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, 2023

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 001-39782

4D Molecular Therapeutics, Inc.

(Exact name of registrant as specified in its Charter)

Delaware	**47-3506994**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
5858 Horton Street #455 Emeryville, CA	**94608**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (510) 505-2680

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

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

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

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

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☒		

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

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

As of November 2, 2023, there were 42,753,607 shares of 4D Molecular Therapeutics, Inc.'s common stock outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements concerning our business, operations and financial performance and condition, as well as our plans, objectives and expectations for our business operations and financial performance and condition. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “predict,” “potential,” “positioned,” “seek,” “should,” “target,” “will,” “would” and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the success, cost and timing of our development activities, preclinical studies and clinical trials, including our clinical trials for 4D-150, 4D-710, 4D-310, 4D-125 and 4D-110;
- the number, size and design of our planned clinical trials, and what regulatory authorities may require to obtain marketing approval;
- the timing of Investigational New Drug Application (“IND”) enabling studies and results from such studies;
- the timing and success of lead optimization for our product candidates in lead optimization;
- the translation of our preclinical results and data into future clinical trials in humans;
- the timing of any manufacturing runs for materials to be used in patient trials;
- the timing or likelihood of regulatory filings and approvals;
- our ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of any approved product candidate;
- our ability to obtain funding for our operations, including funding necessary to develop and commercialize our product candidates;
- the rate and degree of market acceptance of our product candidates, if approved;
- the success of competing products or platform technologies that are or may become available;
- our plans and ability to establish sales, marketing and distribution infrastructure to commercialize any product candidates for which we obtain approval;
- future agreements with third parties in connection with the commercialization of our product candidates;
- the size and growth potential of the markets for our product candidates, if approved for commercial use, and our ability to serve those markets;
- existing regulations and regulatory developments in the United States and foreign countries;
- the expected potential benefits of strategic collaboration agreements, including our relationships with Astellas Gene Therapies, Inc., uniQure biopharma B.V. and Cystic Fibrosis Foundation, and our ability to attract collaborators with development, regulatory and commercialization expertise;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- potential claims relating to our intellectual property and third-party intellectual property;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- the pricing and reimbursement of our product candidates, if approved;

- the potential effects of public health emergencies, including the COVID-19 pandemic, to our preclinical and clinical programs and our business;
- our ability to attract and retain key managerial, scientific and medical personnel;
- the accuracy of our estimates regarding expenses, capital requirements and needs for additional financing;
- our financial performance; and
- our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act.

These forward-looking statements are based on management's current expectations, estimates, forecasts and projections about our business and the industry in which we operate and management's beliefs and assumptions and are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. As a result, any or all of our forward-looking statements in this Quarterly Report on Form 10-Q may turn out to be inaccurate. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section titled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. Potential investors are urged to consider these factors carefully in evaluating the forward-looking statements. These forward-looking statements speak only as of the date of this Quarterly Report on Form 10-Q. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future. You should, however, review the factors and risks we describe in the reports we will file from time to time with the SEC after the date of this Quarterly Report on Form 10-Q.

4D Molecular Therapeutics, Inc.

Condensed Balance Sheets (Unaudited)
(In thousands, except share and per share amounts)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 275,678	$ 52,351
Marketable securities	43,986	161,203
Prepaid expenses and other current assets	8,579	6,957
Total current assets	328,243	220,511
Marketable securities, long-term	—	4,908
Property and equipment, net	20,738	22,262
Operating lease right-of-use assets, net	11,950	13,085
Other assets	682	1,080
Total assets	$ 361,613	$ 261,846
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 4,713	$ 3,322
Accrued and other current liabilities	10,194	8,870
Deferred revenue	130	884
Operating lease liabilities, current portion	3,126	2,655
Total current liabilities	18,163	15,731
Deferred revenue, net of current portion	1,096	1,076
Derivative liability	369	212
Operating lease liabilities, long-term portion	12,031	13,469
Other liabilities	20	21
Total liabilities	31,679	30,509
Commitments and contingencies (Note 9)		
Stockholders' equity		
Preferred stock, $0.0001 par value, 10,000,000 shares authorized at September 30, 2023 and December 31, 2022; no shares issued and outstanding at September 30, 2023 and December 31, 2022	—	—
Common stock, $0.0001 par value, 300,000,000 shares authorized at September 30, 2023 and December 31, 2022; 42,752,452 and 32,626,627 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	4	3
Additional paid-in-capital	712,440	547,020
Accumulated other comprehensive gain (loss)	534	(1,196)
Accumulated deficit	(383,044)	(314,490)
Total stockholders' equity	329,934	231,337
Total liabilities and stockholders' equity	$ 361,613	$ 261,846

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

4D Molecular Therapeutics, Inc.

Condensed Statements of Operations (Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Revenue:				
Collaboration and license revenue	$ 20,204	$ 500	$ 20,742	$ 1,882
Operating expenses:				
Research and development (includes $0 for each of the three months ended September 30, 2023 and 2022, respectively, and $0 and $134 for the nine months ended September 30, 2023 and 2022, respectively, attributable to related parties)	25,066	18,940	71,068	58,753
General and administrative	9,112	8,055	25,889	24,441
Total operating expenses	34,178	26,995	96,957	83,194
Loss from operations	(13,974)	(26,495)	(76,215)	(81,312)
Other income (expense):				
Interest income	3,876	795	7,831	1,235
Other income (expense), net	(158)	9	(170)	(38)
Total other income (expense), net	3,718	804	7,661	1,197
Net loss	$ (10,256)	$ (25,691)	$ (68,554)	$ (80,115)
Net loss per share, basic and diluted	$ (0.24)	$ (0.79)	$ (1.81)	$ (2.48)
Weighted-average shares outstanding used in computing net loss per share, basic and diluted	42,256,629	32,385,791	37,884,363	32,305,074

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

4D Molecular Therapeutics, Inc.

Condensed Statements of Comprehensive Loss (Unaudited)
(In thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Net loss	$ (10,256)	$ (25,691)	$ (68,554)	$ (80,115)
Other comprehensive loss:				
Net unrealized gain (loss) on marketable securities	189	(24)	1,730	(1,428)
Total comprehensive loss	$ (10,067)	$ (25,715)	$ (66,824)	$ (81,543)

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

4D Molecular Therapeutics, Inc.

Condensed Statements of Stockholders' Equity (Unaudited)
(In thousands, except share amounts)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Gain (Loss)	Deficit	Equity
Balances at December 31, 2022	32,626,627	$ 3	$ 547,020	$ (1,196)	$ (314,490)	$ 231,337
Issuance of common stock upon exercise of stock options	122,207	—	1,135	—	—	1,135
Issuance of common stock for the ATM offering program, net of issuance costs	487,934	—	9,647	—	—	9,647
Stock-based compensation expense	—	—	4,221	—	—	4,221
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized gain on marketable securities	—	—	—	926	—	926
Net loss	—	—	—	—	(28,682)	(28,682)
Balances at March 31, 2023	33,236,768	$ 3	$ 562,045	$ (270)	$ (343,172)	$ 218,606
Issuance of common stock upon exercise of stock options	72,217	—	894	—	—	894
Issuance of common stock upon public offering, net of issuance costs	8,625,000	1	129,207	—	—	129,208
Issuance of common stock - 2020 ESPP	82,637	—	658	—	—	658
ATM offering costs	—	—	(212)	—	—	(212)
Stock-based compensation expense	—	—	4,969	—	—	4,969
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized gain on marketable securities	—	—	—	615	—	615
Net loss	—	—	—	—	(29,616)	(29,616)
Balances at June 30, 2023	42,016,622	$ 4	$ 697,583	$ 345	$ (372,788)	$ 325,144
Issuance of common stock upon exercise of stock options	125,152	—	1,040	—	—	1,040
Issuance of common stock for the ATM offering program	610,678	—	9,701	—	—	9,701
ATM offering costs	—	—	(468)	—	—	(468)
Stock-based compensation expense	—	—	4,562	—	—	4,562
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized gain on marketable securities	—	—	—	189	—	189
Net loss	—	—	—	—	(10,256)	(10,256)
Balances at September 30, 2023	42,752,452	$ 4	$ 712,440	$ 534	$ (383,044)	$ 329,934

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Loss	Deficit	Equity
Balances at December 31, 2021	32,224,524	$ 3	$ 526,523	$ (423)	$ (206,996)	$ 319,107
Issuance of common stock upon exercise of stock options	39,541	—	330	—	—	330
Stock-based compensation expense	—	—	3,933	—	—	3,933
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized loss on marketable securities	—	—	—	(998)	—	(998)
Net loss	—	—	—	—	(26,338)	(26,338)
Balances at March 31, 2022	32,264,065	$ 3	$ 530,808	(1,421)	$ (233,334)	$ 296,056
Issuance of common stock upon exercise of stock options and warrants	51,261	—	138	—	—	138
Stock-based compensation expense	—	—	4,322	—	—	4,322
Issuance of common stock - 2020 ESPP	61,552	—	471	—	—	471
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized loss on marketable securities	—	—	—	(406)	—	(406)
Net loss	—	—	—	—	(28,086)	(28,086)
Balances at June 30, 2022	32,376,878	$ 3	$ 535,761	(1,827)	$ (261,420)	$ 272,517
Issuance of common stock upon exercise of stock options	15,500	—	100	—	—	100
Stock-based compensation expense	—	—	4,520	—	—	4,520
Vesting of common stock warrants issued for services	—	—	22	—	—	22
Net unrealized loss on marketable securities	—	—	—	(24)	—	(24)
Net loss	—	—	—	—	(25,691)	(25,691)
Balances at September 30, 2022	32,392,378	$ 3	$ 540,403	$ (1,851)	$ (287,111)	$ 251,444

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

4D Molecular Therapeutics, Inc.

Condensed Statements of Cash Flows (Unaudited)
(In thousands)

	Nine Months Ended September 30,	
	2023	**2022**
Cash flows from operating activities		
Net loss	$ (68,554)	$ (80,115)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	13,752	12,775
Vesting of common stock warrants in return for services	66	66
Change in fair value of derivative liability	157	(1)
Depreciation and amortization	3,102	1,389
Amortization of right-of-use assets	1,134	1,103
Net amortization (accretion) of premium (discount) on marketable securities	(765)	1,290
Changes in operating assets and liabilities		
Accounts receivable	—	47
Prepaid expenses and other current assets	(1,622)	3,693
Other assets	(69)	(451)
Accounts payable	1,984	(3,151)
Accrued and other liabilities	1,374	662
Deferred revenue	(734)	(1,882)
Operating lease liabilities	(967)	104
Net cash used in operating activities	(51,142)	(64,471)
Cash flows from investing activities		
Purchases of marketable securities	(20,156)	(253,821)
Maturities of marketable securities	144,777	282,993
Acquisition of property and equipment	(2,222)	(10,443)
Net cash provided by investing activities	122,399	18,729
Cash flows from financing activities		
Issuance of common stock upon the exercise of stock options and warrants	3,069	568
Issuance of common stock for the ATM offering program, net of issuance costs	19,135	—
Issuance of common stock upon public offering, net of issuance costs	129,208	—
Issuance of common stock - 2020 ESPP	658	471
Net cash provided by financing activities	152,070	1,039
Net increase (decrease) in cash and cash equivalents	223,327	(44,703)
Cash and cash equivalents, beginning of period	52,351	153,001
Cash and cash equivalents, end of period	$ 275,678	$ 108,298
Supplemental disclosures of noncash investing and financing information		
Deferred ATM costs reclassification	$ 468	$ —
Purchases of property and equipment in accounts payable and accrued and other liabilities	$ 345	$ 297

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

1. The Company

4D Molecular Therapeutics, Inc. (the “Company”) was formed as a limited liability company in September 2013 under the name 4D Molecular Therapeutics, LLC. The Company changed its name and converted into a corporation which was incorporated in the state of Delaware in March 2015. The Company is a clinical-stage biotherapeutics company harnessing the power of directed evolution for targeted genetic medicines.

Initial Public Offering

In December 2020, the Company sold and issued 9,660,000 shares of common stock at a price to the public of $23.00 per share, which included shares sold upon the underwriters’ exercise of their overallotment option to purchase 1,260,000 additional shares. The Company received an aggregate of $204.7 million in net proceeds, after deducting underwriting discounts and commissions and offering costs.

Upon the closing of the Company's initial public offering in December 2020 (the "IPO"), all outstanding shares of redeemable convertible preferred stock automatically converted into 11,575,984 shares of common stock. Subsequent to the closing of the IPO, there were no shares of redeemable convertible preferred stock outstanding.

2021 Follow On Public Offering

In November 2021, the Company completed an underwritten public offering ("2021 Offering") in which 4,750,000 shares of the Company's common stock were sold at an offering price of $25.00 per share pursuant to an effective Registration Statement on Form S-1. The net proceeds from the 2021 Offering were $111.1 million, after deducting underwriting discounts and commissions and estimated offering expenses.

2023 Follow On Public Offering

In May 2023, the Company completed its third underwritten public offering (the “2023 Offering”) in which 8,625,000 shares of the Company’s common stock were sold at an offering price of $16.00 per share pursuant to an effective Registration Statement on Form S-3. The net proceeds from the 2023 Offering were $129.2 million, after deducting underwriting discounts and commissions and offering expenses.

Liquidity

The Company has incurred significant losses and negative cash flows from operations and had an accumulated deficit of $383.0 million as of September 30, 2023. The Company believes that its cash and cash equivalents and marketable securities as of September 30, 2023 are sufficient for the Company to fund planned operations for at least one year from the issuance date of these unaudited condensed financial statements. The Company has historically financed its operations primarily through the sale of equity securities, and to a lesser extent, from cash received pursuant to its collaboration and license agreements. To date, none of the Company’s product candidates have been approved for sale, and therefore, the Company has not generated any revenue from product sales. Management expects operating losses and negative cash flows from operations to continue for the foreseeable future. The Company plans to raise additional funding as required based on the status of its clinical trials and projected cash flows. There can be no assurance that, in the event the Company requires additional financing, such financing will be available on terms acceptable to the Company, if at all. Failure to generate sufficient cash flows from operations, raise additional capital and reduce discretionary spending should additional capital not become available could have a material adverse effect on the Company’s ability to achieve its business objectives.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed financial statements have been prepared in accordance with United States Generally Accepted Accounting Principles ("U.S. GAAP") and the rules and regulations of the United States Securities and Exchange Commission ("SEC") for interim reporting.

Certain information and note disclosures normally included in annual financial statements prepared in accordance with U.S. GAAP have been condensed or omitted. Accordingly, the unaudited condensed 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, 2022 filed with the SEC.

The accompanying financial information for the three and nine months ended September 30, 2023 and 2022 is unaudited. The unaudited condensed financial statements have been prepared on the same basis as the annual audited financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's financial position as of September 30, 2023 and December 31, 2022 and its results of operations and cash flows for the nine months ended September 30, 2023 and 2022. The results for interim periods are not necessarily indicative of the results expected for the full fiscal year or any other periods.

Use of Estimates and Judgments

The preparation of condensed financial statements in conformity with U.S. GAAP requires management to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses; and disclosure of contingent assets and liabilities as of the date of the financial statements. Such estimates include the determination of useful lives for property and equipment, the contract term, transaction price and costs of collaboration agreements, as well as estimates of the fair value of common stock (prior to the IPO), stock options and the derivative instrument and income tax uncertainties. Actual results could differ from those estimates.

Due to the coronavirus ("COVID-19") pandemic, the war in Ukraine, rising interest rates and inflation, there has been uncertainty and disruption in the global economy and financial markets. The Company is not aware of any specific event or circumstance that would require an update to its estimates or judgments or a revision of the carrying value of its assets or liabilities as of September 30, 2023. While there was not a material impact to the Company's unaudited condensed financial statements as of September 30, 2023, these estimates may change, as new events occur and additional information is obtained, as well as other factors that could result in material impacts to the unaudited condensed financial statements in future reporting periods.

Segment Information

The Company operates and manages its business as one reportable and operating segment. The Company's chief executive officer, who is the chief operating decision maker, reviews financial information on a company-wide basis for purposes of allocating resources and assessing financial performance.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents, marketable securities and accounts receivable. The Company's cash is held at two financial institutions in the United States of America. The Company's cash equivalents are invested in money market funds. The Company also invests in U.S. Treasuries, U.S. government sponsored agencies, commercial paper and corporate bonds. The Company has not experienced any losses on its deposits of cash and cash equivalents. Such deposits may, at times, exceed federally insured limits.

The Company's partners in collaboration and license agreements who represent 10% or more of the Company's total revenue are as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Customer A	*	*	*	*
Customer B	*	99%	*	98%
Customer C	99%	*	96%	*
Total	99%	99%	96%	98%

* Less than 10%

The Company did not have accounts receivable from its partners in collaboration and license agreements as of September 30, 2023 and December 31, 2022.

The Company's total revenues by geographic region, based on the location of the customer, are as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Netherlands	$ 41	$ 497	$ 566	$ 1,849
United States	20,163	3	20,176	33
Total revenue	$ 20,204	$ 500	$ 20,742	$ 1,882

Acquisitions

The Company first determines whether a set of assets acquired constitute a business and should be accounted for as a business combination. If the assets acquired do not constitute a business, the Company accounts for the transaction as an asset acquisition where the cost of the acquisition is allocated to the assets acquired and liabilities based on their relative fair values. In-process research and development (IPR&D) projects with no alternative future use are recorded as research and development expense upon acquisition, and contingent consideration obligations incurred in connection with an asset acquisition are recorded when it is probable that they will occur and they can be reasonably estimated. Business combinations are accounted for by means of the acquisition method of accounting. Under the acquisition method, assets acquired, including IPR&D projects, and liabilities assumed are recorded at their respective fair values as of the acquisition date. The excess of the fair value of consideration transferred over the fair value of the net assets acquired is recorded as goodwill.

Other Risks and Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, protection of proprietary technology, dependence on key personnel, suppliers for key raw materials, contract manufacturing organizations ("CMOs") and contract research organizations ("CROs"), compliance with government regulations and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies, clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance and reporting.

There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained or maintained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services

of its employees, consultants and other third parties (including for clinical trials and some aspects of research and preclinical testing).

Recently Adopted Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments-Credit Losses (Topic 326), Measurement of Credit Losses on Financial Instruments, as clarified in subsequent amendments. ASU 2016-13 changes the impairment model for certain financial instruments. The new model is a forward-looking expected loss model and will apply to financial assets subject to credit losses and measured at amortized cost and certain off-balance sheet credit exposures. This includes loans, held-to-maturity debt securities, loan commitments, financial guarantees and net investments in leases, as well as trade receivables. For available-for-sale debt securities with unrealized losses, credit losses will be measured in a manner similar to today, except that the losses will be recognized as allowances rather than reductions in the amortized cost of the securities. In October 2019, the FASB voted to delay the effective date of this standard. Topic 326 will be effective for the Company for fiscal years beginning after December 15, 2022, including interim periods within those fiscal years. Early adoption is permitted. The Company adopted ASU 2016-13 on January 1, 2023 and the adoption did not have a material impact on the Company's condensed consolidated financial statements and related disclosures.

3. Fair Value Measurements

The following tables represent the Company's fair value hierarchy for financial assets and financial liabilities measured at fair value on a recurring basis as of September 30, 2023 and December 31, 2022 (in thousands):

	Basis for Fair Value Measurements			Fair Value as of September 30, 2023
	Level 1	Level 2	Level 3	
Assets				
Money market funds	$ 250,041	$ —	$ —	$ 250,041
Commercial paper	—	24,687	—	24,687
Corporate bonds	—	6,279	—	6,279
U.S. Treasuries	—	4,969	—	4,969
U.S. government sponsored agencies	—	8,051	—	8,051
Total	$ 250,041	$ 43,986	$ —	$ 294,027
Liabilities				
Derivative liability	$ —	$ —	$ 369	$ 369
Total	$ —	$ —	$ 369	$ 369

	Basis for Fair Value Measurements			Fair Value as of December 31, 2022
	Level 1	Level 2	Level 3	
Assets				
Money market funds	$ 44,447	$ —	$ —	$ 44,447
Commercial paper	—	48,872	—	48,872
Corporate bonds	—	47,012	—	47,012
U.S. Treasuries	—	63,059	—	63,059
U.S. government sponsored agencies	—	7,168	—	7,168
Total	$ 44,447	$ 166,111	$ —	$ 210,558
Liabilities				
Derivative liability	$ —	$ —	$ 212	$ 212
Total	$ —	$ —	$ 212	$ 212

Level 3 Inputs

The fair value of the derivative liability is based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the derivative liability was determined using a present value analysis with multiple scenarios. In determining the fair value

of the derivative liability, the inputs impacting fair value include the change of control payment to CFF, the probability of a change of control event, the product status at time of a change of control event and the discount rate. See Note 14 for further discussion on the embedded derivative.

There were no transfers between Level 1, 2 and 3 during the periods presented.

The following tables set forth a summary of the changes in the fair value of the Company's Level 3 financial instrument during the nine-month periods ended September 30, 2023 and 2022 (in thousands):

	Derivative Liability
Balance as of December 31, 2022	$ 212
Change in fair value included in other income (expense), net	157
Balance as of September 30, 2023	$ 369

	Derivative Liability
Balance as of December 31, 2021	$ 214
Change in fair value included in other income (expense), net	(1)
Balance as of September 30, 2022	$ 213

4. Marketable Securities

Marketable securities, which are classified as available-for-sale, consisted of the following as of September 30, 2023 and December 31, 2022 (in thousands):

	Amortized Cost Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value as of September 30, 2023
Short-term marketable securities:				
Commercial paper	$ 24,452	$ 312	$ (77)	$ 24,687
Corporate bonds	6,302	—	(23)	6,279
U.S. treasuries	4,827	142	—	4,969
U.S. government sponsored agencies	7,871	195	(15)	8,051
Total short-term marketable securities	$ 43,452	$ 649	$ (115)	$ 43,986

	Amortized Cost Basis	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value as of December 31, 2022
Short-term marketable securities:				
Commercial paper	$ 43,988	$ —	$ (24)	$ 43,964
Corporate bonds	47,565	—	(553)	47,012
U.S. treasuries	63,502	1	(444)	63,059
U.S. government sponsored agencies	7,185	12	(29)	7,168
Total short-term marketable securities	$ 162,240	$ 13	$ (1,050)	$ 161,203
Long-term marketable securities:				
Commercial paper	$ 5,067	$ —	$ (159)	$ 4,908
Total long-term marketable securities	$ 5,067	$ —	$ (159)	$ 4,908

All marketable securities held as of September 30, 2023 had contractual maturities of less than one year. There have been no material realized gains or losses on marketable securities for the periods presented.

Aggregate fair values of marketable securities with unrealized losses and gains were as follows as of September 30, 2023 and December 31, 2022 (in thousands):

	September 30, 2023	December 31, 2022
Aggregate fair value of marketable securities in a continuous loss position for less than twelve months	$ 12,783	$ 97,472
Aggregate fair value of marketable securities in a continuous loss position for more than twelve months	11,232	53,716
Aggregate fair value of marketable securities in unrealized gain position	19,971	14,923
Total marketable securities	$ 43,986	$ 166,111

The Company manages credit risk associated with its investment portfolio through its investment policy, which limits purchases to high-quality issuers and also limits the amount of its portfolio that can be invested in a single issuer. The Company did not record an allowance for credit losses or other impairment charges related to its marketable securities for any period presented. The Company has determined that (i) it does not have the intent to sell any of these investments, and (ii) it is not more likely than not that it will be required to sell any of these investments before recovery of the entire amortized cost basis. The Company further considered the maximum unrealized loss amounts both at the individual instrument level, $0.1 million, as well as in aggregate, $0.1 million, as of September 30, 2023, as immaterial. These unrealized losses were not attributed to credit risk and were associated with changes in market conditions. The Company periodically reviews its marketable securities for indications of credit losses. The Company anticipates that it will recover the entire amortized cost basis of such securities, and therefore, no credit loss existed as of September 30, 2023.

5. Property and Equipment, Net

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

	September 30, 2023	December 31, 2022
Machinery and equipment	$ 11,995	$ 11,191
Leasehold improvements	16,902	16,902
Furniture and fixtures	566	566
Office equipment	240	239
Computer equipment and software	836	616
Construction in progress	1,030	476
Total property and equipment	31,569	29,990
Less: Accumulated depreciation and amortization	(10,831)	(7,728)
Property and equipment, net	$ 20,738	$ 22,262

All property and equipment are maintained in the United States. Depreciation expense was $1.1 million and $0.5 million for the three months ended September 30, 2023 and 2022, respectively, and $3.1 million and $1.4 million for the nine months ended September 30, 2023 and 2022, respectively.

6. Accrued and Other Current Liabilities

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

	September 30, 2023	December 31, 2022
Payroll and related expenses	$ 6,223	$ 5,689
Accrued clinical and preclinical study costs	1,776	1,355
Consulting and professional	2,032	1,444
Other accrued expenses	163	382
Total accrued and other current liabilities	$ 10,194	$ 8,870

7. Research and Collaboration Arrangements

Collaboration and license revenue for each period was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
uniQure	$ 41	$ 497	$ 566	$ 1,849
CFF	155	3	168	33
Astellas	20,008	—	20,008	—
Total revenue	$ 20,204	$ 500	$ 20,742	$ 1,882

Deferred revenue is summarized as follows (in thousands):

	September 30, 2023	December 31, 2022
uniQure	$ —	$ 566
CFF	1,226	1,394
Total deferred revenue	$ 1,226	$ 1,960

The total amount of revenue in the nine months ended September 30, 2023, which was included in deferred revenue at January 1, 2023, was $0.7 million. The total amount of revenue in the nine months ended September 30, 2022, which was included in deferred revenue at January 1, 2022, was $1.9 million.

uniQure

In January 2014, the Company and uniQure biopharma B.V. (“uniQure”) entered into a Collaboration and License Agreement (the “uniQure Agreement”) to collaborate on the discovery and non-clinical research activities related to the Company’s Therapeutic Vector Evolution platform in order to generate and validate vectors for gene delivery to treat diseases within the central nervous system and liver (together, the “uniQure Field”).

The uniQure Agreement provided uniQure with a research license as well as an exclusive development and commercialization license for each project variant selected for further development. The initial research term is three years with an option for uniQure to extend the research term one time for an additional year. Once the Company’s research plan has concluded, uniQure is solely responsible for the continued development, manufacturing and commercialization of the project variants as potential product candidates. In October 2016, uniQure exercised its option to extend the research term for an additional year to January 2018. The Company was also required to work exclusively with uniQure in the uniQure Field (the “uniQure Exclusivity Clause”).

Pursuant to the uniQure Agreement, the Company received upfront payments of $0.2 million, and was entitled to receive (i) contingent payments for the achievement of research and development milestones of up to $5.0 million for each licensed product selected under the arrangement, and (ii) royalties in the single digit range on future sales of the potential product candidates and sublicense consideration in the low teens to low thirties range on any future sublicensing arrangements. The Company also received capped research and development service fees based on contractual full-time employee rates per year. In connection with the performance obligations under the uniQure Agreement, the founders of 4D Molecular Therapeutics, LLC received equity options to purchase an aggregate of 609,744 of uniQure ordinary shares that vested over the initial three-year term of the agreement.

The upfront payment of $0.2 million was recorded as deferred revenue and was recognized on a ratable basis over the estimated performance period of four years. Payments and reimbursements for research costs were recognized on an as-incurred basis. The options to purchase uniQure shares were deemed to be a noncash component of the arrangement consideration, as the vesting of options is linked to the uniQure Agreement and there is a requirement for the holders of the options to provide services under the agreement. The fair value of the uniQure options, which was estimated to be $10.6 million, was recognized ratably as revenue over the estimated performance period of four years and the associated compensation expense related to the stock options was recorded as research and development expense.

In August 2019, the Company and uniQure entered into an Amended and Restated Collaboration and License Agreement (the “Amended uniQure Agreement”), which amended and restated the uniQure

Agreement, and a separate Collaboration and License Agreement (the “Second uniQure Agreement”). Under these agreements, the Company agreed to transfer incremental rights and services to uniQure in exchange for uniQure eliminating the uniQure Exclusivity Clause and transferring other rights back to the Company.

Under the Amended uniQure Agreement, uniQure continues to have an exclusive license to select AAV capsid variants (the “Selected Variants”) in the uniQure Field. uniQure continues to be solely responsible, at its cost, to develop and commercialize the compounds and products containing the Selected Variants. The amended uniQure Agreement eliminated the uniQure Exclusivity Clause in the uniQure Agreement. Furthermore, the contingent payments that the Company was entitled to from uniQure for the achievement of research and development milestones of up to $5.0 million for each licensed product selected under the uniQure Agreement were eliminated and sublicense consideration on any future sublicensing arrangements was reduced from the low teens to low thirties percentages to mid-single digit to mid-twenties percentages.

Under the Second uniQure Agreement, the parties agreed to research and develop new AAV capsid variants (the “New Variants”) that are not Selected Variants that affect certain targets selected by uniQure (the “uniQure Targets”) in the uniQure Field. The Company is solely responsible, at its cost, for the research of the New Variants. The Company granted uniQure an exclusive license to a certain number of the New Variants (the “uniQure New Variants”) that affect the uniQure Targets. uniQure is solely responsible, at its cost, to develop and commercialize the compounds and products containing the uniQure New Variants that affect the uniQure Targets (the “Licensed Products”). The Company retains all rights to New Variants in the uniQure Field that affect targets other than the uniQure Targets.

Under both the Amended uniQure Agreement and the Second uniQure Agreement, uniQure will be required to pay the Company royalties on worldwide annual net sales of Licensed Products at a mid-single digit percentage rate, subject to certain specified reductions. uniQure will also be required to pay the Company sublicensing consideration for sublicensing the Company’s intellectual property rights licensed under the Amended uniQure Agreement or the Second uniQure Agreement to third parties at a rate between the mid-single digit to mid-twenties. The Company has reciprocal obligations, at the same percentage rates as uniQure, to pay uniQure royalties and sublicensing consideration for sublicensing certain intellectual property rights licensed under the Amended uniQure Agreement or the Second uniQure Agreement to third parties.

The Company concluded that the Amended uniQure Agreement and the Second uniQure Agreement should be accounted for as one combined contract that should be accounted for as a separate contract from the uniQure Agreement given that the incremental licensed intellectual property rights and research and development services are distinct from the rights and services previously transferred to uniQure under the uniQure Agreement and the transaction price increased by an amount that equals the standalone selling price of the incremental rights and services to be transferred to uniQure under the Amended uniQure Agreement and Second uniQure Agreement.

Neither party was required to pay monetary consideration in connection with the execution of the Amended uniQure Agreement or the Second uniQure Agreement or for subsequent performance by the parties under those agreements, notwithstanding the potential future royalty and sublicense consideration described above. The fair value of the non-monetary consideration given by uniQure to the Company, for the intellectual property right is $5.1 million. This intellectual property right is considered to be an in-process research and development asset with no alternative future use and, accordingly, was written off as acquired in-process research and development expense in the year ended December 31, 2019.

The incremental transaction price described in the paragraph above was recorded as deferred revenue given that the Company identified one single combined performance obligation under ASC 606, which includes the licenses to the New Variants, research services and participation in the joint steering committee (“JSC”). Revenue is being recognized using the input method based on actual costs incurred as a percentage of total budgeted costs as the Company completes its performance obligation. The Company completed its performance obligation during the third quarter of 2023 and the deferred revenue was recognized as revenue in the same period.

The Company determined the transaction price using the risk adjusted net present value analysis (“rNPV”) methodology to value the elimination of the uniQure exclusivity clause and other material rights received by the Company, including the potential royalties the Company would receive from uniQure. The rNPVs incorporate estimates and assumptions including the number of products the Company and uniQure

would develop, the risk-adjusted probability of successfully developing a biopharmaceutical product, the probability that uniQure will develop a product, the research and development costs, the potential worldwide sales and associated commercialization costs, corporate tax rate, and discount rate.

The Company recognized an immaterial amount and $0.6 million during the three and nine months ended September 30, 2023, respectively, under both of the Amended uniQure Agreement and the Second uniQure Agreement. During the three and nine months ended September 30, 2022, the Company recognized $0.5 million and $1.8 million, respectively, under both of the Amended uniQure Agreement and the Second uniQure Agreement. During the nine months ended September 30, 2022, the decrease in total budgeted costs for the remaining performance obligation resulted in a $1.8 million increase in revenue recognized in the nine months ended September 30, 2022 related to the performance obligation partially satisfied prior to January 1, 2022. As of September 30, 2023 and December 31, 2022, deferred revenue relating to uniQure was zero and $0.6 million, respectively. There were no amounts due from uniQure under the uniQure Agreement, Amended uniQure Agreement or Second uniQure Agreement as of September 30, 2023 and December 31, 2022. As of September 30, 2023 and December 31, 2022, the aggregate amount of the transaction price allocated to the remaining performance obligation was zero and $0.6 million, respectively.

CFF

In September 2016, the Company entered into an award agreement for the Optimized Adeno-Associated Virus for Lung Epithelia Gene Delivery Development Program with CFF, a non-profit organization dedicated to finding a cure for cystic fibrosis, an inherited disorder that causes disease in the pulmonary airways leading to morbidity and mortality. Under this agreement, CFF contributes funding to help advance the Company's CF research program. The September 2016 grant award agreement was incorporated into a new grant award agreement with the CFF in September 2017 with the same objectives, which was subsequently amended in August 2018 and February 2021. In August 2023, the Company executed a third amendment to the agreement (the "August 2023 Amendment"), which modified the research plan, increased the aggregate milestone payments from $3.5 million to $6.3 million and extended the estimated project completion date. The aforementioned September 2017 agreement and three amendments are collectively referred to as the "CFF Agreement". The August 2023 Amendment represents a contract modification to an existing contract under Topic 606, given the amendment did not include any additional goods or services, and the remaining research activities are not distinct from those previously provided. The August 2023 Amendment did not impact the transaction price, given the increased award amount relates to variable consideration for future milestones that are fully constrained. Accordingly, the contract modification did not result in a revenue adjustment. As of September 30, 2023 and December 31, 2022, the Company had achieved milestones totaling $1.7 million under the CFF Agreement. The remaining award amount will be paid by CFF based on achievement of certain development milestones by the Company.

The Company expects to make payments to CFF equal to six times the actual award received by the Company in three installments within the first four years of the first commercial sale of a product developed under this agreement. The Company also has agreed to make future sales-based milestone payments to CFF of up to three times the actual award received upon achieving specified commercialization milestones with respect to the first of any product developed utilizing any compound covered under the CFF Agreement. The CFF Agreement also requires the Company to pay to CFF royalties of a mid-single digit percentage, up to six times the actual award received, on any amounts received by the Company from the sale, license or transfer to a third-party of rights in the technology developed as a result of this collaboration. Any such royalty payments shall be credited against the payments owed by the Company upon first commercial sale. In the event of a change of control of the Company, CFF will receive certain payments, depending on the timing of the change of control and the size of the transaction.

To date, the Company has not developed a commercial product in connection with the CFF Agreement, and it has not licensed, sold or otherwise transferred to another party the product developed under the CFF Agreement or the underlying technology.

If at any time prior to the first commercial sale of a product developed as a result of the CFF Agreement, the Company ceases to use commercially reasonable efforts to develop or commercialize any product under the CFF Agreement for a continuous period of 180 consecutive days and fails to present a reasonable plan to resume commercially reasonable efforts, the Company will grant to CFF an irrevocable,

exclusive worldwide interruption license under all of the Company's interest in the research plan technology to exploit such product. Any third-party license granted by the Company shall be subject to such interruption license.

The Company identified one performance obligation within the CFF grant agreement for research activities. The Company's contract with CFF does not include a significant financing component.

The Company concluded that the transaction price should not include the variable consideration related to future research milestones as they were considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal of cumulative revenue in the future. The Company re-evaluates the transaction price and estimated period of performance at each reporting period.

Revenue recognized during the three and nine months ended September 30, 2023 was $0.2 million and $0.2 million, respectively, while revenue recognized during the three and nine months ended September 30, 2022 was immaterial. As of September 30, 2023 and December 31, 2022, deferred revenue relating to the CFF Agreement was $1.2 million and $1.4 million, respectively. There were no accounts receivable from CFF under the CFF Agreement as of September 30, 2023 and December 31, 2022. As of September 30, 2023 and December 31, 2022, the aggregate amount of the transaction price allocated to the remaining performance obligation was $1.2 million and $1.4 million, respectively. Based on current timelines, the deferred revenue is expected to be recognized as revenue over the next one to three years as the Company performs research services through the completion of IND-enabling studies.

The obligation to make payments to CFF upon a change of control meets the definition of an embedded derivative that is required to be bifurcated and separately accounted for as a derivative liability. See Note 14 for further discussion of the embedded derivative.

8. License Arrangements

Astellas Gene Therapies, Inc.

On July 5, 2023, the Company entered into a licensing agreement (the "License Agreement") with Astellas Gene Therapies, Inc. ("AGT"), pursuant to which the Company granted to AGT a license to utilize its intravitreal retinotropic R100 vector ("4D Vector") to develop and commercialize licensed compounds and licensed products for one genetic target implicated in rare monogenic ophthalmic disease(s), with options to add up to two additional targets implicated in rare monogenic ophthalmic diseases after paying additional option exercise fees. Under the terms of the License Agreement, the Company has provided its 4D vector technology to Astellas to deliver Astellas' genetic payloads for the treatment of rare monogenic diseases. Astellas will conduct all subsequent research, development, manufacturing, and commercialization activities. As partial consideration for the rights and licenses granted to AGT by the Company under this Agreement, AGT paid the Company an upfront amount of $20 million, which was received in July 2023. The Company may receive potential future option fees and milestones of up to $942.5 million including potential near-term development milestones of $15 million for the initial target. In addition, the Company is entitled to receive mid-single digit to double-digit, sub-teen royalties on net sales of all licensed products.

Under the License Agreement, the Company's performance obligation is to grant and make available to AGT the Licensed IP and Licensed Know-How (each as defined in the License Agreement) with respect to the 4D Vector. In connection with the grant of the license, in July 2023, the Company delivered to AGT the Transferred Material (as defined in the License Agreement).

Aevitas Therapeutics, Inc. and The Trustees of the University of Pennsylvania

On April 21, 2023, the Company entered into an agreement (the "Agreement") with Aevitas Therapeutics, Inc. ("Aevitas"), pursuant to which the Company acquired all of Aevitas' worldwide rights to short-form human complement factor H (sCFH), which the Company plans to use for its 4D-175 product candidate research program. The asset purchase was accounted for as an asset acquisition. As consideration for the Agreement, the Company shall pay Aevitas up to approximately $144 million in cash upon certain late-stage development, regulatory and sales milestones being achieved plus royalties in the

low single digits range on sales of 4D-175. In addition, as part of the Agreement, the Company was assigned a License Agreement for sCFH with the Trustees of the University of Pennsylvania, under which the Company shall pay the University of Pennsylvania up to approximately $42 million in cash upon certain late-stage development, regulatory and sales milestones being achieved plus royalties in the low single digits range on sales of 4D-175. No upfront consideration was paid under the Agreement.

As of September 30, 2023, the Company has not recorded a liability related to contingent consideration for future milestone and royalty payments to either Aevitas or the University of Pennsylvania, as the achievement of such milestones has not occurred and was not deemed probable and product sales have not commenced.

Regents of the University of California

The Company has exclusive, worldwide license agreements (the "UC Agreements") with the Regents of the University of California (the "UC Regents") relating to the use of certain patents and intellectual property surrounding its core technologies, including Therapeutic Vector Evolution. Pursuant to each of the UC Agreements executed prior to January 2019, the Company was obligated to pay a (i) non-refundable license fee of $5,000 upon execution, (ii) a non-refundable license fee of $5,000 each year thereafter, until sales of a licensed product are made and royalties are paid to the UC Regents, (iii) reimbursement of domestic and foreign patent filing, prosecution and maintenance fees, and (iv) either $50,000 or issuance of a 3% equity interest in the Company upon the closing of the first qualified financing at the option of the UC Regents. The Company's first qualified financing occurred in 2015 and at the election of the UC Regents, the Company issued the UC Regents in January 2016 an amount of common stock equal to 6% of the equity interests in the Company pursuant to the applicable clause in each of the UC Agreements.

