bankruptcy. To the extent allowed under applicable Law, either Party shall have the right to terminate this Agreement in the event of the commencement of any proceeding in or for bankruptcy, insolvency, dissolution or winding up by or against the other Party (other than pursuant to a corporate restructuring) that is not dismissed or otherwise disposed of within sixty (60) days thereafter.

(c) Termination for Futility. uniQure shall have the right terminate this Agreement immediately upon written notice to 4DMT summarizing the basis for such termination if, at any point prior to the first (1<sup>st</sup>) anniversary of the Effective Date, the JRSC determines that (i) it would be futile to continue the Research Program, including if the JRSC determines that any Delivery Success Criteria cannot be met through use of the 4DMT Intellectual Property following the reasonable efforts of 4DMT to achieve such Delivery Success Criteria or (ii) 4DMT is not making *bona fide* efforts to achieve the timelines set forth in the Research Plan.

(d) Termination for Convenience. uniQure shall have the right terminate this Agreement at any time after the Research Term, for any reason or for no reason, by giving 4DMT ninety (90) days' prior written notice thereof.

### 10.3 Effect of Termination

(a) If uniQure terminates this Agreement under Section 10.2(a) or Section 10.2(b):

(i) uniQure's licenses pursuant to this Agreement shall continue; provided however that uniQure shall continue to fulfill uniQure's payment obligations with respect to royalties and Sublicense Consideration under ARTICLE VI; and provided further that uniQure may reduce such payment obligations by the amount of monetary damage suffered by uniQure as a direct result of 4DMT's Default, as determined (A) in a final decision of the arbitrators in accordance with Section 11.2 or, with respect to an Excluded Claim, a court of competent jurisdiction, which decision is not appealable or has not been appealed within the time allowed for appeal, or (B) by the Parties in a settlement agreement;

(ii) 4DMT shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination of this Agreement;

(iii) For clarity, uniQure shall be released of its ongoing diligence obligations under Section 4.4 (if any) and uniQure and 4DMT shall be released of their disclosure and information exchange obligations under ARTICLE III and ARTICLE IV;

- (iv) For clarity, the JRSC and its subcommittees shall not meet anymore; and

Notwithstanding the foregoing, if such termination is under Section 10.2(a) solely with respect to one or more given Indication(s), then uniQure's licenses pursuant to Section 5.1 will not terminate but the Field is automatically narrowed to exclude the relevant Indication(s); the license granted to 4DMT under Section 5.2(b) shall be automatically adjusted to include the relevant Indication(s) rather than all fields of use; and uniQure's obligations under subsection (ii) shall be limited to copies of 4DMT's Confidential Information and 4DMT Intellectual Property and Materials that relate solely to the relevant Indication(s).

(b) Upon termination of this Agreement by uniQure under Section 10.2(c) or Section 10.2(d), or by 4DMT under Section 10.2(a) or Section 10.2(b):

(i) For clarity, uniQure's licenses pursuant to Section 5.1 and Step-In Rights under Section 4.4 shall terminate as of the effective date of such termination;

(ii) Effective as of the effective date of such termination, the license granted to 4DMT under Section 5.2(b) shall be automatically expanded to include the New Capsid Variants and all fields of use;

(iii) uniQure shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to 4DMT, copies of all 4DMT's Confidential Information and 4DMT Intellectual Property and all Materials provided by 4DMT; except that uniQure may retain one copy of the 4DMT Confidential Information solely for legal archive purposes;

(iv) 4DMT shall, within [\*\*\*] ([\*\*\*)] days after the effective date of such termination, return or cause to be returned to uniQure, copies of all uniQure's Confidential Information and uniQure Intellectual Property and all Materials provided by uniQure, except that 4DMT may retain one copy of uniQure's Confidential Information solely for legal archive purposes and to exercise the licenses granted to 4DMT which survive termination or are granted upon termination of this Agreement; and

(v) For a period of [\*\*\*] ([\*\*\*)] months, if termination occurs after Regulatory Approval of Royalty Bearing Products, uniQure and its Affiliates shall be entitled to finish work in progress and to sell any of the Royalty Bearing Products remaining in inventory in accordance with the terms of this Agreement to the extent such Royalty Bearing Products were being sold in the Territory at the time of termination, provided that such sales shall be subject to the royalty provisions of this Agreement.

#### 10.4 Effect of Expiration or Termination; Survival.

(a) Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination. Any expiration or termination of this Agreement shall be without prejudice to the rights of either Party against the other accrued or accruing under this Agreement prior to expiration or termination, including the obligation to pay royalties for Royalty Bearing Product(s) sold prior to such expiration or



termination. Termination of this Agreement shall be in addition to, and shall not prejudice, the Parties' remedies at law or in equity, including the Parties' ability to receive legal damages or equitable relief with respect to any breach of this Agreement, regardless of whether or not such breach was the reason for the termination.