Pursuant to an agreement with the UC Regents executed in January 2019 the Company paid a non-refundable license fee of $50,000 to the UC Regents upon execution of the agreement. The Company is obligated to pay a non-refundable license fee of $5,000 on the one-year anniversary of the contract effective date and each year thereafter, until sales of a licensed product are made and royalties are paid to the UC Regents.

In addition, the Company is obligated to make certain contingent payments including (i) development milestones up to $3.1 million, (ii) low single digit royalties on the net sales of its developed products that consists of a minimum annual royalty of up to $0.1 million per year for the term of the agreement beginning in the first calendar year after the year in which net sales first occurred, and (iii) sublicense consideration in the mid-teens to the mid-twenties-range on any future sublicensing arrangements the Company may enter into with third-party licensees.

Regents of the University of California and The Trustees of the University of Pennsylvania

In July 2021, the Company entered into an exclusive license agreement with the UC Regents and the Trustees of the University Pennsylvania to license intellectual property related to certain vectors. In July 2021, the Company paid a non-refundable license fee of $100,000 to the UC Regents upon execution of the agreement. The Company is obligated to pay a non-refundable license maintenance fee of $10,000 on the one-year anniversary of the contract effective date and each year thereafter, except years for which the Company has paid royalties on the net sales of a licensed product. In addition, the Company is obligated to make certain contingent payments including (i) development milestones up to $3.9 million, (ii) low single digit percentage rate royalties on the net sales of its licensed products that consists of a minimum annual royalty of up to $0.1 million per year for the term of the agreement beginning in the first calendar year after the year in which net sales first occurred and (iii) sublicense consideration in the mid-single digits to the low twenties percentage rate range on any future sublicensing arrangements the Company may enter into with third-party licensees. On February 22, 2023, the Company provided 90 days written notice to the UC Regents and the Trustees of the University Pennsylvania to terminate said agreement in its entirety at-will. Accordingly, the effective termination date of this agreement is May 23, 2023.

The Company did not incur any expense under the provisions of the outstanding license agreements during the three and nine months ended September 30, 2023. During the three and nine months ended September 30, 2022, the Company incurred immaterial expenses under the provisions of the outstanding license agreements.

9. Commitments and Contingencies

Operating Lease Commitments

5980 Horton Street Building Lease

In May 2015, the Company executed a lease agreement for office and laboratory space in Emeryville, California. In January 2016, the Company executed the first amendment to the lease agreement for additional rentable office and laboratory space which extended the lease term to March 31, 2023 and updated the option to renew the lease at market rent from the prior additional term of three years to five years. In October 2018, the Company executed a second amendment to extend the lease to September 2026. Additionally, the second amendment provided a tenant improvement allowance of $0.2 million, which was paid to the Company in November 2018.

The 5980 Horton Street Building Lease is considered an operating lease under ASC 842 as it does not meet the criteria of a finance lease. As of September 30, 2023, the operating lease right-of-use asset and operating lease liability were $1.3 million and $1.5 million, respectively. As of December 31, 2022, the operating lease right-of-use asset and operating lease liability were $1.6 million and $1.9 million, respectively. The discount rate used to determine the lease liability was 7.5%.

5858 Horton Street Building Lease and Expansion

In October 2018, the Company executed a second lease agreement for additional office and laboratory space in Emeryville, California. The new lease has an initial term of 87 months beginning on the rent commencement date with the option to renew the lease for one additional term of five years at market rent. The Company did not have to pay rent until October 2019. This lease agreement also provided for a tenant improvement allowance of $0.4 million, which was paid to the Company in December 2019. The Company amortizes the tenant improvement allowance on a straight-line basis over the remaining term of the lease as a reduction of rent expense.

In May 2019, the Company amended the second lease agreement executed in October 2018 to add additional office, manufacturing and laboratory space (the “Expansion”). The amendment extended the term of the lease to December 31, 2029. The Company did not have to pay rent until December 2019. The lease agreement also included a tenant improvement allowance, which was increased to $2.3 million in February 2021 pursuant to a second amendment to the second lease agreement. As of September 30, 2023, the Company has received $2.3 million of this tenant improvement allowance. The tenant improvement allowance funds improvements to the additional office, manufacturing and laboratory space, which the Company has determined to be lessee owned. The allowance is treated as a reduction of lease payments used to measure the lease liability.

The 5858 Horton Street Building Lease and Expansion are considered operating leases under ASC 842 as they do not meet the criteria of a finance lease. As of September 30, 2023, the operating lease right-of-use asset and operating lease liability was $10.7 million and $13.6 million, respectively. As of December 31, 2022, the operating lease right-of-use asset and operating lease liability was $11.5 million and $14.3 million, respectively. The discount rate used to determine the lease liability was 7.8%.

Neither of the leases include a general option for the Company to terminate the leases.

The following table summarizes the components of lease expense for the three and nine months ended September 30, 2023 and 2022, which are included in operating expenses in the Company’s condensed statements of operations (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Operating lease cost	$ 698	$ 697	$ 2,095	$ 2,090
Variable lease cost	311	260	933	751
Total	$ 1,009	$ 957	$ 3,028	$ 2,841

Variable lease payments include amounts relating to common area maintenance and are recognized in the condensed statements of operations as incurred.

The following table summarizes supplemental information related to operating leases:

	September 30, 2023	December 31, 2022
Weighted-average remaining lease term (in years)	6.0	6.7
Weighted-average discount rate:	7.8%	7.8%

The following table summarizes the maturities of lease liabilities as of September 30, 2023 (in thousands):

2023 (remaining three months)	$ 773
2024	3,149
2025	3,244
2026	3,135
2027	2,810
Thereafter	5,875
Total future minimum lease payments	18,986
Less: Amount representing interest	(3,829)
Present value of future minimum lease payments	15,157
Less: Current portion of operating lease liabilities	3,126
Long-term portion of operating lease liabilities	$ 12,031

Indemnification Agreements

In the ordinary course of business, the Company enters into agreements that may include indemnification provisions, such as with vendors and other parties. Pursuant to such agreements, the Company may indemnify, hold harmless and defend an indemnified party for losses suffered or incurred by the indemnified party. Some of the provisions will limit losses to those arising from third-party actions. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amount of future payments the Company could be required to make under these provisions is not determinable. The Company has never incurred material costs to defend lawsuits or settle claims related to these indemnification provisions. The Company has also entered into indemnification agreements with its directors and officers that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers to the fullest extent permitted by Delaware corporate law. The Company currently maintains directors' and officers' liability insurance that would generally enable it to recover a portion of any future amounts paid. The Company believes the estimated fair value of its indemnification agreements in excess of applicable insurance coverage is not material.

Legal Proceedings

From time to time, the Company may become involved in legal proceedings arising from the ordinary course of its business. If applicable, the Company records a legal liability when it believes that it is both probable that a liability may be imputed, and the amount of the liability can be reasonably estimated. Significant judgment by the Company is required to determine both probability and the estimated amount. There are no material legal proceedings outstanding at September 30, 2023.

10. Common Stock

As of September 30, 2023 and December 31, 2022, the Company's certificate of incorporation authorized the Company to issue 300,000,000 shares of common stock, at the par value of $0.0001 per share. The holder of each share of common stock is entitled to one vote per share.

Common stockholders are entitled to dividends, if and when declared by the board of directors. To date, no dividends on common stock have been declared by the board of directors.

As of September 30, 2023 and December 31, 2022, the Company has reserved common stock, on an as-converted basis, for future issuance as follows:

	September 30, 2023	December 31, 2022
Issuance of common stock under the 2020 Equity Incentive Award Plan	3,181,340	2,112,039
Issuance of common stock under the 2020 Employee Stock Purchase Plan	380,636	413,273
Exercise of options issued and outstanding	6,021,383	5,778,929
Exercise of common stock warrants	53,669	53,669
Total common stock reserved for future issuance	9,637,028	8,357,910

Funding Agreement with CFF — In April 2020, CFF made a $10.0 million investment in the Company's Series C redeemable convertible preferred stock financing. In return for the investment, CFF received shares of Series C redeemable convertible preferred stock, and the Company and CFF entered into a Funding Agreement (the "Funding Agreement"). Pursuant to the terms of the Funding Agreement, except in the event of a technical failure, the $10.0 million received from CFF will be used to advance the development program for 4D-710, the Company's lead product in cystic fibrosis, or any other therapeutic approved by the Program Advisory Group ("PAG") to alleviate pulmonary complications of cystic fibrosis (the "Funding Agreement Product").

CFF committed to provide an additional $4.0 million of funding upon acceptance of an Investigational New Drug ("IND") application or its equivalent to allow for human testing of the Funding Agreement Product ("Acceptance").

In October 2021, the IND was cleared by the U.S. Food and Drug Administration and CFF made the additional investment of $4.0 million in cash for the issuance of 125,715 shares of the Company's common stock to CFF.

The Company was committed to providing an amount equal to the funding provided by CFF to be used solely to advance the Funding Agreement Product. As of September 30, 2023, the funding commitment has been fulfilled. Under the terms of the Funding Agreement, neither the $10.0 million investment in the Series C redeemable convertible preferred stock, which converted to common stock as of December 31, 2020, nor the $4.0 million of funding upon Acceptance are restricted as to withdrawal or usage.

11. Stock-based Compensation

2020 Equity Incentive Award Plan

In December 2020, the Company adopted the 2020 Incentive Award Plan ("2020 Plan"), which became effective on December 10, 2020. The 2020 Plan initially reserved 2,606,546 shares of common stock for the issuance of stock options, stock appreciation rights, restricted stock awards, restricted stock units, performance bonus awards, performance stock units, dividend equivalents or other stock or cash based award granted to employees, directors and consultants of the Company. The number of shares reserved for future issuance under the 2020 Plan will increase annually on the first day of each fiscal year beginning in 2021 and ending in 2030 by the lesser of (i) 5% of the shares of common stock outstanding (on an as converted basis) on the last day of the immediately preceding fiscal year and (ii) such number of shares of common stock as determined by the Company's board of directors, provided, however, no more than 18,000,000 shares of the Company's common stock may be issued upon the exercise of incentive stock options. On January 1, 2023, an additional 1,631,331 shares of common stock became available for issuance under the 2020 Plan, as a result of the operation of the automatic annual increase provision. All options are exercisable over a period not to exceed the contractual term of ten years from the date the stock options were issued. As of September 30, 2023 there were 2,857,922 shares available for grant under the 2020 Plan.

Following the effectiveness of the 2020 Plan, the Company will not make any further grants under the 2015 Equity Incentive Plan (the "2015 Plan"). However, the 2015 Plan continues to govern the terms of options that remain outstanding under the 2015 Plan. As of September 30, 2023, there were 323,418 shares available for grant under the 2015 Plan.

2015 Equity Incentive Plan

The 2015 Plan provided for grants of stock options, stock appreciation rights, restricted stock and restricted stock unit awards to employees, directors and consultants of the Company. As of September 30, 2023, options to purchase 1,829,240 shares of common stock were outstanding under the 2015 Plan. All options are exercisable over a period not to exceed the contractual term of ten years from the date the stock options were issued and are granted at prices not less than the estimated fair market value of the Company's common stock on the grant date as determined by the board of directors.

No additional grants will be made under the 2015 Plan, and all outstanding grants under the 2015 Plan that are repurchased, forfeited, expire or are cancelled are returned to the 2015 Plan and are not available for grant under the 2020 Plan.

Employee Stock Purchase Plan

In December 2020, the Company adopted the 2020 Employee Stock Purchase Plan (the "2020 ESPP"). Under the 2020 ESPP, 252,337 shares of the Company's common stock were initially reserved for employee purchases of the Company's common stock under terms and provisions established by the Company's board of directors and approved by the Company's stockholders. The number of shares reserved for future issuance under the 2020 ESPP will increase annually on the first day of each fiscal year beginning in 2021 and ending in 2030 by the lesser of (i) 1% of the shares of common stock outstanding (on an as converted basis) on the last day of the immediately preceding fiscal year and (ii) such number of shares of common stock as determined by the Company's board of directors, provided, however, no more than 15,000,000 shares the Company's common stock may be issued under the 2020 ESPP. On December 9, 2022, the Company's Board of Directors approved an increase of 50,000 shares of common stock under the 2020 ESPP, effective as of January 1, 2023.

Under the 2020 ESPP the Company's employees may purchase common stock through payroll deductions at a price equal to 85% of the lower of the fair market value of the stock at the beginning of the offering period or at the end of each applicable purchase period. The 2020 ESPP provides for a series of overlapping 24-month offering periods comprising four six-month purchase periods. The initial offering period under the 2020 ESPP is longer than 24 months, commencing February 15, 2021 and ending on May 14, 2023. Contributions under the 2020 ESPP are limited to a maximum of 15% of an employee's eligible compensation.

Stock Options

The following table summarizes the stock options activity for the nine months ended September 30, 2023:

	Number of Shares Available for Grant	Number of Shares Underlying Outstanding Options
Balances at December 31, 2022	2,112,039	5,778,929
Options authorized	1,631,331	—
Options granted	(1,226,579)	1,226,579
Options exercised	—	(319,576)
Options expired	60,746	(60,746)
Options forfeited	603,803	(603,803)
Balances at September 30, 2023	3,181,340	6,021,383
Options exercisable at September 30, 2023		3,146,872
Options vested and expected to vest at September 30, 2023		6,021,383

The Company estimates the fair value of stock options using the Black-Scholes option pricing model. The fair value of employee and nonemployee options is recognized on a straight-line basis over the

requisite service period of the awards. The fair value of the Company's stock options was estimated using the following assumptions for the three and nine months ended September 30, 2023 and 2022:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Expected term	6.0 - 6.1 years	5.9 - 6.1 years	6.0 - 6.1 years	5.9 - 6.1 years
Expected volatility	80.6% - 82.2%	81.3% - 81.9%	80.6% - 84.4%	80.0% - 81.9%
Risk-free interest rate	4.1% - 4.4%	2.9% - 3.9%	3.5% - 4.4%	1.5% - 3.9%
Expected dividend yield	—%	—%	—%	—%

Expected Term. The expected term for employee options is calculated using the simplified method as the Company does not have sufficient historical information to provide a basis for this estimate. The simplified method is based on the average of the vesting tranches and the contractual life of each grant. The expected term for nonemployee options is the contractual term of the options.

Expected Volatility. The expected volatility is based on a mix of the Company's historical volatility and the historical volatility of comparable companies with similar attributes to the Company, including industry, stage of life cycle, size and financial leverage. For each grant, the Company measured historical volatility over a period equivalent to the expected term.

Risk-free Interest Rate. The risk-free interest rate is based on the yield available on U.S. Treasury zero-coupon issues whose term is similar in duration to the expected term of the respective stock option.

Expected Dividend Yield. The Company has not paid and does not anticipate paying any dividends on its common stock in the future. Accordingly, the Company has estimated the dividend yield to be zero.

Stock-Based Compensation Expense

The following table is a summary of stock-based compensation expense incurred for employees and nonemployees by function (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Research and development	$ 2,314	$ 2,438	$ 7,463	$ 6,931
General and administrative	2,248	2,082	6,289	5,844
Total stock-based compensation expense	$ 4,562	$ 4,520	$ 13,752	$ 12,775

12. Common Stock Warrants

In 2016, the Company issued a warrant for 45,000 shares of the Company's common stock to a service provider with an exercise price of $1.14 per share, of which 15,000 warrant shares become exercisable upon completion of an offering of securities in a private placement by the Company with net proceeds in excess of $25.0 million and 30,000 warrant shares become exercisable upon completion of an IPO by the Company. The warrant expires in 2023. The fair value of the warrant was determined at the issuance date using the Black-Scholes option pricing model. 15,000 of these warrant shares became exercisable upon the completion of the Series B financing in 2018 and 30,000 of these warrants became exercisable upon completion of the IPO in 2020. All 45,000 shares of this warrant were exercised in October 2021.

In May 2018, the Company issued a warrant for 23,669 shares of the Company's common stock to a service provider with an exercise price of $3.19 per share. The fair value of the warrant was determined

at the issuance date using the Black-Scholes option pricing model, expires in 2025 and upon issuance was fully vested.

In December 2020, the Company issued a warrant for 30,000 shares of the Company's common stock to a service provider with an exercise price of $18.00 per share. This warrant vests over a period of four years and expires in 2027. The fair value of the warrant was determined at the issuance date using the Black-Scholes option pricing model.

In April 2021, the Company issued a warrant for 40,000 shares of the Company's common stock to a former employee with an exercise price of $9.41 per share as a result of a settlement agreement with such former employee. The warrant was exercised in May 2021.

There was no material expense related to the above warrant shares during each of the three and nine months ended September 30, 2023 and 2022.

13. Net Loss Per Share, Basic and Diluted

The following table sets forth the computation of basic and diluted net loss per share (in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Numerator				
Net loss	$ (10,256)	$ (25,691)	$ (68,554)	$ (80,115)
Denominator				
Weighted-average shares outstanding used in computing net loss per share, basic and diluted	42,256,629	32,385,791	37,884,363	32,305,074
Net loss per share, basic and diluted	$ (0.24)	$ (0.79)	$ (1.81)	$ (2.48)

The following outstanding shares of potentially dilutive securities were excluded from the computation of diluted net loss per share for the periods presented because including them would have been antidilutive:

	September 30,	
	2023	2022
Options issued and outstanding	6,021,383	6,035,456
ESPP shares expected to be issued	269,102	—
Common stock warrants	53,669	53,669
Total	6,344,154	6,089,125

14. Derivative Liability

The Company identified an embedded derivative resulting from the change of control provision in the CFF Agreement. Embedded derivatives that are required to be bifurcated from the underlying host instrument are accounted for and valued as separate financial instruments. At the inception of the derivative in 2017, the Company recognized this derivative as a liability, and revenue was reduced by the initial fair value of the derivative liability. The Company remeasures the derivative liability to fair value at each reporting period and records the change in fair value of the derivative liability as other income (expense), net. The Company uses a present value analysis with multiple scenarios, which incorporates assumptions and estimates to value the derivative instrument. The Company assesses these assumptions and estimates on a periodic basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement include the change of control payment to CFF (range of zero to $18.9 million at September 30, 2023 and zero to $10.6 million at December 31, 2022), the probability of a change of control event, the probability of the product achieving development or commercial status at time of change of control (range of 4.8% to 17.2% at September 30, 2023 and December 31, 2022) and the discount rate (15.0% at September 30, 2023 and December 31, 2022). The Company

determined the estimated fair value of this liability as of the inception date of the CFF Agreement and concluded that the amount was immaterial. The Company determined the fair value of this derivative liability was $0.4 million and $0.2 million as of September 30, 2023 and December 31, 2022, respectively.

15. Related Party Transactions

In April 2019, the Company entered into two sponsored research agreements ("SRAs") with the UC Regents to conduct research in a research facility on the U.C. Berkeley campus, under the direction of David Schaffer, Ph.D., a co-founder and former director and Chief Scientific Advisor of the Company. The SRAs had a three year term that ended in May 2022. Under the SRAs, the Company had an option to license (on a royalty-bearing basis) all intellectual property generated under the SRAs. The total amount the Company was committed to pay to the UC Regents under the SRAs was $1.4 million, which was fully paid as of March 31, 2022. In March 2021, the Company entered into another sponsored research agreement with the UC Regents to conduct research in laboratories on the U.C. Berkeley campus that are under the direction of David Schaffer, Ph.D., and another U.C. Berkeley professor covering investigations into how machine learning approaches may enhance AAV capsid engineering (the "Machine Learning SRA"). Pursuant to the Machine Learning SRA, the Company committed to pay the UC Regents a total of $1.4 million, of which $0.4 million was paid as of March 31, 2022. The Machine Learning SRA had a three-year term ending in 2024. The Company could terminate the Machine Learning SRA for convenience and without cause with 60 days' notice. The Machine Learning SRA was terminated by the Company in March 2022. While the Machine Learning SRA was between the Company and the UC Regents, the payments under the SRA were used to fund the lab under the direction of David Schaffer, Ph.D. As of September 30, 2023 and December 31, 2022, there were no accounts payable related to the SRAs and Machine Learning SRA. Any patent prosecution costs incurred under the Machine Learning SRA and the SRAs were borne by the Company. During the three and nine months ended September 30, 2023, the Company did not record any expense related to these agreements. During the three and nine months ended September 30, 2022, the Company recorded zero and $0.3 million, respectively, of expenses related to these agreements

As of February 2022, the Machine Learning SRA and the SRAs ceased to be related party transactions upon the resignation of David Schaffer, Ph.D., from the board of directors of the Company.

16. **401(k) Plan**

In 2014, the Company adopted a 401(k) plan for all employees who have met certain eligibility requirements. The 401(k) plan allows employees to make pre-tax and post-tax contributions up to the maximum allowable amount set by the Internal Revenue Service. The Company made contributions to the 401(k) plan for all eligible participants and recorded contribution expenses of $0.2 million for each of the three months ended September 30, 2023 and 2022. For the nine months ended September 30, 2023 and 2022, the Company recorded contribution expenses of $0.9 million and $0.7 million, respectively.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and the related notes included elsewhere in this Quarterly Report on Form 10-Q (this "report"). This discussion and analysis and other parts of this report contain forward-looking statements based upon current beliefs, plans and expectations related to future events and our future financial performance that involve risks, uncertainties and assumptions, such as statements regarding our intentions, plans, objectives, expectations, forecasts and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under the section titled "Risk Factors" and elsewhere in this report.

Overview

We are a genetic medicines company with three novel, highly targeted next generation AAV vectors currently in human clinical studies. We seek to unlock the full potential of genetic medicines using its proprietary invention platform, Therapeutic Vector Evolution, which combines the power of the Nobel Prize-winning technology, directed evolution, with approximately one billion synthetic AAV capsid-derived sequences to invent customized and evolved vectors for use in our product candidates.. We believe key features of our targeted and evolved vectors will help us to potentially create targeted genetic medicine product candidates with improved therapeutic profiles. These profiles will allow us to treat a broad range of large market diseases, unlike most current genetic medicines that generally focus on rare or small market diseases.

We have built a deep portfolio of genetic medicine product candidates, with three novel, highly targeted next generation AAV vectors currently in the clinic. This portfolio includes five product candidates in clinical trials: 4D-150 for the treatment of wet age-related macular degeneration ("wet AMD") and diabetic macular edema ("DME"), 4D-710 for the treatment of cystic fibrosis lung disease, 4D-310 for the treatment of Fabry disease cardiomyopathy, 4D-125 for the treatment of X-linked retinitis pigmentosa ("XLRP") and 4D-110 for the treatment of choroideremia. In addition, we have two product candidates in preclinical studies: 4D-175 for geographic atrophy ("GA") and 4D-725 for alpha-1 antitrypsin deficiency lung disease.

We have funded our operations primarily through the sale and issuance of equity securities and to a lesser extent from cash received pursuant to our collaboration and license agreements.

In May 2023, we completed an underwritten public offering (the "2023 Offering") in which 8,625,000 shares of our common stock were sold at an offering price of $16.00 per share pursuant to the Company's effective shelf registration statement on Form S-3. The net proceeds from the 2023 Offering were $129.2 million after deducting underwriting discounts and commissions and offering expenses.

In July 2023, we entered into a licensing agreement (the "License Agreement") with Astellas Gene Therapies, Inc. ("AGT") for our intravitreal retinotropic R100 vector to develop and commercialize products for genetic targets implicated in rare monogenic ophthalmic disease. AGT paid the Company an upfront amount of $20.0 million and may receive potential future option fees and milestones of up to $942.5 million including potential near-term development milestones of $15.0 million for the initial target.

We have incurred significant operating losses and expect that our operating losses will increase significantly as we, among other things, continue to advance our product candidates through preclinical and clinical development, seek regulatory approval, and prepare for, and, if approved, proceed to commercialization; broaden and improve our platform; acquire, discover, validate and develop additional product candidates; maintain, protect and enforce our intellectual property portfolio; and hire additional personnel.

Our net losses were $10.3 million and $25.7 million for the three months ended September 30, 2023 and 2022, respectively, and $68.6 million and $80.1 million for the nine months ended September 30, 2023 and 2022, respectively. As of September 30, 2023, we had an accumulated deficit of $383.0 million. We do not expect positive cash flows from operations in the foreseeable future. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

We do not have any products approved for sale and have not generated any revenue from product sales since our inception. Our ability to generate product revenue will depend on the successful

development, regulatory approval and eventual commercialization of one or more of our product candidates, if approved.

We will require substantial additional funding to support our continuing operations and further the development of our product candidates. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through the sale of equity, debt financings, or other capital sources, which could include income from collaborations, strategic partnerships, or other strategic arrangements, for the foreseeable future. Adequate funding may not be available when needed or on terms acceptable to us, or at all. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic, the war in Ukraine, rising interest rates, inflation and otherwise. If we fail to obtain necessary capital when needed on acceptable terms, or at all, it could force us to delay, limit, reduce or terminate our product development programs, commercialization efforts or other operations. Insufficient liquidity may also require us to relinquish rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Recent Developments

We presented positive interim clinical and lung biopsy biomarker data for 4D-710 from patients in our Phase 1/2 AEROW clinical trial in November 2023. The interim results included:

- Aerosolized 4D-710 was generally well-tolerated with no inflammation in any lung biopsy across Cohorts 1 & 2 (1E15 & 2E15 vg; n=7) with up to 17 months follow-up;
- Robust, reproducible, supraphysiological CFTR expression across all participants and all lung tissue samples collected (n=34), significantly exceeding target profile; and
- Durable clinical activity demonstrated by improvements in CFQ-R-RD & ppFEV1 through 12 months in Cohort 1.

We also recently announced alignment with the U.S. Food and Drug Administration (“FDA”) on a plan to lift the clinical hold on the Phase 1/2 INGLAXA clinical trial for 4D-310 for Fabry disease cardiomyopathy, including initiation of a single non-human primate safety study evaluating intravenous (“IV”) 4D-310 combined with a rituximab/sirolimus (“R/S”) immunosuppressive regimen and amendment of the INGLAXA protocol to minimize risk of atypical hemolytic uremic syndrome (aHUS) associated with IV AAV dosing, including addition of R/S immunosuppressive regimen.

Components of Results of Operations

Revenue

Our revenue to date has been generated through payments from our collaboration and license agreements, primarily from upfront and milestone payments and expense reimbursement. We have not generated any revenue from the sale of approved products and do not expect to do so for the foreseeable future.

In July 2023, we entered into the License Agreement with AGT where we provided our 4D vector technology to AGT to deliver AGT’s genetic payloads for the treatment of rare monogenic diseases. As partial consideration for the rights and licenses granted to AGT under License Agreement, we received an upfront payment of $20.0 million which we recognized as revenue during the quarter ended September 30, 2023.

In August 2019, we amended our agreement with uniQure and entered into a separate new collaboration and license agreement with uniQure. Neither party was required to pay monetary consideration in connection with the amendment or new agreement. We determined the incremental transaction price of the amendment and new agreement to be $5.1 million and recorded the amount as deferred revenue in August 2019. We began recognizing revenue related to uniQure in 2020 and recognized the remaining revenue under the agreement during the third quarter of 2023. We recognized immaterial revenue during the three months ended September 30, 2023, and $0.6 million during the nine

months ended September 30, 2023 related to this agreement. See Note 7, Research and Collaboration Agreements, to our unaudited condensed financial statements included elsewhere in this report for further discussion regarding the accounting treatment of this agreement.

Future collaboration and license revenue is highly dependent on the successful development and commercialization of products by our collaboration partners, which is uncertain, and revenue may fluctuate significantly from period to period. Additionally, we may never receive the consideration from our license agreements that is contemplated for option fees, development and sales-based milestone payments or royalties on sales of licensed products, given the contingent nature of these payments.

Operating Expenses

Research and Development

Our research and development expenses primarily consist of costs incurred for the discovery and preclinical and clinical development of our product candidates. These expenses include salaries and personnel-related costs, including stock-based compensation of our scientific personnel performing research and development activities; laboratory supplies; research materials; fees paid to CROs to execute preclinical studies and clinical trials; fees paid to CMOs to manufacture materials for preclinical studies and clinical trials; fees related to obtaining technology licenses; consulting costs; costs related to seeking regulatory approval of our product candidates; and allocated facility-related costs, information technology costs, depreciation expense, and other overhead.

We expense all research and development costs in the periods in which they are incurred. We have entered into various agreements with CROs and CMOs. Costs of certain activities are recognized based on an evaluation of the progress to completion of specific tasks. Payments made prior to the receipt of goods or services that will be used or rendered for future research and development activities are deferred and capitalized as prepaid expenses and other current assets on our balance sheet. The capitalized amounts are recognized as expense as the goods are delivered or the related services are performed.

We do not allocate our costs by product candidate, as a significant amount of research and development expenses includes internal costs, such as salary and other personnel-related expenses, laboratory supplies and allocated overhead, and external costs, such as fees paid to third parties to conduct research and development activities on our behalf, none of which are tracked by product candidate. In particular, with respect to internal costs, several of our departments support multiple product candidate research and development programs and, therefore, the costs cannot be allocated to a particular product candidate or development program.

At this time, we cannot reasonably estimate or know the nature, timing or estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our product candidates. We expect our research and development expenses to increase for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, as our product candidates advance into later stages of development, as we begin to conduct larger clinical trials, as we seek regulatory approvals for any product candidates that successfully complete clinical trials, and incur expenses associated with hiring additional personnel to support our research and development efforts. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. See the section titled “Risk Factors” for additional risks regarding regulatory development and approval.

General and Administrative

Our general and administrative expenses consist primarily of personnel-related expenses, including salaries, employee benefit costs and stock-based compensation expense for our personnel in executive, finance and accounting, legal, human resources, business development, and other administrative functions. General and administrative expenses also include professional fees for legal, patent, consulting, accounting and tax services, allocated overhead, including rent, equipment, depreciation, information technology costs

and utilities, and other general operating expenses not otherwise classified as research and development expenses.

We expect our general and administrative expenses to increase as a result of increased personnel-related costs, patent costs for our product candidates, consulting, legal and accounting services associated with maintaining compliance with stock exchange listing and requirements of the SEC, investor relations costs, director and officer insurance premiums, and other costs associated with being a public company.

Other Income (Expense), Net

Our other income (expense), net primarily consists of interest income earned on our cash equivalents and marketable securities and adjustments for the change in the fair value of our derivative liability which must be remeasured at each reporting date.

Results of Operations

Comparison of the Three and NIne Months Ended September 30, 2023 and 2022

The following tables summarize our results of operations for the periods indicated (dollars in thousands):

	Three Months Ended September 30,			
	2023	2022	$ Change	% Change
Revenue				
Collaboration and license revenue	$ 20,204	$ 500	$ 19,704	3941 %
Operating Expenses:				
Research and development	25,066	18,940	6,126	32 %
General and administrative	9,112	8,055	1,057	13 %
Total operating expenses	34,178	26,995	7,183	27 %
Loss from operations	(13,974)	(26,495)	12,521	(47)%
Other Income, Net	3,718	804	2,914	362 %
Net loss	$ (10,256)	$ (25,691)	$ 15,435	(60)%

	Nine Months Ended September 30,			
	2023	2022	$ Change	% Change
Revenue				
Collaboration and license revenue	$ 20,742	$ 1,882	$ 18,860	1002 %
Operating Expenses:				
Research and development	71,068	58,753	12,315	21 %
General and administrative	25,889	24,441	1,448	6 %
Total operating expenses	96,957	83,194	13,763	17 %
Loss from operations	(76,215)	(81,312)	5,097	(6)%
Other Income, Net	7,661	1,197	6,464	540 %
Net loss	$ (68,554)	$ (80,115)	$ 11,561	(14)%

Revenue

Revenue increased by $19.7 million, or 3,941%, from the three months ended September 30, 2022 to the three months ended September 30, 2023 mainly due to the upfront fees received from the License Agreement with Astellas.

Revenue increased by $18.9 million, or 1,002%, from the nine months ended September 30, 2022 to the nine months ended September 30, 2023 mainly due to the upfront fees received from the License Agreement with Astellas which was partially offset by lower revenue from uniQure in 2023.

Research and Development Expenses

The following table provides a breakout of research and development expenses for the periods indicated (dollars in thousands):

	Three Months Ended September 30,			
	2023	2022	$ Change	% Change
Research and development trials and consumables expenses	$ 11,500	$ 6,677	$ 4,823	72 %
Payroll and personnel expenses	9,661	9,560	101	1 %
Facilities and other research and development expenses	3,905	2,703	1,202	44 %
Total research and development expenses	$ 25,066	$ 18,940	$ 6,126	32 %

	Nine Months Ended September 30,			
	2023	2022	$ Change	% Change
Research and development trials and consumables expenses	$ 28,485	$ 22,704	5,781	25 %
Payroll and personnel expenses	31,548	28,112	3,436	12 %
Facilities and other research and development expenses	11,035	7,937	3,098	39 %
Total research and development expenses	$ 71,068	$ 58,753	$ 12,315	21 %

Research and development expenses increased by $6.1 million, or 32%, from the three months ended September 30, 2022 to the three months ended September 30, 2023. The increase was primarily due to the following:

- a $4.8 million increase in clinical trial costs mainly due to increased clinical trial activity for our product candidates, primarily 4D-150;
- a $1.2 million increase in facilities and other research and development expenses mainly due to higher fixed assets depreciation, repairs and maintenance services and consultant expenses; and
- a $0.1 million increase in payroll and personnel expenses.

Research and development expenses increased by $12.3 million, or 21%, from the nine months ended September 30, 2022 to the nine months ended September 30, 2023. The increase was primarily due to the following:

- a $5.8 million increase in clinical trial costs mainly due to increased clinical trial activity for our product candidates, primarily 4D-150;
- a $3.1 million increase in facilities and other research and development expenses mainly due to higher fixed assets depreciation and consultant expenses; and
- a $3.4 million increase in payroll and personnel expenses, including a $0.5 million increase in employee stock-based compensation, due to increased headcount of research and development personnel.

General and Administrative Expenses

General and administrative expenses increased by $1.1 million, or 13%, from the three months ended September 30, 2022 to the three months ended September 30, 2023. The increase was primarily due to:

- a $0.2 million increase in outside services;
- a $0.5 million increase in payroll and personnel expenses, which includes a $0.2 million increase in employee stock-based compensation due to increased headcount of general and administrative personnel; and
- a $0.4 million increase in facilities and other expenses mainly due to higher consulting services and audit and tax fees.

General and administrative expenses increased by $1.4 million, or 6%, from the nine months ended September 30, 2022 to the nine months ended September 30, 2023. The increase was primarily due to:

- a $1.0 million increase in outside services;
- a $0.2 million increase in payroll and personnel expenses; and
- a $0.2 million increase in facilities and other expenses mainly due to higher facilities expenses and audit and tax fees which was partially offset by lower business insurance expense.

Other Income, Net

Other income, net increased by $2.9 million, or 362%, from the three months ended September 30, 2022 to the three months ended September 30, 2023. It increased by $6.5 million, or 540%, from the nine

months ended September 30, 2022 to the nine months ended September 30, 2023. The increase is primarily attributable to higher market yields on our cash equivalents and marketable securities.

Liquidity and Capital Resources

Sources of Liquidity

We have funded our operations primarily through the sale and issuance of our equity securities, including from the sale of our common stock in our IPO, 2021 Offering and 2023 Offering, as well as the sale of our Series A, Series A-1, Series B and Series C redeemable preferred stock, and to a lesser extent from cash received pursuant to our collaboration and license agreements.

In December 2020, we completed our IPO. We issued and sold 9,660,000 shares of common stock at a price to the public of $23.00 per share. The aggregate net proceeds from our IPO were $204.7 million after deducting underwriting discounts and commissions and other offering costs.

In November 2021, we completed our 2021 Offering in which 4,750,000 shares of our common stock were sold at an offering price of $25.00 per share. The net proceeds from the Follow-on Offering were $111.1 million after deducting underwriting discounts and commissions and offering expenses.

In March 2022, we filed a Registration Statement on Form S-3 covering the offering of up to $300.0 million of common stock, preferred stock, debt securities, warrants and units, which was declared effective by the SEC in April 2022 (the “S-3 Registration Statement”). In March 2022, we also entered into an Open Market Sales Agreement (the "Sales Agreement") with Jefferies LLC as sales agent to sell shares of our common stock, from time to time, with aggregate gross sales proceeds of up to $100.0 million pursuant to the S-3 Registration Statement as an “at-the-market” offering under the Securities Act (the "2022 ATM Offering Program"). As of September 30, 2023, 1.1 million shares of the Company's common stock were sold pursuant to the Sales Agreement for net proceeds to the Company of $19.1 million, after deducting issuance costs. As of September 30, 2023, $80.1 million of common stock remained available for sale under the Sales Agreement.

In May 2023, we completed the 2023 Offering in which 8,625,000 shares of our common stock were sold at an offering price of $16.00 per share. The net proceeds from the 2023 Offering were $129.2 million after deducting underwriting discounts and commissions and offering expenses.

In July 2023, we entered into the License Agreement with AGT where we provided our 4D vector technology to AGT to deliver AGT’s genetic payloads for the treatment of rare monogenic diseases. As partial consideration for the rights and licenses granted to AGT under the License Agreement, we received an upfront payment of $20.0 million.

Future Funding Requirements

We have experienced recurring net losses and had an accumulated deficit of $383.0 million as of September 30, 2023. Our transition to profitability is dependent upon the successful development, approval and commercialization of our product candidates and those of our collaboration partners and achieving a level of revenue adequate to support our cost structure. We expect to continue to incur losses for the foreseeable future.

We expect that our research and development and general and administrative expenses will continue to increase for the foreseeable future. Additionally, we expect our capital expenditures will increase significantly in the future for costs associated with building commercial manufacturing capacity. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amount of increased capital we will need to raise to support our operations and the outlays and operating expenditures necessary to complete the development of our product candidates and build additional manufacturing capacity, and we may use our available capital resources sooner than we currently expect.

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

- the progress of our current and future product candidates through preclinical and clinical development;
- potential delays in our preclinical studies and clinical trials, whether current or planned;
- expanding our manufacturing facilities and working with our contract manufacturers to scale up the manufacturing processes for our product candidates;
- continuing our research and discovery activities;
- continuing the development of our Therapeutic Vector Evolution platform;
- initiating and conducting additional preclinical, clinical or other studies for our product candidates;
- changing or adding additional contract manufacturers or suppliers;
- seeking regulatory approvals and marketing authorizations for our product candidates;
- establishing sales, marketing and distribution infrastructure to commercialize any products for which we obtain approval;
- acquiring or in-licensing product candidates, intellectual property and technologies;
- making milestone, royalty or other payments due under any current or future collaboration or license agreements;
- obtaining, maintaining, expanding, protecting and enforcing our intellectual property portfolio;
- attracting, hiring and retaining qualified personnel;
- potential delays or other issues related to our operations;
- meeting the requirements and demands of being a public company;
- defending against any product liability claims or other lawsuits related to our products; and
- the impact of the COVID-19 pandemic and adverse macroeconomic conditions such as, but not limited to, higher inflation and increased interest rates, each of which may exacerbate the magnitude of the factors discussed above.

We believe that our existing cash and cash equivalents and marketable securities will allow us to fund our planned operations for at least one year from the date of the issuance of this report.

We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing sooner than currently projected, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. See the section titled “Risk Factors” for additional risks associated with our substantial capital requirements.

We do not have any committed external sources of funds. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources to complete the clinical development for the product candidates for treatment of wet AMD, DME, GA, cystic fibrosis lung disease, alpha-1 antitrypsin deficiency lung disease, Fabry disease cardiomyopathy, XLRP, choroideremia or any other indication we may pursue. If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Any future debt financing into which we enter would result in fixed payment obligations and may involve agreements that include grants of security interests on our assets and restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, granting liens over our assets, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Any debt financing or additional equity that we raise may contain terms that could adversely affect our common stockholders. Further, additional funds may not be available when we need them, on terms that are acceptable to us, or at all. Our ability to raise additional capital may be adversely impacted by

potential worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic, the war in Ukraine, rising interest rates and inflation.

If we are unable to obtain additional funding, we expect to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or investment in internal manufacturing capabilities, which could adversely affect our business. If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to future revenue streams or product candidates or grant licenses on terms that may not be favorable to us.

Summary Statement of Cash Flows

The following is a summary of cash flows for the periods indicated below (in thousands):

	Nine Months Ended September 30,	
	2023	2022
Net cash used in operating activities	$ (51,142)	$ (64,471)
Net cash provided by investing activities	122,399	18,729
Net cash provided by financing activities	152,070	1,039
Net increase (decrease) in cash and cash equivalents	$ 223,327	$ (44,703)

Net Cash Used in Operating Activities

Net cash used in operating activities was $51.1 million for the nine months ended September 30, 2023. This was primarily due to the net loss of $68.6 million, adjusted for noncash charges of $17.4 million and net changes in operating assets and liabilities of $0.1 million. Noncash charges included $13.8 million of stock-based compensation expense, $3.1 million of depreciation and amortization, $1.1 million for the amortization of operating lease right-of-use assets and $0.2 million change in fair value of derivative liability, offset by $0.8 million net amortization/accretion of premium/discount on marketable securities. Net changes in operating assets and liabilities included a $1.0 million decrease in operating lease liabilities, $0.7 million decrease in deferred revenue and a $1.6 million increase in prepaid expenses and other current assets which were offset by a $2.0 million increase in accounts payable and $1.4 million increase in accrued and other liabilities.

Net cash used in operating activities was $64.5 million for the nine months ended September 30, 2022. This was primarily due to the net loss of $80.1 million, adjusted for noncash charges of $16.6 million. Noncash charges included $12.8 million of stock-based compensation expense, $1.4 million of depreciation and amortization, $1.3 million of amortization of the premium on marketable securities, and $1.1 million for the amortization of operating lease right-of-use assets. Net changes in operating assets and liabilities included a $3.1 million decrease in accounts payable and a $1.9 million decrease in deferred revenue which were partially offset by a $3.7 million decrease in prepaid expenses and other current assets.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was $122.4 million for the nine months ended September 30, 2023. This was due to $144.8 million in maturities of marketable securities, offset by purchases of marketable securities of $20.2 million and purchases of property and equipment of $2.2 million.

Net cash provided by investing activities was $18.7 million for the nine months ended September 30, 2022. This was due to maturities of marketable securities of $283.0 million, partially offset by purchases of marketable securities of $253.8 million and purchases of property and equipment of $10.4 million.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $152.1 million for the nine months ended September 30, 2023. This was primarily due to proceeds from the issuance of common stock upon public offering of $129.2 million, proceeds from the issuance of common stock from the ATM offering program of $19.1

million, proceeds from the issuance of common stock from the exercise of stock options of $3.1 million, and proceeds from the issuance of common stock from the Company's 2022 Employee Stock Purchase Plan ("ESPP") purchases of $0.7 million.

Net cash provided by financing activities was $1.0 million for the nine months ended September 30, 2022. This was due to proceeds from the exercise of stock options and warrants of $0.6 million and issuance of common stock under the ESPP of $0.5 million.

Contractual Obligations, Commitments and Contingencies

Our commitments include obligations under vendor contracts to provide research services and other purchase commitments with our vendors. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations and other third parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided. These amounts are not fixed and determinable.

As of September 30, 2023, our principal commitments consisted of obligations under our operating leases for our headquarters. Please see Note 9, Commitments and Contingencies, to our unaudited condensed financial statements included elsewhere in this report for further details.

Critical Accounting Policies and Significant Judgments and Estimates

Our financial statements have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenue and expenses during the reported periods. We evaluate these estimates and assumptions on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

During the three and nine months ended September 30, 2023, there were no changes to our critical accounting policies and significant judgments and estimates as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2022.

Recent Accounting Pronouncements

See Note 2, Summary of Significant Accounting Policies, to our unaudited condensed financial statements included elsewhere in this report for information.

Off-Balance Sheet Arrangements

Since our inception, we have not engaged in any off-balance sheet arrangements as defined in the rules and regulations of the SEC.

JOBS Act

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). Under the JOBS Act, emerging growth companies may delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, while we are an emerging growth company, we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies. As a result, our financial statements may not

be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We will remain an emerging growth company until the earlier of (i) December 31, 2025, (ii) the last day of the year in which we have total annual gross revenue of at least $1.07 billion, (iii) the last day of the year in which we are deemed to be a “large accelerated filer” as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded $700.0 million as of the last business day of the second fiscal quarter of such year, or (iv) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period. An emerging growth company may take advantage of specified reduced reporting requirements and is relieved of certain other significant requirements that are otherwise generally applicable to public companies. As an emerging growth company:

- we will present only two years of audited financial statements for any interim period, and related management’s discussion and analysis of financial condition and results of operations;
- we will avail ourselves of the exemption from the requirement to obtain an attestation and report from our auditors on the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act of 2002 (the “Sarbanes-Oxley Act”);
- we will provide less extensive disclosure about our executive compensation arrangements;
- we will not require stockholder non-binding advisory votes on executive compensation or golden parachute arrangements; and
- we will take advantage of extended transition periods to comply with new or revised accounting standards, delaying the adoption of these accounting standards until they would apply to private companies.

As a result, the information in this report in the future may be different than what you might receive from other public reporting companies.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Sensitivity and Effects of Inflation

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of September 30, 2023, we had cash and cash equivalents and marketable securities of $319.7 million, consisting of bank deposits, interest-bearing money market funds, and marketable securities, for which the fair value would be affected by changes in the general level of U.S. interest rates. However, due to the short to medium-term maturities of our cash equivalents and marketable securities, an immediate 10% change in interest rates would not have a material effect on the fair value of our cash equivalents or marketable securities.

We do not believe that inflation or interest rate changes have had a significant impact on our results of operations for any periods presented herein.

Item 4. Controls and Procedures.

Evaluation of our Disclosure Controls and Procedures

We maintain “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission’s rules and forms and (2) accumulated and communicated to our management, including our principal executive and principal financial and accounting officers, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our principal executive officer and principal financial and accounting officer, evaluated the effectiveness of our disclosure controls and procedures at the end of the period covered by this report. Based upon such evaluation, our principal executive officer and principal financial and accounting officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Changes in Internal Control over Financial Reporting

There has been no change in our internal control over financial reporting during the quarter ended September 30, 2023, that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings.

The legal proceedings information set forth in Note 9 *Commitments and Contingencies* of the notes to our unaudited condensed financial statements in Part I, Item I of this report is incorporated by reference.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this report, including our financial statements and the related notes and the section of this report "Management's Discussion and Analysis of Financial Condition and Results of Operations," before deciding whether to invest in our common stock. If any of the following risks actually occur, our business, reputation, financial condition, results of operations, revenue and future prospects could be seriously harmed. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that adversely affect our business. Unless otherwise indicated, references to our business being seriously harmed in these risk factors and elsewhere will include harm to our business, reputation, financial condition, results of operations, future prospects and stock price. If our business is seriously harmed, the market price of our common stock could decline, and you could lose part or all of your investment.

Risk Factor Summary

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. The following is a summary of the principal risks that could seriously harm our business, all of which are more fully described below. This summary should be read in conjunction with the other risk factors included in this "Risk Factors" section and should not be relied upon as an exhaustive summary of the material risks facing our business.

- We are in the early stages of drug development and have a very limited operating history and no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.
- We have had recurring net losses, and we expect to continue to incur significant net losses for the foreseeable future.
- We will require substantial additional capital to finance our operations. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs or future commercialization efforts.
- All of our product candidates are based on a novel AAV genetic medicine technology with which there is limited regulatory and clinical experience to date, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Further, the regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied therapeutic modalities.
- Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise seriously harm our business.
- Adverse public perception or regulatory scrutiny of genetic medicine technology may negatively impact the developmental progress or commercial success of products that we develop alone or with collaborators.
- Our clinical trials may fail to demonstrate substantial evidence of the safety and efficacy of our product candidates, which would prevent, delay or limit the scope of regulatory approval and commercialization.
- The regulatory approval processes of the FDA, EMA and comparable foreign regulatory authorities are lengthy, expensive, time consuming, and inherently unpredictable. If we are

ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be substantially harmed.

- Our employees, independent contractors, consultants, research or commercial partners or collaborators and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.
- Our success depends on our ability to protect our intellectual property and our proprietary technologies.
- Our rights to develop and commercialize our product candidates are subject in part to the terms and conditions of licenses granted to us by others, and the patent protection, prosecution and enforcement for some of our product candidates may be dependent on our licensors.

Risks Related to Our Limited Operating History, Financial Condition and Capital Requirements

We are in the early stages of drug development and have a very limited operating history and no products approved for commercial sale, which may make it difficult to evaluate our current business and predict our future success and viability.

We are a clinical-stage genetic medicine company pioneering the development of product candidates using our targeted and evolved AAV vectors. We commenced operations in September 2013, have no products approved for commercial sale and have not generated any product revenue. Drug development is a highly uncertain undertaking and involves a substantial degree of risk. If our product candidates are not successfully developed and approved, we may never generate any product revenue. To date, we have not completed any clinical trials (including any pivotal clinical trial), obtained marketing approval for any product candidates, manufactured commercial scale quantities of any of our product candidates or arranged for a third party to do so on our behalf, or conducted sales and marketing activities necessary for successful product commercialization. Our limited operating history as a company and early stage of drug development make any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early-stage biopharmaceutical companies in rapidly evolving fields, and we have not yet demonstrated an ability to successfully overcome such risks and difficulties. If we do not address these risks and difficulties successfully, our business will be seriously harmed.

We have had recurring net losses, and we expect to continue to incur significant net losses for the foreseeable future.

We have incurred recurring net losses, including net losses of $10.3 million and $25.7 million for the three months ended September 30, 2023 and 2022, respectively, and $68.6 million and $80.1 million for the nine months ended September 30, 2023 and 2022, respectively. As of September 30, 2023, we had an accumulated deficit of $383.0 million.

We have devoted substantially all of our financial resources and efforts on research and development activities, including for our product candidates and our Therapeutic Vector Evolution platform. We do not expect to generate revenue from product sales for several years, if at all. We continue to incur significant research and development and other expenses related to our ongoing operations. The amount of our future net losses will depend, in part, on the level of our future expenditures and our ability to generate revenue. Moreover, our net losses may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- progress our current and any future product candidates through preclinical and clinical development;

- expand our manufacturing facilities and work with our contract manufacturers to scale up the manufacturing processes for our product candidates;
- continue our research and discovery activities;
- continue the development of our Therapeutic Vector Evolution platform;
- initiate and conduct additional preclinical, clinical or other studies for our product candidates;
- change or add additional contract manufacturers or suppliers;
- seek regulatory approvals and marketing authorizations for our product candidates;
- establish sales, marketing and distribution infrastructure to commercialize any products for which we obtain approval;
- acquire or in-license product candidates, intellectual property and technologies;
- make milestone, royalty or other payments due under any current or future collaboration or license agreements;
- obtain, maintain, expand, protect and enforce our intellectual property portfolio;
- attract, hire and retain qualified personnel;
- experience any delays or encounter other issues related to our operations;
- meet the requirements and demands of being a public company;
- are adversely impacted by general economic conditions, such as rising inflation and increased interest rates;
- defend against any product liability claims or other lawsuits related to our products; and
- experience delays in our preclinical studies and clinical trials, whether current or planned, due to the novel coronavirus ("COVID-19") pandemic or other similar pandemics or public health emergencies.

Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' deficit and working capital. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

We will require substantial additional capital to finance our operations. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs or future commercialization efforts.

Developing biopharmaceutical products, including conducting preclinical studies and clinical trials, is a very time consuming, expensive and uncertain process that takes years to complete. Our operations have required substantial amounts of cash since inception. To date, we have financed our operations primarily through the sale of equity securities and to a lesser extent from cash received pursuant to our collaboration and license agreements. We have initiated clinical trials, which are ongoing, and have additional product candidates in preclinical development that may enter clinical development. Developing our product candidates is expensive, and we expect to continue to spend substantial amounts as we fund our early stage research projects, continue preclinical and clinical development of our product candidates and, in particular, advance our product candidates through clinical trials. Even if we are successful in developing our product candidates, obtaining regulatory approvals and launching and commercializing any product candidate will require substantial funding.

As of September 30, 2023, we had $319.7 million in cash and cash equivalents and marketable securities. In May 2023 we closed the 2023 Offering which provided net proceeds of $129.2 million.

Based on our current operating plan, we believe that our existing cash, cash equivalents and marketable securities will allow us to fund our planned operations for at least one year from the date of the issuance of the unaudited condensed financial statements included in this Quarterly Report on Form 10-Q.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. Our ability to raise additional capital may be adversely impacted if global economic conditions continue to worsen as a result of volatility in credit and financial markets (including due to the residual effects of the COVID-19 pandemic) and to product supply chains. If adequate funds are not available to us on a timely basis, we may be required to:

- delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities or eliminate one or more of our development programs altogether; or
- delay, limit, reduce or terminate our efforts to establish manufacturing and sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates, or reduce our flexibility in developing or maintaining our sales and marketing strategy.

We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to, or jointly own some aspects of, our product candidates or technologies that we would otherwise pursue on our own. We do not expect to realize revenue from sales of products or royalties from licensed products in the foreseeable future, if at all, and unless and until a product candidate is clinically tested, approved for commercialization and successfully marketed.

We will be required to seek additional funding in the future and currently intend to do so through collaborations, public or private equity offerings or debt financings, credit or loan facilities or a combination of one or more of these funding sources. Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution, and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, is likely to involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of our equity securities received any distribution of our corporate assets.

If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates or one or more of our other research and development initiatives. Any of the above events could seriously harm our business and cause the price of our common stock to decline.

Due to the significant resources required for the development of our product candidates, and depending on our ability to access capital, we must prioritize development of certain product candidates. Moreover, we may expend our limited resources on product candidates that do not yield a successful product and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Due to the significant resources required for the development of our product candidates, in particular our product candidates in IND-enabling studies and those in clinical trials, we must decide which product candidates and indications to pursue and advance and the amount of resources to allocate to each. Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates or therapeutic areas may not lead to the development of any viable commercial product and may divert resources away from better opportunities. Similarly, our potential decisions to delay, terminate or collaborate with third parties in respect of certain product candidates may subsequently also prove to be less than optimal and could cause us to miss valuable opportunities. If we make incorrect determinations regarding the viability or market potential of any of our product candidates or misread trends in the biopharmaceutical industry, in particular for ophthalmology, cardiology and pulmonology, and cardiology diseases, our business could be seriously harmed. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases that may later prove

to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights.

The amount of our future losses is uncertain and our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

Our quarterly and annual operating results may fluctuate significantly, which makes it difficult for us to predict our future operating results. These fluctuations may occur due to a variety of factors, many of which are outside of our control and may be difficult to predict, including:

- the timing and success or failure of preclinical studies and clinical trials for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or collaboration partners;
- the timing and cost of, and level of investment in research, development and commercialization activities, which may change from time to time;
- the timing of receipt of approvals from regulatory authorities in the United States and internationally;
- the timing and status of enrollment and safety and efficacy readouts for our clinical trials;
- the cost of manufacturing, as well as building out our supply chain, which may vary depending on the quantity of production, the cost of continuing to establish and scale up our internal manufacturing capabilities, and the terms of any agreements we enter into with third-party suppliers;
- timing and amount of any option, milestone, royalty or other payments due under any current or future collaboration or license agreement;
- coverage and reimbursement policies with respect to our genetic medicine product candidates and potential future drugs that compete with our products, if approved;
- expenditures that we may incur to acquire, develop or commercialize additional products and technologies;
- the level of demand for our genetic medicine products, if approved, which may vary significantly over time;
- future accounting pronouncements or changes in our accounting policies; and
- the impact from general macroeconomic trends, such as higher inflation and increased interest rates.

For example, most of our collaboration and license revenue for the year ended December 31, 2021 was from Roche. However, Roche terminated its collaboration and license agreement with us effective September 16, 2021. The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

This variability and unpredictability could also result in our failing to meet the expectations of financial analysts or investors for any period. If our revenue or operating results fall below or if operating expenses or other costs are higher than the expectations of analysts or investors or below or above, as the case may be, any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

Risks Related to the Research, Discovery, Development and Commercialization of Our Product Candidates

All of our product candidates are based on a novel AAV genetic medicine technology with which there is limited regulatory and clinical experience to date, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Further, the regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied therapeutic modalities.

All of our product candidates are based on genetic medicine technology, and our future success depends on the successful development of this novel therapeutic approach. We cannot assure you that any development problems we or other genetic medicine companies experience in the future related to genetic medicine technology will not cause significant delays or unanticipated costs in the development of our product candidates, or that such development problems can be solved. In addition, the clinical study requirements of the FDA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied therapeutic modalities. Further, as we are developing novel treatments for diseases in which there is limited clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, European Medicines Agency ("EMA") or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. To date, few genetic medicine products have been approved by the FDA or comparable foreign regulatory authorities, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the United States, the European Union or other jurisdictions. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

Regulatory requirements governing genetic medicine products have evolved and may continue to change in the future. For example, the FDA has established the Office of Therapeutic Products within its Center for Biologics Evaluation and Research ("CBER") to consolidate the review of genetic medicine and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. These and other regulatory review agencies, committees and advisory groups and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions.

The National Institutes of Health ("NIH") Guidelines for Research Involving Recombinant DNA Molecules ("NIH Guidelines") require supervision of human gene transfer trials, including evaluation and assessment by an Institutional Biosafety Committee ("IBC"), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to the public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

We are subject to significant regulatory oversight by the FDA, and, in addition, the applicable IBC and Institutional Review Board ("IRB"), of each institution at which we or our collaborators conduct clinical trials of our product candidates, or a central IRB if appropriate, need to review and approve the proposed clinical trial.

Similarly, the EMA governs the development of gene therapies in the European Union and may issue new guidelines concerning the development and marketing authorization for genetic medicine products and require that we comply with these new guidelines.

Changes in applicable regulatory guidelines may lengthen the regulatory review process, require us to perform additional studies or trials, 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.

As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of such product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all and could seriously harm our business.

Adverse public perception or regulatory scrutiny of genetic medicine technology may negatively impact the developmental progress or commercial success of product candidates that we develop alone or with collaborators.

The developmental and commercial success of our current product candidates, or any that we develop alone or with collaborators in the future, will depend in part on public acceptance of the use of genetic medicine technology, including the use of AAVs, for the prevention or treatment of human diseases. Adverse public perception of gene therapies may negatively impact our ability to raise capital or enter into strategic agreements for the development of product candidates.

Genetic medicine remains a novel technology. The commercial success of our genetic medicine product candidates, if successfully developed and approved, may be adversely affected by claims that genetic medicine is unsafe, unethical or immoral. This may lead to unfavorable public perception and the inability of any of our product candidates to gain the acceptance of the public or the medical community. Unfavorable public perceptions may also adversely impact our or our collaborators' ability to enroll clinical trials for our product candidates. Moreover, success in commercializing any product candidates that receive regulatory approval will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of such product candidates in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

Publicity of any adverse events in, or unfavorable results of, preclinical studies or clinical trials for any current or future product candidates, or with respect to the studies or trials of our competitors or of academic researchers utilizing similar technologies, even if not ultimately attributable to our technology or product candidates, could negatively influence public opinion. Negative public perception about the use of AAV technology in human therapeutics, whether related to our technology or a competitor's technology, could result in increased governmental regulation, delays in the development and commercialization of product candidates or decreased demand for the resulting products, any of which may seriously harm our business.

Our product candidates may cause undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authorities.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries, and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions. It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in previous trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. Less common adverse effects may not become

evident until investigational products are tested in large-scale, Phase 3 clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval.

If any serious adverse events occur, clinical trials or commercial distribution of any product candidates or products we develop alone or with collaborators could be suspended or terminated, and our business could be seriously harmed. Treatment-related side effects could also affect patient recruitment and the ability of enrolled patients to complete the trial or result in potential liability claims. Regulatory authorities could order us or our collaborators to cease further development of, deny approval of, or require us to cease selling any product candidates or products for any or all targeted indications. If we or our collaborators elect, or are required, to delay, suspend or terminate any clinical trial or commercialization efforts, the commercial prospects of such product candidates or products may be harmed, and our ability to generate product revenues from them or other product candidates that we develop may be delayed or eliminated. Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects or adverse events caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label including “boxed” warnings, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to change the way the product is administered or conduct additional clinical trials or post-approval studies;
- we may be required to create a Risk Evaluation and Mitigation Strategy (“REMS”), which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers and/or other elements to assure safe use;
- the product may become less competitive;
- we may be subject to fines, injunctions or the imposition of criminal penalties;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could seriously harm our business.

Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have no products approved for commercial sale, and we have never generated any revenue from product sales, and we may never generate product revenue or be profitable.

We have no products approved for commercial sale and have not generated any revenue from product sales. We do not anticipate generating any revenue from product sales until after we have successfully completed clinical development and received regulatory approval for the commercial sale of a product candidate, which will not occur for several years, if ever.

Our ability to generate revenue and achieve profitability depends significantly on many factors, including:

- successfully completing research and preclinical and clinical development of our product candidates;
- obtaining regulatory approvals and marketing authorizations for product candidates for which we successfully complete clinical development and clinical trials;
- developing a sustainable and scalable manufacturing process for our product candidates, as well as establishing and maintaining commercially viable supply relationships with third parties that can provide adequate products and services to support clinical activities and any commercial demand for our product candidates;

- identifying, assessing, acquiring and/or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- the prevalence, duration and severity of potential side effects or other safety issues experienced with our product candidates or future approved products, if any;
- launching and successfully commercializing product candidates for which we obtain marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales, marketing and distribution infrastructure;
- obtaining and maintaining an adequate price for our product candidates, both in the United States and in foreign countries where our product candidates are commercialized;
- obtaining adequate reimbursement for our product candidates or procedures using our product candidates from payors;
- the convenience and durability of our treatment or dosing regimen;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of our product candidates, or any future product candidates, if approved, including relative to alternative and competing treatments;
- patient demand for any of our product candidates that may be approved;
- addressing any competing technological and market developments;
- the effects of any public health emergencies, including the COVID-19 pandemic, which may result in delays to patient enrollment, patients discontinuing their treatment or follow up visits or changes to trial protocols;
- maintaining, protecting, expanding and enforcing our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when we will be able to generate any meaningful revenue or achieve or maintain profitability, if ever. In addition, our expenses could increase beyond our current expectations if we are required by the FDA or foreign regulatory agencies, to perform studies in addition to those that we currently anticipate, or if there are any delays in any of our or our collaborators' clinical trials or the development of any of our product candidates. Even if one or more of our product candidates are approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate and ongoing compliance efforts.

Even if we are able to generate revenue from the sale of any approved products, we may not become profitable, and we will need to obtain additional funding through one or more equity or debt financings in order to continue operations. Revenue from the sale of any product candidate for which regulatory approval is obtained will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price and whether we own the commercial rights for that territory. If the number of addressable patients is not as large as we anticipate, the indication approved by regulatory authorities is narrower than we expect, the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines or the price and available third-party reimbursement are lower than anticipated, we may not generate significant revenue from sales of such product candidate, even if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis.

Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations and cause a decline in the value of our common stock, all or any of which may seriously harm our business.

We may encounter substantial delays in our clinical trials or may not be able to conduct or complete our clinical trials on the timelines we expect, if at all.

Clinical testing is expensive, time consuming, and subject to uncertainty. We cannot guarantee that any clinical trials will be initiated or conducted as planned or completed on schedule, if at all. We also cannot be sure that submission of an IND or a clinical trial application ("CTA") will result in the FDA or other regulatory authority, as applicable, allowing clinical trials to begin in a timely manner, if at all. Moreover, even if these trials begin, issues may arise that could delay, suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our future clinical trials may not be successful. Events that may prevent successful or timely initiation or completion of clinical trials include:

- inability to generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation or continuation of clinical trials;
- delays in reaching a consensus with regulatory agencies on study design or implementation of the clinical trials;
- delays or failure in obtaining regulatory authorization to commence a trial;
- delays in reaching agreement on acceptable terms with prospective contract research organizations ("CROs") and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- refusal of the FDA to accept data from clinical trials in geographies outside the United States;
- delays in identifying, recruiting and training suitable clinical investigators;
- delays in identifying, recruiting, and enrolling patients who meet the requirements of our clinical trials;
- delays in obtaining required IRB approval at each clinical trial site;
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing;
- insufficient or inadequate supply or quality of product candidates or other materials necessary for use in clinical trials, or delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND or amendment, CTA or amendment, or equivalent foreign application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; or a negative finding from an inspection of our clinical trial operations or study sites;
- developments on trials conducted by competitors for related technology that raise FDA or foreign regulatory authority concerns about risk to patients of the technology broadly; or if the FDA or a foreign regulatory authority finds that the investigational protocol or plan is clearly deficient to meet its stated objectives;
- delays caused by patients withdrawing from clinical trials or failing to return for post-treatment follow-up;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical trial protocols;
- failure to perform in accordance with the FDA's or any other regulatory authority's good clinical practice ("GCP") requirements or applicable regulatory guidelines in other countries;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;

- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- the cost of clinical trials of our product candidates being greater than we anticipate;
- clinical trials of our product candidates producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development of such product candidates;
- transfer of manufacturing processes to larger-scale facilities operated by a contract CMO or by us, and delays or failure by our CMOs or us to make any necessary changes to such manufacturing process;
- adverse impacts from public health emergencies, such as the COVID-19 pandemic;
- third parties being unwilling or unable to satisfy their contractual obligations to us; and
- adverse public perception or regulatory scrutiny of genetic medicine technology may negatively impact the developmental progress or commercial success of products that we develop alone or with collaborators.

Patient enrollment, a determinative factor in the timing of clinical trials, is affected by many factors including the severity of and difficulty of diagnosing the disease under investigation, knowledge of the disease in the medical community and availability of effective diagnostic methods, size and distribution of the patient population and process for identifying subjects, access of patients to medical professionals experienced in their disease, our ability to effectively disseminate information about our clinical trials to the patient population and access of patients to such information, eligibility and exclusion criteria for the trial in question, design of the trial protocol, availability, efficacy of, and our ability to compete with approved and standard of care therapies or other clinical trials for the disease or condition under investigation, perceived risks and benefits of the product candidate under trial or testing, availability of genetic testing for potential patients, efforts to facilitate timely enrollment in clinical trials, patient referral practices of physicians, ability to obtain and maintain subject consent, the risk that enrolled subjects will drop out before completion of the trial, the ability to monitor patients adequately during and after treatment, the time and financial commitments required of patients to enroll in our trials beyond the costs covered by the company, and the proximity and availability of and access to clinical trial sites for prospective patients. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required, or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may seriously harm our business.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Delays in the completion of any clinical trial of our product candidates will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or

lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates and could seriously harm our business.

The limited number of patients who have the diseases for which many of our product candidates are being studied may make it more difficult for us to enroll or complete clinical trials or may result in findings in our clinical trials that do not reach levels of statistical significance sufficient for marketing approval.

Many of the conditions for which we plan to evaluate our current product candidates in clinical trials are rare genetic diseases. Accordingly, there are limited patient pools from which to draw for clinical trials. In addition to the rarity of these diseases, the eligibility criteria of our clinical trials will further limit the pool of available study participants as we will require that patients have specific characteristics that we can measure to assure their disease is either severe enough or not too advanced to include them in a trial. We or our collaborators may not be able to initiate or continue clinical trials on a timely basis or at all for any of our product candidates if we or our collaborators are unable to locate and enroll a sufficient number of eligible patients to participate in the trials as required by applicable regulations or as needed to provide appropriate statistical power for a given trial. Similarly, because many of the conditions we intend to treat are rare in nature, we plan to design and conduct clinical trials utilizing a small number of patients in order to evaluate the safety and therapeutic activity of our product candidates. Conducting trials in smaller subject populations increases the risk that any safety or efficacy issues observed in only a few patients could prevent such trials from reaching statistical significance or otherwise meeting their specified endpoints, which could require us to conduct additional clinical trials, or delay or prevent our product candidates from receiving regulatory approval, which would seriously harm our business.

Research and development of biopharmaceutical products is inherently risky. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized or if they will ever be successfully commercialized.

We are at an early stage of development of our product candidates. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize our product candidates, and we may fail to do so for many reasons, including the following:

- our product candidates may not successfully complete preclinical studies or clinical trials;
- a product candidate may on further study be shown to have harmful side effects or other characteristics that indicate it does not meet applicable regulatory criteria;
- our competitors may develop therapeutics that render our product candidates obsolete or less attractive;
- our competitors may develop platform technologies that render our Therapeutic Vector Evolution platform technology obsolete or less attractive;
- the product candidates and Therapeutic Vector Evolution platform technology that we develop may not be sufficiently covered by intellectual property for which we hold exclusive rights or may be covered by third-party patents or other intellectual property or exclusive rights;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable or commercially attractive;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all;
- if a product candidate obtains regulatory approval, we may be unable to establish sales and marketing capabilities, or successfully market such approved product candidate;
- delays in our clinical development plans due to public health emergencies, such as the COVID-19 pandemic; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors.

If any of these events occur, we or our collaborators may be forced to abandon our development efforts for a product candidate or candidates, which would seriously harm our business. Failure of a product candidate may occur at any stage of preclinical or clinical development, and, because our product candidates and our Therapeutic Vector Evolution platform technology are in an early stage of development, there is a relatively higher risk of failure, and we may never succeed in developing marketable products or generating product revenue.

We may not be successful in our efforts to further develop our Therapeutic Vector Evolution platform technology and current product candidates. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. Each of our product candidates is in the early stages of development and will require significant additional clinical development, management of preclinical, clinical, and manufacturing activities, regulatory approval, adequate manufacturing supply, a commercial organization, and significant marketing efforts before we generate any revenue from product sales, if at all. Any clinical trials that we may conduct may not demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates. If the results of our ongoing or future clinical trials are inconclusive with respect to the efficacy of our product candidates or if we do not meet the clinical endpoints with statistical significance or if there are safety concerns or adverse events associated with our product candidates, we may be prevented or delayed in obtaining marketing approval for our product candidates.

If any of our product candidates successfully completes clinical trials, we generally plan to seek regulatory approval to market our product candidates in the United States, the European Union, and in additional foreign countries where we believe there is a viable commercial opportunity. We have never commenced, compiled or submitted an application seeking regulatory approval to market any product candidate. We may never receive regulatory approval to market any product candidates even if such product candidates successfully complete clinical trials, which would seriously harm our business. To obtain regulatory approval in countries outside the United States, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, purity, potency, chemistry, manufacturing and controls, clinical trials, commercial sales, pricing and distribution of our product candidates. We may also rely on our collaborators or collaboration partners to conduct the required activities to support an application for regulatory approval, and to seek approval, for one or more of our product candidates. We cannot be sure that our collaborators or collaboration partners will conduct these activities successfully or do so within the timeframe we desire. Even if we (or our collaborators or collaboration partners) are successful in obtaining approval in one jurisdiction, we cannot assure you that we will obtain approval in any other jurisdictions. Failure to obtain approval for our product candidates in multiple jurisdictions will seriously harm our business.

Even if we receive regulatory approval to market any of our product candidates, we cannot assure you that any such product candidate will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. Any approval we may obtain could be for indications or patient populations that are not as broad as intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We may also be required to perform additional or unanticipated clinical trials to obtain approval or be subject to additional post-marketing testing requirements to maintain approval. In addition, regulatory authorities may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a REMS. The failure to obtain timely regulatory approval of product candidates, any product marketing limitations or a product withdrawal would seriously harm our business.

Investment in biopharmaceutical product development involves significant risk that any product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval, and become commercially viable. We cannot assure you that we will be able to successfully advance any of our product candidates through the development process or, if approved, successfully commercialize any of our product candidates.

Public health crises such as pandemics or similar outbreaks have affected and could continue to seriously and adversely affect our preclinical and clinical trials, business, financial condition and results of operations.

In response to the COVID-19 pandemic, “shelter in place” orders and other public health guidance measures were implemented, and may be implemented again to the extent eased, across much of the United States and Europe, including in the locations of our offices, clinical trial sites, key vendors and partners. We expect that our clinical development program timelines could be negatively affected by the COVID-19 pandemic or residual effects thereof, which could seriously harm our business. Further, in response to COVID-19 health guidance measures, we have implemented a work-from-home policy for all staff members excluding those working in certain laboratory and manufacturing functions and those necessary to maintain minimum basic operations. Our increased reliance on personnel working from home may negatively impact productivity, or disrupt, delay or otherwise seriously harm our business. For example, with our personnel working from home, some of our research activities that require our personnel to be in our laboratories will be delayed.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could seriously harm our business.

The ability of the FDA to review and/or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA’s ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA’s ability to perform routine functions. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs and biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the COVID-19, the FDA postponed most inspections of domestic and foreign manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations of domestic facilities where feasible, the FDA has continued to monitor and implement changes to its inspectional activities to ensure the safety of its employees and those of the firms it regulates as it adapts to the evolving COVID-19 pandemic, and any resurgence of the virus or emergence of new variants may lead to further inspectional delays. Regulatory authorities outside the United States may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could seriously harm our business.

Our clinical trials may fail to demonstrate substantial evidence of the safety and efficacy of our product candidates, which would prevent, delay or limit the scope of regulatory approval and commercialization, which could seriously harm our business.

Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we or our collaborators must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that our product candidates are both safe and effective for use in each target indication. Further, because our product candidates are subject to regulation as biological drug products, we will need to demonstrate that they are safe, pure, and potent for use in their target indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies of our product candidates may not be predictive of the results of early-stage or later-stage clinical trials, and results of early clinical trials of our product candidates may not be predictive of the results of later-stage

clinical trials. The results of clinical trials in one set of patients or disease indications may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization.

We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. We cannot be certain that our ongoing and planned clinical trials or any other future clinical trials will be successful. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could seriously harm our business.

In addition, even if such clinical trials are successfully completed, we cannot assure you that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant additional resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for any of our product candidates, the terms of such approval may limit the scope and use of our product candidate, which may also limit its commercial potential.

Interim, "top-line" and preliminary data from studies or trials that we announce or publish from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we publicly disclose top-line or preliminary data from preclinical studies and clinical trials, which are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the top-line or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, top-line and preliminary data should be viewed with caution until the final data are available.

From time to time, we also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Adverse differences between preliminary, top-line or interim data and final data could seriously harm our business.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what we determine to be material information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure. Any information we determine not to disclose may ultimately be deemed significant by you or others with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product candidate or our business. If the top-line or interim data that we report differ from final results, or if others, including regulatory authorities, disagree with the

conclusions reached, our ability to obtain approval for, and commercialize, product candidates may be harmed, which could seriously harm our business.

We may not be successful in our efforts to continue to create a pipeline of product candidates or to develop commercially successful products. If we fail to successfully identify and develop additional product candidates, our commercial opportunity may be limited, which could seriously harm our business.

One of our strategies is to identify and pursue preclinical and clinical development and commercialization of additional product candidates through our Therapeutic Vector Evolution platform technology. Our Therapeutic Vector Evolution platform technology may not produce a pipeline of viable product candidates, or our competitors may develop platform technologies that render our Therapeutic Vector Evolution platform technology obsolete or less attractive. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make them unmarketable or unlikely to receive marketing approval. Identifying, developing and obtaining regulatory approval and commercializing additional product candidates will require substantial funding and is prone to the risks of failure inherent in drug development. If we are unable to successfully identify, acquire, develop and commercialize additional product candidates, our commercial opportunity may be limited, which could seriously harm our business.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from major pharmaceutical companies and biotechnology companies worldwide. 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.

There are a number of large pharmaceutical and biotechnology companies that are currently pursuing the development of products for the treatment of the indications for which we have product candidates, including wet AMD, diabetic macular adema, cystic fibrosis lung disease, Fabry disease, XLRP, and choroideremia. Certain of our competitors have commercially approved products for the treatment of the diseases that we are pursuing or may pursue in the future, including Regeneron, Roche, Sanofi, Takeda and Vertex. These drugs are well established therapies and are widely accepted by physicians, patients and third-party payors, which may make it difficult to convince these parties to switch to our product candidates. Companies that we are aware are developing therapeutics in the ophthalmology, cardiology and pulmonology disease areas include large companies with significant financial resources, such as Allergan, Novartis, Pfizer, Roche, Sanofi, Takeda and Vertex, and biopharmaceutical companies such as Adverum, Amicus, Kodiak Sciences, Krystal, REGENXBIO, Sangamo, and Spirovant. In addition to competition from other companies targeting ophthalmology, pulmonology, and cardiology any products we may develop may also face competition from other types of therapies, such as gene-editing therapies and drug delivery devices.

Many of our current or potential competitors, either alone or with their strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our product candidates. 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 any products that we may develop. Furthermore, currently approved

products could be discovered to have application for treatment of ophthalmology, cardiology and pulmonology indications, which could give such products significant regulatory and market timing advantages over any of our product candidates. Our competitors also may obtain FDA, EMA or other regulatory approval for their products more rapidly than we may obtain approval for ours. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing against competitors any product candidates we may develop.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any product candidates we may develop, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing and commercial support infrastructure to sell, or participate in sales activities with our collaborators for some of our product candidates if and when they are approved.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force or reimbursement specialists is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and other commercialization capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our commercialization personnel.

Factors that may inhibit our efforts to commercialize any approved product on our own include:

- our inability to recruit and retain adequate numbers of effective sales, marketing, reimbursement, compliance, customer service, medical affairs and other support personnel;
- our inability to recruit and build a commercial infrastructure;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future approved products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement, and other acceptance by payors;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercialization organization.

If we enter into arrangements with third parties to perform sales, marketing, commercial support and distribution services, our product revenue or the profitability of product revenue may be lower than if we were to market and sell any products we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates if approved and our business would be seriously harmed.

Even if any product candidates we develop receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in pivotal clinical trials and published in peer-reviewed journals;
- the potential and perceived advantages compared to alternative treatments;
- the ability to offer our products for sale at competitive prices;
- the ability to offer appropriate patient access programs, such as co-pay assistance;
- sufficient third-party coverage or reimbursement;
- the extent to which physicians recommend our products to their patients;
- convenience and ease of dosing and administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA, EMA or other regulatory agencies;
- product labeling or product insert requirements of the FDA, EMA or other comparable foreign regulatory authorities, including any limitations, contraindications or warnings contained in a product's approved labeling;
- restrictions on how the product is distributed;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support; and
- the prevalence and severity of any side effects.

If any product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenue, and we may not become profitable and our business could be seriously harmed.

Risks Related to Manufacturing

Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise seriously harm our business.

We currently have a development, manufacturing and testing agreement and cooperation agreement with Catalent to manufacture supplies of our product candidates in the future. Our product candidates require processing steps that are more complex than those required for most chemical and protein pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory, which could delay or prevent the initiation of clinical trials or receipt of regulatory approvals. We may encounter problems

achieving adequate quantities and quality of clinical-grade materials that meet FDA, or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs.

In addition, FDA and other comparable foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA or other comparable foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise seriously harm our business.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing 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 process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies, which could limit our access to additional attractive development programs. Problems in third-party manufacturing processes or facilities also could restrict our ability to meet market demand for our products. Additionally, should our agreement with Catalent or agreements with other parties with whom we have manufacturing agreements be terminated for any reason, there are a limited number of manufacturers who would be suitable replacements, and it would take a significant amount of time to transition the manufacturing to a replacement.

Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process may delay or disrupt our commercialization efforts.

Before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA to market our product using the manufacturing process and facility we proposed in our marketing application. In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before any of our product candidates can obtain marketing approval, if ever. In order to obtain approval of a BLA for our product candidates, we will need to ensure that all of our manufacturing processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

Delays in developing our manufacturing capabilities or failure to achieve operating efficiencies from it may require us to devote additional resources and management time to manufacturing operations and may delay our product development timelines.