(b) The provisions of ARTICLE I, ARTICLE VII (but not Sections 7.4(c) nor 7.6-7.9), ARTICLE VIII, ARTICLE XI, ARTICLE XII, and Sections 4.5, 5.2(b), 5.4, 5.5, 5.6, 9.4, 9.5, 9.6, 9.7, 9.8, 9.9, 9.10, 10.3 and 10.4 shall survive any expiration or termination of this Agreement, and with respect to those Royalty Bearing Products in such countries for which uniQure retains a Development and Commercialization license after the expiration or termination of this Agreement, the provisions of ARTICLE VI shall also survive as to uniQure Products in such countries.

## **ARTICLE XI**

### **DISPUTE RESOLUTION**

11.1 Seeking Consensus. If any dispute arises out of, in connection with or related to this Agreement, including disputes over the interpretation, performance, enforcement or breach of this Agreement, including any dispute that is not within the jurisdiction of the JRSC, (a "Dispute"), excluding any dispute resolved in accordance with Section 2.3(c) (subject to Section 2.3(d)), then upon the written request of either Party, the matter shall be referred to the Executives, who shall meet in a good faith effort to resolve the dispute within [\*\*\*] ([\*\*\*]) days. If the Parties' Executives cannot agree on a resolution of the Dispute within such [\*\*\*] ([\*\*\*]) day period, then it shall be resolved pursuant to the remaining provisions of this ARTICLE XI.

11.2 Arbitration. If the Parties do not fully settle a Dispute pursuant to Section 2.3 (only as to those matters that may be referred to arbitration) or 11.1, as applicable, and a Party wishes to pursue the matter, each such Dispute that is not an Excluded Claim (as defined below) shall be finally resolved by binding arbitration in accordance with the Rules of Arbitration of the ICC (International Chamber of Commerce) and judgment on the arbitration award may be entered in any court having jurisdiction thereof.

(a) The arbitration shall be conducted by a panel of three (3) persons. Within [\*\*\*] ([\*\*\*]) days after initiation of arbitration, each Party shall select one person to act as arbitrator and the two Party-selected arbitrators shall select a third arbitrator within [\*\*\*] ([\*\*\*]) days after their appointment. If the arbitrators selected by the Parties are unable or fail to agree upon the third arbitrator, the third arbitrator shall be appointed by the ICC. The place of arbitration shall be New York City, New York, and all proceedings and communications shall be in English.

(b) Either Party may apply to the arbitrators for interim injunctive relief until the arbitration award is rendered or the Dispute is otherwise resolved. Either Party also may, without waiving any remedy under this Agreement, seek from any court having jurisdiction any injunctive or provisional relief necessary to protect the rights or property of that Party pending the arbitration award. The scope of the authority of the arbitrators shall be limited to the strict application of law. The arbitrators shall have no authority to award punitive or any other type of damages not measured by a Party's compensatory damages, except as permitted by Section 9.10.

Each Party participating in an arbitration pursuant to the terms of this Agreement shall, [\*\*\*]. The arbitrators shall have the power to award recovery of all costs (including reasonable attorney's fees, administrative fees, arbitrators' fees and court costs) to the prevailing Party.

(c) Neither Party shall be required to give general discovery of documents, but may be required to produce documents or testimony that are relevant or considered relevant by the arbitrators to the Dispute. It is the objective and intent of the Parties that any arbitration proceeding be conducted in such a manner that a decision will be rendered by the arbitrators within [\*\*\*] ([\*\*\*) days after the third arbitrator is appointed to the panel, and the Parties and the panel selected in the manner provided above will adopt rules and procedures intended to implement such objective and intent.

(d) Except to the extent necessary to confirm or vacate an award or as may be required by Law (including applicable securities laws or the rules of any stock exchange on which a Party's securities may then be listed), neither a Party nor an arbitrator may disclose the existence, content, or results of arbitration without the prior written consent of both Parties. In no event shall arbitration be initiated after the date when commencement of a legal or equitable proceeding based on the dispute, controversy or claim would be barred by the applicable New York statute of limitations.

(e) The Parties agree that any payment made pursuant to this Agreement pending resolution of the Dispute shall be refunded or credited if the arbitrators or court determines that such payments are not due.

As used in this Section 11.2, the term "Excluded Claim" shall mean a Dispute that concerns (a) the validity, enforceability, scope or infringement of a patent, trademark or copyright; or (b) any antitrust, anti-monopoly or competition law or regulation, whether or not statutory.

## ARTICLE XII

### MISCELLANEOUS

12.1 Governing Law. This Agreement shall be governed by and construed in accordance with the Laws of the State of New York, other than any principle of conflict or choice of laws that would cause the application of the Laws of any other jurisdiction.