We have recently completed the build out of approximately 17,000 square feet of laboratory and manufacturing space at our headquarters in Emeryville, California, a large portion of which we plan to devote to manufacturing activities for our clinical trials under cGMP. We may face delays in the production of clinical supply at our manufacturing facility and cannot guarantee when our facility will be able to produce sufficient quantities of product candidates needed to support our planned clinical trials. Any delays in developing our internal manufacturing capabilities may disrupt or delay the supply of our product candidates if we have not maintained a sufficient back-up supply of such product candidates through third-party manufacturers. Moreover, changing manufacturing facilities during the clinical development process may also require that we or our collaborators conduct additional studies, make notifications to regulatory authorities, make additional filings to regulatory authorities, and obtain regulatory authority approval for the

new facilities, which may be delayed or which we may never receive. We will further need to comply with the FDA's and applicable foreign regulatory authorities' cGMP requirements for the production of product candidates for clinical trials and, if approved, commercial supply, and will be subject to FDA and comparable foreign regulatory authority inspection. These requirements include the qualification and validation of our manufacturing equipment and processes. We may not be able to develop or acquire the internal expertise and resources necessary for compliance with these requirements.

In order to develop internal manufacturing expertise, we may be forced to devote greater resources and management time than anticipated, particularly in areas relating to operations, quality, regulatory, facilities and information technology. We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing processes. If we experience unanticipated employee shortage or turnover in any of these areas, we may not be able to effectively manage our ongoing manufacturing operations and we may not achieve the operating efficiencies that we anticipate from developing these capabilities, which may negatively affect our product development timeline or result in difficulties in maintaining compliance with applicable regulatory requirements. Any such problems could result in the delay, prevention or impairment of clinical development and commercialization of our product candidates and would seriously harm our business.

We currently rely and expect to continue to rely on third parties to conduct product manufacturing for certain of our product candidates, and these third parties may not perform satisfactorily.

Although we are in process of expanding internal manufacturing capabilities, we currently rely, and expect to continue to rely, on third parties for the production of some of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities. The facilities used by us and our contract manufacturers to manufacture certain of our product candidates must be reviewed by the FDA pursuant to inspections that will be conducted after we submit a BLA to the FDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the cGMP for manufacture of our products. If we or our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, we will not be able to obtain and/or maintain regulatory approval for our products as manufactured at their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

In addition, we rely on additional third parties to manufacture plasmids used in the manufacture of our product candidates and to perform quality testing, and reliance on these third parties entails risks to which we would not be subject if we manufactured the plasmids ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or service provider.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates. Some of these events could be the basis for FDA or European Union Member State regulatory authority action, including injunction, recall, seizure or total or partial suspension of product manufacture.

Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our research studies, preclinical, and clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process, such as plasmids, are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could seriously harm our business.

We depend on third-party suppliers for key raw materials used in our manufacturing processes, and the loss of these third-party suppliers or their inability to supply us with adequate raw materials could seriously harm our business.

We rely on third-party suppliers for the raw materials required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of raw materials involve several risks, including limited control over pricing, availability, quality and delivery schedules. As a small company, our negotiation leverage is limited, and we are likely to get lower priority than our competitors who are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements. Any interruption in supply of raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supplier in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would seriously harm our business.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

The regulatory approval processes of the FDA, EMA and comparable foreign regulatory authorities are lengthy, expensive, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, we will be unable to generate product revenue and our business will be seriously harmed.

We and any collaborators are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Foreign regulatory authorities impose similar requirements. The time required to obtain approval by the FDA and comparable foreign regulatory authorities is unpredictable, typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. We have not submitted for or obtained regulatory approval for any product candidate. We and any collaborators must complete additional preclinical or nonclinical studies and clinical trials to demonstrate the safety and efficacy of our product candidates in humans to the satisfaction of the regulatory authorities before we will be able to obtain these approvals, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including but not limited to the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design, implementation or results of our or our collaborators' clinical trials;
- the FDA or comparable foreign regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use of our products;
- the population studied in the clinical program may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;
- we or our collaborators may be unable to demonstrate to the FDA, or comparable foreign regulatory authorities that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our or our collaborators' interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other submission or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our or our collaborators' clinical data insufficient for approval.

This lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market any of our product candidates, which would seriously harm our business.

In addition, even if we or our collaborators were to obtain approval, regulatory authorities may approve any of our product candidates for fewer or more limited indications than we request, may impose significant limitations in the form of narrow indications, warnings, or a REMS. Regulatory authorities or other authorities responsible for pricing negotiations may not approve the price we or our collaborators intend to charge for products we may develop, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Any of the foregoing scenarios could seriously harm our business.

We may attempt to secure approval from the FDA or comparable foreign regulatory authorities through the use of accelerated approval pathways. If we are unable to obtain such approval, we may be required to conduct additional clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA may seek to withdraw accelerated approval.

We may in the future seek an accelerated approval for one or more of our product candidates. Under the accelerated approval program, the FDA may grant accelerated approval to a product candidate designed to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on

irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, confirmatory studies to verify and describe the drug's clinical benefit. If such confirmatory studies fail to confirm the drug's clinical benefit, or if the sponsor fails to conduct required confirmatory studies in a timely manner, the FDA may withdraw its approval of the drug on an expedited basis. In addition, in December 2022, President Biden signed an omnibus appropriations bill to fund the U.S. government through fiscal year 2023. Included in the omnibus bill is the Food and Drug Omnibus Reform Act of 2022, which among other things, provided FDA new statutory authority to mitigate potential risks to patients from continued marketing of ineffective drugs previously granted accelerated approval. Under these provisions, the FDA may require a sponsor of a product seeking accelerated approval to have a confirmatory trial underway prior to such approval being granted.

Prior to seeking accelerated approval for any of our product candidates, we intend to seek feedback from the FDA and will otherwise evaluate our ability to seek and receive accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit a BLA for accelerated approval or any other form of expedited development, review or approval. Furthermore, if we decide to submit an application for accelerated approval for any of our product candidates, there can be no assurance that such submission or application will be accepted or that any expedited development, review or approval will be granted on a timely basis, or at all. The FDA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our product candidate would result in a longer time period to commercialization of such product candidate, if any, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

Even if we or our collaborators obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

If one of our product candidates is approved, it will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post- market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA and comparable foreign regulatory authority requirements, including ensuring that quality control and manufacturing procedures conform to cGMP regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any approved marketing application. Accordingly, we and others with whom we work must continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, and quality control.

We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs and biologics are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products "off-label" for indications or uses for which they do not have approval, though we may share truthful and not misleading information that is otherwise consistent with our product's approved labeling. The holder of an approved application must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical studies to verify the safety and efficacy of our products in general or in specific patient subsets. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval or label restrictions.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our manufacturing facility or our contract manufacturers' facilities; or
- seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, our business will be seriously harmed.

Moreover, the policies of the FDA and of other regulatory authorities may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

We have received Fast Track designation for 4D-310 for the treatment of Fabry disease, and for 4D-125 for the treatment of patients with inherited retinal dystrophies due to defects in the RPGR gene, including XLRP, and we may seek Fast Track designation for certain future product candidates. However, we may not be able to obtain such designations, and there is no guarantee that 4D-310 or 4D-125 will experience a faster regulatory review or obtain regulatory approval.

If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this disease condition, the product sponsor may apply for Fast Track designation. The sponsor of a Fast Track product candidate has opportunities for more frequent interactions with the applicable FDA review team during product development and, once a BLA is submitted, the application may be eligible for priority review. A BLA for a Fast Track product candidate may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. We have received Fast Track designation for 4D-310 for the treatment of Fabry disease, and for 4D-125 for the treatment of patients with inherited retinal dystrophies due to defects in the RPGR gene, including XLRP and we may receive Fast Track designation for other product candidates in the future; however, we may not experience a faster development, review or approval process, and receipt of the designation does not increase the likelihood that the FDA will approve 4D-310 or 4D-125 for any indication. In addition, the FDA may rescind the Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

We have received orphan drug designation for 4D-110 for the treatment of choroideremia as well as 4D-125 for the treatment of patients with inherited retinal dystrophies due to defects in the RPGR gene, including XLRP, and for 4D-310 for the treatment of Fabry disease, and we may seek orphan drug designation for certain future product candidates. However, we may be unable to obtain such designations or to maintain the benefits associated with orphan drug designation, including market exclusivity, which may cause our revenue, if any, to be reduced.

We have received orphan drug designation in the United States for 4D-110 for the treatment of choroideremia and for 4D-310 for the treatment of Fabry disease. In the European Union, we have received orphan drug designation for 4D-125 for the treatment of patients with inherited retinal dystrophies due to defects in the RPGR gene, including XLRP. Although we may seek orphan product designation for some or all of our other product candidates, we may never receive such designations. Under the Orphan Drug Act, the FDA may designate a drug or biologic product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation must be requested before submitting a BLA. In the European Union, the EMA's Committee for Orphan Medicinal Products ("COMP"), grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA.

In addition, if a product receives the first FDA approval for the disease or condition for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same disease or condition for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for a disease or condition broader than the orphan designated disease or condition and may be lost if the FDA later determines that the request for designation was materially defective. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Even if we obtain orphan drug designation, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug or biologic nor gives the drug or biologic any advantage in the regulatory review or approval process.

If the product candidates that we or our collaborators may develop receive regulatory approval in the United States or another jurisdiction, they may never receive approval in other jurisdictions,

which would limit market opportunities for such product candidates and seriously harm our business.

Approval of a product candidate in the United States by the FDA or by the requisite regulatory agencies in any other jurisdiction does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions. The approval process varies among countries and may limit our or our collaborators' ability to develop, manufacture, promote and sell product candidates internationally. Failure to obtain marketing approval in international jurisdictions would prevent the product candidates from being marketed outside of the jurisdictions in which regulatory approvals have been received. In order to market and sell product candidates in the European Union and many other jurisdictions, we and our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and may involve additional preclinical studies or clinical trials both before and after approval. In many countries, any product candidate for human use must be approved for reimbursement before it can be approved for sale in that country. In some cases, the intended price for such product is also subject to approval. Further, while regulatory approval of a product candidate in one country does not ensure approval in any other country, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. If we or our collaborators fail to comply with the regulatory requirements in international markets or to obtain all required marketing approvals, the target market for a particular potential product will be reduced, which would limit our ability to realize the full market potential for the product and seriously harm our business.

Enacted and future healthcare legislation may increase the difficulty and cost for us to obtain marketing approval of and to commercialize our product candidates and may affect the prices we may set.

In the United States, the European Union and other jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes to the healthcare system that could affect our future results of operations. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively the "ACA") was enacted, which substantially changed the way healthcare is financed by both governmental and private payors. Among the provisions of the ACA, those of greatest importance to the pharmaceutical and biotechnology industries include the following:

- an annual, non-deductible fee payable by any entity that manufactures or imports certain branded prescription drugs and biologic agents (other than those designated as orphan drugs), which is apportioned among these entities according to their market share in certain government healthcare programs;
- new requirements to report certain financial arrangements with physicians and teaching hospitals, including reporting "transfers of value" made or distributed to prescribers and other healthcare providers and reporting investment interests held by physicians and their immediate family members;
- an increase to the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and an extension the rebate program to individuals enrolled in Medicaid managed care organizations;
- a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- a licensure framework for follow-on biologic products;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and

- establishment of a Center for Medicare & Medicaid Innovation ("CMMI") at the Centers for Medicare & Medicaid Services ("CMS") to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

Since its enactment, there have been judicial, U.S. Congressional and executive branch challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. It is unclear how other healthcare reform measures, if any, will impact our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions of Medicare payments to providers. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, CMS may develop new payment and delivery models, such as bundled payment models. In addition, recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under government payor programs, and review the relationship between pricing and manufacturer patient assistance programs. Most recently, on August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). On August 29, 2023, CMS announced the list of the first ten drugs that will be subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. In addition, in response to an executive order from the Biden administration, the Department of Health and Human Services released a report outlining three new models for testing by CMMI which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. We also expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures and could seriously harm our business.

Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could seriously harm our business. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our product candidates or put pressure on our product pricing. Furthermore, there has

been increased interest by third-party payors and governmental authorities in reference pricing systems and publication of discounts and list prices. Prescription drugs and biological products that are in violation of these requirements will be included on a public list. These reforms could reduce the ultimate demand for our product candidates or put pressure on our product pricing and could seriously harm our business.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our product candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product candidates, if approved. In markets outside of the United States and European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or judicial action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Even if we are able to commercialize any product candidates, due to unfavorable pricing regulations and/or third-party coverage and reimbursement policies, we may not be able to offer such products at competitive prices which would seriously harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to successfully commercialize any products that we may develop also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Government authorities currently impose mandatory discounts for certain patient groups, such as Medicare, Medicaid and Veterans Affairs (“VA”) hospitals, and may seek to increase such discounts at any time. Future regulation may negatively impact the price of our products, if approved. Increasingly, other third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. For genetic medicine and other products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs.

We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, that the level of reimbursement will be sufficient.

Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. In order to get reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the medicine is approved by the FDA, EMA or other comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could seriously harm our business.

Even if we obtain FDA approval for any of our product candidates, we may never obtain approval or commercialize such products outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval procedures vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approvals could result in significant delays, difficulties and costs for us and may require additional preclinical studies or clinical trials which would be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. Satisfying these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. In addition, our failure to obtain regulatory approval in any country may delay or have negative effects on the process for regulatory approval in other countries. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, our ability to realize the full market potential of our products will be harmed.

We currently have no sales organization. If we are unable to establish sales capabilities on our own or through third parties, we may not be able to market and sell any products effectively, if approved, or generate product revenue.

We currently do not have a marketing or sales organization. In order to commercialize any product, if approved, in the United States and foreign jurisdictions, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these

services, and we may not be successful in doing so. In advance of any of our product candidates receiving regulatory approval, we expect to establish a sales organization with technical expertise and supporting distribution capabilities to commercialize each such product candidate, which will be expensive and time-consuming. We have no prior experience in the marketing, sale and distribution of pharmaceutical products, and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain, and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may choose to collaborate with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. If we are unable to enter into such arrangements on acceptable terms or at all, we may not be able to successfully commercialize our product candidates. If we are not successful in commercializing products, either on our own or through arrangements with one or more third parties, we may not be able to generate any future product revenue and we would incur significant additional losses and our business would be seriously harmed.

Our business operations and current and future relationships with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers will be subject to applicable healthcare regulatory laws, which could expose us to penalties and seriously harm our business.

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payors, patient organizations and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute our product candidates, if approved. Such laws include:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or providing any remuneration (including any kickback, bribe, or certain rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under any U.S. federal healthcare program, such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the U.S. federal civil and criminal false claims and civil monetary penalties laws, including the civil federal False Claims Act, which can be enforced through civil whistleblower or *qui tam* actions, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, to the U.S. federal government, claims for payment or approval that are false or fraudulent, knowingly making, using or causing to be made or used, a false record or statement material to a false or fraudulent claim, or from knowingly making a false statement to avoid, decrease or conceal an obligation to pay money to the U.S. federal government. Pharmaceutical manufacturers can cause false claims to be presented to the U.S. federal government by engaging in impermissible marketing practices, such as the off-label promotion of a product for an indication for which it has not received FDA approval. In addition, the government may assert that a claim including items and services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement, in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the U.S. federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation;

- the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices;
- the U.S. Public Health Service Act, which prohibits, among other things, the introduction into interstate commerce of a biological product unless a biologics license is in effect for that product;
- the U.S. Physician Payments Sunshine Act and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to CMS information related to certain payments and other transfers of value to physicians (as defined by statute), certain other non-physician practitioners (including physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiology assistants, and certified nurse midwives) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- analogous U.S. state laws and regulations, including: state anti-kickback and false claims laws, which may apply to our business practices, including but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by any third-party payor, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information, which requires tracking gifts and other remuneration and items of value provided to healthcare professionals and entities; and state and local laws requiring the registration of pharmaceutical sales representatives; and
- similar healthcare laws in the European Union and other jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers.

We may also be subject to additional federal laws, such as the U.S. Foreign Corrupt Practices Act of 1977, as amended, which prohibits, among other things, U.S. companies and their employees and agents from authorizing, promising, offering, or providing, directly or indirectly, corrupt or improper payments or anything else of value to foreign government officials, employees of public international organizations and foreign government owned or affiliated entities, candidates for foreign political office, and foreign political parties or officials thereof, and federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Ensuring that our internal operations and future business arrangements with third parties comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including certain of our advisory board arrangements with physicians, some of whom are compensated in the form of stock or stock options, do not comply with current or future statutes, regulations, agency guidance or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that may apply to us, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs and imprisonment, which could affect our ability to operate our business. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

We use and generate materials that may expose us to material liability.

Our research programs involve the use of hazardous materials and chemicals. We are subject to foreign, federal, state and local environmental and health and safety laws and regulations governing, among other matters, the use, manufacture, handling, storage and disposal of hazardous materials and waste products such as human tissue samples that may have the potential to transmit diseases. We may incur significant costs to comply with these current or future environmental and health and safety laws and regulations. In addition, we cannot completely eliminate the risk of contamination or injury from hazardous materials and may incur material liability as a result of such contamination or injury. In the event of an accident, an injured party may seek to hold us liable for any damages that result. Any liability could exceed the limits or fall outside the coverage of our workers' compensation, property and business interruption insurance and we may not be able to maintain insurance on acceptable terms, if at all. We currently carry no insurance specifically covering environmental claims.

Compliance with governmental regulations regarding the treatment of animals used in research could increase our operating costs, which would adversely affect the commercialization of our products.

The Animal Welfare Act ("AWA") is the federal law that covers the treatment of certain animals used in research. Currently, the AWA imposes a wide variety of specific regulations that govern the humane handling, care, treatment and transportation of certain animals by producers and users of research animals, most notably relating to personnel, facilities, sanitation, cage size, and feeding, watering and shipping conditions. Third parties with whom we contract are subject to registration, inspections and reporting requirements under the AWA. Furthermore, some states have their own regulations, including general anti-cruelty legislation, which establish certain standards in handling animals. Comparable rules, regulations, and or obligations exist in many foreign jurisdictions. If we or our contractors fail to comply with regulations concerning the treatment of animals used in research, we may be subject to fines and penalties and adverse publicity, and our operations could be adversely affected.

Risks Related to Our Reliance on Third Parties

We expect to rely on third parties to conduct our clinical trials and some aspects of our research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, clinical data assessments and analysis organizations, medical institutions and clinical investigators, to conduct some aspects of our research, preclinical testing and clinical trials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities, but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCP for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We are also required to register ongoing clinical trials and to post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for any product candidates we may develop and will not be able to, or may be delayed in our efforts to, successfully commercialize our medicines.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development, marketing approval or commercialization of any product candidates, producing additional losses and depriving us of potential product revenue.

We rely on various CROs to obtain NHPs for use in vector discovery and preclinical development work. In February 2023, we were informed that Charles River Laboratories ("Charles River"), one of our primary suppliers of NHPs, received a subpoena from the United States Department of Justice with respect to its importation of NHPs from Cambodia. Charles River reported that it has voluntarily suspended NHP shipments from Cambodia at this time. If we are unable to secure adequate supply of NHPs from Charles River or other CROs, or the shortage of NHPs causes the price of NHPs to rise substantially, certain of our vector discovery projects and preclinical development efforts will be delayed, and the cost of conducting discovery projects and preclinical development activities may substantially increase. Such delays or cost increases could materially adversely affect our discovery and preclinical development activities and our business.

We may depend on collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to realize the market potential of those product candidates.

We have sought, and may in the future seek third-party collaborators for the research, development and commercialization of certain product candidates we may develop. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies, regional, national and international pharmaceutical companies, biotechnology companies and academic institutions. If we enter into any such arrangements with any third parties, we will likely have shared or limited control over the amount and timing of resources that our collaborators dedicate to the development or potential commercialization of any product candidates we may seek to develop with them. Our ability to generate revenue from these arrangements with commercial entities will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our product candidates we may develop, pose the following risks to us:

- collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not properly obtain, maintain, enforce or defend intellectual property or proprietary rights relating to our product candidates or may use our proprietary information in such a way as to expose us to potential litigation or other intellectual property related proceedings, including proceedings challenging the scope, ownership, validity and enforceability of our intellectual property;
- collaborators may own or co-own intellectual property covering our product candidates that result from our collaboration with them, and in such cases, we may not have the exclusive right to commercialize such intellectual property or such product candidates;
- disputes may arise with respect to the ownership of intellectual property developed pursuant to collaborations;
- we may need the cooperation of our collaborators to enforce or defend any intellectual property we contribute to or that arises out of our collaborations, which may not be provided to us;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborators may decide not to pursue development and commercialization of any product candidates we develop or may elect not to continue or renew development or

commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, 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 independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if 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;
- collaborators with marketing and distribution rights to one or more product candidates may not commit sufficient resources to the marketing and distribution of such product candidates;
- we may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control;
- collaborators may undergo a change of control and the new owners may decide to take the collaboration in a direction which is not in our best interest;
- collaborators may become party to a business combination transaction and the continued pursuit and emphasis on our development or commercialization program by the resulting entity under our existing collaboration could be delayed, diminished or terminated;
- collaborators may become bankrupt, which may significantly delay our research or development programs, or may cause us to lose access to valuable technology, devices, materials, know-how or intellectual property of the collaborator relating to our products and product candidates;
- key personnel at our collaborators may leave, which could negatively impact our ability to productively work with our collaborators;
- collaborations may require us to incur short and long-term expenditures, issue securities that dilute our stockholders, or disrupt our management and business;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates or our Therapeutic Vector Evolution platform technology; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

We may face significant competition in seeking appropriate collaborations. Recent business combinations among biotechnology and pharmaceutical companies have resulted in a reduced number of potential collaborators. In addition, the negotiation process is time-consuming and complex, and we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate or delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop product candidates or bring them to market and generate product revenue which could seriously harm our business.

If we enter into collaborations to develop and potentially commercialize any product candidates, we may not be able to realize the benefit of such transactions if we or our collaborator elect not to exercise the rights granted under the agreement or if we or our collaborator are unable to successfully integrate a product candidate into existing operations and company culture. In addition, if our agreement with any of our collaborators terminates, our access to technology and intellectual property licensed to us by that collaborator may be restricted or terminate entirely, which may delay our continued development of our product candidates utilizing the collaborator's technology or intellectual property or require us to stop

development of those product candidates completely. We may also find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. Any collaborator may also be subject to many of the risks relating to product development, regulatory approval, and commercialization described in this “Risk Factors” section, and any negative impact on our collaborators may adversely affect us.

Risks Related to Our Intellectual Property

Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Our commercial success depends in part on our ability to obtain and maintain patent protection and trade secret protection for our product candidates, proprietary technologies and their uses as well as our ability to operate without infringing upon the proprietary rights of others. We generally seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates, proprietary technologies and their uses that are important to our business. Our patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issue from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our patent applications or those of our licensors will result in additional patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents issued will not be infringed, designed around or invalidated by third parties. Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. Further, we have three granted patents (U.S. patent nos. 11,136,557, 11,634,691 and 10,988,519) and four pending patent applications (U.S. patent application nos. 16/486,681, 17,468,290, 17/156,023 and 18/184,184), that were made with government support, that may be subject, under certain circumstances, to march-in-rights under 35 U.S.C. 203, which is a right that allows the government, in certain limited circumstances, to force a party with a license to intellectual property funded, at least in part, by the government, to grant a license to such property to another entity. U.S. patent nos. 11,136,557 and 11,634,691 were made with the support of U.C. Berkeley and relate to our A101 vector of our 4D-710 and 4D-725 product candidates. U.S. patent no. 10,988,519 was made with the support of University of Pennsylvania and relates to our short-form human complement factor H (sCFH) payload of our 4D-175 product candidate. The degree of future protection for our proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our product candidates and proprietary technologies and erode or negate any competitive advantage we may have, which could seriously harm our business.

We and our licensors have applied, and we intend to continue applying, for patents covering aspects of our product candidates, proprietary technologies and their uses that we deem appropriate. However, we may not be able to apply for patents on certain aspects of our current or future product candidates, proprietary technologies and their uses in a timely fashion, at a reasonable cost, in all jurisdictions, or at all, and any potential patent coverage we obtain may not be sufficient to prevent substantial competition.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators or licensors will be successful in protecting our product candidates, proprietary technologies and their uses by obtaining and defending patents. These risks and uncertainties include the following:

- the U.S. Patent and Trademark Office (“USPTO”) and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;

- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use and sell our potential product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict with our patent applications, either by claiming the same methods or devices or by claiming subject matter that could dominate our patent position;
- any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our product candidates, proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our licensors' patent applications for any patent application with an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

The patent prosecution process is also expensive and time-consuming, 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. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

The patent position of biopharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. Moreover, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents, if issued, patents obtained by our collaborators or the patent rights that we license from others, may be challenged in the courts or patent offices in the United States and abroad. Once granted, patents may remain open to opposition, interference, re-examination, post-grant review, inter partes review, nullification or derivation action in court or before patent offices or similar proceedings for a given period after allowance or grant, during which time third parties can raise objections against such initial grant. Such challenges 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 products or product candidates, or limit the duration of the patent protection of our product candidates. 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 intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any of our patents covering our product candidates are invalidated or found unenforceable, or if a court found that valid, enforceable patents held by third parties covered one or more of our product candidates, our competitive position could be harmed or we could be required to incur significant expenses to enforce or defend our rights. If we initiate lawsuits to protect or enforce our patents, or litigate against third-party claims, such proceedings would be expensive and would divert the attention of our management and technical personnel.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates;
- any of our pending patent applications or those of our licensors may issue as patents;
- others will not or may not be able to make, use, offer to sell, or sell products that are the same as or similar to our own but that are not covered by the claims of the patents that we own or license;
- we will be able to successfully commercialize our product candidates on a substantial scale, if approved, before the relevant patents that we own or license expire;
- we or our licensors were the first to make the inventions covered by each of the patents and pending patent applications that we own or license;
- we or our licensors were the first to file patent applications for these inventions;
- others will not develop similar or alternative technologies that do not infringe the patents we own or license;
- any of the patents we own or license will be found to ultimately be valid and enforceable;
- any patents issued to us or our licensors will provide a basis for an exclusive market for our commercially viable products or will provide us with any competitive advantages;
- a third party may not challenge the patents we own or license and, if challenged, a court would hold that such patents are valid, enforceable and infringed;
- we may develop or in-license additional proprietary technologies that are patentable;
- the patents of others will not have an adverse effect on our business;
- our competitors do not conduct research and development activities in countries where we do not have enforceable patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we will develop additional proprietary technologies or product candidates that are separately patentable; or
- our commercial activities or products will not infringe upon the patents of others.

Where we obtain licenses from or collaborate with third parties, 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, or such activities, if controlled by us, may

require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could seriously harm our business.

Furthermore, our owned and in-licensed intellectual property rights may be subject to a reservation of rights by one or more third parties. For example, the research resulting in certain of our in-licensed patent rights and technology was funded in part by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could seriously harm our business.

The lives of our patents may not be sufficient to effectively protect our product candidates and business.

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after its first effective non-provisional filing date. Although various extensions may be available, the life of a patent, and the protection it affords, are limited. Even if patents covering our product candidates, proprietary technologies and their uses are obtained, once the patent life has expired, we may be open to competition. In addition, although upon issuance in the United States a patent's life can be extended based on certain delays caused by the USPTO and clinical development, this extension can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. 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. If we do not have sufficient patent life to protect our product candidates, proprietary technologies and their uses, our business would be seriously harmed.

If we are unable to protect the confidentiality of our trade secrets, our business would be seriously harmed.

We rely on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information to maintain our competitive position. We have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with employees and consultants. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant or third party with authorized access. Our security measures may not prevent an employee, consultant, collaborator or third party from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our product candidates that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated

a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets will over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions.

Though our agreements with third parties typically restrict the ability of our employees, collaborators, licensors, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we have relied on, and in future expect to rely on third parties in the development, manufacture, and distribution of our product candidates and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets 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. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

Our rights to develop and commercialize our product candidates are subject in part to the terms and conditions of licenses granted to us by others, and the patent protection, prosecution and enforcement for some of our product candidates may be dependent on our licensors.

We currently are reliant upon licenses of certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology, including technology related to our product candidates. For example, we rely on our exclusive license agreements with i) U.C. Berkeley for certain rights with respect to the intellectual property covering certain compositions of matter and methods of use of certain AAV variants related to our 4D-710 and 4D-725 product candidates, and ii) University of Pennsylvania for certain rights with respect to the intellectual property covering certain compositions of matter and methods of use of the short-form human complement factor H (sCFH) payload related to our 4D-175 product candidate. These and other licenses we may enter into in the future may not provide adequate rights to use such intellectual property and technology in all relevant fields of use or in all territories in which we may wish to develop or commercialize our technology and product candidates in the future. As a result, we may not be able to develop and commercialize our technology and product candidates in fields of use and territories for which we are not granted rights pursuant to such licenses.

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 seriously harm our business.

In some circumstances, we may not have the right to control the preparation, filing, prosecution and enforcement 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 our licensors or collaborators will prosecute, maintain, enforce and defend such intellectual property rights in a manner consistent with the best interests of our business, including by taking reasonable measures to protect the confidentiality of know-how and trade secrets, or by paying all applicable prosecution and maintenance fees related to intellectual property registrations for any of our product candidates. We also cannot be certain that our licensors have drafted or prosecuted the patents and patent applications licensed to us in compliance with applicable laws and

regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such applications. If they fail to do so, this could cause us to lose rights in any applicable intellectual property that we in-license, and as a result our ability to develop and commercialize products or product candidates may be adversely affected and we may be unable to prevent competitors from making, using and selling competing products.

Our current licenses, and our future licenses likely will, impose various royalty payments, milestones, and other obligations on us. If we fail to comply with any of these obligations, we may be required to pay damages and the licensor may have the right to terminate the license. Termination by the licensor would cause us to lose valuable rights, and could prevent us from developing and commercializing our product candidates and proprietary technologies. Our business would be seriously harmed if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any current or future licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of royalty obligations we would be required to pay on the sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in product candidates that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize any product candidates, we may be unable to achieve or maintain profitability.

If our trademarks and trade names, whether registered in the future or unregistered now, are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Any trademarks we may register in the future or any current unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names, to the extent any are registered, to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could seriously harm our business.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make genetic medicine products that are similar to our product candidates or utilize similar genetic medicine technology but that are not covered by the claims of the patents that we own or have exclusively licensed;

- we or our licensors or future collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or have exclusively licensed;
- we or our licensors or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or have exclusively licensed may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could seriously harm our business.

Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

Any future collaborations that we enter into may not be successful, such as our collaboration agreement with Pfizer that was terminated in 2018, with AstraZeneca that concluded in 2020 without AstraZeneca exercising its option and with Roche that was terminated without cause effective September 2021. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products and product candidates that compete directly or indirectly with our product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products and product candidates may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future product candidates or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may adversely affect the price of our common stock and may result in a need for additional capital to pursue further development or commercialization of the applicable current or future product candidates;

- collaborators may own or co-own intellectual property covering our product candidates that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Risks Related to Our Operations

We are highly dependent on our key personnel, and if we are not successful in attracting, motivating and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract, motivate and retain highly qualified managerial, scientific and medical personnel. The loss of the services provided by any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements, could result in delays in the development of our product candidates and harm our business.

We conduct our operations at our facilities in Emeryville, California, in a region that is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel is intense and the turnover rate can be high, which may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. We expect that we may need to recruit talent from outside of our region, and doing so may be costly and difficult.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock option grants. The value to employees of these equity grants that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. In addition, our employees are employed at-will, which means that any of our employees could leave our employment at any time, with or without notice. If we are unable to attract, incentivize and retain quality personnel on acceptable terms, or at all, it could seriously harm our business.

We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.

As of September 30, 2023, we had 144 full-time employees. As our development plans and strategies develop, we must add a significant number of additional managerial, operational, financial and other personnel. Future growth will impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, retaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical and FDA review process for our current and future product candidates, while complying with our contractual obligations to contractors and other third parties;
- expanding our operational, financial and management controls, reporting systems and procedures; and
- managing increasing operational and managerial complexity.

Our future financial performance and our ability to continue to develop and, if approved, commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth. Our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to manage these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services. There can be no assurance that the services of these independent organizations, advisors and consultants will continue to

be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed, or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, if at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Unfavorable global economic conditions 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. Portions of our future clinical trials may be conducted outside of the United States and unfavorable economic conditions resulting in the weakening of the U.S. dollar would make those clinical trials more costly to operate. Furthermore, a severe or prolonged economic downturn, including a recession or depression resulting from the lingering effects of the COVID-19 pandemic or other factors such as increased inflation or closure of or liquidity issues at financial institutions (including, for example, Silicon Valley Bank) could result in a variety of risks to our business, including weakened demand for our product candidates or any future product candidates, if approved, and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy or political disruption, including any international trade disputes, could also strain our manufacturers or suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our potential products. Any of the foregoing could seriously harm our business, and we cannot anticipate all of the ways in which the political or economic climate and financial market conditions could seriously harm our business.

If we engage in acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may engage in various acquisitions and strategic partnerships in the future, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of indebtedness or contingent liabilities;
- the issuance of our equity securities which would result in dilution to our stockholders;
- assimilation of operations, intellectual property, products and product candidates of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing product candidates and initiatives in pursuing such an acquisition or strategic partnership;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired intellectual property, technology and/or products sufficient to meet our objectives or even to offset the associated transaction and maintenance costs.

In addition, if we undertake such a transaction, we may incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our information technology systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches and other disruptions.

We collect and maintain information in digital form that is necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business. In the ordinary course of our business, we collect, store and transmit large amounts of confidential information, including intellectual property, proprietary business information and personal information of customers and our employees and contractors. It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. Despite the implementation of security measures, our internal information technology systems and those of our collaborators, future CROs and other contractors and consultants may be vulnerable to attack, damage and interruption from computer viruses and malware (e.g. ransomware), malicious code, natural disasters, terrorism, war, telecommunication and electrical failures, hacking, cyberattacks, phishing attacks and other social engineering schemes, denial or degradation of service attacks, and sophisticated nation-state and nation-state supported actors.

The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We and our third party service providers and partners may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may experience security breaches that may remain undetected for an extended period. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. Our third party service providers and partners are also subject to these heightened risks. The costs to us to investigate and mitigate network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service, negative publicity and other harm to our business and our competitive position.

We and certain of our service providers have experienced certain cyberattacks and security incidents from time to time. Although to our knowledge we have not experienced any significant system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss, corruption or unauthorized disclosure of our trade secrets, personal data or other proprietary or sensitive information or other similar disruptions. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If a security breach or other incident were to result in the unauthorized access to or unauthorized use, disclosure, release or other processing of clinical trial data or personal data, it may be necessary to notify individuals, governmental authorities, supervisory bodies, the media, and other parties pursuant to privacy and security laws.

Likewise, we rely on our third-party research institution collaborators for research and development of our product candidates and other third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their information technology systems could also seriously harm our business. Any security compromise affecting us, our partners or our industry, whether real or perceived, could harm our reputation, erode confidence in the effectiveness of our security measures, and lead to regulatory scrutiny. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary or personal information, we could incur liability, our competitive position could be harmed, and the further development and commercialization of our product candidates could be delayed. Any losses, costs or liabilities may not be covered by, or may exceed the coverage limits of, any applicable insurance policies.

Business disruptions could seriously harm our business.

Our operations, and those of our CROs, CMOs, suppliers, and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are partly uninsured. In addition, we rely on our third-party research institution collaborators for conducting research and development of our product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could seriously harm our business.

All of our operations including our corporate headquarters are located in multiple facilities in Emeryville, California. Damage or extended periods of interruption to our corporate, development or research facilities due to fire, earthquake and other natural disaster, power loss, communications failure, unauthorized entry or other events could cause us to cease or delay development of some or all of our product candidates. Although we maintain property damage and business interruption insurance coverage on these facilities, our insurance might not cover all losses under such circumstances and our business could be seriously harmed by such delays and interruption.

Actual or perceived failures to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal data, such as information that we may collect in connection with clinical trials in the United States and abroad. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties and damage to our reputation, any of which could seriously harm our business.

As our operations and business grow, we may become subject to or affected by new or additional data protection laws and regulations and face increased scrutiny or attention from regulatory authorities. In the United States, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and regulations implemented thereunder (collectively, “HIPAA”), imposes, among other things, certain standards on certain health care providers, health plans, and health care clearinghouses, known as covered entities, as well as their business associates that perform certain services that involve creating, receiving, maintaining or transmitting individually identifiable health information for or on behalf of such covered entities, and their covered subcontractors. We may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. Depending on the facts and circumstances, we could be subject to significant penalties if we violate HIPAA.

Certain states have also adopted comparable privacy and security laws and regulations, which govern the privacy, processing and protection of health-related and other personal information. Such laws and regulations will be subject to interpretation by various courts and other governmental authorities, thus creating potentially complex compliance issues for us and our future customers and strategic partners. For example, California enacted the CCPA on June 28, 2018, which went into effect on January 1, 2020. The CCPA creates individual privacy rights for California consumers and increases the privacy and security obligations of entities handling certain personal data. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that has increased the likelihood of, and risks associated with data breach litigation. The CCPA may increase our compliance costs and potential liability,

and many similar laws have been proposed at the federal level and in other states. Further, the CPRA generally went into effect on January 1, 2023 and significantly amends the CCPA. It imposes additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It also created a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. Additional compliance investment and potential business process changes may be required. Similar laws have passed in other states and are continuing to be proposed at the state and federal level, reflecting a trend toward more stringent privacy legislation in the United States. The enactment of such laws could have potentially conflicting requirements that would make compliance challenging. In the event that we are subject to or affected by HIPAA, the CCPA, the CPRA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

In Europe, the GDPR went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the European Economic Area ("EEA"). Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant undertaking, whichever is greater. In addition to fines, a breach of the GDPR may result in regulatory investigations, reputational damage, orders to cease or change our data processing activities, enforcement notices, assessment notices (for a compulsory audit) and/or civil claims (including class actions). Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EEA and the United States remains uncertain. Case law from the Court of Justice of the European Union ("CJEU") states that reliance on the standard contractual clauses - a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism - alone may not necessarily be sufficient in all circumstances and that transfers must be assessed on a case-by-case basis. On October 7, 2022, President Biden signed an Executive Order on 'Enhancing Safeguards for United States Intelligence Activities' which introduced new redress mechanisms and binding safeguards to address the concerns raised by the CJEU in relation to data transfers from the EEA to the United States and which formed the basis of the new EU-US Data Privacy Framework ("DPF"), as released on December 13, 2022. The European Commission adopted its Adequacy Decision in relation to the DPF on July 10, 2023, rendering the DPF effective as a GDPR transfer mechanism to U.S. entities self-certified under the DPF. The DPF also introduced a new redress mechanism for EU citizens which addresses a key concern in the previous CJEU judgments and may mean transfers under standard contractual clauses are less likely to be challenged in future. We expect the existing legal complexity and uncertainty regarding international personal data transfers to continue. In particular, we expect the DPF Adequacy Decision to be challenged and international transfers to the United States and to other jurisdictions more generally to continue to be subject to enhanced scrutiny by regulators. As a result, we may have to make certain operational changes and we will have to implement revised standard contractual clauses and other relevant documentation for existing data transfers within required time frames.

Further, from January 1, 2021, companies have had to comply with the GDPR and also the United Kingdom GDPR, or the UK GDPR, which, together with the amended UK Data Protection Act 2018, retains the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, i.e., fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Although we work to comply with applicable laws, regulations and standards, our contractual obligations and other legal obligations, these requirements are evolving and may be modified, interpreted and applied in an inconsistent manner from one jurisdiction to another, and may conflict with one another or other legal obligations with which we must comply. Any failure or perceived failure by us or our employees, representatives, contractors, consultants, CROs, collaborators, or other third parties to comply with such requirements or adequately address privacy and security concerns, even if unfounded, could result in additional cost and liability to us, damage our reputation, and seriously harm our business.

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

Under Sections 382 and 383 of the United States Internal Revenue Code of 1986, as amended (the "Code"), if a corporation undergoes an "ownership change" (generally defined as a greater than 50-percentage-point cumulative change (by value) in the equity ownership of certain stockholders over a rolling three-year period), the corporation's ability to use its pre-change net operating loss carryforwards ("NOLs") and other pre-change tax attributes to offset its post-change taxable income or taxes may be limited. If finalized, Treasury Regulations currently proposed under Section 382 of the Code may further limit our ability to utilize our pre-change NOLs or other pre-change tax attributes if we undergo a future ownership change. We have experienced ownership changes in the past. We may also experience ownership changes as a result of any future shifts in our stock ownership, some of which are outside our control. As a result, our ability to use our NOLs and other pre-change tax attributes to offset post-change taxable income or taxes may be subject to limitation. We will be unable to use our NOLs or other tax attributes if we do not attain profitability sufficient to offset our available NOLs or other tax attributes prior to their expiration, to the extent subject to expiration.

Changes in tax laws or regulations that are applied adversely to us or our customers may seriously harm our business.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of any of our future domestic and foreign earnings. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us.

Risks Related to Ownership of Our Common Stock

The market price of our common stock may be volatile, which could result in substantial losses for investors.

Some of the factors that may cause the market price of our common stock to fluctuate include:

- results from, and any delays in, our clinical trials for our clinical-stage product candidates or any other future clinical development programs;
- the success of existing or new competitive products or technologies;
- commencement or termination of collaborations for our product candidates;
- failure or discontinuation of any of our product candidates;
- failure to develop our Therapeutic Vector Evolution platform technology;
- results of preclinical studies, clinical trials or regulatory approvals of product candidates of our competitors, or announcements about new research programs or product candidates of our competitors;
- regulatory or legal developments in the United States and other countries, including sanctions imposed by either the U.S. or foreign governments;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the commencement of litigation;
- the level of expenses related to any of the research programs or product candidates that we may develop;
- the results of our efforts to develop additional product candidates or products;

- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders, or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- the impact of political instability, natural disasters, war and/or events of terrorism, such as the war between Ukraine and Russia, and the corresponding tensions created from such conflict between Russia, the United States and countries in Europe as well as other countries such as China;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry, and market conditions; and
- the other factors described in this “Risk Factors” section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced significant price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Further, the stock market in general has been highly volatile due to the COVID-19 pandemic, macroeconomic factors such as higher inflation and increased interest rates and political uncertainty in the United States. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. Since the completion of our initial public offering in December 2020, the price of our common stock has been volatile, and we expect such volatility to continue. Following periods of such volatility in the market price of a company’s securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management’s attention and resources from our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We currently have research coverage by seven financial analysts. If one or more of these analysts should drop research coverage of us or if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Sales of a substantial number of shares of our common stock in the public market could occur at any time, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales or the perception in the market that the holders of a large number of shares of our common stock intend to sell shares, could reduce the market price of our common stock.

Further, shares issued upon the exercise of stock options outstanding under our equity incentive plans, or pursuant to future awards granted under those plans, will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the “Securities Act”).

Moreover, holders of an aggregate of approximately 1.9 million shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We will seek additional capital through one or a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. We, and indirectly, our stockholders, will bear the cost of issuing and servicing such securities. Because our decision to issue debt or equity securities in any future offering will depend on market conditions and other factors beyond our control, we cannot predict or estimate the amount, timing or nature of any future offerings. To the extent that we raise additional capital through the sale of equity securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Additionally, any future collaborations we enter into with third parties may provide capital in the near term but limit our potential cash flow and revenue in the future. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

Insiders have substantial influence over us, which could limit your ability to affect the outcome of key transactions, including a change of control.

Our directors, executive officers, holders of more than 5% of our outstanding stock and their respective affiliates beneficially own shares representing approximately 56% of our outstanding common stock as of September 30, 2023. As a result, these stockholders, if they act together, will be able to influence our management and affairs and all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

We are an "emerging growth company" and a "smaller reporting company" and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, as an "emerging growth company" the JOBS Act allows us to delay adoption of new or revised accounting pronouncements applicable to public companies until such pronouncements are made applicable to private companies. We have elected to use this extended transition period under the JOBS Act. As a result, our financial statements may not be comparable to the financial statements of issuers who are required to comply with the effective dates for new or revised accounting standards that are applicable to public companies, which may make comparison of our financials to those of other public companies more difficult.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) December 31, 2025, (2) the last day of the year in which we have total annual gross revenue of at least $1.07 billion, (3) the last day of the year in which

we are deemed to be a “large accelerated filer” as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded $700.0 million as of the last business day of the second fiscal quarter of such year or (4) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

Even after we no longer qualify as an “emerging growth company,” we may still qualify as a “smaller reporting company,” which would allow us to continue to take advantage of many of the same exemptions from disclosure requirements, including, among other things, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, presenting only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and reduced disclosure obligations.

We incur significant costs as a result of operating as a public company, and our management devotes substantial time to public company compliance initiatives. We may fail to comply with the rules that apply to public companies, including Section 404 of the Sarbanes-Oxley Act of 2002, which could result in sanctions or other penalties that would seriously harm our business.

We incur significant legal, accounting and other expenses as a public company, including costs resulting from public company reporting obligations under the Exchange Act and regulations regarding corporate governance practices. The listing requirements of the Nasdaq Global Select Market and the rules of the Securities and Exchange Commission (“SEC”) require that we satisfy certain corporate governance requirements relating to director independence, filing annual and interim reports, stockholder meetings, approvals and voting, soliciting proxies, conflicts of interest and a code of conduct. Our management and other personnel will need to devote a substantial amount of time to ensure that we comply with all of these requirements. Moreover, the reporting requirements, rules and regulations have and will likely continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly. Any changes we make to comply with these obligations may not be sufficient to allow us to satisfy our obligations as a public company on a timely basis, or at all. These reporting requirements, rules and regulations, coupled with the increase in potential litigation exposure associated with being a public company, could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors or board committees or to serve as executive officers, or to obtain certain types of insurance, including directors’ and officers’ insurance, on acceptable terms.

We are subject to Section 404 of The Sarbanes-Oxley Act of 2002 (“Section 404”) and the related rules of the SEC, which generally require our management and independent registered public accounting firm to report on the effectiveness of our internal control over financial reporting. Section 404 requires an annual management assessment of the effectiveness of our internal control over financial reporting. However, for so long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404. Once we are no longer an emerging growth company or, if prior to such date, we opt to no longer take advantage of the applicable exemption, we will be required to include an opinion from our independent registered public accounting firm on the effectiveness of our internal controls over financial reporting.

During the course of our review and testing, we may identify deficiencies and be unable to remediate them before we must provide the required reports. Furthermore, if we have a material weakness in our internal controls over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. We or, when required to do so, our independent registered public accounting firm may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting, which could harm our operating results, cause investors to lose confidence in our reported financial information and cause the trading price of our stock to fall. In addition, as a public company we will be required to file accurate and timely quarterly and annual reports with the SEC under the Exchange Act. In order to report our results of operations and financial statements on an accurate and timely basis, we will depend in part on CROs to provide timely and accurate notice of their costs to us.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws and Delaware law might discourage, delay or prevent a change in control of our company or changes in our management and, therefore, depress the market price of our common stock.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could depress the market price of our common stock by acting to discourage, delay or prevent a change in control of our company or changes in our management that the stockholders of our Company may deem advantageous. These provisions, among other things:

- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- eliminate cumulative voting in the election of directors;
- authorize our board of directors to issue shares of preferred stock and determine the price and other terms of those shares, including preferences and voting rights, without stockholder approval;
- provide our board of directors with the exclusive right to elect a director to fill a vacancy or newly created directorship;
- provide that our directors may be removed only for cause;
- permit stockholders to only take actions at a duly called annual or special meeting and not by written consent;
- prohibit stockholders from calling a special meeting of stockholders;
- provide for a staggered board, which will result in only a few directors being up for re-election in each calendar year;
- require that stockholders give advance notice to nominate directors or submit proposals for consideration at stockholder meetings;
- authorize our board of directors, by a majority vote, to amend the bylaws;
- require the affirmative vote of at least 66 2/3% or more of the outstanding shares of our common stock to amend many of the provisions described above; and
- limit the liability of, and provide indemnification to, our directors and officers.

In addition, Section 203 of the General Corporation Law of the State of Delaware (“DGCL”) prohibits a publicly-held Delaware corporation from engaging in a business combination with an interested stockholder, generally a person which together with its affiliates owns, or within the last three years has owned, 15% of our voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in a prescribed manner.

Any provision of our certificate of incorporation, bylaws, or Delaware law that has the effect of delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our capital stock and could also affect the price that some investors are willing to pay for our common stock.

Claims for indemnification by our directors and officers may reduce our available funds to satisfy successful third-party claims against us and may reduce the amount of money available to us.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that we will indemnify our directors and officers, in each case to the fullest extent permitted by Delaware law.

In addition, as permitted by Section 145 of the Delaware General Corporation Law, our amended and restated bylaws and our indemnification agreements that we have entered into with our directors and officers provide that:

- We will indemnify our directors and officers for serving us in those capacities or for serving other business enterprises at our request, to the fullest extent permitted by Delaware law. Delaware law provides that a corporation may indemnify such person if such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the registrant and, with respect to any criminal proceeding, had no reasonable cause to believe such person's conduct was unlawful.
- We may, in our discretion, indemnify employees and agents in those circumstances where indemnification is permitted by applicable law.
- We are required to advance expenses, as incurred, to our directors and officers in connection with defending a proceeding, except that such directors or officers shall undertake to repay such advances if it is ultimately determined that such person is not entitled to indemnification.
- We will not be obligated pursuant to our amended and restated bylaws to indemnify a person with respect to proceedings initiated by that person against us or our other indemnitees, except with respect to proceedings authorized by our board of directors or brought to enforce a right to indemnification.
- The rights conferred in our amended and restated bylaws are not exclusive, and we are authorized to enter into indemnification agreements with our directors, officers, employees and agents and to obtain insurance to indemnify such persons.
- We may not retroactively amend our amended and restated bylaw provisions to reduce our indemnification obligations to directors, officers, employees and agents.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for certain disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware (or, in the event that the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware or other state courts of the State of Delaware) is the exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of fiduciary duty, any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine; provided that, the exclusive forum provision will not apply to suits brought to enforce any liability or duty created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; and provided further that, if and only if the Court of Chancery of the State of Delaware dismisses any such action for lack of subject matter jurisdiction, such action may be brought in another state or federal court sitting in the State of Delaware. Our amended and restated certificate of incorporation and amended and restated bylaws will also provide that the federal district courts of the United States of America will be the exclusive forum for the resolution of any complaint asserting a cause of action against us or any of our directors, officers, employees or agents and arising under the Securities Act. Nothing in our amended and restated certificate of incorporation and amended and restated bylaws precludes stockholders that assert claims under the Exchange Act from bringing such claims in state or federal court, subject to applicable law.

This choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, other employees or stockholders, which may discourage lawsuits with respect to such claims, although our stockholders will not be deemed to have waived our compliance with federal securities laws and the rules and regulations thereunder. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. While the Delaware courts have determined that such

choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions, and there can be no assurance that such provisions will be enforced by a court in those other jurisdictions. If a court were to find the choice of forum provision that will be contained in our amended and restated certificate of incorporation and amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could seriously harm our business.

We do not currently intend to pay dividends on our common stock, and, consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.

We do not currently intend to pay any cash dividends on our common stock for the foreseeable future. We currently intend to invest our future earnings, if any, to fund our growth. Therefore, you are not likely to receive any dividends on your common stock for the foreseeable future. Since we do not intend to pay dividends, your ability to receive a return on your investment will depend on any future appreciation in the market value of our common stock. There is no guarantee that our common stock will appreciate or even maintain the price at which our holders have purchased it.

Unfavorable conditions in the global economy caused by global political unrest or conflicts, including the ongoing conflict between Russia and Ukraine and in Israel and Gaza , may exacerbate certain risks we face.

Political unrest, international conflict, terrorism or war, such as Russia's invasion of Ukraine and the armed conflict in Israel and Gaza, and the global response to these conflicts, including the imposition of sanctions by the United States and other countries, could create or exacerbate risks facing our business. We have evaluated our operations and partner contracts, and we currently do not expect existing conflicts to directly have a significant effect on our financial condition or results of operations. However, if hostilities persist, escalate or expand, risks that we have identified in this Quarterly Report on Form 10-Q may be materially increased. For example, if our supply arrangements or clinical operations are disrupted due to expanded sanctions or involvement of countries where we have operations or relationships, our business could be materially disrupted. Further, the use of cyberattacks could expand as part of the ongoing conflicts, which could adversely affect our ability to maintain or enhance our cyber security measures. These and other risks are described more fully in this "Risk Factors" section.

General Risk Factors

Any legal proceedings or claims against us could be costly and time-consuming to defend and could harm our reputation regardless of the outcome.

We may in the future become subject to legal proceedings and claims that arise in the ordinary course of business, such as disputes or employment claims made by our current or former employees. Any litigation, whether meritorious or not, could harm our reputation, will increase our costs and may divert management's attention, time and resources, which may in turn seriously harm our business. Insurance might not cover such claims, might not provide sufficient payments to cover all the costs to resolve one or more such claims, and might not continue to be available on terms acceptable to us. A claim brought against us that is uninsured or underinsured could result in unanticipated costs and could seriously harm our business.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk when and if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we

may incur substantial liabilities or be required to cease commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased or interrupted demand for our products;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate; and
- a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Our employees, independent contractors, consultants, research or commercial partners or collaborators and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, research or commercial partners or other collaborators, including the foundations we work with, and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to: comply with the laws of the FDA, EMA and other comparable foreign regulatory authorities; provide true, complete and accurate information to the FDA, EMA and other comparable foreign regulatory authorities; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. In particular, research, sales, marketing, education and other business arrangements in the healthcare industry are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, educating, marketing and promotion, sales and commission, certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such

actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could seriously harm our business, including the imposition of significant fines or other sanctions.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could seriously harm our business.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations, and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at our current or past facilities and at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research, product development and manufacturing efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation 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 carry specific biological or hazardous waste insurance coverage, and our property, casualty, and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could seriously harm our business.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Reliance on third parties to conduct clinical trials, assist in research and development and to manufacture our product candidates, will at times require us to share trade secrets with them. We seek to protect our proprietary technology by in part entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors 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, including our 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 independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may seriously harm our business.

We may fail to comply with any of our obligations under existing or future agreements pursuant to which we license or have otherwise acquired intellectual property rights or technology, which could result in the loss of rights or technology that are material to our business.

We are party to various agreements that we depend on to operate our business. Our rights to use currently licensed intellectual property, or intellectual property to be licensed in the future, are or will be subject to the continuation of and our compliance with the terms of these agreements. These agreements

are complex, and certain provisions in such agreements may be susceptible to multiple interpretations which could lead to disputes, including but not limited to those regarding:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our proprietary technology and product candidates infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights;
- diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the creation or use of intellectual property by us or our counterparties, alone or jointly;
- the scope and duration of our payment obligations;
- rights upon termination of such agreement; and
- the scope and duration of exclusivity obligations of each party to the agreement.

The resolution of any contractual interpretation dispute that may arise, if unfavorable to us, could seriously harm our business. Such resolution could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, increase what we believe to be our financial or other obligations under the relevant agreement, or decrease the third party's financial or other obligations under the relevant agreement.

If disputes over intellectual property rights that we have licensed or acquired from third parties prevent or impair our ability to maintain our current license agreements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we fail to comply with our obligations under current or future license agreements, these agreements may be terminated or the scope of our rights under them may be reduced and we might be unable to develop, manufacture or market any product that is licensed under these agreements.

Litigation or other proceedings or third-party claims of intellectual property infringement could require us to spend significant time and money and could prevent us from selling our products.

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts. We cannot assure you that our operations do not, or will not in the future, infringe existing or future patents or other proprietary rights of third parties.

Third parties may have or obtain patents or other proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and future approved products, if any, or impair our competitive position. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, reexaminations, inter partes review proceedings and post-grant review proceedings before the USPTO and/or corresponding foreign patent offices. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. Further, we may incorrectly

determine that our technologies or product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or abroad that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our product candidates.

As the biotechnology industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. Our competitors in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our product candidates. We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. Patent applications in the United States and elsewhere are typically published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Certain U.S. patent applications that will not be filed outside the United States can remain confidential until patents issue. In addition, patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived. Furthermore, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our product candidates or the use of our product candidates. As such, there may be applications of others now pending or recently revived patents of which we are unaware. These applications may later result in issued patents, or the revival of previously abandoned patents, that will prevent, limit or otherwise interfere with our ability to make, use or sell our product candidates. As a result, we may be unaware of third-party patents that may be infringed by commercialization of our product candidates, and cannot be certain that we were the first to file a patent application related to a product candidate or technology. Moreover, because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be infringing; and/or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all.

Although no third party has asserted a claim of patent infringement against us as of the date of this report, others may hold proprietary rights that could prevent our product candidates or any future products from being marketed. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our product candidates or proprietary technologies could subject us to potential liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market our product candidates or any future products. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be time-consuming and a substantial diversion of management and employee resources from our business.

We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Even if such licenses are available, we could incur substantial costs related to royalty payments for licenses obtained from third parties, which could negatively affect our gross margins, and the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. In addition, we cannot be certain that we could redesign our product candidates or proprietary technologies to avoid infringement, if necessary, or on a cost-effective basis. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates or any future products which could seriously harm our business. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology.

If we collaborate with third parties in the development of technology in the future, our 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 intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. Also, we may be obligated under our agreements with our collaborators, licensors, suppliers and others to indemnify and hold them harmless for damages arising from intellectual property infringement by us.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged.

Competitors may infringe our intellectual property rights or those of our licensors. To prevent infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in a patent infringement proceeding, a court or administrative tribunal may decide that a patent we own or license is not valid, is unenforceable and/or is not infringed. If we or any of our potential future collaborators were to initiate legal proceedings against a third party to enforce a patent directed at one of our product candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign patent offices and may result in the revocation, cancellation, or amendment of any foreign patents we hold in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we could lose at least part, and perhaps all, of the patent protection on an affected product candidate. Such a loss of patent protection would seriously harm our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO, or equivalent actions brought in foreign jurisdictions, may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be seriously harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace and seriously harm our business.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will depend in part on our ability to acquire, license or use these proprietary rights. We may be unable to acquire or license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights 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 a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We have collaborated with a U.S. academic institution and may in the future collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. These institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of that program and our business could be seriously harmed.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we, our employees or consultants have wrongfully used or disclosed alleged confidential information or trade secrets of their former or concurrent employers or former or current clients.

As is common in the biotechnology and biopharmaceutical industries, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other biotechnology or biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may become subject to claims that we, our employees or a consultant inadvertently or otherwise

used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. We may also be subject to claims that patents and applications we have filed to protect inventions of our employees or consultants, even those related to one or more of our product candidates, are rightfully owned by their former or concurrent employer or former or current client. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, which could seriously harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management and other employees.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

Our agreements with employees and consultants provide that any inventions conceived by an individual in the course of rendering services to us shall be our exclusive property. Although our policy is to have all such individuals complete these agreements, 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 may not be self-executing and despite such agreement, such inventions may become assigned to third parties. 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. We may also be subject to claims that former employees, consultants, or other third parties have an ownership interest in our patents or other intellectual property. In addition, we may face claims by third parties that our agreements with employees or consultants obligating them to assign intellectual property to us are ineffective or in conflict with prior or competing contractual obligations of assignment, which could result in ownership disputes regarding intellectual property we have developed or will develop and interfere with our ability to capture the commercial value of such intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Such an outcome could seriously harm our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distraction to our management and other employees.

If we do not obtain patent term extension for our product candidates, our business may be seriously harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of any of our product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension may also be available in certain foreign countries upon regulatory approval of our product candidates. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration of the applicable product, and our business may be seriously harmed.

Changes in patent law in the United States or in other countries could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

Our patent rights may be affected by developments or uncertainty in the U.S. or foreign patent statutes, patent case laws, USPTO rules and regulations or in the rules and regulations of foreign patent offices.

There are a number of recent changes to the U.S. patent laws that may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. For example, on September 16, 2011, the Leahy-Smith America Invents Act ("Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes a number of significant changes to the U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a "first to file" system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the USPTO, and may become involved in post-grant proceedings including opposition, derivation, reexamination, inter partes review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position. This could have a negative impact on some of our intellectual property and could increase uncertainties surrounding obtaining and enforcement or defense of our issued patents. In addition, Congress may pass patent reform legislation that is unfavorable to us. The Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents we might obtain in the future. Similarly, statutory or judicial changes to the patent laws of other countries may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Complying with these laws and regulations could limit our ability to obtain new patents in the future that may be important for our business.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending all current and future patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but enforcement is not as strong as that in the United States. These products may compete with our product candidates, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights. For example, some foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. In addition, some countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our business may be seriously harmed.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent

agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We employ reputable professionals and rely on such third parties to help us comply with these requirements and effect payment of these fees with respect to the patents and patent applications that we own, and if we license intellectual property we may have to rely upon our licensors to comply with these requirements and effect payment of these fees with respect to any patents and patent applications that we license. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case.

Our business could be negatively impacted by corporate citizenship and ESG matters and/or our reporting of such matters.

Institutional, individual, and other investors, proxy advisory services, regulatory authorities, consumers and other stakeholders are increasingly focused on environmental, social and governance (“ESG”) practices of companies. As we look to respond to evolving standards for identifying, measuring, and reporting ESG metrics, our efforts may result in a significant increase in costs and may nevertheless not meet investor or other stakeholder expectations and evolving standards or regulatory requirements, which may negatively impact our financial results, our reputation, our ability to attract or retain employees, our attractiveness as an investment or business partner, or expose us to government enforcement actions, private litigation, and actions by stockholders or stakeholders.

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

Recent Sales of Unregistered Securities

None.

Use of Proceeds from Our IPO

On December 15, 2020, we closed our IPO, in which we issued and sold 9,660,000 shares of common stock at a price to the public of $23.00 per share. We received net proceeds of $204.7 million, after deducting underwriting discounts and commissions of $15.6 million and offering costs of $1.9 million.

None of the expenses associated with the IPO were paid to directors, officers, or persons owning 10% or more of any class of equity securities, or to their associates, or to our affiliates, other than payments in the ordinary course of business to officers for salaries. Goldman Sachs & Co. LLC, BofA Securities and Evercore ISI acted as book-running managers for the offering.

Shares of our common stock began trading on the Nasdaq Global Select Market on December 11, 2020. The shares were registered under the Securities Act on a Registration Statement on Form S-1 (File No. 333-250150), which was declared effective by the SEC on December 11, 2020.

There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus dated December 11, 2020, filed with the SEC pursuant to Rule 424(b)(4) under the Securities Act.

Item 3. Default Upon Senior Securities

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
3.1	Amended and Restated Certificate of Incorporation, as currently in effect.	8-K	12/15/20	3.1	
3.2	Amended and Restated Bylaws, as currently in effect.	8-K	6/21/22	3.1	
4.1	Description of Securities of the Registrant.	10-K	3/15/23	4.1	
4.2	Form of Common Stock Certificate.	S-1/A	12/7/20	4.2	
4.3	Amended and Restated Investors' Rights Agreement, dated as of April 29, 2020, among the Registrant and the investors party thereto.	S-1/A	12/7/20	4.3	
10.1^	License Agreement between the Registrant and Astellas Gene Therapies, Inc., dated as of July 5, 2023				X
31.1	Certification of the Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
31.2	Certification of the Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				X
32.1*	Certifications of the Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				X
101.INS	Inline XBRL Instance Document				X
101.SCH	Inline XBRL Taxonomy Extension Schema Document				X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document				X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document				X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document				X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document				X
104	The cover page from the Company's Quarterly Report on Form 10-Q for the three months ended September 30, 2023, has been formatted in Inline XBRL.				X

^ Pursuant to Item 601(b)(10)(iv) of Regulation S-K, certain portions of this exhibit have been omitted because the Company has determined that the information is not material and is the type that the Company treat as private or confidential.

* The certifications attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of 4D Molecular Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

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

4D Molecular Therapeutics, Inc.

Date: November 9, 2023

By: /s/ David Kirn

David Kirn, M.D.
Chief Executive Officer and Director
Principal Executive Officer

Date: November 9, 2023

By: /s/ Uneek Mehra

Uneek Mehra
Chief Financial and Business Officer
Principal Financial and Accounting Officer

Certain confidential information contained in this document, marked by brackets and asterisk, has been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K, because the Company treats such information as private or confidential and the omitted information is not material.

Exhibit 10.1

Execution Version

LICENSE AGREEMENT

between

4D MOLECULAR THERAPEUTICS, INC.

and

ASTELLAS GENE THERAPIES, INC.

Dated as of July 5, 2023

LICENSE AGREEMENT

This License Agreement (the "**Agreement**") is made and entered into effective as of July 5, 2023 (the "**Effective Date**") by and between 4D Molecular Therapeutics, Inc., a Delaware corporation ("**Licensor**"), and Astellas Gene Therapies, Inc. (f/k/a Audentes Therapeutics, Inc.), a Delaware corporation ("**AGT**"). Licensor and AGT are sometimes referred to herein individually as a "**Party**" and collectively as the "**Parties**."

RECITALS

WHEREAS, Licensor Controls (as defined below) certain intellectual property rights with respect to the 4D Vector (as defined below);

WHEREAS, AGT has expertise in the development and commercialization of biopharmaceutical products, including AAV gene therapy products; and

WHEREAS, Licensor wishes to grant, and AGT wishes to take, a license under such intellectual property rights to develop and commercialize Licensed Compounds and Licensed Products that incorporate the 4D Vector, in each case in accordance with the terms and conditions set forth below.

NOW, THEREFORE, in consideration of the premises and the mutual promises and conditions hereinafter set forth, and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties, intending to be legally bound, do hereby agree as follows:

ARTICLE 1
DEFINITIONS

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

1.1"**4D Vector**" means Licensor's proprietary R100 AAV capsid variant, as further defined on Schedule 1.1 by the capsid protein sequence referenced on Schedule 1.1 ([*]).

1.2"**4D Vector DMF**" has the meaning set forth in Section 3.5.1(b).

1.3"**AAV**" means adeno-associated virus, including its recombinant forms.

1.4"**Acquirer**" has the meaning set forth in Section 1.27.

1.5"**Acquirer Competing Product**" has the meaning set forth in Section 6.9.2.

1.6"**Accounting Standards**" means, with respect to a Party, the maintenance by such Party of its records and books of accounts in accordance with United States Generally Accepted Accounting Principles or International Financial Reporting Standards.

1.7"**Additional Target**" has the meaning set forth in Section 2.1.

1.8"**Additional Target Fee**" has the meaning set forth in Section 7.1.2.

1.9"**Additional Target Identification Notice**" means a written notice sent by AGT to Licensor stating that AGT is formally selecting an Additional Target as an AGT Target in accordance with Section 2.2.

1.10"**Affiliate**" means, with respect to a Party, any Person that, directly or indirectly, through one or more intermediaries, controls, is controlled by or is under common control with such Party. For purposes of this definition, "control" and, with correlative meanings, the terms "controlled by" and "under common control with" means (a) the possession, directly or indirectly, of the power to direct the management or policies of a Person, whether through the ownership of voting securities, by contract relating to voting rights or corporate governance, or otherwise; or (b) the ownership, directly or indirectly, of more than fifty percent (50%) of the voting securities or other ownership interest of a Person (or, with respect to a limited partnership or other similar entity, its general partner or controlling entity). For clarity, once a Person ceases to be an Affiliate of a Party, then, without any further action, such Person shall cease to have any rights, including license and sublicense rights, under this Agreement by reason of being an Affiliate of such Party.

1.11"**Agreement**" has the meaning set forth in the preamble hereto.

1.12"**AGT**" has the meaning set forth in the preamble hereto.

1.13"**AGT Payload**" means, with respect to an AGT Target, a DNA sequence or DNA sequences (regardless of whether in genomic, complementary or other form) [*] that has a therapeutic effect on a disease in the Field when packaged into a 4D Vector, administered to a patient, and delivered to the appropriate cells.

1.14"**AGT Target**" means the Initial Target and the Additional Targets (in each case, unless and until replaced by a Substitute Target as set forth in this Agreement).

1.15"**AGT Vector Improvement Patents**" means all Patents to the extent claiming Know-How that both (a) [*] and (b) comprises [*], in each case that is Controlled by AGT or its Affiliates at any time during the Term.

1.16"**AGT Vector/Payload Patent**" means a Patent owned by AGT or its Affiliates or Sublicensees that [*].

1.17"**Antitrust Event**" means (a) any required filing under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 (15 U.S.C. §18a) or (b) any investigations related to monopolization or restraint of trade or lessening or impeding competition are initiated by a governmental authority, in each case (a) and (b), where such filing or investigation(s) arises from the entering into of this Agreement or the designation of an AGT Target under this Agreement.

1.18"**Applicable Law**" means federal, state, local, national, and supra-national laws, statutes, rules, and regulations, including any rules, regulations, guidelines, or other requirements of the Regulatory Authorities, major national securities exchanges, or major securities listing organizations, that may be in effect from time to time during the Term and applicable to a particular activity or country or other jurisdiction hereunder.

1.19"**Audit Expert**" has the meaning set forth in Section 7.10.

1.20"**Bankruptcy Code**" has the meaning set forth in Section 13.5.1.

1.21"**Biosimilar Application**" has the meaning set forth in Section 8.3.4.

1.22"**Biosimilar Product**" means, with respect to a Licensed Product, on a country-by-country basis, a biologic product (a) whose licensing, approval, or marketing authorization relies in whole or in part on a prior approval, licensing, or marketing authorization granted such Licensed Product or any data generated in support thereof; (b) that is "biosimilar" to such Licensed Product, as the term "biosimilar" is defined in 42 U.S.C. § 262(i)(2); or (c) is approved by the applicable Regulatory Authority as biosimilar or interchangeable with such Licensed Product (including by the FDA as set forth at 42 USC 262(k)(4), the EMA as set forth in CHMP/437/04 and similar regulations in other jurisdictions). A Licensed Product licensed, marketed, sold, manufactured, or produced by AGT, its Affiliates or Sublicensees will not constitute a Biosimilar Product.

1.23"**Breaching Party**" has the meaning set forth in Section 13.2.1.

1.24"**Business Day**" means a day other than (a) a Saturday or Sunday, (b) any day on which banking institutions in New York, New York and Tokyo, Japan are authorized or required by Applicable Law to remain closed, or (c) [*].

1.25"**Calendar Quarter**" means each successive period of three (3) calendar months commencing on January 1, April 1, July 1, and October 1, except that the first Calendar Quarter of the Term shall commence on the Effective Date and end on the day immediately prior to the first to occur of January 1, April 1, July 1 or October 1 after the Effective Date, and the last Calendar Quarter shall end on the last day of the Term.

1.26"**Calendar Year**" means each successive period of twelve (12) calendar months commencing on January 1 and ending on December 31, except that the first Calendar Year of the Term shall commence on the Effective Date and end on December 31 of the year in which the Effective Date occurs, and the last Calendar Year of the Term shall commence on January 1 of the year in which the Term ends and end on the last day of the Term.

1.27"**Change of Control**" means, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent more than fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation, (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of more than fifty percent (50%) of the combined voting power of the outstanding securities of such Party, or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's business to which the subject matter of this Agreement relates. Any such Third Party, together with any affiliates of such Third Party existing immediately prior to the consummation of the Change of Control, shall be an "**Acquirer**" for purposes of this Agreement. For clarity, an "Acquirer" of a Party shall exclude the Party and all of its Affiliates existing immediately prior to the consummation of the Change of Control.

1.28"**Clinical Data**" means all Know-How with respect to any compound or product and made, collected, or otherwise generated under or in connection with Clinical Studies, including any data (including raw data), reports, and results with respect thereto.

1.29"**Clinical Studies**" means Phase I, Phase II, Phase III, Pivotal Studies, and such other tests and studies in human subjects that are required by Applicable Law, or otherwise recommended by the Regulatory Authorities, to obtain or maintain Regulatory Approvals for a Licensed Product for one or more indications, including tests or studies that are intended to expand the product labeling for such Licensed Product with respect to such indication.

1.30"**Combination Product**" means a Licensed Product that (a) is comprised of or contains one or more Licensed Compounds as active ingredient(s) together with one or more other active ingredients (that are not Licensed Compounds) and (b) is sold for single price, regardless if such other active ingredients are in the same or different formulations, co-shipped or shipped separately, co-packaged or packaged separately or co-administered or administered separately.

1.31"**Commercialization**" means any and all activities directed to the preparation for sale of, offering for sale of, or sale of a compound or product, including activities related to marketing, promoting, distributing, importing and exporting such compound or product, including Manufacturing in support thereof, and in each, interacting with Regulatory Authorities regarding any of the foregoing. When used as a verb, "**to Commercialize**" and "**Commercializing**" means to engage in Commercialization, and "**Commercialized**" has a corresponding meaning.

1.32"**Commercially Reasonable Efforts**" means, with respect to the performance of Development, Commercialization, or Manufacturing activities with respect to a Licensed Product by a Party (including its Affiliates where applicable), the carrying out of such activities using efforts and resources [*] of comparable size in connection with the development, manufacture or commercialization of pharmaceutical products of similar market potential at a similar stage of development or commercialization in its product lifecycle [*]. "Commercially Reasonable Efforts" shall be determined on a country-by-country (or region-by-region, where applicable) basis, [*]. Commercially Reasonable Efforts requires, with respect to a task under this Agreement, that a Party reasonably and in good faith: [*].

1.33"**Competing Product**" means any [*]. Competing Product expressly excludes any Licensed Compound or Licensed Product.

1.34"**Conditioning Regimen**" means any compound, product, or regimen [*] whether given prior to, at the same time as, or after administration of Licensed Product. For clarity, Licensed Products do not constitute a Conditioning Regimen.

1.35"**Confidential Information**" means any Know-How provided orally, visually, in writing or other form by or on behalf of one Party (or an Affiliate or representative of such Party) to the other Party (or to an Affiliate or representative of such Party) in connection with this Agreement, whether prior to, on, or after the Effective Date, including Know-How disclosed by or on behalf of the Parties or their Affiliates pursuant to the Existing CDA, relating to the terms of this Agreement, the AGT Payload, the 4D Vector, or any Licensed Compound, Licensed Product (including the Regulatory Documentation and regulatory data), any Exploitation of any Licensed

Compound or Licensed Product, any Know-How with respect thereto developed by or on behalf of the disclosing Party or its Affiliates, or the scientific, regulatory or business affairs or other activities of either Party. Notwithstanding the foregoing, Joint Know-how shall be deemed to be the Confidential Information of both Parties, and both Parties shall be deemed to be the receiving Party and the disclosing Party with respect thereto.

1.36"**Control**" means, with respect to any item of Know-How, Regulatory Documentation, material, Patent, or other property right, the possession of the right, whether directly or indirectly, and whether by ownership, license, covenant not to sue or otherwise (other than by operation of the license and other grants in Section 6.1), to grant a license, sublicense or other right (including the right to reference Regulatory Documentation) to or under such Know-How, Regulatory Documentation, material, Patent, or other property right as provided for herein without violating the terms of any agreement with any Third Party.

1.37"**Convicted Individual**" or "**Convicted Entity**" has the meaning set forth in Section 11.2.14.

1.38"**Debarred Entity**" has the meaning set forth in Section 11.2.14.

1.39"**Default Notice**" has the meaning set forth in Section 13.2.1.

1.40"**Development**" means all activities related to research, pre-clinical and other non-clinical testing, test method development and stability testing, toxicology, formulation, process development, manufacturing scale-up, qualification and validation, quality assurance/quality control, Clinical Studies, including Manufacturing in support thereof, statistical analysis and report writing, the preparation and submission of Drug Approval Applications, regulatory affairs with respect to the foregoing and all other activities necessary or reasonably useful or otherwise requested or required by a Regulatory Authority as a condition or in support of obtaining or maintaining a Regulatory Approval. When used as a verb, "**Develop**" means to engage in Development. For purposes of clarity, Development shall include any submissions and activities required in support thereof, required by Applicable Laws or a Regulatory Authority as a condition or in support of obtaining a pricing or reimbursement approval for an approved Licensed Product.

1.41"**Dispute**" has the meaning set forth in Section 14.6.

1.42"**Distributor**" has the meaning set forth in Section 6.6.

1.43"**Dollars**" or "**$**" means United States dollars.

1.44"**Drug Approval Application**" means a New Drug Application or a Biologics License Application as defined in the FFDCA, or any corresponding foreign application in the Territory, including, with respect to the EU5, a Marketing Authorization Application filed with the EMA or with the applicable Regulatory Authority of a country in the EU5 with respect to the mutual recognition or any other national approval procedure.

1.45"**Effective Date**" has the meaning set forth in the preamble hereto.

1.46"**EMA**" means the European Medicines Agency and any successor agency(ies) or authority having substantially the same function.

1.47"**EU5**" means Germany, Italy, Spain, France, and the United Kingdom.

1.48"**EU5 Pricing and Reimbursement Approval**" means, with respect to a Licensed Product in an EU5 country, the approval, agreement, determination, or decision of the applicable Regulatory Authority for such country establishing the price or level of reimbursement for such Licensed Product, as required in such country prior to sale of such Licensed Product in such country.

1.49"**Excluded Licensor IP**" means Patents and Know-How (which Know-How has not been publicly disclosed) that are Controlled by Licensor or its Affiliates as of the Effective Date or during the Term [*]. For clarity, Excluded Licensor IP does not include [*].

1.50"**Excluded Entity**" or "**Excluded Individual**" has the meaning set forth in Section 11.2.14.

1.51"**Existing CDA**" means the Mutual Nondisclosure Agreement between the Parties (or their respective Affiliates), dated August 22, 2022.

1.52"**Existing Licensor Patents**" has the meaning set forth in Section 11.2.1.

1.53"**Existing Licensor Regulatory Documentation**" means Regulatory Documentation [*] and that are Controlled by Licensor or any of its Affiliates as of the Effective Date. "Existing Licensor Regulatory Documentation" excludes, for clarity, any other Regulatory Documentation relating to [*].

1.54"**Exploit**" or "**Exploitation**" means (a) with regards to a product (other than the 4D Vector component of such product), to make, have made, import, export, use, have used, sell, have sold, or offer for sale, including to Develop, Commercialize, register, modify, enhance, improve, Manufacture, have Manufactured, hold, or keep (whether for disposal or otherwise), or otherwise handle or dispose of, and (b) with regards to the 4D Vector, solely for use in Licensed Compounds and Licensed Products, to (i) make, have made, import, export, use, have used, sell, have sold, or offer for sale, including to Develop, Commercialize, register, Manufacture, have Manufactured, hold, or keep (whether for disposal or otherwise), or otherwise handle or dispose of and (ii) [*].

1.55"**FDA**" means the United States Food and Drug Administration and any successor agency(ies) or authority having substantially the same function.

1.56"**FDA's Disqualified/Restricted List**" has the meaning set forth in Section 11.2.14.

1.57"**FFDCA**" means the United States Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

1.58"**Field**" means any and all treatment, diagnosis or prophylaxis of Monogenic Rare Disorder(s).

1.59"**First Commercial Sale**" means, with respect to a Licensed Product and a country, the first sale for monetary value for use or consumption by the end user of such Licensed Product in such country after Regulatory Approval for such Licensed Product has been obtained in such country and where such sale results in a recordable Net Sale in accordance with the applicable Accounting Standards. Sales prior to receipt of Regulatory Approval for such Licensed Product, such as so-called "treatment IND sales," "named patient sales," and "compassionate use sales," shall not be construed as a First Commercial Sale.

1.60"**GLP Toxicity Study**" means, with respect to a Licensed Product, (a) an in vivo preclinical toxicology study [*].