12.2 Waiver. Waiver by a Party of a breach hereunder by the other Party shall not be construed as a waiver of any succeeding breach of the same or any other provision. No delay or omission by a Party to exercise or avail itself of any right, power or privilege that it has or may have hereunder shall operate as a waiver of any right, power or privilege by such Party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the Party granting the waiver.

12.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address specified in this Section 12.3 and shall be: (a) delivered personally; (b) transmitted by facsimile; (c) sent by registered or certified mail, return receipt requested, postage prepaid; or (d) sent via a reputable international

overnight delivery service. Any such notice, instruction or communication shall be deemed to have been delivered (i) upon receipt if delivered by hand, (ii) when transmitted with electronic confirmation of receipt, if transmitted by facsimile (if such transmission is on a Business Day; otherwise, on the next Business Day following such transmission), provided that an original document is sent via an internationally recognized overnight delivery service (receipt requested), (iii) three (3) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, or (iv) one (1) Business Day after it is sent via a reputable international overnight delivery service.

If to 4DMT, to: 4D Molecular Therapeutics, Inc.  
5858 Horton St  
Emerystation North, Suite 460  
Emeryville, CA 94608  
Attention: CEO  
Facsimile:

with a copy to: Latham & Watkins LLP  
140 Scott Drive  
Menlo Park, CA 94025  
Attention: [\*\*\*]  
Facsimile: [\*\*\*]

And a required email [\*\*\*]  
copy to:

If to uniQure, to: uniQure biopharma B.V.  
P.O. Box 22506  
1100 DA Amsterdam  
The Netherlands  
Attention: CEO  
Facsimile: [\*\*\*]

with a copy to: [\*\*\*]

or to such other address as the Party to whom notice is to be given may have furnished to the other Party in writing in accordance herewith.

12.4 Entire Agreement; Amendment. This Agreement (including its Exhibits and Schedules) contains the complete understanding of the Parties with respect to the subject matter hereof and supersedes all prior understandings and writings relating to such subject matter. In particular, it supersedes and replaces the Prior Confidentiality Agreement and any and all term sheets relating to the transactions contemplated by this Agreement and exchanged between the Parties or their Affiliates prior to the Effective Date. No amendment, change or addition to this Agreement will be effective or binding on either Party unless reduced to writing and duly executed on behalf of both Parties.

12.5 Headings. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement.

12.6 Severability. If any provision or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement shall be construed as if such clause or portion thereof had never been contained in this Agreement, and there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by applicable Law.

12.7 Assignment. Neither this Agreement nor any right or obligation hereunder may be assigned or otherwise transferred by any Party without the consent of the other Party; provided, however, that any Party may, without such consent, assign this Agreement, in whole or in part: (a) to any of its respective Affiliates; provided that the assigning Party shall remain jointly and severally liable with such Affiliate in respect of all obligations so assigned, or (b) to any successor in interest by way of merger, acquisition or sale of all or substantially all of its assets to which this Agreement relates (an "M&A Event"). Any assignment not in accordance with this Section 12.7 shall be void. Each Party agrees that, notwithstanding any provision of this Agreement to the contrary, neither the assignment of this Agreement by a Party in connection with an M&A Event, nor the occurrence of such M&A Event (whether or not a formal assignment of this Agreement occurs), shall provide the non-assigning Party with rights or access to any intellectual property or technology of the acquirer of the assigning Party or its Affiliates that were not Affiliates of the assigning Party prior to such M&A Event. If uniQure assigns its rights and obligations hereunder to an Affiliate or Third Party outside the United States or The Netherlands pursuant to this Section 12.7, and if such Affiliate or Third Party shall be required by applicable Law to withhold additional taxes from or in respect of any amount payable under this Agreement as a result of such assignment, then any such amount payable under this Agreement shall be increased to take into account the additional taxes withheld as may be necessary so that, after making all required withholdings, 4DMT receives an amount equal to the sum it would have received had no such assignment been made.

12.8 Counterparts. This Agreement may be executed in any number of counterparts, each of which shall be deemed an original but all of which together shall constitute one and the same instrument. Signatures provided by facsimile transmission or in Adobe Portable Document Format (PDF) sent by electronic mail shall be deemed to be original signatures.

12.9 Force Majeure. No Party shall be liable for failure of or delay in performing obligations (other than payment obligations) set forth in this Agreement, and no Party shall be deemed in breach of its obligations, if such failure or delay is due to a natural disaster, explosion, fire, flood, tornado, thunderstorm, hurricane, earthquake, war, terrorism, riot, embargo, loss or shortage of power, labor stoppage, substance or material shortage, events caused by reason of laws of any Governmental Authority, events caused by acts or omissions of a Third Party or any other cause reasonably beyond the control of such Party, if the Party affected gives prompt notice of any such cause to the other Party. The Party giving such notice shall thereupon be excused from such of its obligations hereunder as it is thereby disabled from performing for so long as it

is so disabled, provided, however, that such affected Party commences and continues to use its Commercially Reasonable Efforts to cure such cause.