1.61"**Good Laboratory Practices**" or "**GLP**" means the applicable then-current good laboratory practice standards required by the applicable Regulatory Authorities or Applicable Law, including those promulgated or endorsed by the FDA, as defined in U.S. 21 C.F.R. Part 58 and such other comparable regulatory standards in jurisdictions outside the United States, as they may be updated from time to time.

1.62"**Governmental Authority**" means any multinational, federal, state, local, municipal or other governmental authority of any nature (including any governmental division, prefecture, subdivision, department, agency, bureau, branch, office, commission, council, court or other tribunal), in each case, having jurisdiction over the applicable subject matter.

1.63"**IND**" means an application filed with a Regulatory Authority for authorization to commence Clinical Studies, including (a) an Investigational New Drug Application as defined in the FFDCA or any successor application or procedure filed with the FDA, (b) any equivalent of an Investigational New Drug Application described in the foregoing clause (a) in other countries or regulatory jurisdictions, (e.g., clinical trial application ("**CTA**")) and (c) all supplements, amendments, variations, extensions and renewals thereof that may be filed with respect to the foregoing.

1.64"**Indemnification Claim Notice**" has the meaning set forth in Section 12.3.

1.65"**Indemnified Party**" has the meaning set forth in Section 12.3.

1.66"**[*] Action**" has the meaning set forth in Section 6.2.1.

1.67"**Initial Target**" means the Target known as [*].

1.68"**Initiation**" means, with respect to a Clinical Study, the first dosing of the first human subject in such Clinical Study, subject to Section 1.92 in the case of Pivotal Studies.

1.69"**Joint Know-How**" means Know-How and inventions that are conceived, discovered, developed, or otherwise made jointly by or on behalf of Licensor or its Affiliates, on the one hand, and AGT or its Affiliates, on the other hand, in connection with the work conducted under or in connection with this Agreement, whether or not patented or patentable.

1.70"**Joint Patents**" means Patents covering or claiming any invention within the Joint Know-How.

1.71"**Know-How**" means all knowledge of a technical, scientific, business and other nature, including know-how, technology, means, methods, processes, practices, formulae, instructions, skills, techniques, procedures, experiences, ideas, technical assistance, designs, drawings, assembly procedures, computer programs, apparatuses, specifications, data, results and other material, regulatory data, and other biological, chemical, pharmacological, toxicological, pharmaceutical, physical and analytical, pre-clinical, clinical, safety, manufacturing and quality control data and information, including study designs and protocols, reagents (*e.g.*, plasmids, proteins, cell lines, assays and compounds) and biological methodology; in each case (whether or not confidential, proprietary, patented or patentable, of commercial advantage or not) in written, electronic or any other form now known or hereafter developed.

1.72"**Knowledge**" with respect to Licensor, means [*]. For the purposes of this definition of "Knowledge," [*].

1.73"**Licensed Compound**" means an active pharmaceutical ingredient comprising (a) the 4D Vector and (b) an AGT Payload.

1.74"**Licensed IP**" means the Licensor Patents and Licensor Know-How, but excluding the Excluded Licensor IP.

1.75"**Licensed Product**" means any product that is or contains at least one Licensed Compound alone or together with one or more other active ingredients, in any and all forms, presentations, delivery systems, dosages and formulations.

1.76"**Licensor**" has the meaning set forth in the preamble hereto.

1.77"**Licensor Indemnitees**" has the meaning set forth in Section 12.1.

1.78"**Licensor Know-How**" means all Know-How that is (a) Controlled by Licensor or any of its Affiliates as of the Effective Date or at any time during the Term and (b) necessary or reasonably useful for the Exploitation of the 4D Vector for use in a Licensed Compound, including Licensor's safety data relating to the 4D Vector. For clarity, Licensor Know-How excludes any Know-How in the Excluded Licensor IP, Transferred Materials Know-How, and any Joint Know-How.

1.79"**Licensor Patents**" means all of the Patents that are (a) owned or Controlled by Licensor or any of its Affiliates as of the Effective Date or at any time during the Term and (b) necessary or reasonably useful (or, with respect to Patent applications, would be necessary or reasonably useful if such Patent applications were to issue as Patents) for the Exploitation of the 4D Vector for use in a Licensed Compound. "Licensor Patents" includes the Existing Licensor Patents. "Licensor Patents" excludes any Patents in the Excluded Licensor IP and any Joint Patents.

1.80"**Losses**" has the meaning set forth in Section 12.1.

1.81"**Manufacture**" and "**Manufacturing**" means all activities related to the synthesis, making, production, processing, purifying, formulating, filling, finishing, packaging, labeling, shipping, and holding of the 4D Vector for use in any Licensed Compound, any Licensed Compound, any Licensed Product, or any intermediate or component thereof, including process development, process qualification and validation/audit, scale-up, pre-clinical, clinical and commercial production and analytic development, product characterization, stability testing, quality assurance, and quality control. When used as a verb, "**Manufactured**" has a corresponding meaning.

1.82"**Monogenic Rare Disorder**" means, with respect to a disorder, that (a) one or more mutations in a single gene causes such disorder and (b) such disorder has [*]. The following are not Monogenic Rare Disorders: any [*].

1.83"**Net Sales**" means, with respect to a Licensed Product for any period, the gross invoice price of a particular Licensed Product sold or otherwise transferred to a Third Party (other than a Sublicensee, but including wholesalers [*]) by AGT, its Affiliates, or Sublicensees for consideration, reduced by the following amounts, all as calculated in accordance with Accounting Standards, consistently applied:

1.83.1[*];

1.83.2[*];

1.83.3[*]; and

1.83.4[*].

[*]. For purposes of calculating Net Sales, all Net Sales shall be converted into Dollars in accordance with Section 7.6.

If a Licensed Product is a Combination Product, the Net Sales for such Combination Product shall be calculated as follows:

[*].

1.84"**Non-Breaching Party**" has the meaning set forth in Section 13.2.1.

1.85"**Party**" and "**Parties**" has the meaning set forth in the preamble hereto.

1.86"**Patent Challenge**" means any action or proceeding in any patent office, court, or other appropriate legal forum, in each case, commenced by AGT or its Affiliates or Sublicensees challenging the validity or enforceability of any Licensor Patent.

1.87"**Patents**" means (a) all national, regional and international patents and patent applications, including provisional patent applications, (b) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from either of these, including divisionals, continuations, continuations-in-part, provisionals, converted provisionals and continued prosecution applications, (c) any and all patents

that have issued or in the future issue from the foregoing patent applications ((a) and (b)), including utility models, petty patents and design patents and certificates of invention, and (d) any and all extensions or restorations by existing or future extension or restoration mechanisms (including any supplementary protection certificates and the like) of the foregoing patents or patent applications ((a), (b), and (c)), and (e) any similar rights including so-called pipeline protection or any importation, revalidation, confirmation or introduction patent or registration patent or patent of additions to any of such foregoing patent applications and patents.

1.88"**Person**" means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, unincorporated association, joint venture or other similar entity or organization, including a government or political subdivision, department, or agency of a government.

1.89"**Phase I**" means a human clinical trial of a product, the principal purpose of which is a preliminary determination of safety, tolerability, pharmacological activity or pharmacokinetics in healthy individuals or patients or a similar clinical study prescribed by the Regulatory Authorities, including the trials referred to in 21 C.F.R. §312.21(a), as amended.

1.90"**Phase II**" means a human clinical trial of a product, the principal purpose of which is a determination of safety and efficacy in the target patient population, which is prospectively designed to generate sufficient data that may permit commencement of pivotal clinical trials, or a similar clinical study prescribed by the Regulatory Authorities, including the trials referred to in 21 C.F.R. §312.21(b), as amended.

1.91 "**Phase III**" means a human clinical trial of a product on a sufficient number of subjects in an indicated patient population that is designed to establish that such product is safe and efficacious for its intended use and to determine the benefit/risk relationship, warnings, precautions, and adverse reactions that are associated with such product in the dosage range to be prescribed, which trial is intended to support marketing approval of such product, or a similar clinical study prescribed by the Regulatory Authorities, including the trials referred to in 21 C.F.R. §312.21(c), as amended.

1.92"**Pivotal Study**" means a Clinical Study (whether or not designated a Phase III) for a product that is [*] the basis for submitting an application for, and obtaining Regulatory Approval of, such product for an indication without requiring any additional pre-approval Clinical Studies, based on [*].

1.93 "**Product Trademarks**" means the Trademark(s) Controlled by AGT or its Affiliates or its or their respective Sublicensees and used for the Commercialization of Licensed Products in the Territory and any registrations thereof or any pending applications relating thereto in the Territory (excluding, in any event, any Trademarks that include any corporate name or logo of Licensor or its Affiliates).

1.94"**Rare Ophthalmology Target**" means any of the Targets set forth on Schedule 1.94.

1.95 "**Regulatory Approval**" means, with respect to a country or other jurisdiction in the Territory, any and all approvals (including approvals of Drug Approval Applications), licenses, registrations, or authorizations of any Regulatory Authority necessary to Commercialize a product in such country or other jurisdiction, including, where applicable, (a) pricing or reimbursement approval in such country or other jurisdiction, (b) pre- and post-approval marketing authorizations (including any prerequisite Manufacturing approval or authorization related thereto), and (c) approval of product labeling. For clarity, subsection (a) shall be excluded from the "Regulatory Approval" definition for purposes of determining achievement of the milestone event set forth in Section 7.2.1(e).

1.96"**Regulatory Authority**" means any applicable supra-national, federal, national, regional, state, provincial, or local governmental or regulatory authority, agency, department, bureau, commission, council, or other entities (*e.g.*, the FDA or EMA) regulating or otherwise exercising authority with respect to activities contemplated in this Agreement, including the Exploitation of the Licensed Compound or Licensed Products in the Territory.

1.97"**Regulatory Documentation**" means all (a) applications (including all INDs and Drug Approval Applications and other major regulatory filings), registrations, licenses, authorizations, and approvals (including Regulatory Approvals), (b) correspondence and reports submitted to or received from Regulatory Authorities (including minutes and official contact reports relating to any communications with any Regulatory Authority) and all supporting documents with respect thereto, including all regulatory drug lists, advertising and promotion documents, adverse event files, and complaint files, and (c) Clinical Data and data contained or relied upon in any of the foregoing, in each case ((a), (b), and (c)) to the extent relating to the 4D Vector or to any Licensed Compound or Licensed Product.

1.98"**Regulatory Exclusivity**" means, with respect to a Licensed Product and country, any exclusive marketing rights or data exclusivity rights (other than a Patent) conferred by the applicable Regulatory Authority in such country with respect to such Licensed Product that provides AGT, its Affiliates or Sublicensees with the exclusive right to market and sell such Licensed Product in such country.

1.99"**Reporter Genes**" shall mean (a) [*] or (b) [*].

1.100[*].

1.101"**Reserved Target Candidates**" means, subject to Section 2.5, the Targets known as (a) [*], (b) [*], (c) [*], and (d) [*] additional Rare Ophthalmology Target that is not an Unavailable Target and that is deemed a Reserved Target Candidate in accordance with Section 2.5.1.

1.102"**Reserved Target Candidate Identification Notice**" means a written notice sent by AGT to Licensor stating that AGT is formally selecting a Rare Ophthalmology Target as a Reserved Target Candidate in accordance with Section 2.5.1.

1.103"**Royalty Term**" means, with respect to each Licensed Product and each country in the Territory, the period beginning on the date of the First Commercial Sale of such Licensed Product in such country, and ending on the later to occur of (a) (i) the expiration, invalidation, or

abandonment date of the last Licensor Patent or Joint Patent that includes a Valid Claim that claims the composition of matter or method of use of such Licensed Product in such country or (ii) the expiration, invalidation, or abandonment date of the last AGT [*] Patent that includes a Valid Claim that claims the composition of matter of such Licensed Product in such country, (b) the tenth (10th) anniversary of the First Commercial Sale of such Licensed Product in such country, or (c) the expiration of any Regulatory Exclusivity for such Licensed Product in such country.

1.104"**Segregated**" has the meaning set forth in Section 6.9.2.

1.105"**Sublicensee**" means a Person, other than an Affiliate or a Distributor, that is granted a sublicense by AGT under the grants in Section 6.1 as provided in Section 6.4.

1.106"**Substitute Target**" has the meaning set forth in Section 2.1.

1.107"**Substitute Target Fee**" has the meaning set forth in Section 7.1.3.

1.108"**Target**" means (a) a human gene associated with a Monogenic Rare Disorder, including [*] and variants thereof or (b) [*] of Licensed Compounds or Licensed Products. For clarity, [*].

1.109"**Target Substitution Notice**" means a written notice sent by AGT to Licensor stating that AGT is formally designating a Substitute Target as an AGT Target in accordance with Section 2.3 and identifying the Target to be replaced.

1.110"**Tax**" means any present or future taxes, levies, imposts, duties, tariffs, charges, assessments, or fees of any nature imposed by a governmental authority in the exercise of its taxing power (including interest, penalties, and additions thereto), including value-added Tax and withholding Tax.

1.111"**Term**" has the meaning set forth in Section 13.1.1.

1.112"**Terminated Product**" means each Licensed Product with respect to which this Agreement is terminated by AGT pursuant to Section 13.3, or, if this Agreement is terminated in its entirety, all the Licensed Products.

1.113"**Terminated Target**" means each AGT Target for which this Agreement has been terminated or, if this Agreement is terminated in its entirety, all the AGT Targets.

1.114"**Territory**" means the entire world.

1.115"**Third Party**" means any Person other than Licensor, AGT and their respective Affiliates.

1.116"**Third Party Claims**" has the meaning set forth in Section 12.1.

1.117"**Trademark**" means any word, name, symbol, color, designation or device or any combination thereof that functions as a source identifier, including any trademark, trade dress,

brand mark, service mark, trade name, brand name, logo, business symbol or domain names, whether or not registered.

1.118"**Transferred Materials**" has the meaning set forth in Section 3.1.

1.119"**Transferred Materials Know-How**" means all Know-How embodied within the Transferred Materials.

1.120"**Unavailable Target**" means, with respect to a Rare Ophthalmology Target proposed as a Reserved Target Candidate by AGT in accordance with ARTICLE 2, that (a) [*], or (b) [*], in each case (a) and (b) [*]. For clarity, a Rare Ophthalmology Target would not be an Unavailable Target if [*].

1.121"**United States**" or "**U.S.**" means the United States of America and its territories and possessions (including the District of Columbia and Puerto Rico).

1.122"**Valid Claim**" means (a) a claim of any issued and unexpired Patent whose validity, enforceability, or patentability has not been affected by any of the following: (i) irretrievable lapse, abandonment, revocation, dedication to the public, or disclaimer; or (ii) a holding, finding, or decision of invalidity, unenforceability, or non-patentability by a court, governmental agency, national or regional patent office, or other appropriate body that has competent jurisdiction, such holding, finding, or decision being final and unappealable or unappealed within the time allowed for appeal, or (b) a claim in a pending Patent; *provided, however*, that, [*].

ARTICLE 2
TARGETS

2.1Generally. Up to three (3) AGT Targets may be designated by AGT under this Agreement, subject to this ARTICLE 2 below. The Initial Target has been designated as an AGT Target as of the Effective Date. AGT shall have the right, but not the obligation, to designate up to two (2) Reserved Target Candidates as AGT Targets in accordance with this ARTICLE 2, below (upon being deemed an AGT Target as set forth in this ARTICLE 2, below, each, an "**Additional Target**"). In addition, AGT shall have the right to substitute one (1) AGT Target with a Reserved Target Candidate in accordance with this ARTICLE 2, below (upon being deemed an AGT Target as set forth in this ARTICLE 2, below, a "**Substitute Target**").

2.2Selecting Additional Targets.

2.2.1 At any time on or prior to [*], AGT shall have the right, but not the obligation, to select up to two (2) Reserved Target Candidates as AGT Targets, subject to Sections 2.2.2 and 2.2.3, by providing an Additional Target Identification Notice to Licensor. For each proposed Additional Target that is deemed an AGT Target in accordance with Section 2.4, AGT shall pay a one-time Additional Target Fee as specified in Section 7.1.2 (*provided* that the amount of any fee that was prepaid pursuant to Section 2.2.2 is creditable against such payment).

2.2.2 If, on or prior to [*], AGT has not selected at least one (1) Reserved Target Candidate for inclusion in this Agreement as an Additional Target, then AGT shall lose the right

to designate one (1) of the two (2) Additional Targets if (a) within [*] after receipt of written notice thereof from Licensor given promptly after such [*], AGT fails to provide written notice to Licensor that it wishes to retain the right to designate two (2) Additional Targets in the [*] year of the Agreement or (b) AGT fails to pre-pay Licensor the first Additional Target Fee within [*] days after receipt of an invoice for such amount from Licensor delivered after AGT provides a written notice pursuant to foregoing clause (a).

2.2.3 Notwithstanding anything to the contrary in this ARTICLE 2, if AGT would like to designate the Reserved Target Candidate known as [*] to be an Additional Target, AGT must do so on or prior to [*], and if AGT does not designate the Reserved Target Candidate known as [*] as an Additional Target within such period, it shall be removed from the list of Reserved Target Candidates (without substitution with another Target), and shall no longer be subject to this Agreement.

2.3Target Substitution.

2.3.1 At any time prior to the [*], AGT shall have the right to substitute [*] AGT Target with a Reserved Target Candidate in accordance with this Section 2.3 by providing a Target Substitution Notice to Licensor. If a proposed Substitute Target is deemed an AGT Target in accordance with Section 2.4, AGT shall pay one-time Substitute Target Fee to Licensor as specified in Section 7.1.3.

2.3.2 Upon replacement of an AGT Target with a Substitute Target, (a) the replaced AGT Target shall (i) no longer be considered an AGT Target, (ii) be removed from the list of Reserved Target Candidates, (iii) no longer be subject to this Agreement, and (b) the Substitute Target shall be deemed an AGT Target for all purposes of this Agreement.

2.4Inclusion of Additional Targets and Substitute Target.

2.4.1 If AGT delivers an Additional Target Identification Notice or Target Substitution Notice, then within [*] after Licensor's receipt of each such notice, Licensor shall provide AGT with [*].

2.4.2 Unless Sections 2.4.3 or 2.4.4 apply, then such Target shall be deemed an Additional Target or Substitute Target, as applicable, and an AGT Target and, if applicable, the Target being replaced shall no longer be an AGT Target, in each case as of the date of [*].

2.4.3 [*].

2.4.4 [*].

2.5Reserved Target Candidates.

2.5.1 Selecting the Remaining Reserved Target Candidates.

(a) For as long as AGT has the right to request [*] Additional Target or Substitute Target under this Agreement, AGT shall have the right to include an additional Rare Ophthalmology Target(s) as a Reserved Target Candidate by providing a Reserved Target

Candidate Identification Notice to Licensor. If AGT delivers a Reserved Target Candidate Identification Notice, then, promptly (and in any event, within [*]) after Licensor's receipt of each such notice, Licensor shall provide AGT with written notice of whether or not such proposed Rare Ophthalmology Target is an Unavailable Target.

(b) If Licensor's notice under Section 2.5.1(a) indicates that the proposed Rare Ophthalmology Target is an Unavailable Target, then AGT shall have the right to select another Rare Ophthalmology Target as the proposed Reserved Target Candidate, and the terms of this Section 2.5.1 shall again apply. AGT shall request such alternative Rare Ophthalmology Target to be a Reserved Target Candidate on or before [*].

(c) If Licensor's notice under Section 2.5.1(a) indicates that the proposed Rare Ophthalmology Target is not an Unavailable Target, then together with such notice Licensor shall provide [*].

(d) Unless subsections (e) or (f) below apply, then such Rare Ophthalmology Target shall be deemed Reserved Target Candidate as of the date of [*].

(e) [*].

(f) [*].

2.5.2 Notice of Rare Ophthalmology Target [*]. For so long as AGT retains the right to include an Additional Target or Substitute Target under this Agreement, Licensor shall promptly (and in any event within [*] Business Days) provide written notice to AGT of any Rare Ophthalmology Target [*].

2.5.3 Integrity of Reserved Target Candidates. Prior to (a) the [*] with respect to the Reserved Target Candidate known as [*] or (b) the [*] with respect to any other Reserved Target Candidate, [*].

2.6Antitrust Events. If any Antitrust Events are initiated by any governmental authority or are required under Applicable Laws in connection with the designation of an Additional Target or Substitute Target, then Licensor shall reasonably cooperate, at AGT's cost and expense, with AGT in connection with any such Antitrust Events.

ARTICLE 3
DEVELOPMENT AND REGULATORY

3.1[*] **Transfer.** As soon as possible and, in any event, no later than [*], Licensor shall, at AGT's expense, deliver to AGT, at an address specified by AGT, at least [*] of [*] in accordance with this Agreement (the "**Transferred Materials**"). The Transferred Materials shall be Manufactured by Licensor or its Affiliates (or its' or its Affiliates' designated manufacturer) according to the applicable current good manufacturing practices pursuant to Applicable Law and shipped under appropriate conditions (including temperature control if needed).

3.2Development Activities. AGT (itself or through its Affiliates, Sublicensees or subcontractors) shall have the sole right to Develop the Licensed Compounds and Licensed

Products in the Field in the Territory in accordance with this Agreement at its own cost and expense.

3.3Diligence. During the Term, AGT (itself or through its Affiliates, Sublicensees or subcontractors) shall use Commercially Reasonable Efforts to Develop and obtain Regulatory Approval (including EU5 Pricing and Reimbursement Approval to the extent required for sale in the applicable EU5 country) for at least one (1) Licensed Product directed to each AGT Target in the United States and at least two (2) countries within the EU5.

3.4Reports. On a Licensed Product–by–Licensed Product basis, prior to the First Commercial Sale of a Licensed Product, AGT shall provide Licensor with a [*] written report [*] summarizing the status, progress, and results of its research and Development activities with respect to such Licensed Product under this Agreement during [*], which shall cover such subject matter at a reasonable level of detail. Additionally, at Licensor's request, the Parties will hold teleconference calls to discuss the status, progress, and results of AGT's research and Development activities under this Agreement [*]. In such diligence reports, AGT shall disclose [*].

3.5Regulatory Matters.

3.5.1 Regulatory Activities.

(a) As between the Parties, AGT shall have the [*] right to prepare, obtain, maintain, and hold the Drug Approval Applications (including the setting of the overall regulatory strategy therefor), other Regulatory Approvals and other submissions, and to conduct communications with the Regulatory Authorities, for Licensed Compounds and Licensed Products in the Field in the Territory (which shall include filings of or with respect to INDs and other filings or communications with the Regulatory Authorities with respect to Development activities). Licensor shall support AGT, at AGT's cost and expense, as may be reasonably necessary, in obtaining Regulatory Approvals for the Licensed Compounds and Licensed Products, and in the activities in support thereof, including by promptly providing safety data (including, for clarity, serious adverse event reports) for the 4D Vector (including such data and reports arising from AAV-based gene therapy products that utilize the 4D Vector) to AGT in order for AGT (or its Affiliates or Sublicensees) to obtain Regulatory Approvals, in each case to the extent in Licensor's or its Affiliates' possession and Control.

(b) All Regulatory Documentation (including all Regulatory Approvals and product labeling) [*] the Licensed Compounds and Licensed Products with respect to the Field and the Territory developed by or on behalf of AGT or its Affiliates or Sublicensees (and for clarity, not by Licensor or its Affiliates or (sub)licensees) shall be owned by, and shall be the sole property and held in the name of, AGT or its designated Affiliate, Sublicensee or other designee; *provided* that, for clarity, Licensor shall retain ownership of (i) its safety information and 4D Vector drug master files ("**4D Vector DMF**") and other documentation or information relating to the 4D Vector developed by or on behalf of Licensor or its Affiliates and provided to AGT pursuant to ARTICLE 9 or this Section 3.5.1 and (ii) its own regulatory documentation relating to the 4D Vector developed by or on behalf of Licensor or its Affiliates.

(c) Upon each Party's reasonable request, the other Party shall provide the requesting Party with safety information or safety data relating to the 4D Vector, to the extent such information is in such Party's possession and Control and is required by the applicable Regulatory Authority in connection with the requesting Party obtaining or maintaining Regulatory Approval for Licensed Products (if AGT is the requesting Party) or other products controlled by Licensor or its Affiliates (if Licensor is the requesting Party); *provided*, *however*, that, if the information requested relates to analytical and quality control data, stability data, or other chemistry, manufacturing, and control data, then, at the providing Party's option (and in all cases if such disclosure is precluded by Section 4.2), and upon prior written notice to the requesting Party, the providing Party shall provide such information or data directly to the applicable Regulatory Authority, in which case, the providing Party shall notify the requesting Party in writing once such information or data has been provided. The providing Party shall use reasonable efforts to provide such data prior to any timelines requested by the applicable Regulatory Authority. Notwithstanding the foregoing, this Section 3.5.1(c) shall not require either Party to disclose to the other Party or any Regulatory Authority (a) [*] or (b) in the case of Licensor, any data or information comprising the Excluded Licensor IP or, in the case of AGT, any Manufacturing information, in each case, except to the extent required by Applicable Law.

(d) Licensor hereby grants to AGT a right of reference to, and a right to use and rely upon, (i) any Regulatory Documentation solely to the extent containing safety information relevant to the 4D Vector that is Controlled by Licensor or its Affiliates and (ii) the 4D Vector DMF. Licensor shall execute such documents or instruments reasonably required to provide such right of reference or right of use promptly following written request by AGT. AGT hereby grants to Licensor a right of reference to, and a right to use and rely upon, any Regulatory Documentation generated by or on behalf of AGT or its Affiliates or Sublicensees in connection with this Agreement solely to the extent containing safety information relevant to the 4D Vector that is Controlled by AGT or its Affiliates. AGT shall execute such documents or instruments reasonably required to provide such right of reference or right of use promptly following written request by Licensor.

(e) AGT (or its Affiliate or Sublicensee) shall have the sole (as between the Parties) right to make the final determination whether to voluntarily implement any recall, market suspension, or market withdrawal of its, its Affiliate or Sublicensee's Licensed Compounds and Licensed Products in the Territory. For all recalls, market suspensions or market withdrawals undertaken pursuant to this Section 3.5.1(e), AGT (or its Affiliate or Sublicensee) shall be solely responsible for the execution thereof.

ARTICLE 4
MANUFACTURING

4.1Supply of 4D Vectors and Licensed Products. As between the Parties, AGT (or its Affiliate, Sublicensee, or designated subcontractor, as applicable) shall have the sole right, at its expense, to Manufacture (or have Manufactured) and supply (including process development and analytical development thereof) (a) the 4D Vector for use in Licensed Compounds and Licensed Products and (b) the Licensed Compounds and Licensed Products for Development or Commercialization in Field in the Territory in accordance with this Agreement.

4.2No Disclosure of Excluded Licensor IP by Licensor. Licensor shall use its reasonable efforts not (and shall cause its Affiliates to use their reasonable efforts not) to disclose to AGT any Excluded Licensor IP that has not been publicly disclosed or trade secrets of Licensor or its Affiliates (other than any trade secrets in the Transferred Materials Know-How).

ARTICLE 5
COMMERCIALIZATION

5.1Commercialization Activities. AGT (itself or through its Affiliates, Sublicensees, or other Third Parties) shall have the sole right to Commercialize the Licensed Compounds and Licensed Products in the Field in the Territory in accordance with this Agreement at its own cost and expense, including [*].

5.2Diligence. During the Term, after receipt of Regulatory Approval (including EU5 Pricing and Reimbursement Approvals to the extent required for sale in the applicable EU5 country) for such Licensed Product, AGT shall use Commercially Reasonable Efforts to Commercialize at least one Licensed Product directed to each AGT Target in the United States and at least two countries within the EU5.

5.3Product Trademarks. AGT shall have the sole right to determine the Product Trademarks to be used with respect to the sale of Licensed Products in the Territory. AGT shall own all right, title, and interest to its Product Trademarks in the Territory, and shall have the sole right with respect to the registration, prosecution, maintenance, and enforcement thereof. Notwithstanding the foregoing, to the extent required by Applicable Law in a country or other jurisdiction in the Territory, the promotional materials, packaging, and product labeling used by AGT and its Affiliates in connection with the Licensed Compounds and Licensed Products in such country or other jurisdiction shall contain (a) the corporate name of Licensor (and to the extent required, Licensor grants AGT a license, with the right to sublicense, to use the same), and (b) the logo and corporate name of the manufacturer (if other than AGT or an Affiliate).

ARTICLE 6
GRANT OF RIGHTS

6.1Grants to AGT. Licensor (on behalf of itself and its Affiliates) hereby grants to AGT:

6.1.1 an exclusive (including with regard to Licensor and its Affiliates) license (or sublicense), with the right to grant sublicenses in accordance with Section 6.4, under the Licensed IP and Licensor’s interest in the Joint Patents and the Joint Know-How to Exploit the Licensed Compounds and Licensed Products (and the 4D Vector solely for use in Licensed Compounds and Licensed Products) in the Field in the Territory;

6.1.2 up to and until [*], a non-exclusive license, with the right to grant sublicenses in accordance with Section 6.4, under the Licensed IP to [*]; and

6.1.3 a non-exclusive, royalty-free, fully paid license, with the right to grant sublicenses in accordance with Section 6.4, [*].

6.2Licensor Covenant [*]**.** Licensor (on behalf of itself and its Affiliates) hereby covenants that:

6.2.1 at no time will it, its successors or its assigns, directly or indirectly, alone or by, with, or through others, cause, induce, consent to, or authorize, or voluntarily assist, participate, or cooperate in the commencement, maintenance, or prosecution of, commence, maintain, or prosecute any action or proceeding of any kind or nature whatsoever (including any suit, complaint, grievance, demand, claim, cause of action in, of, or before any Governmental Authority) (collectively, an "**[*] Action**") against AGT, its Affiliates or Sublicensees or its or their distributors, customers, manufacturers or other Third Parties acting on their behalf (i.e. to the extent such party's actions or omissions are by, on behalf of, or for the benefit of AGT, its Affiliates or Sublicensees) asserting [*];

6.2.2 it shall use Commercially Reasonable Efforts [*] set forth in foregoing Section 6.2.1; and

6.2.3 [*], as applicable, to comply with the terms of Section 6.2.1 and Section 6.2.2.

This covenant shall be binding upon, and inure to the benefit of, the Parties, their successors, and assigns. Notwithstanding the foregoing, the covenants in this Section 6.2 are subject to the covenant in Section 11.4 and do not permit [*].

6.3AGT Covenant [*]. AGT (on behalf of itself and its Affiliates) hereby covenants that:

6.3.1 at no time will it, its successors or its assigns, directly or indirectly, alone or by, with, or through others, cause, induce, consent to, or authorize, or voluntarily assist, participate, or cooperate in the commencement, maintenance, or prosecution of, commence, maintain, or prosecute any [*] Action against Licensor or its Affiliates or licensees or its or their distributors, customers, manufacturers or other Third Parties acting on their behalf (i.e. to the extent such party's actions or omissions are by, on behalf of, or for the benefit of Licensor, its Affiliates or licensees) asserting [*];

6.3.2 it shall use Commercially Reasonable Efforts [*] set forth in foregoing Section 6.3.1; and

6.3.3 [*], as applicable, to comply with the terms of Section 6.3.1 and Section 6.3.2.

This covenant shall be binding upon, and inure to the benefit of, the Parties, their successors, and assigns. Notwithstanding the foregoing, the covenant in this Section 6.3 does not [*].

6.4Sublicenses. AGT shall have the right to grant sublicenses (or further rights of reference or covenants not to sue), through multiple tiers of Sublicensees, under the licenses, rights of reference and covenants not to sue granted in Sections 3.5.1(d), 6.1.1, 6.1.2, 6.1.3 and 6.2, to its Affiliates and Third Parties; provided that such right to sublicense [*]. Licensor shall have the right to grant sublicenses under the rights of reference and covenants not to sue granted in Sections

3.5.1(d) and 6.3. Each such sublicense to a Third Party shall be subject to a written agreement that is consistent with the terms and conditions of this Agreement (including with respect to each Party's obligations relating to confidentiality and non-use of information, and consistent with the intellectual property provisions, set forth in this Agreement). Each Party shall remain directly responsible for fulfillment of all of its obligations under this Agreement, regardless of whether any such obligation is sublicensed to any of its Affiliates or sublicensees, unless successfully performed by an Affiliate or sublicensee.

6.5Subcontracting. AGT shall have the right to subcontract any of its activities under this Agreement to its Affiliates or Third Parties. AGT shall cause any Third Party subcontractor engaged by it to be bound by written obligations of confidentiality and non-use at least as protective of Licensor and Licensor's Confidential Information as the terms of this Agreement prior to such subcontractor gaining access to any of Licensor's Confidential Information. AGT shall remain directly responsible for any of its obligations under this Agreement that have been delegated or subcontracted to any subcontractor (unless successfully performed by a subcontractor) and shall be directly responsible for the performance of its subcontractors.

6.6Distributorships. AGT shall have the right, in its sole discretion, to appoint its Affiliates, and AGT and its Affiliates shall have the right, in their sole discretion, to appoint any other Persons to distribute[*] the Licensed Products, subject to Section 6.5. Where AGT or its Affiliates appoints such a Person and such Person is not an Affiliate of AGT, that Person shall be a "**Distributor**" for purposes of this Agreement.

6.7Retention of Rights; No Implied Licenses.

6.7.1 For clarity, subject to Licensor's exclusivity obligations under Section 6.9, Licensor retains the right to practice under the Licensor Patents, Licensor Know-How, Joint Patents, Joint Know-How, Transferred Materials and Transferred Materials Know-How (a) to Exploit products (other than Licensed Compounds and Licensed Products) that incorporate the 4D Vector in the Field and in the Territory and (b) for any other purpose outside of the scope of the licenses granted under Section 6.1.

6.7.2 Except as set forth in this Agreement, neither Party shall acquire any license or other intellectual property interest, by implication or otherwise, under or to any intellectual property Controlled by the other Party or its Affiliates. Notwithstanding anything to the contrary in this Agreement, the licenses or covenant not to sue granted by Licensor under this Agreement do not include rights to any vectors other than the 4D Vector or any payloads that are Controlled by Licensor or its Affiliates.

6.8Confirmatory Patent License. Licensor shall, and shall procure that its Affiliates shall, if requested to do so by AGT and at AGT's sole cost and expense, (a) promptly enter into confirmatory license agreements in a form consistent with this Agreement and reasonably requested by AGT for purposes of recording the licenses granted under this Agreement with such patent offices in the Territory as AGT considers appropriate and (b) assist AGT in registering or filing such licenses.

6.9Exclusivity.

6.9.1 Prior to the seventh (7th) anniversary of the Effective Date, Licensor shall not, and shall cause its Affiliates not to (a) Exploit any Competing Product in any country or other jurisdiction in the Territory, or (b) license, authorize, appoint, or otherwise enable any Third Party to Exploit any Competing Product in any country or other jurisdiction in the Territory.

6.9.2 Notwithstanding Section 6.9.1, if Licensor or any of its Affiliates undergoes a Change of Control and, on the date of the closing of such Change of Control, the Acquirer owns or has in-licensed a Competing Product ("**Acquirer Competing Product**"), then Licensor or its Affiliate or Acquirer, as applicable, will not be in breach of Section 6.9.1 as a result of such Acquirer Competing Product, if the Acquirer Competing Product and activities related thereto are promptly Segregated (and remain Segregated during the period set forth in Section 6.9.1). Promptly following the closing date of a Change of Control of Licensor (or its successor) involving an Acquirer that has an Acquirer Competing Product, Licensor shall provide AGT with written notice of such Change of Control to AGT. "**Segregated**" means, with respect to an Acquirer Competing Product, to (a) [*]; and (b) [*].

6.9.3 Licensor shall not, and shall cause its Affiliates not to, grant a license to any Third Party under the Excluded Licensor IP to Exploit Licensed Compounds or Licensed Products (or the 4D Vector for use in Licensed Compounds or Licensed Products) in the Field in the Territory.

ARTICLE 7
PAYMENTS AND RECORDS

7.1Fees.

7.1.1 Upfront Payment. As partial consideration for the rights and licenses granted to AGT by Licensor under this Agreement, AGT shall pay Licensor an upfront amount of Twenty Million Dollars ($20,000,000) within [*] Business Days after the Effective Date.

7.1.2 Additional Target Fee. In further consideration of the rights granted by Licensor to AGT hereunder and subject to the terms and conditions set forth in this Agreement, AGT will pay to Licensor a fee of [*] within [*] days after receipt of an invoice from Licensor delivered after each Additional Target that is deemed to be an AGT Target in accordance with Section ARTICLE 2 (each, an "**Additional Target Fee**"). For clarity, (a) if AGT has prepaid an Additional Target Fee pursuant to Section 2.2.2, then no additional fee shall be due when the first Additional Target is deemed to be an AGT Target and (b) the maximum aggregate amount payable by AGT under Section 2.2.2 and this Section 7.1.2 is [*] if two (2) Targets are deemed to be Additional Targets in accordance with ARTICLE 2.

7.1.3 Substitute Target Fee. In further consideration of the rights granted by Licensor to AGT hereunder and subject to the terms and conditions set forth in this Agreement AGT will pay to Licensor a one-time fee of [*] within [*] days after receipt of an invoice from Licensor delivered after the Substitute Target is deemed to be an AGT Target in accordance with ARTICLE 2 ("**Substitute Target Fee**").

7.2Development and Regulatory Milestones.

7.2.1 In further consideration of the rights granted by Licensor to AGT hereunder and subject to the terms and conditions set forth in this Agreement, on an AGT Target–by–AGT Target basis, AGT shall pay to Licensor each milestone payment set forth below after the achievement of each of the following milestones:

(a) [*];

(b) [*];

(c) [*];

(d) [*];

(e) [*]; and

(f) [*].

7.2.2 AGT shall promptly notify Licensor in writing of the achievement of each milestone event set forth above and shall make the corresponding milestone payment within [*] days after receipt by AGT of an invoice from Licensor delivered after such notice.

If a milestone event set forth in Section [*] is achieved prior to the achievement of any preceding milestone event set forth in Section [*] for the same AGT Target (*i.e.*, if a lower-listed milestone event is achieved before a milestone event that is listed earlier in Section 7.2.1), then upon achievement of the relevant milestone event, all preceding milestone events for such AGT Target set forth in Section 7.2.1 shall become due and payable if not previously paid for such AGT Target; *provided* that, for clarity, the achievement of the milestone event set forth in Section [*] will not, solely due to such achievement, trigger the payment set forth in Section [*]. Each milestone payment in this Section 7.2 shall be payable only upon the first achievement of such milestone with respect to an AGT Target and no amounts shall be due for subsequent or repeated achievements of such milestone, whether for the same or a different Licensed Product against such AGT Target. The maximum aggregate amount payable by AGT pursuant to this Section 7.2 per AGT Target, if all milestone events are achieved, is [*].

7.3Net Sales-Based Milestones.

7.3.1 In further consideration for the rights and licenses granted to AGT by Licensor under this Agreement, on an AGT Target–by–AGT Target basis, subject to Section 7.3.4, following the first achievement of each sales milestone event set forth in the left hand column of the table immediately below for an AGT Target, AGT shall make the corresponding one-time milestone payment in the corresponding amount set forth in the right hand column of the table immediately below.

Sales Milestone Event (per AGT Target)	One Time Net Sales-Based Milestone Payment Amount
[*]	[*]
[*]	[*]
[*]	[*]

7.3.2 AGT shall promptly notify Licensor in writing of the achievement of each milestone event set forth above following the end of the Calendar Year in which such milestone was achieved and shall make the corresponding milestone payment within [*] days after receipt by AGT of an invoice from Licensor promptly delivered after such notice.

7.3.3 Each milestone payment in this Section 7.3 shall be payable a maximum of one time per AGT Target, and no amounts shall be due for subsequent or repeated achievements of such milestone in subsequent Calendar Years by such AGT Target. The maximum aggregate amount payable by AGT pursuant to this Section 7.3 is [*] per AGT Target.

7.3.4 During any period during which the Royalty Term for a Licensed Product in a country has expired, Net Sales of such Licensed Product in such country shall be excluded from Net Sales for purposes of determining whether a sales milestone event in Section 7.3.1 has been achieved.

7.4Royalties.

7.4.1 Royalty Rates. As further consideration for the rights granted to AGT hereunder, subject to Sections 7.4.2 and 7.4.3 and the other terms and conditions of this Agreement, on an AGT Target–by–AGT Target basis, AGT shall pay to Licensor a royalty on Net Sales in the Territory during each Calendar Year at the following rates:

Net Sales in the Territory of Licensed Products against each AGT Target in a Calendar Year	Royalty Rate
[*]	[*]
[*]	[*]
[*]	[*]

7.4.2 Royalty Term. AGT shall have no obligation to pay any royalty with respect to Net Sales of any Licensed Product in any country during any period during which the Royalty Term for such Licensed Product in such country has expired. With respect to each Licensed Product in each country in the Territory, during any period during which the Royalty Term for such Licensed Product in such country has expired, Net Sales of such Licensed Product in such country shall be excluded from Net Sales, including for purposes of calculating the Net Sales thresholds and ceilings set forth in Section 7.4.1.

7.4.3 Reductions. Notwithstanding the foregoing:

(a) on a Licensed Product–by–Licensed Product and country-by-country basis, if in a particular Calendar Quarter during the Royalty Term for such Licensed Product one or more Third Parties commences selling a Biosimilar Product in a country and the aggregate unit sales of such Licensed Product in such country in such Calendar Quarter has [*] in such country (as measured against the aggregate unit sales for such Licensed Product in such country in the Calendar Quarter immediately preceding the first sale of the applicable Biosimilar Product in such country), then the royalties payable on the Net Sales of such Licensed Product in such country shall be reduced by [*] of the amounts otherwise payable to Licensor under Section 7.4.1 [*].

(b) On a country-by-country and Licensed Product–by–Licensed Product basis, if AGT, its Affiliates, or its Sublicensees [*] a license from a Third Party under such Third Party's Patents for the Exploitation of the 4D Vector for use in one or more Licensed Products (which Patents are not specific to any AGT Payload) in the Field in a country of the Territory (in accordance with Section 8.5), then AGT shall have the right to credit [*] of all royalties and other amounts paid to such Third Party with respect to such Licensed Product and such country pursuant to such license agreement for the license to Exploit such Third Party's Patents in connection with such Licensed Product in such country against any royalties owed to Licensor under Section 7.4.1 with respect to such Licensed Product and such country.

(c) On a country-by-country and Licensed Product–by–Licensed Product basis, if there is not at least one (1) Valid Claim in (i) a Licensor Patent or Joint Patent that claims [*] Licensed Product (ii) an AGT [*] Patent that [*] in such country, then the royalties payable on the Net Sales of such Licensed Product in such country shall be reduced by [*] of the royalties otherwise payable under Section 7.4.1 for such Licensed Product and such country.

(d) In no event shall the cumulative reductions under this Section 7.4.3 reduce the royalties payable by AGT to Licensor on the Licensed Products, on a country-by-country and Licensed Product–by–Licensed Product basis, in any Calendar Quarter by more than [*] of the amounts otherwise payable under Section 7.4.1, with respect to such Licensed Product and such country; [*].

7.5Royalty Payments and Reports. AGT shall calculate all amounts payable to Licensor pursuant to Section 7.4 at the end of each Calendar Quarter, which amounts shall be converted to Dollars, in accordance with Section 7.6. AGT shall pay to Licensor the royalty amounts due with respect to a given Calendar Quarter within [*] days after the end of such Calendar Quarter. Each payment of royalties due to Licensor shall be accompanied by a report setting forth, on a country-by-country and Licensed Product–by–Licensed Product basis, [*].

7.6Mode of Payment; Conversion. All payments to Licensor under this Agreement shall be made by deposit of Dollars in the requisite amount to such bank account as Licensor may from time to time designate by notice to AGT. For the purpose of calculating any sums due under, or otherwise reimbursable pursuant to, this Agreement (including the calculation of Net Sales expressed in currencies other than Dollars), AGT shall convert any amount expressed in a foreign currency into Dollar equivalents using its, its Affiliate's or Sublicensee's standard conversion methodology consistent with Accounting Standards.

7.7Withholding Taxes. Where any sum due to be paid to either Party hereunder is subject to any withholding or similar Tax, the Parties will use their reasonable efforts to do all such acts and things and to sign all such documents as shall enable them to take advantage of any applicable double taxation agreement or treaty, including, for the avoidance of doubt, a payee providing to the payor the documentation required by the jurisdiction of the payor to establish the payee's entitlement to the benefits of the applicable double taxation agreement or treaty prior to the payment. In the event there is no applicable double taxation agreement or treaty, or if an applicable double taxation agreement or treaty reduces but does not eliminate such withholding or similar Tax, the payor shall pay such Tax to the appropriate government authority, deduct the amount paid from the amount due to payee and secure and send to payee the best available evidence of such payment. If AGT assigns or otherwise transfers its rights and obligations hereunder to an Affiliate or Third Party and if such Affiliate or Third Party is required by Applicable Law to withhold any additional Tax from or in respect of any amount payable under this Agreement as a result of such assignment or transfer, then any such amount payable under this Agreement shall be increased to take into account the additional Tax withheld as may be necessary so that, after making all required withholdings, Licensor receives an amount equal to the sum it would have received as if such assignment or transfer had not occurred.

7.8Interest on Late Payments. If any payment due to either Party under this Agreement is not paid when due, then such paying Party shall pay interest thereon (before and after any judgment) at an annual rate equal to the greater of (a) [*] or (b) [*], in each case (a) and (b), compounded monthly (or the highest rate allowed under Applicable Law), such interest to run from the date on which payment of such sum became due until payment thereof in full together with such interest.

7.9Audit. AGT shall, and shall cause its Affiliates to, keep complete books and records pertaining to Net Sales of Licensed Products in sufficient detail to calculate all amounts payable hereunder. Such records shall be kept for (a) [*] following the end of the Calendar Quarter to which they pertain, or (b) such longer period as may be required under Applicable Law. Licensor shall have the right to have an independent, nationally recognized, certified public accountant reasonably acceptable to AGT audit AGT's records to confirm Net Sales, royalties, and Net Sales-based milestone payments for a period covering not more than [*] years following the Calendar Quarter to which they pertain. Such audits may be conducted during normal business hours upon reasonable prior written notice to AGT, and, except for cause, may not (a) be conducted more than [*] in any [*] period or (b) [*]. The accountant shall disclose to Licensor only whether the reports are correct or not, and the specific details concerning any discrepancies and shall not disclose AGT's Confidential Information to Licensor, except to the extent such disclosure is necessary to convey such discrepancies. The accountant shall enter into a customary confidentiality agreement with AGT. If there is no dispute regarding such audit and such audit concludes that (i) additional amounts were owed by AGT, then AGT shall pay the additional amounts, together with interest from the date originally due as provided in Section 7.8 within [*] days after invoicing following the accountant's report, or (ii) excess payments were made by AGT, then AGT may credit such excess payments against payment payable to Licensor under this Agreement (or, if insufficient future payments to Licensor are anticipated under this Agreement, Licensor shall pay such amounts within [*] days after invoicing by AGT). Licensor shall bear the cost of such audit unless such audit discloses an underpayment by AGT of more than [*] of the

amount of royalties or other payments due under this Agreement for the audited period, in which case, AGT shall bear the reasonable fees paid by Licensor to such auditor for such audit.

7.10Audit Dispute. In the event of a dispute with respect to any audit under Section 7.9, Licensor and AGT shall work in good faith to resolve the disagreement. If the Parties are unable to reach a mutually acceptable resolution of any such dispute within [*] days of either Party notifying the other of such dispute, the dispute shall be submitted for resolution to a certified public accounting firm jointly selected by each Party's certified public accountants or to such other Person as the Parties shall mutually agree (the "**Audit Expert**"). The decision of the Audit Expert shall be final and the costs of such arbitration as well as the initial audit shall be borne between the Parties in such manner as the Audit Expert shall determine. Not later than [*] days after such decision and in accordance with such decision, AGT shall pay the additional amounts (including, if the Audit Expert's decision resolves the dispute in favor of Licensor, any reasonable fees paid by Licensor to such auditor for the resolution of such dispute), together with interest from the date originally due as provided in Section 7.8, or the Licensor shall reimburse the excess payments, as applicable.

7.11Confidentiality. The receiving Party shall treat all Confidential Information subject to review under this ARTICLE 7 in accordance with the confidentiality provisions of ARTICLE 10 and the Parties shall cause the Audit Expert to enter into a reasonably acceptable confidentiality agreement with the audited Party obligating such firm to retain all such financial information in confidence pursuant to such confidentiality agreement.

7.12No Other Compensation. Each Party hereby agrees that the terms of this Agreement fully define all consideration, compensation, and benefits, monetary or otherwise, to be paid, granted, or delivered by one Party to the other Party in connection with the transactions contemplated herein. Neither Party previously has paid or entered into any other commitment to pay, whether orally or in writing, any of the other Party's employees, directly or indirectly, any consideration, compensation or benefits, monetary or otherwise, in connection with the transaction contemplated herein.

ARTICLE 8
INTELLECTUAL PROPERTY

8.1Ownership of Intellectual Property.

8.1.1 Ownership of IP. Subject to the rights and licenses granted under this Agreement, as between the Parties, each Party shall own and retain all right, title, and interest in and to any and all Know-How, Patents and other intellectual property Controlled by such Party as of the Effective Date or arising or acquired outside the scope of this Agreement.

8.1.2 Ownership of Foreground IP.

(a) Subject to the rights and licenses granted under this Agreement, as between the Parties, [*], except to the extent that [*].

(b) [*]. Each Party will have an equal and undivided joint ownership interest in and to Joint Know-How and Joint Patents. Each Party may exercise its ownership rights

in and to such Joint Know-How and Joint Patents, including the right to license and sublicense or otherwise to exploit through multiple tiers, transfer or encumber its ownership interest, without any duty of accounting or other obligation to, or consent required from (where consent is required by Applicable Law, such consent is deemed hereby granted), the other Party, but subject to the licenses and other rights granted to the other Party under this Agreement and the other terms and conditions of this Agreement. Each Party will grant and hereby does grant to the other Party all further permissions, consents, and waivers with respect to, and all licenses under, Joint Know-How and Joint Patents, throughout the world, necessary to effectuate the foregoing.

8.1.3 United States Law. The determination of whether Know-How and inventions are conceived, discovered, developed, or otherwise made by a Party for the purpose of allocating proprietary rights (including Patent, copyright or other intellectual property rights) therein in accordance with this Agreement, shall, for purposes of this Agreement, be made in accordance with Applicable Law in the United States as such law exists as of the Effective Date irrespective of where such conception, discovery, development, or making occurs.

8.2Maintenance and Prosecution of Patents.

8.2.1 Patent Prosecution and Maintenance of Licensor Patents. Licensor shall have the sole right, through the use of internal or outside counsel of its own choice, prepare, file, prosecute, and maintain the Licensor Patents worldwide, at Licensor's sole cost and expense. [*].

8.2.2 Patent Prosecution and Maintenance of AGT [*] Patents. AGT shall have the sole right (but not the obligation), through the use of internal or outside counsel of its own choice, prepare, file, prosecute, and maintain the AGT [*] Patents worldwide, at AGT's sole cost and expense. [*].

8.2.3 Patent Prosecution and Maintenance of Joint Patents.

(a) AGT shall have the first right (but not the obligation), through the use of internal or outside counsel, to prepare, file, prosecute, and maintain the Joint Patents worldwide, at AGT's sole cost and expense. AGT [*] keep Licensor [*] informed of [*] with regard to the preparation, filing, prosecution, and maintenance of Joint Patents, including by providing Licensor with a copy of material communications to and from any patent authority in the Territory regarding such Joint Patents, and by providing Licensor drafts of any material filings or responses to be made to such patent authorities in the Territory [*] in advance of submitting such filings or responses so as to allow for a reasonable opportunity for Licensor to review and comment thereon. AGT shall consider in good faith the requests and suggestions of Licensor with respect to such AGT drafts and with respect to strategies for filing and prosecuting the Joint Patents in the Territory, [*]. AGT [*] inform Licensor of any adversarial patent office proceeding or sua sponte filing, including a request for, or filing or declaration of, any interference, opposition, or reexamination relating to a Joint Patent in the Territory. The Parties shall thereafter consult and cooperate to determine a course of action with respect to any such proceeding in the Territory and AGT shall consider in good faith all comments, requests and suggestions provided by Licensor. AGT shall not initiate any such adversarial patent office proceeding relating to a Joint Patent in the Territory without first consulting Licensor.

(b) If AGT decides not to prepare, file, prosecute, or maintain a Joint Patent in a country or other jurisdiction in the Territory, AGT shall provide reasonable prior written notice to Licensor of such intention (which notice shall, in any event, be given no later than [*] days prior to the next deadline for any action that may be taken with respect to such Joint Patent in such country or other jurisdiction). Licensor shall then have the option, in its sole discretion, to assume the control and direction of the preparation, filing, prosecution, and maintenance of such Joint Patent at its expense in such country or other jurisdiction. In such case, upon Licensor's written election provided no later than [*] days after such notice by AGT, Licensor may, in its sole discretion, continue or discontinue prosecution and maintenance of such Joint Patent.

(c) Each Party agrees to cooperate fully in the preparation, filing, prosecution, and maintenance of Joint Patents under this Section 8.2.3 at its own cost. Such cooperation includes (i) executing all papers and instruments, or requiring its employees or contractors, to execute such papers and instruments, so as enable the other Party to apply for and to prosecute patent applications in any country as permitted by this Section 8.2.3; and (ii) promptly informing the other Party of any matters coming to such Party's attention that may affect the preparation, filing, prosecution, or maintenance of any such patent applications.

8.2.4 Patent Term Extension and Supplementary Protection Certificate. AGT shall have the sole right to obtain and maintain, and make decisions regarding patent term extensions and supplementary protection certificates and any other extensions that are now or become available in the future, wherever applicable, with respect to the Licensed Products in any country or other jurisdiction and for applying, at its cost, with respect to (i) any Patents owned or controlled by AGT or its Affiliates and (ii) any Joint Patents. Licensor shall have the sole right to obtain and maintain, and make decisions regarding, any patent term extensions, supplementary protection certificates and their equivalent with respect to any Licensor Patent, at its own cost; [*].

8.2.5 Regulatory Patent Listings. AGT shall have the sole right to make all filings with Regulatory Authorities in the Territory with respect to any Patent listings for the Licensed Products. AGT shall consult with Licensor and consider Licensor's comments in good faith with respect to the listing of any Licensor Patents or Joint Patents, and shall notify Licensor of any such listing of any Licensor Patents or Joint Patents promptly after they are made.

8.3Enforcement and Defense of Patents.

8.3.1 Enforcement and Defense of Licensor Patents. Each Party shall promptly notify the other Party in writing of any alleged or threatened (a) infringement of any Licensor Patent by a Third Party (including alleged or threatened infringement based on the development, commercialization, or an application to market a product containing any Licensed Compound in the Territory) or (b) assertion of any invalidity or unenforceability of any of any Licensor Patent by any Third Party, in each case (a) and (b) in the Territory of which such Party becomes aware. Licensor shall have the first right, but not the obligation, to prosecute any the foregoing and Licensor shall retain control of the prosecution or defense of such claim, suit or proceeding. If Licensor prosecutes or defends any the foregoing with respect to any Licensor Patent, AGT shall, at the written request of Licensor, join as a party to such claim, suit, or proceeding in the Territory and participate with its own counsel at its own expense; *provided* that Licensor shall retain control

of the prosecution or defense of such claim, suit, or proceeding. If Licensor does not take commercially reasonable steps to prosecute or defend with respect to a Licensor Patent to the extent it claims or covers a Licensed Compound, (i) within [*] days following the first applicable notice provided above or (ii) *provided* that such date occurs after the first such notice is provided, [*] before the time limit, if any, set forth in appropriate laws and regulations for filing of such actions, whichever comes first, then AGT may request from Licensor consent to prosecute or defend such claim at AGT's own expense; *provided* that Licensor may withhold such consent in its reasonable discretion.

8.3.2 Enforcement and Defense of AGT [*] Patents. Each Party shall promptly notify the other Party in writing of any alleged or threatened (a) infringement of any AGT [*] Patent by a Third Party (including alleged or threatened infringement based on the development, commercialization, or an application to market a product containing any Licensed Compound in the Territory) or (b) assertion of any invalidity or unenforceability of any of any AGT [*] Patent by any Third Party, in each case (a) and (b) in the Territory of which such Party becomes aware. AGT shall have the sole right, but not the obligation, to prosecute any of the foregoing and AGT shall retain control of the prosecution or defense of such claim, suit or proceeding.

8.3.3 Enforcement and Defense of Joint Patents. Each Party shall promptly notify the other Party in writing of any alleged or threatened (a) infringement of any Joint Patent by a Third Party (including alleged or threatened infringement based on the development, commercialization, or an application to market a product containing any Licensed Compound in the Territory) or (b) assertion of any invalidity or unenforceability of any of the Joint Patents by any Third Party, in each case in the Territory of which such Party becomes aware. AGT shall have the first right, but not the obligation, to prosecute any the foregoing and AGT shall retain control of the prosecution or defense of such claim, suit or proceeding. If AGT prosecutes or defends any the foregoing with respect to any Joint Patent, Licensor shall, at the written request of AGT, join as a party to such claim, suit, or proceeding in the Territory and participate with its own counsel at AGT's expense; *provided* that AGT shall retain control of the prosecution or defense of such claim, suit, or proceeding. If AGT does not take commercially reasonable steps to prosecute or defend with respect to a Joint Patent, then (i) within [*] days following the first applicable notice provided above or (ii) *provided* that such date occurs after the first such notice is provided, [*] before the time limit, if any, set forth in appropriate laws and regulations for filing of such actions, whichever comes first, then Licensor may request from AGT consent to prosecute or defend such claim at Licensor's own expense; *provided* that AGT may withhold such consent in its reasonable discretion.

8.3.4 Patent Exclusivity Listings. If Licensor receives a copy of application or submission filed with a Regulatory Authority for Regulatory Approval of a Biosimilar Product (a "**Biosimilar Application**") naming a Licensed Product as a reference product or otherwise becomes aware that such a Biosimilar Application has been filed (such as in an instance described in Section 351(l)(9)(C) of the PHSA), Licensor shall, within [*], notify and provide AGT with copies of such communication. AGT shall have the sole right to designate the outside counsel and in-house counsel who shall receive confidential access to the Biosimilar Application. AGT shall have the sole right to list any Patents, including Joint Patents and Licensor Patents, insofar as they claim or cover the applicable Licensed Product as required pursuant the PHSA, to respond to any communications with respect to such lists from the filer of the Biosimilar Application, and to

negotiate with the filer of the Biosimilar Application as to whether to utilize a different mechanism for information exchange than that specified in the PHSA, and AGT shall have the sole right to identify Patents or respond to communications under any equivalent or similar listing in any other jurisdiction in the Territory. AGT shall [*] with respect to any such listing of any Licensor Patents or Joint Patents, and shall [*] of any such listing of any Licensor Patents or Joint Patents [*].

8.3.5 Cooperation. The Parties agree to cooperate fully in any infringement or defense action pursuant to this Section 8.3. Where a Party brings such an action or defends a claim, the other Party shall, to the extent required by Applicable Law (or otherwise necessary for standing purposes), furnish a power of attorney solely for such purpose or shall join in, or be named as a necessary party to, such action Unless otherwise set forth herein, the Party entitled to bring any patent infringement litigation or defend any invalidity or unenforceability claim in accordance with this Section 8.3 shall have the right to settle such claim; *provided* that neither Party shall have the right to settle any litigation under this Section 8.3 in a manner that admits to the invalidity or unenforceability of any Licensor Patent or Joint Patent (or otherwise affects the scope, validity or enforceability of such Licensor Patent or Joint Patent), that imposes any costs or liability on, or involves any admission of liability, wrongdoing or fault by, the other Party, without the express written consent of the other Party and [*]. The Party commencing the litigation or defending any claims shall provide the other Party with copies of all pleadings and other documents filed with the court and shall consider reasonable input from the other Party during the course of the proceedings.

8.3.6 Recovery. Any recovery realized as a result of such litigation described in Section 8.3.1 and 8.3.2 (whether by way of settlement or otherwise) shall be first allocated to reimburse the Parties for their costs and expenses in making such recovery (which amounts shall be allocated pro rata if insufficient to cover the totality of such expenses). Any remainder after such reimbursements are made shall be [*].

8.4Infringement Claims by Third Parties. If the Manufacture, sale, or use of a Licensed Compound or Licensed Product in the Territory pursuant to this Agreement results in, or may result in, any claim, suit, or proceeding by a Third Party alleging patent infringement by AGT (or its Affiliates or Sublicensees) of such Third Party's intellectual property rights specifically as a result of the use of a 4D Vector in such Licensed Compound or Licensed Product, AGT shall [*] notify Licensor thereof in writing. AGT shall have the sole right, but not the obligation, to defend and control the defense of any such claim, suit, or proceeding at its own expense (but subject to deduction as provided below), using counsel of its own choice. Licensor may participate in any such claim, suit, or proceeding with counsel of its choice at its own expense. Without limitation of the foregoing, if Licensor is required by Applicable Law or is otherwise necessary to be joined as a party to any such action, Licensor shall execute all papers and perform such acts as shall be reasonably required, at AGT's expense. AGT shall keep Licensor reasonably informed of all material developments in connection with any such claim, suit, or proceeding.

8.5Third Party Licenses. As between the Parties, AGT has the [*] right (but not the obligation), at its expense (subject to the royalty reduction set forth in Section 7.4.3(b)), to obtain licenses under any Third Party intellectual property to Exploit the Licensed Compounds and Licensed Products. Notwithstanding the foregoing, if [*].

8.6Inventor's Remuneration. Each Party shall be solely responsible for any remuneration that may be due such Party's inventors under any applicable inventor remuneration laws.

8.7Patent Marking. AGT shall, and shall require its Affiliates and Sublicensees to, mark all Licensed Products (or their packaging) sold by it or them hereunder in a reasonable manner consistent with industry custom and practice with appropriate patent numbers or indicia to the extent permitted by Applicable Law.

ARTICLE 9
PHARMACOVIGILANCE AND SAFETY

9.1Safety Reporting and Pharmacovigilance Agreement. The Parties shall comply with any and all Applicable Laws in connection with the collection and reporting of safety data related to respective Party's (or its Affiliate's or sublicensee's) products incorporating the 4D Vector. Prior to Initiation of a Phase I for each Licensed Product directed to each AGT Target, the Parties shall enter into an agreement to initiate a process for the mutual exchange of safety data (including post-marketing spontaneous reports received by each Party and its Affiliates) in a mutually agreed format in order to monitor the safety of such Licensed Product and any products incorporating the 4D Vector and to meet reporting requirements with any applicable Regulatory Authority.

9.2Safety Information. All safety Know-How (including, for clarity, serious adverse event reports) in the Control of Licensor or its Affiliates with respect to the 4D Vector (including Conditioning Regimens applied to Clinical Studies of any products incorporating the 4D Vector), that were made available to AGT or its Affiliates as of the Effective Date or that is provided during the Term pursuant to Section 3.5.1 or this ARTICLE 9 may be used solely as necessary or reasonably useful for AGT, its Affiliate or Sublicensees to obtain or maintain Regulatory Approvals for Licensed Products or to comply with its pharmacovigilance responsibilities for the use of the 4D Vector in a Licensed Product or to Exploit a Licensed Product in accordance with this Agreement. With respect to safety Know-How related to the 4D Vector generated or obtained by either Party after the Effective Date (including [*]), to the extent such safety Know-How is in the Control of that Party or its Affiliates, each Party shall provide the other Party with all such safety Know-How (and such other Party shall have the right to use such safety Know-How) as is necessary to comply with its pharmacovigilance responsibilities for (a) in the case of AGT, the use of the 4D Vector with Licensed Products or (b) in the case of Licensor, the use of the 4D Vector with products that are not Licensed Products. Any such safety Know-How shall be the disclosing Party's Confidential Information and would be provided on an "as is where is" basis without any representation or warranty. For purposes of this Section, safety information shall include any adverse drug experiences, including from pre-clinical or clinical laboratory, animal toxicology and pharmacology studies, Clinical Studies, and commercial experiences.

9.3Safety Databases. Licensor shall be the holder of any global safety database related to any products incorporating the 4D Vector (other than Licensed Products). AGT shall be solely accountable for reporting all safety information required to be submitted to the concerned Regulatory Authorities, ethics committees, institutional review boards or investigators as required by Applicable Laws related to Licensed Products. Where applicable and when applicable, AGT

shall be solely responsible for pharmacovigilance activities related to Licensed Products in the respective country(ies) according to Applicable Laws. Licensor shall be solely responsible for reporting all safety information required to be submitted to the concerned Regulatory Authorities, ethics committees, institutional review boards or investigators as required by Applicable Laws related to any products incorporating the 4D Vector (other than the Licensed Product).

ARTICLE 10
CONFIDENTIALITY AND NON-DISCLOSURE

10.1Confidentiality Obligations. At all times during the period set forth in Section 10.7, each Party shall, and shall cause its officers, directors, employees and agents to, keep confidential and not publish or otherwise disclose to a Third Party and not use, directly or indirectly, for any purpose, any Confidential Information furnished or otherwise made known to it, directly or indirectly, by the other Party, except to the extent such disclosure or use is expressly permitted by the terms of this Agreement or such use is reasonably necessary for the performance of, or the exercise of its rights under, this Agreement. Notwithstanding the foregoing, to the extent the receiving Party can demonstrate by documentation or other competent proof, the confidentiality and non-use obligations under this Section 10.1 with respect to any Confidential Information shall not include any information that:

10.1.1was publicly known and made generally available in the public domain prior to the time of disclosure to the receiving Party by the disclosing Party;

10.1.2becomes publicly known and made generally available after disclosure to the receiving Party by the disclosing Party other than as a result of any wrongful act or a breach of this Agreement on the part of the receiving Party;

10.1.3is in the possession of the receiving Party, without confidentiality restrictions, at the time of disclosure by the disclosing Party as shown by the receiving party's files and records immediately prior to the time of disclosure; *provided* that the foregoing exception shall not apply to Joint Know-How;

10.1.4is obtained by the receiving Party from a Third Party not under confidentiality obligations and without a breach of any obligations of confidentiality; or

10.1.5was independently developed by the receiving Party without use of or benefit from Disclosing Party's Confidential Information, as shown by the receiving Party's files and records immediately prior to the time of disclosure; *provided* that the foregoing exception shall not apply to Joint Know-How.

10.2Permitted Disclosures. The applicable Party may disclose Confidential Information to the extent that such disclosure is:

10.2.1in the reasonable opinion of the receiving Party's legal counsel, required to be disclosed pursuant to Applicable Law; *provided* that the receiving Party shall first have given prompt written notice (and to the extent possible, at least [*] notice) to the disclosing Party and given the disclosing Party a reasonable opportunity to take whatever action it deems necessary to protect its Confidential Information. In the event that no protective order or other remedy is

obtained, or the disclosing Party waives compliance with the terms of this Agreement, the receiving Party shall furnish only that portion of Confidential Information which the receiving Party reasonably determines is legally required to be disclosed;

10.2.2made by or on behalf of AGT or, solely in the case of safety Know-How provided to Licensor under Section 9.2, Licensor to the Regulatory Authorities in connection with any filing, application, or request for Regulatory Approval; *provided* that reasonable measures shall be taken to assure confidential treatment of such Confidential Information to the extent practicable and consistent with Applicable Law;

10.2.3made by or on behalf of AGT or, solely in the case of safety Know-How provided to Licensor under Section 9.2, Licensor to a patent authority as may be reasonably necessary or useful for purposes of obtaining, defending or enforcing a Patent; *provided* that (a) [*] unless [*] and (b) reasonable measures shall be taken to assure confidential treatment of such Confidential Information, to the extent such protection is available;

10.2.4made to its or its Affiliates' financial and legal advisors who have a need to know such disclosing Party's Confidential Information and are either under professional codes of conduct giving rise to expectations of confidentiality and non-use or under written agreements of confidentiality and non-use, in each case, at least as restrictive as those set forth in this Agreement; *provided* that the receiving Party shall remain responsible for any failure by such financial and legal advisors, to treat such Confidential Information as required under this Article; or

10.2.5made by a Party or its Affiliates or (sub)licensees to its or their advisors, consultants, clinicians, vendors, service providers, contractors, existing or prospective collaboration partners, licensees, sublicensees, or other Third Parties as may be necessary or useful in connection with (a) with respect to Licensor, use of safety Know-How disclosed by AGT pursuant to Section 9.2, and (b) with respect to AGT, the Exploitation of the Licensed Compounds, Licensed Products (and the 4D Vector solely for use in Licensed Compounds and Licensed Products), or otherwise in connection with the performance of its obligations or exercise of its rights under this Agreement; *provided* that such Persons shall be subject to obligations of confidentiality and non-use with respect to such Confidential Information substantially similar to the obligations of confidentiality and non-use of the receiving Party pursuant to this ARTICLE 11.

10.3Use of Name. Except as expressly provided herein, neither Party nor its Affiliates shall mention or otherwise use the name, logo, or Trademark of the other Party or any of its Affiliates (or any abbreviation or adaptation thereof) in any publication, press release, public marketing and promotional material, or other form of publicity without the prior written approval of such other Party in each instance. The restrictions imposed by this Section 10.3 shall not prohibit either Party or its Affiliates from making any disclosure identifying the other Party that, in the opinion of the disclosing Party's counsel, is required by Applicable Law or the rules of a stock exchange on which the securities of the disclosing Party or its Affiliate are listed (or to which an application for listing has been submitted); *provided* that such Party shall submit the proposed disclosure identifying the other Party in writing to the other Party as far in advance as reasonably practicable (and in no event less than [*] prior to the anticipated date of disclosure) so as to provide a reasonable opportunity to comment thereon.

10.4Public Announcements. Subject to the remainder of this Section 10.4, neither Party shall issue any public announcement, press release, or other public disclosure regarding this Agreement or its subject matter without the other Party's prior written consent, except (a) for the joint press release in the form attached hereto as Schedule 10.4, which will be issued promptly after the Effective Date, (b) for any such disclosure that is reasonably determined by the disclosing Party to be required by Applicable Law or the rules of a stock exchange on which the securities of the disclosing Party are listed (or to which an application for listing has been submitted), or (c) to the extent information regarding this Agreement or its subject matter has already been publicly disclosed (other than as a result of a breach of this Agreement), each Party may subsequently disclose the same information to the public without the consent of the other Party, *provided* that such information remains true, accurate, and up to date. In the event a Party is, in the opinion of its counsel, required by Applicable Law or the rules of a stock exchange on which its securities are listed (or to which an application for listing has been submitted) to make such a public disclosure, such Party shall submit the proposed disclosure in writing to the other Party as far in advance as reasonably practicable (and in no event less than [*] prior to the anticipated date of disclosure) so as to provide a reasonable opportunity to comment thereon. Except as otherwise set forth in this Agreement with respect to Confidential Information of the other Party, (i) AGT, its Sublicensees and its and their respective Affiliates shall have the right to publicly disclose research, development, and commercial information (including with respect to regulatory matters) regarding the Licensed Compounds and Licensed Products, and (ii) Licensor, its licensees and its and their respective Affiliates shall have the right to publicly disclose research, development, and commercial information (including with respect to regulatory matters) generated by Licensor, its licensees and its and their respective Affiliates regarding the 4D Vector.

10.5Publications. Each Party acknowledges that scientific publications must be strictly monitored to prevent any adverse effect from premature publication of results of the Development activities hereunder. Accordingly, Licensor shall not publish, present, or otherwise disclose, and shall cause its Affiliates and subcontractors and its and their employees and agents not to disclose, any material related to the Exploitation of the Licensed Compounds or Licensed Products without the prior written consent of AGT, and, for clarity, public announcements or other public disclosure or publications by AGT (and its Sublicensees) are subject to Section 10.6, if applicable. For clarity, this Section 10.5 shall not affect Licensor's rights to publish, present, or otherwise disclose any information relating to (a) the 4D Vector independent of its use in Licensed Compounds or Licensed Products in the Field in the Territory, or (b) Licensor's other products containing the 4D Vector.

10.6Licensor Review. If AGT desires to issue any public announcement or other public disclosure or publication containing information specific to the 4D Vector (i.e., independent of its use in Licensed Compounds or Licensed Products), including [*] in each case, other than as permitted under Sections 10.4, AGT will provide Licensor with a copy of such public announcement, publication or other public disclosure. AGT will specify with each such public announcement, publication, or other public disclosure, taking into account the urgency of the matter being disclosed, a reasonable period of time (which will be no less than [*] unless earlier disclosure is required by Applicable Law) within which Licensor may provide any comments on such announcement or disclosure. If Licensor notifies AGT in writing that it does not have any comments to such public announcement, publication, or other public disclosure, then AGT may issue such public announcement, publication or other public disclosure in the form provided to

Licensor. If Licensor provides any comments, the Parties will consult on such public announcement, publication, or public disclosure and AGT will consider Licensor's comments in good faith to prepare a mutually acceptable disclosure, provided that Licensor will have final decision-making authority with to the extent any such public announcement, publication or other public disclosure contains information specific to 4D Vector (i.e., independent of its use in Licensed Compounds or Licensed Products). For clarity, AGT shall not be required to seek the permission of Licensor to disclose (a) the joint press release in the form attached hereto Agreement as Schedule 10.4 or (b) to issue any public announcement, publication or other public disclosure solely to the extent the information contained therein has already been publicly disclosed (other than as a result of a breach of this Agreement), provided that such information remains true, accurate, and up to date as of such time of the disclosure.

10.7Survival. All Confidential Information shall continue to be subject to the terms of this Agreement during the Term and for the period of [*] years after expiration or termination of this Agreement in its entirety, *except* that, with respect to Confidential Information comprising a trade secret of a Party, such obligations shall continue thereafter for as long as such Confidential Information qualifies as a trade secret under Applicable Law, *provided* such Party holding such Confidential Information as a trade secret notifies the other Party thereof.

ARTICLE 11
REPRESENTATIONS AND WARRANTIES

11.1Mutual Representations and Warranties. Licensor and AGT each represents and warrants to the other, as of the Effective Date, as follows:

11.1.1Organization. It is duly organized, validly existing, and in good standing under the laws of the jurisdiction of its organization, and has all requisite power and authority, corporate or otherwise, to execute, deliver, and perform this Agreement.

11.1.2Authorization. The execution and delivery of this Agreement and the performance by it of the transactions contemplated hereby have been duly authorized by all necessary corporate action, and do not violate (a) such Party's charter documents, bylaws, or other organizational documents, (b) in any material respect, any agreement, instrument, or contractual obligation to which such Party is bound, (c) any requirement of any Applicable Law, or (d) any order, writ, judgment, injunction, decree, determination, or award of any court or governmental agency presently in effect applicable to such Party.

11.1.3Binding Agreement. This Agreement is a legal, valid, and binding obligation of such Party enforceable against it in accordance with its terms and conditions, subject to the effects of bankruptcy, insolvency, or other laws of general application affecting the enforcement of creditor rights, judicial principles affecting the availability of specific performance, and general principles of equity (whether enforceability is considered a proceeding at law or equity).

11.1.4No Inconsistent Obligation. It is not under any obligation, contractual or otherwise, to any Person that conflicts with or is inconsistent in any material respect with the

terms of this Agreement, or that would impede the diligent and complete fulfillment of its obligations hereunder.

11.2 Additional Representations and Warranties of Licensor. Licensor further represents and warrants to AGT, as of the Effective Date, as follows:

11.2.1 All Licensor Patents existing as of the Effective Date are listed on Schedule 11.2.1 (the "**Existing Licensor Patents**"). All Existing Licensor Patents are (a) subsisting and, to Licensor's Knowledge, are not invalid or unenforceable, in whole or in part, (b) are being diligently prosecuted in the respective patent offices in the Territory in accordance with Applicable Law, and (c) have been filed and maintained properly and correctly and all applicable fees have been paid on or before the due date for payment.

11.2.2 There are no claims, judgments, or settlements against, or amounts with respect thereto, owed by Licensor or any of its Affiliates relating to the Existing Licensor Regulatory Documentation, the Existing Licensor Patents, or the Licensor Know-How. No claim or litigation has been brought or threatened by any Person alleging, and Licensor has no Knowledge of any claim, whether or not asserted, that the Existing Licensor Patents or the Licensor Know-How are invalid or unenforceable. To Licensor's Knowledge, the Exploitation of the 4D Vector as contemplated herein, does not and will not violate, infringe, misappropriate, or otherwise conflict or interfere with any intellectual property or proprietary right of any Person. To Licensor's Knowledge, no Person is infringing or threatening to infringe or misappropriating or threatening to misappropriate the Existing Licensor Patents or the Licensor Know-How in connection with any Competing Product in the Field.

11.2.3 Licensor is the sole and exclusive owner of the entire right, title and interest in the Existing Licensor Patents and the Licensor Know-How free of any encumbrance, lien, or claim of ownership by any Third Party.

11.2.4 Licensor is entitled to grant the licenses to AGT specified herein under the terms and conditions of this Agreement. The Existing Licensor Patents represent all Patents within Licensor's or its Affiliates' ownership or Control relating to the Exploitation of the 4D Vector in connection with a Licensed Compound or Licensed Product in the Field.

11.2.5 Except for [*], neither Licensor nor its Affiliates have licensed, authorized, appointed, or otherwise enabled any Third Party to directly or indirectly, research, develop, commercialize, or otherwise exploit any AAV-based gene therapy product that is directed to the Initial Target, any of the Reserved Target Candidates as defined in subsections (a)-(c) of the definition thereof, or any Rare Ophthalmology Target in any country or other jurisdiction in the Territory.

11.2.6 Licensor has the right to use all Licensor Know-How and Existing Licensor Patents necessary for AGT to Manufacture and use the 4D Vector as contemplated herein. Licensor has the right to provide the Transferred Materials to AGT for use as contemplated in this Agreement.

11.2.7 Licensor has provided to AGT copies of [*] provided to or made available to AGT by Licensor prior to the Effective Date, are true, complete, and correct.

11.2.8 Licensor and its Affiliates have generated, prepared, maintained, and retained all material safety data in its or its Affiliates possession and Control with respect to the 4D Vector that is required to be maintained or retained pursuant to and in accordance with Applicable Law, and all such information is true, complete, and correct and what it purports to be. Licensor has made available to AGT all material safety Licensor Know-How in the possession and Control of Licensor or its Affiliates with respect to the 4D Vector.

11.2.9[*] any Existing Licensor Patents or any Licensor Know-How has assigned and has executed an agreement assigning its entire right, title, and interest in and to such Existing Licensor Patents and Licensor Know-How to Licensor. To Licensor's Knowledge, no current officer, employee, agent, or consultant of Licensor or any of its Affiliates is in violation of any term of any assignment or other agreement regarding the protection of Patents or other intellectual property or proprietary information of Licensor related to the Existing Licensor Patents or Licensor Know-How.

11.2.10 Licensor has [*] all Licensor Know-How developed or delivered by any Third Party under any agreements between Licensor and any such Third Party with respect to the 4D Vector, and Licensor [*] under each such agreement to transfer such Licensor Know-How to AGT and its designees and to [*] such Licensor Know-How in the Exploitation of the Licensed Compounds or Licensed Products in the Field.

11.2.11 Neither Licensor nor any of its Affiliates, nor any of its or their respective officers, employees, or agents has [*].

11.2.12 Licensor has conducted, and has caused its contractors and consultants to conduct, any and all Development of the Licensor Know-How and the 4D Vector in accordance with Applicable Law. [*]

11.2.13 There are no amounts that are (or, under any agreements existing as of the Effective Date, will be) required to be paid to a Third Party as a result of the use of the 4D Vector in the Development or Commercialization of the Licensed Compounds or Licensed Products in the Field that arise out of [*].