12.10 Third Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, other than a 4DMT Indemnatee under Section 9.5 or uniQure Indemnatee under Section 9.6. No such Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against either Party.

12.11 Relationship of the Parties. Each Party shall bear its own costs incurred in the performance of its obligations hereunder without charge or expense to the other, except as expressly provided in this Agreement. Neither Party shall have any responsibility for the hiring, termination or compensation of the other Party's employees or for any employee compensation or benefits of the other Party's employees. No employee or representative of a Party shall have any authority to bind or obligate the other Party for any sum or in any manner whatsoever, or to create or impose any contractual or other liability on the other Party without said other Party's approval. For all purposes, and notwithstanding any other provision of this Agreement to the contrary, the legal relationship under this Agreement of each Party to the other Party shall be that of independent contractor. Nothing in this Agreement shall be construed to establish a relationship of partners or joint ventures between the Parties.

12.12 Performance by Affiliates. To the extent that this Agreement imposes obligations on Affiliates of a Party or permits a Party to exercise its rights or perform its obligations through its Affiliates, such Party agrees to cause its Affiliates to perform such obligations and shall guarantee performance of this Agreement by its Affiliates. If any disagreement arises out of the performance of this Agreement by an Affiliate of a Party, or the alleged failure of an Affiliate to comply with the conditions and obligations of this Agreement, the Party seeking to resolve such dispute shall have the right do so directly with the other Party, without any obligation to first pursue an action against, or recovery from, the Affiliate which is alleged to have caused a breach of this Agreement.

12.13 Construction. Each Party acknowledges that it has been advised by counsel during the course of negotiation of this Agreement, and, therefore, that this Agreement shall be interpreted without regard to any presumption or rule requiring construction against the Party causing this Agreement to be drafted. Any reference in this Agreement to an ARTICLE, Section, subsection, paragraph, clause, or Schedule shall be deemed to be a reference to any article, section, subsection, paragraph, clause, schedule or exhibit, of or to, as the case may be, this Agreement. Except where the context otherwise requires, (a) wherever used, the use of any gender will be applicable to all genders; (b) the word "or" is used in the inclusive sense (and/or); (c) any definition of or reference to any agreement, instrument or other document refers to such agreement, instrument other document as from time to time amended, supplemented or otherwise modified (subject to any restriction on such amendments, supplements or modifications set forth herein or therein); (d) any reference to any Law refers to such Law as from time to time enacted, repealed or amended; (e) the words "herein", "hereof" and "hereunder", and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof; and (f) the words "include", "includes" and "including" shall be deemed to be followed by the phrase "but not limited to", "without limitation" or words of similar import.

[Signature page follows]

**IN WITNESS WHEREOF**, the Parties have executed this Collaboration and License Agreement as of the New CLA Effective Date.

**UNIQUE BIOPHARMA B.V.**

**4D MOLECULAR THERAPEUTICS, INC.**

BY: s/s Lilly Burggraaf  
NAME: Lilly Burggraaf  
TITLE: Vice President, Global Human Resources

BY: s/s David Kirn  
NAME: David Kirn, MD  
TITLE: Chief Executive Officer

**SCHEDULE 1**  
**DRAFT INVOICE**  
[\*\*\*]

SCHEDULE 1.47 - Page 1

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**SCHEDULE 2**  
**RESEARCH PLAN**

**[\*\*\*]**

SCHEDULE 1.66- Page 1

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**SCHEDULE 3**

**SELECTED CAPSID VARIANTS**

**[\*\*\*]**

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**Certification of Chief Executive Officer**

I, Matthew Kapusta, certify that:

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

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta  
Chief Executive Officer  
October 28, 2019

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**Certification of Chief Financial Officer**

I, Matthew Kapusta, certify that:

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

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta  
*Principal Financial Officer*  
October 28, 2019

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

In connection with this Quarterly Report of uniQure N.V. (the "Company") on Form 10-Q for the period ended September 30, 2019, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew Kapusta, Chief Executive Officer and Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

1       the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934;  
and

2       the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

By: /s/ MATTHEW KAPUSTA

Matthew Kapusta  
Chief Executive Officer and  
Chief Financial Officer  
October 28, 2019

*A signed original of this written statement required by Section 906 has been provided to uniQure N.V. and will be retained by uniQure N.V. and furnished to the SEC or its staff upon request.*

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