11.2.14 Licensor and its Affiliates have never been, are not currently, nor are they the subject of a proceeding that could lead to it or its Affiliates becoming a Debarred Entity, Excluded Entity or Convicted Entity and it and its Affiliates shall not use in any capacity, in connection with the obligations to be performed under this Agreement, any person who is a Debarred Individual, Excluded Individual or a Convicted Individual. For purposes of this provision, the following definitions shall apply:

(a) A "**Debarred Individual**" is an individual who has been debarred by the FDA pursuant to 21 U.S.C. §335a (a) or (b) from providing services in any capacity to a person that has an approved or pending drug or biological product application.

(b) A "**Debarred Entity**" is a corporation, partnership or association that has been debarred by the FDA pursuant to 21 U.S.C. §335a (a) or (b) from submitting or assisting in the submission of any abbreviated drug application, or a subsidiary or Affiliate of a Debarred Entity.

(c) An "**Excluded Individual**" or "**Excluded Entity**" is (i) an individual or entity, as applicable, who has been excluded, debarred, suspended or is otherwise ineligible to participate in federal health care programs such as Medicare or Medicaid by the Office of the Inspector General (OIG/HHS) of the U.S. Department of Health and Human Services, or (ii) is an individual or entity, as applicable, who has been excluded, debarred, suspended or is otherwise ineligible to participate in federal procurement and non-procurement programs, including those produced by the U.S. General Services Administration (GSA).

(d) A "**Convicted Individual**" or "**Convicted Entity**" is an individual or entity, as applicable, who has been convicted of a criminal offense that falls within the ambit of 21 U.S.C. §335a (a) or 42 U.S.C. §1320a - 7(a), but has not yet been excluded, debarred, suspended, or otherwise declared ineligible.

(e) "**FDA's Disqualified/Restricted List**" is the list of clinical investigators restricted from receiving investigational drugs, biologics, or devices if the FDA has determined that the investigators have repeatedly or deliberately failed to comply with regulatory requirements for studies or have submitted false Know-How to the study sponsor or the FDA.

11.2.15 The inventions claimed or covered by the Existing Licensor Patents (a) were not conceived, discovered, developed, or otherwise made in connection with any research activities funded, in whole or in part, by the federal government of the United States or any agency thereof, and (b) are not a "subject invention" as that term is described in 35 U.S.C. Section 201(f).

11.2.16 Neither Licensor nor its Affiliates have disclosed to AGT or its Affiliates any Excluded Licensor IP or any trade secrets of Licensor or its Affiliates other than [*] during diligence regarding the negotiation of this Agreement.

11.2.17 The representations and warranties of Licensor in this Agreement, and the Know-How, documents and materials [*].

11.3Licensor Covenants.

11.3.1 Licensor covenants that, as of the Effective Date, neither Licensor nor any of its Affiliates has granted, and during the Term, neither Licensor nor its Affiliates shall grant, any right or license to any Third Party that would conflict with or limit the scope of any of the rights or licenses granted to AGT hereunder.

11.3.2 Licensor covenants that it has obtained or will obtain written agreements from each of its employees, consultants, contractors and agents who perform research or development activities related to the 4D Vector or otherwise participate in the exploitation of the 4D Vector, or are involved in the generation of Know-How or Patents that are necessary or reasonably useful for the exploitation of the 4D Vector, which agreements will obligate such persons to obligations of confidentiality and non-use and to assign inventions in a manner consistent with the provisions of this Agreement.

11.3.3 Licensor covenants that it shall comply with Applicable Law in the course of performing its obligations or exercising its rights pursuant to this Agreement.

11.4AGT Covenants.

11.4.1 Unless and until the 4D Vector sequence becomes publicly identifiable, AGT covenants that it shall not, and shall not permit any of its Affiliates or authorize any of its Sublicensees to, [*]. For clarity, the foregoing does not restrict AGT, its Affiliates or Sublicensees from [*] for purposes of [*].

11.4.2 Unless otherwise agreed by the Parties (or their respective Affiliates) or AGT, its Affiliates or Sublicensees otherwise have the right to do so (e.g., through a sublicense from a Third Party that has such rights from Licensor), AGT covenants that it shall not, and shall not authorize any of its Affiliates or Sublicensees to, (a) [*] or (b) [*], in each case, outside the scope of the licenses or covenant not to sue or other rights granted to AGT in this Agreement.

11.4.3AGT covenants that it has obtained or will obtain written agreements from each of its employees, consultants, contractors, and agents who perform research or development activities pursuant to this Agreement or otherwise participate in the Exploitation of the 4D Vector pursuant to this Agreement, which agreements will obligate such persons to obligations of confidentiality and non-use and to assign inventions in a manner consistent with the provisions of this Agreement.

11.4.4AGT covenants that it shall comply with Applicable Law in the course of performing its obligations or exercising its rights pursuant to this Agreement.

11.5DISCLAIMER OF WARRANTIES. EXCEPT FOR THE EXPRESS WARRANTIES SET FORTH HEREIN, NEITHER PARTY MAKES ANY REPRESENTATIONS OR GRANTS ANY WARRANTIES, EXPRESS OR IMPLIED, EITHER IN FACT OR BY OPERATION OF LAW, BY STATUTE OR OTHERWISE, AND EACH PARTY SPECIFICALLY DISCLAIMS ANY OTHER WARRANTIES, WHETHER WRITTEN OR ORAL, OR EXPRESS OR IMPLIED, INCLUDING ANY WARRANTY OF QUALITY, MERCHANTABILITY, OR FITNESS FOR A PARTICULAR USE OR PURPOSE OR ANY WARRANTY AS TO THE VALIDITY OF ANY PATENTS OR THE NON-INFRINGEMENT OF ANY INTELLECTUAL PROPERTY RIGHTS OF THIRD PARTIES.

ARTICLE 12
INDEMNITY

12.1Indemnification of Licensor. AGT shall indemnify Licensor, its Affiliates and its and their respective directors, officers, employees, and agents (the "**Licensor Indemnitees**") and defend and save each of them harmless, from and against any and all losses, damages, liabilities, penalties, costs, and expenses (including reasonable attorneys' fees and expenses) (collectively, "**Losses**") in connection with any and all suits, investigations, claims, or demands of Third Parties (collectively, "**Third Party Claims**") incurred by or rendered against the Licensor Indemnitees arising from or occurring as a result of: (a) the material breach by AGT or its Affiliates of this Agreement (including any representation, warranty or covenant in this Agreement); (b) the gross negligence, reckless or willful misconduct on the part of AGT or its Affiliates or their respective directors, officers, employees, and agents in performing its or their obligations under this Agreement, or (c) the Exploitation of Licensed Compounds and Licensed Products by AGT or its

Affiliates, in each case (a) through (c), except to the extent Licensor has an obligation to indemnify AGT pursuant to Section 12.2.

12.2Indemnification of AGT. Licensor shall indemnify AGT, its Affiliates and their respective directors, officers, employees, and agents, and defend and save each of them harmless, from and against any and all Losses in connection with any and all Third Party Claims arising from or occurring as a result of: (a) the material breach by Licensor or its Affiliates of this Agreement (including any representation, warranty or covenant in this Agreement); (b) the gross negligence, reckless or willful misconduct on the part of Licensor or its Affiliates or its or their respective directors, officers, employees, and agents in performing its obligations under this Agreement; (c) Licensor's or its Affiliate's or (sub)licensee's use or other Exploitation of the 4D Vector, other than the Exploitation of Licensed Compounds, Licensed Products, or the AGT Payloads by AGT or its Affiliates under this Agreement, or (d) Licensor's or its Affiliate's or (sub)licensee's use or reliance on any Regulatory Documentation or safety data for which rights are granted by AGT to Licensor under this Agreement, in each case (a) through (d), except to the extent AGT has an obligation to indemnify Licensor pursuant to Section 12.1.

12.3Notice of Claim. All indemnification claims in respect of a Party, its Affiliates, or their respective directors, officers, employees, and agents shall be made solely by such Party to this Agreement (the "**Indemnified Party**"). The Indemnified Party shall give the indemnifying Party prompt written notice (an "**Indemnification Claim Notice**") of any Losses or discovery of fact upon which such Indemnified Party intends to base a request for indemnification under this ARTICLE 12, but in no event shall the indemnifying Party be liable for any Losses that result from any delay in providing such notice. Each Indemnification Claim Notice must contain a description of the claim and the nature and amount of such Loss (to the extent that the nature and amount of such Loss is known at such time). The Indemnified Party shall furnish promptly to the indemnifying Party copies of all papers and official documents received in respect of any Losses and Third Party Claims.

12.4Control of Defense.

12.4.1In General. Subject to the provisions of Section 8.4, at its option based on reasonable determination that it is obligated under Section 12.1 or 12.2, as applicable, to assume the defense of any Third Party Claim, the indemnifying Party may assume the defense of such Third Party Claim by giving written notice to the Indemnified Party within [*] days after the indemnifying Party's receipt of an Indemnification Claim Notice. The assumption of the defense of a Third Party Claim by the indemnifying Party shall not be construed as an acknowledgment that the indemnifying Party is liable to indemnify the Indemnified Party in respect of the Third Party Claim, nor shall it constitute a waiver by the indemnifying Party of any defenses it may assert against the Indemnified Party's claim for indemnification. Upon assuming the defense of a Third Party Claim, the indemnifying Party may appoint as lead counsel in the defense of the Third Party Claim any legal counsel selected by the indemnifying Party which shall be reasonably acceptable to the Indemnified Party. If the indemnifying Party assumes the defense of a Third Party Claim, the Indemnified Party shall immediately deliver to the indemnifying Party all original notices and documents (including court papers) received by the Indemnified Party in connection with the Third Party Claim. Should the indemnifying Party assume the defense of a Third Party Claim, except as provided in Section 12.4.2, the indemnifying Party shall not be liable to the

Indemnified Party for any legal expenses subsequently incurred by such Indemnified Party in connection with the analysis, defense or settlement of the Third Party Claim unless specifically requested in writing by the indemnifying Party. To the extent it is ultimately determined by a court of competent jurisdiction that the indemnifying Party is not obligated to indemnify, defend or hold harmless the Indemnified Party from and against the Third Party Claim, the Indemnified Party shall reimburse the indemnifying Party for any Losses incurred by the indemnifying Party in its defense of the Third Party Claim.

12.4.2Right to Participate in Defense. Without limiting Section 12.4.1, any Indemnified Party shall be entitled to participate in, but not control, the defense of such Third Party Claim and to employ counsel of its choice for such purpose; *provided* that such employment shall be at the Indemnified Party's own expense unless (a) the employment thereof, and the assumption by the indemnifying Party of such expense, has been specifically authorized by the indemnifying Party in writing, (b) the indemnifying Party has failed to assume the defense and employ counsel in accordance with Section 12.4.1 (in which case the Indemnified Party shall control the defense), or (c) the interests of the Indemnified Party and the indemnifying Party with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under Applicable Law, ethical rules or equitable principles.

12.4.3Settlement. With respect to any Losses relating solely to the payment of money damages in connection with a Third Party Claim and that shall not result in the Indemnified Party's becoming subject to injunctive or other relief or otherwise adversely affecting the business of the Indemnified Party in any manner, and as to which the indemnifying Party shall have acknowledged in writing the obligation to indemnify the Indemnified Party hereunder, the indemnifying Party shall have the sole right to consent to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss, on such terms as the indemnifying Party, in its sole discretion, shall deem appropriate; *provided, however*, that the indemnifying Party shall not settle any such Third Party Claim without the prior written consent of the Indemnified Party if such settlement does not include a complete release from liability with respect to the Third Party Claim or includes any admission of wrongdoing by the Indemnified Party or that any intellectual property or proprietary right of the Indemnified Party or this Agreement is invalid, narrowed in scope, or unenforceable. With respect to all other Losses in connection with Third Party Claims, where the indemnifying Party has assumed the defense of the Third Party Claim in accordance with Section 12.4.1, the indemnifying Party shall have authority to consent to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss; *provided* that it obtains the prior written consent of the Indemnified Party (which consent shall not be unreasonably withheld, conditioned or delayed). If the indemnifying Party does not assume and conduct the defense of a Third Party Claim as provided above, the Indemnified Party may defend against such Third Party Claim. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, no Indemnified Party shall admit any liability with respect to, or settle, compromise, or dispose of, any Third Party Claim without the prior written consent of the indemnifying Party, which consent shall not to be unreasonably withheld, conditioned, or delayed. The indemnifying Party shall not be liable for any settlement, compromise or other disposition of a Loss by an Indemnified Party that is reached without the written consent of the indemnifying Party, which consent shall not be unreasonably withheld, conditioned, or delayed.

12.4.4Cooperation. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, the Indemnified Party shall, and shall cause each indemnitee to, cooperate in the defense or prosecution thereof and shall furnish such records, information, and testimony, provide such witnesses, and attend such conferences, discovery proceedings, hearings, trials and appeals as may be reasonably requested in connection therewith. Such cooperation shall include reasonable retention by the Indemnified Party of records and information that are reasonably relevant to such Third Party Claim and making the Indemnified Parties and other employees and agents available on a mutually convenient basis to provide additional information and explanation of any material provided hereunder, and the indemnifying Party shall reimburse the Indemnified Party for all its reasonable out-of-pocket expenses in connection therewith.

12.4.5Expenses. Except as provided above, the reasonable and verifiable costs and expenses, including fees and disbursements of counsel, incurred by the Indemnified Party in connection with any Third Party Claim shall be reimbursed on a monthly basis in arrears by the indemnifying Party, without prejudice to the indemnifying Party's right to contest the Indemnified Party's right to indemnification and subject to refund in the event the indemnifying Party is ultimately held not to be obligated to indemnify the Indemnified Party.

12.5Special, Indirect, and Other Losses. EXCEPT (A) FOR WILLFUL MISCONDUCT, (B) FOR A PARTY'S BREACH OF ITS OBLIGATIONS UNDER ARTICLE 10, AND (C) TO THE EXTENT ANY SUCH DAMAGES ARE REQUIRED TO BE PAID TO A THIRD PARTY AS PART OF A CLAIM FOR WHICH A PARTY PROVIDES INDEMNIFICATION UNDER THIS ARTICLE 12, NEITHER PARTY NOR ANY OF ITS AFFILIATES SHALL BE LIABLE FOR INDIRECT, INCIDENTAL, SPECIAL, EXEMPLARY, PUNITIVE OR CONSEQUENTIAL DAMAGES, INCLUDING LOSS OF PROFITS OR BUSINESS INTERRUPTION, HOWEVER CAUSED AND ON ANY THEORY OF LIABILITY, WHETHER IN CONTRACT, TORT, NEGLIGENCE, BREACH OF STATUTORY DUTY OR OTHERWISE IN CONNECTION WITH OR ARISING IN ANY WAY OUT OF THE TERMS OF THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY OR THE USE OF THE 4D VECTOR, LICENSED COMPOUND OR LICENSED PRODUCT, EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGE.

12.6Insurance. Each Party shall have and maintain such type and amounts of insurance covering its obligations under this Agreement as is (a) normal and customary in the pharmaceutical industry generally for parties similarly situated to such Party and its Affiliates and (b) otherwise required by Applicable Law. Notwithstanding the foregoing, AGT may self-insure, in whole or in part, the insurance requirements described above.

ARTICLE 13
TERM AND TERMINATION

13.1Term.

13.1.1Term. This Agreement shall commence on the Effective Date and, unless earlier terminated in accordance herewith, shall continue in force and effect on a Licensed

Product-by-Licensed Product basis, until the expiration of the last-to-expire Royalty Term for such Licensed Product (such period, the "**Term**").

13.1.2Effect of Expiration of the Term. Following the expiration of the Term for a Licensed Product, the grants in Sections 3.5.1(d) and 6.1 for such Licensed Product shall become fully-paid, royalty-free and irrevocable.

13.2Termination for Material Breach.

13.2.1Material Breach. If either Party (the "**Non-Breaching Party**") believes that the other Party (the "**Breaching Party**") has materially breached one or more of its material obligations under this Agreement, then the Non-Breaching Party may deliver notice of such material breach to the Breaching Party (a "**Default Notice**"). If the Breaching Party fails to cure such breach within [*] days [*] after receipt of the Default Notice or if such compliance cannot be fully achieved within such [*] day period [*] and the Breaching Party has failed to commence compliance or has failed to use diligent efforts to achieve full compliance as soon thereafter as is reasonably possible, the Non-Breaching Party may terminate this Agreement upon written notice to the Breaching Party. If the Breaching Party disputes that it has materially breached one of its material obligations under this Agreement, the dispute shall be resolved pursuant to Section 14.6. Notwithstanding the foregoing, in the event of a good faith dispute as to whether a material breach by either Party allowing for termination hereunder has occurred, the foregoing cure period with respect thereto shall be tolled pending final resolution of such dispute in accordance with the terms of this Agreement; *provided* that, if such dispute relates to payment, such tolling of the cure period shall only apply with respect to payment of the disputed amounts, and not with respect to any undisputed amount.

13.2.2Limitations on Termination for Material Breach. Notwithstanding Section 13.2.1, if the material breach and failure to cure contemplated by Section 13.2.1 is with respect to AGT's diligence obligations under this Agreement, Licensor shall not have the right to terminate this Agreement in its entirety, but shall have the right to terminate this Agreement [*].

13.3Termination for Convenience. AGT may terminate this Agreement in its entirety, or with respect to one or more Licensed Products or AGT Targets for any or no reason, upon [*] days' prior written notice to Licensor.

13.4Termination for Insolvency. In the event that either Party (a) files for protection under bankruptcy or insolvency laws, (b) makes an assignment for the benefit of creditors, (c) appoints or suffers appointment of a receiver, manager or trustee over substantially all of its property, (d) proposes a written agreement of composition or extension of its debts, (e) proposes or is a party to any dissolution or liquidation, (f) files a petition under any bankruptcy or insolvency act or has any such petition filed against that is not discharged within [*] days of the filing thereof, or (g) experiences an event analogous to any of the foregoing in any jurisdiction in which any of its assets are situated, then the other Party may terminate this Agreement effective immediately upon written notice to such Party.

13.5Rights in Bankruptcy.

13.5.1Applicability of 11 U.S.C. § 365(n). All rights and licenses granted under or pursuant to this Agreement, including all rights and licenses to use improvements or enhancements developed during the Term, are intended to be, and shall otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code (the "**Bankruptcy Code**") or any analogous provisions in any other country or jurisdiction, licenses of rights to "intellectual property" as defined under Section 101(35A) of the Bankruptcy Code. The Parties agree that the licensee of such intellectual property under this Agreement shall retain and may fully exercise all of its rights and elections under the Bankruptcy Code, including Section 365(n) of the Bankruptcy Code, or any analogous provisions in any other country or jurisdiction. All of the rights granted to either Party under this Agreement shall be deemed to exist immediately before the occurrence of any bankruptcy case in which the other Party is the debtor.

13.5.2Rights of non-Debtor Party in Bankruptcy. If a bankruptcy proceeding is commenced by or against Licensor under the Bankruptcy Code or any analogous provisions in any other country or jurisdiction, [*].

13.5.3AGT Rights in Lieu of Termination. If AGT has the right to terminate this Agreement under Section 13.2, in whole or in part, due to a material breach by Licensor of Section [*] or a material breach by Licensor of Section [*] of which Licensor had Knowledge as of the Effective Date, then AGT may (in its discretion), in lieu of terminating this Agreement (in whole or in part) elect by written notice to Licensor to reduce [*] payable to AGT under this Agreement by [*].

13.6Termination for Patent Challenge. Licensor will have the right to terminate this Agreement upon [*] days' prior written notice to AGT (such notice to include the details of the Patent Challenge) if AGT or any of its Affiliates commences any Patent Challenge and fails to withdraw, rescind or terminate such Patent Challenge within such [*] day period. In the event any Sublicensee of AGT that has a sublicense under the rights granted to AGT pursuant to Section 6.1.1 (and excluding for clarity any subcontractors appointed by AGT pursuant to Section 6.5) commences any Patent Challenge with respect to a Licensor Patent for which it has rights and fails to withdraw, rescind or terminate such Patent Challenge within [*] days after written notice to AGT from Licensor thereof (such notice to include the details of the Patent Challenge), AGT shall, upon written request by Licensor, promptly send written notice to terminate the applicable sublicense agreement to such Sublicensee. Notwithstanding the foregoing, Licensor shall not have the right to terminate this Agreement and AGT shall not be obligated to terminate the applicable sublicense agreement with such Sublicensee if such Patent Challenge is [*].

13.7Effects of Termination.

13.7.1In Entirety. In the event of a termination of this Agreement in its entirety by either Party for any reason, all rights and licenses granted by each Party to the other hereunder shall immediately terminate.

13.7.2With respect to a Terminated Product or Terminated Target. In the event of termination (but not expiration) of this Agreement with respect to a Terminated Product

or Terminated Target (but not in the case of any termination of this Agreement in its entirety), (a) all rights and licenses granted by Licensor to AGT hereunder shall automatically be deemed to be amended to exclude, if applicable, the rights with respect to such Terminated Product or Terminated Target, as applicable, and (b) all diligence and other obligations of AGT with respect to such Terminated Product and Terminated Target, as applicable, shall cease.

13.7.3Sublicenses. If this Agreement terminates, the following shall apply to any AGT sublicense agreement existing as of the effective date of such termination, but only if the Sublicensee is not in material breach of such sublicense agreement at such time: [*].

13.8Accrued Rights; Surviving Obligations.

13.8.1 Except as otherwise expressly provided herein, termination or expiration of this Agreement (either in its entirety or with respect to one or more Terminated Products or Terminated Targets) for any reason in accordance with the provisions hereof shall be without prejudice to any rights that shall have accrued to the benefit of a Party prior to such termination or expiration and shall not limit remedies that may otherwise be available in law or equity. Such termination or expiration shall not relieve a Party from obligations that are expressly indicated to survive the termination or expiration of this Agreement. Without limiting the foregoing, Section 3.5.1(d) (with respect to the rights granted to Licensor thereunder), Section 6.2 (solely in the case of expiration, but not termination), Section 6.3 (but solely in the event of termination of this Agreement by AGT pursuant to Section 13.3 or by Licensor pursuant to Section 13.2, Section 13.4, or Section 13.6, or expiration of this Agreement), Section 6.7, Sections 7.6 through 7.10 and Section 7.12 (solely with respect to payment obligations arising before termination), Section 8.1, Section 8.2.3, Section 11.5, Section 13.1.2, Section 13.7 (as applicable), and this Section 13.8, and ARTICLE 1, ARTICLE 9, ARTICLE 10 (for the period set forth in Section 10.7), ARTICLE 12, and ARTICLE 14 of this Agreement shall survive the termination or expiration of this Agreement for any reason. If this Agreement is terminated with respect to the Terminated Product or Terminated Target, but not in its entirety, then following such termination the foregoing provisions of this Agreement shall remain in effect with respect to the Terminated Product or Terminated Target (to the extent they would survive and apply in the event the Agreement expires or is terminated in its entirety), and all provisions not surviving in accordance with the foregoing shall terminate upon termination of this Agreement with respect to the Terminated Product and be of no further force and effect (and, for purposes of clarity, all provisions of this Agreement shall remain in effect with respect to all AGT Targets other than the Terminated Targets).

13.8.2 Notwithstanding the termination of AGT's licenses and other rights under this Agreement or with respect to a particular Terminated Product or Terminated Target, as the case may be, (a) AGT shall have the right for [*] months after the effective date of such termination to sell or otherwise dispose of all Licensed Product then in its inventory and any in-progress inventory, as though this Agreement had not terminated with respect to such Terminated Product or Terminated Target, as applicable, and such sale or disposition shall not constitute infringement of Licensor's or its Affiliates' Patent or other intellectual property or other proprietary rights and [*]. For purposes of clarity, in case of sales under foregoing clause (a) AGT shall continue to make payments thereon as provided in ARTICLE 7 (as if this Agreement had not terminated with respect to such Licensed Product). In addition, unless otherwise mutually agreed

by the Parties, upon the termination of this Agreement, AGT shall destroy any remaining quantities of Transferred Materials.

ARTICLE 14
MISCELLANEOUS

14.1Force Majeure. Except with respect to payments of monies hereunder, neither Party shall be held liable or responsible to the other Party or be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement when such failure or delay is caused by or results from events beyond the reasonable control of the non-performing Party, including fires, floods, earthquakes, hurricanes, embargoes, shortages, epidemics, pandemics, quarantines, war, acts of war (whether war be declared or not), terrorist acts, insurrections, riots, civil commotion, strikes, lockouts, or other labor disturbances (whether involving the workforce of the non-performing Party or of any other Person), acts of God or acts, omissions or delays in acting by any governmental authority (except to the extent such delay results from the breach by the non-performing Party or any of its Affiliates of any term or condition of this Agreement). The non-performing Party shall notify the other Party of such force majeure within [*] days after such occurrence by giving written notice to the other Party stating the nature of the event, its anticipated duration, and any action being taken to avoid or minimize its effect. The suspension of performance shall be of no greater scope and no longer duration than is necessary and the non-performing Party shall use commercially reasonable efforts to remedy its inability to perform.

14.2Export Control. This Agreement is made subject to any restrictions concerning the export of products or technical information from the United States or other countries that may be imposed on the Parties from time to time. Each Party agrees that it will not export, directly or indirectly, any technical information acquired from the other Party under this Agreement or any products using such technical information to a location or in a manner that at the time of export requires an export license or other governmental approval, without first obtaining the written consent to do so from the appropriate agency or other governmental entity in accordance with Applicable Law.

14.3Assignment.

14.3.1 Without the prior written consent of the other Party, neither Party shall sell, transfer, assign, delegate, pledge, or otherwise dispose of, whether voluntarily, involuntarily, by operation of law or otherwise, this Agreement or any of its rights or duties hereunder; *provided* that either Party may make such an assignment without the other Party's consent to its Affiliate or to a successor, whether in a merger, sale of stock, sale of assets or any other transaction, of the business to which this Agreement relates. With respect to an assignment to an Affiliate, the assigning Party shall remain responsible for the performance by such Affiliate of the rights and obligations hereunder. Any attempted assignment or delegation in violation of this Section 14.3 shall be void and of no effect. All validly assigned and delegated rights and obligations of a Party hereunder shall be binding upon and inure to the benefit of and be enforceable by and against the successors and permitted assigns of such Party, as the case may be. The permitted assignee or transferee shall assume all obligations of its assignor or transferor under this Agreement.

14.3.2 Each Party agrees that, notwithstanding any provisions of this Agreement to the contrary, if this Agreement is assigned by a Party as permitted under this Section 14.3 to an Acquirer in a Change of Control, then the Patents, Know-How or other intellectual property rights of any such Acquirer that (a) were controlled by such Acquirer (and not such Party or any of its Affiliates prior to such Change of Control) immediately prior to such Change of Control (other than as a result of a license or other grant of rights, covenant or assignment by such Party or its other Affiliates to, or for the benefit of, such successor) or (b) are developed by such Acquirer after such Change of Control outside of the scope of this Agreement or through activities that were Segregated, in each case (a) and (b), will be deemed not to be "Controlled" by such Party for purposes of this Agreement.

14.3.3 Notwithstanding the foregoing or anything herein to the contrary, Licensor may assign, pledge, grant a security interest in, encumber, or otherwise transfer or distribute its right to receive milestone payments under Sections 7.2 and 7.3 and royalties under Section 7.4 to a Third Party pursuant to a royalty monetization transaction, royalty buyout, revenue participation, royalty or revenue purchase, synthetic royalty assignment or any similar structure or transfer transaction. AGT agrees that, to the extent necessary for such counterparty to confirm the payments it is entitled to receive with respect to such transaction, Licensor may disclose to the counterparty of such transaction any royalty payments, milestone payments and royalty reports relating to the royalties or milestone interests transferred as part of such transaction permitted under this Section 14.3.3; *provided* that such counterparty is under obligations of confidentiality and non-use with respect to Confidential Information included in such disclosures that are substantially the same as the terms of ARTICLE 10.

14.4Severability. If any provision of this Agreement is held to be illegal, invalid, or unenforceable under any present or future law, and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby, (a) such provision shall be fully severable, (b) this Agreement shall be construed and enforced as if such illegal, invalid, or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement shall remain in full force and effect and shall not be affected by the illegal, invalid, or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid, or unenforceable provision, there shall be added automatically as a part of this Agreement a legal, valid, and enforceable provision as similar in terms to such illegal, invalid, or unenforceable provision as may be possible and reasonably acceptable to the Parties. To the fullest extent permitted by Applicable Law, each Party hereby waives any provision of law that would render any provision hereof illegal, invalid, or unenforceable in any respect.

14.5Governing Law, Jurisdiction and Service.

14.5.1Governing Law. This Agreement or the performance, enforcement, breach, or termination hereof shall be interpreted, governed by, and construed in accordance with the laws of the State of New York, United States (including New York General Obligations Law §5-1401), excluding any conflicts or choice of law rule or principle that might otherwise refer construction or interpretation of this Agreement to the substantive law of another jurisdiction. The Parties agree to exclude the application to this Agreement of the United Nations Convention on Contracts for the International Sale of Goods.

14.5.2Service. Each Party further agrees that service of any process, summons, notice or document by registered mail to its address set forth in Section 14.7.2 shall be effective service of process for any action, suit, or proceeding brought against it under this Agreement in any such court.

14.6Dispute Resolution.

14.6.1 Except for disputes resolved by the procedures set forth in Section 7.10 or 14.10, if a dispute arises between the Parties in connection with or relating to this Agreement or any document or instrument delivered in connection herewith (a "**Dispute**"), it shall be resolved pursuant to this Section 14.6. Any Dispute shall first be referred to a designated senior executive for each of the Parties, each of whom shall have the authority to resolve such Dispute on behalf the Party. The designated executives shall confer in good faith on the resolution of the issue. Any final decision mutually agreed to by the senior executives of each Party shall be conclusive and binding on the Parties. If the senior executives are not able to agree on the resolution of any such Dispute within [*] days (or such other period of time as mutually agreed by the senior executives) after such Dispute was first referred to them, then each of the Parties hereby irrevocably and unconditionally consents to submit to the exclusive jurisdiction of the state and federal courts of New York, New York for any matter arising out of or relating to this Agreement and the transactions contemplated hereby, and (a) agrees not to commence any litigation relating thereto except in such courts, (b) waives any objection to the laying of venue of any matter arising out of this Agreement or the transactions contemplated hereby in the state and federal courts of New York, New York, and (c) waives and agrees not to plead or claim in any such court that any such matter brought in any such court has been brought in an inconvenient forum. The Parties agree that a final judgment in any such matter shall be conclusive and may be enforced in other jurisdictions by suits on the judgment or in any other manner provided by law. Any proceeding brought by either Party under this Agreement shall be exclusively conducted in the English language.

14.6.2 Notwithstanding anything herein to the contrary, nothing in this Section 14.6 shall preclude either Party from seeking interim or provisional relief pursuant to Section 14.10, including a temporary restraining order, preliminary injunction or other interim equitable relief concerning a Dispute, if necessary to protect the interests of such Party. This Section shall be specifically enforceable.

14.7Notices.

14.7.1Notice Requirements. Any notice, request, demand, waiver, consent, approval, or other communication permitted or required under this Agreement shall be in writing, shall refer specifically to this Agreement and shall be deemed given only if (a) delivered by hand, or (b) by internationally recognized overnight delivery service that maintains records of delivery, addressed to the Parties at their respective addresses specified in Section 14.7.2 or to such other address as the Party to whom notice is to be given may have provided to the other Party in accordance with this Section 14.7.1. Such notice shall be deemed to have been given as of the date delivered by hand or on the second Business Day (at the place of delivery) after deposit with an internationally recognized overnight delivery service. This Section 14.7.1 is not intended to govern

the day-to-day business communications necessary between the Parties in performing their obligations under the terms of this Agreement.

14.7.2Address for Notice.

If to AGT, to:

[*]

with a copy (which shall not constitute notice) to:

[*]

If to Licensor, to:

[*]

with a copy (which shall not constitute notice) to:

[*]

with a copy (which shall not constitute notice) to:

[*]

14.8Entire Agreement; Amendments. This Agreement, together with the Schedules attached hereto, and the pharmacovigilance agreement contemplated by Section 9.1, set forth and constitute the entire agreement and understanding between the Parties with respect to the subject matter hereof and all prior agreements, understandings, promises, and representations, whether written or oral, with respect thereto are superseded hereby. For clarity, the Existing CDA shall survive for purposes of discussions unrelated to this Agreement (e.g., non-ophthalmology AAV vectors). Each Party confirms that it is not relying on any representations or warranties of the other Party except as specifically set forth in this Agreement. No amendment, modification, release, or discharge shall be binding upon the Parties unless in writing and duly executed by authorized representatives of both Parties.

14.9English Language. This Agreement shall be written and executed in, and all other communications under or in connection with this Agreement shall be in, the English language. Any translation into any other language shall not be an official version thereof, and in the event of any conflict in interpretation between the English version and such translation, the English version shall control.

14.10Equitable Relief. Each Party acknowledges and agrees that the restrictions set forth in [*] are reasonable and necessary to protect the legitimate interests of the other Party and that such other Party would not have entered into this Agreement in the absence of such restrictions, and that any breach or threatened breach of any provision of such Section or Articles may result in irreparable injury to such other Party for which there shall be no adequate remedy at law. In the event of a breach or threatened breach of any provision of such Section or Articles, the non-breaching Party shall be authorized and entitled to seek from any court of competent

jurisdiction injunctive relief, whether preliminary or permanent, specific performance, and an equitable accounting of all earnings, profits, and other benefits arising from such breach, which rights shall be cumulative and in addition to any other rights or remedies to which such non-breaching Party may be entitled in law or equity. Both Parties agree to waive any requirement that the other (a) post a bond or other security as a condition for obtaining any such relief, and (b) show irreparable harm, balancing of harms, consideration of the public interest, or inadequacy of monetary damages as a remedy. Nothing in this Section 14.10 is intended, or should be construed, to limit either Party's right to equitable relief or any other remedy for a breach of any other provision of this Agreement.

14.11Waiver and Non-Exclusion of Remedies. Any term or condition of this Agreement may be waived at any time by the Party that is entitled to the benefit thereof, but no such waiver shall be effective unless set forth in a written instrument duly executed by or on behalf of the Party waiving such term or condition. The waiver by either Party of any right hereunder or of the failure to perform or of a breach by the other Party shall not be deemed a waiver of any other right hereunder or of any other breach or failure by such other Party, whether of a similar nature or otherwise. The rights and remedies provided herein are cumulative and do not exclude any other right or remedy provided by Applicable Law or otherwise available except as expressly set forth herein.

14.12No Benefit to Third Parties. Except as provided in ARTICLE 12, covenants and agreements set forth in this Agreement are for the sole benefit of the Parties and their respective successors and permitted assigns, and they shall not be construed as conferring any rights on any other Persons.

14.13Further Assurance. Each Party shall duly execute and deliver, or cause to be duly executed and delivered, such further instruments and do and cause to be done such further acts and things, including the filing of such assignments, agreements, documents, and instruments, as may be necessary or as the other Party may reasonably request in connection with this Agreement or to carry out more effectively the provisions and purposes hereof, or to better assure and confirm unto such other Party its rights and remedies under this Agreement.

14.14Relationship of the Parties. It is expressly agreed that Licensor, on the one hand, and AGT, on the other hand, shall be independent contractors and that the relationship between the Parties shall not constitute a partnership, joint venture, or agency. Neither Licensor, on the one hand, nor AGT, on the other hand, shall have the authority to make any statements, representations, or commitments of any kind, or to take any action, which shall be binding on the other, without the prior written consent of the other Party to do so. All persons employed by a Party shall be employees of such Party and not of the other Party and all costs and obligations incurred by reason of any such employment shall be for the account and expense of such Party.

14.15Performance by Affiliates. AGT may use one or more of its Affiliates to perform its obligations and duties hereunder and such AGT Affiliates are expressly granted certain rights herein; *provided* that each such Affiliate shall be bound by the corresponding obligations of AGT and, subject to an assignment to such Affiliate pursuant to Section 14.3, AGT shall remain liable hereunder for the prompt payment and performance of all their respective obligations hereunder.

14.16Counterparts; Electronic Execution. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. This Agreement may be executed by facsimile or electronically transmitted signatures and such signatures shall be deemed to bind each Party as if they were original signatures.

14.17References. Unless otherwise specified, (a) references in this Agreement to any Article, Section or Schedule shall mean references to such Article, Section or Schedule of this Agreement, (b) references in any Section to any clause are references to such clause of such Section, and (c) references to any agreement, instrument, or other document in this Agreement refer to such agreement, instrument, or other document as originally executed or, if subsequently amended, replaced, or supplemented from time to time, as so amended, replaced, or supplemented and in effect at the relevant time of reference thereto.

14.18Schedules. In the event of any inconsistencies between this Agreement and any schedules or other attachments hereto, the terms of this Agreement shall control.

14.19Construction. Except where the context otherwise requires, wherever used, the singular shall include the plural, the plural the singular, the use of any gender shall be applicable to all genders and the word “or” is used in the inclusive sense (and/or). Whenever this Agreement refers to a number of days, unless otherwise specified, such number refers to calendar days. The captions of this Agreement are for convenience of reference only and in no way define, describe, extend, or limit the scope or intent of this Agreement or the intent of any provision contained in this Agreement. The term “including,” “include,” or “includes” as used herein shall mean “including, but not limited to,” and shall not limit the generality of any description preceding such term. The word “will” shall be construed to have the same meaning and effect as the word “shall.” The language of this Agreement shall be deemed to be the language mutually chosen by the Parties and no rule of strict construction shall be applied against either Party hereto. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

{Signature Page Follows}

THIS AGREEMENT IS EXECUTED by the authorized representatives of the Parties as of the Effective Date.

4D Molecular Therapeutics, Inc.	**Astellas Gene Therapies, Inc.**
By:	By:
Name: David Kirn	Name: Morten Sogaard
Title: Chief Executive Officer	Title: President

Schedule 1.1

4D Vector Sequence

[*]

Schedule 1.94

Rare Ophthalmology Targets

Gene	Other Name
[*]	[*]
[*]	[*]
[*]	[*]
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Schedule 10.4

Form of Joint Press Release

Astellas and 4D Molecular Therapeutics (4DMT) Enter into License Agreement to Use 4DMT's Proprietary Intravitreal R100 Vector for Rare Ophthalmic Targets

TOKYO and EMERYVILLE, Calif., July 10, 2023 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Naoki Okamura, "Astellas") and 4D Molecular Therapeutics, Inc. (NASDAQ: FDMT, CEO: David Kirn, MD, "4DMT") today announced a license agreement under which Astellas gains rights to utilize the intravitreal retinotropic R100* vector invented by 4DMT for one genetic target implicated in rare monogenic ophthalmic disease(s), with options to add up to two additional targets implicated in rare monogenic ophthalmic diseases after paying additional option exercise fees.

R100 is an adeno-associated virus (AAV) vector invented by 4DMT for intravitreal delivery. It has the ability to penetrate the internal limiting membrane barrier and to efficiently transduce the entire retina, resulting in robust transgene expression within retinal cells. All three 4DMT clinical-stage ophthalmic product candidates utilize the R100 vector, including 4D-150 for wet age-related macular degeneration and diabetic macular edema.

Under the terms of the agreement, 4DMT will provide its proprietary R100 vector technology to Astellas to deliver Astellas' unique genetic payloads for the treatment of rare monogenic diseases. Astellas will conduct all subsequent research, development, manufacturing, and commercialization activities. 4DMT will receive US$20 million upfront, and potential future option fees and milestones of up to US$942.5 million including potential near-term development milestones of US$15 million for the initial target. In addition, 4DMT is entitled to receive mid-single digit to double-digit, sub-teen royalties on net sales of all licensed products.

"This collaboration with Astellas, a leader in AAV gene therapy, continues to validate R100 for routine intravitreal low dose delivery of genetic payloads for the treatment of retinal diseases," said David Kirn, M.D., Co-Founder and Chief Executive Officer of 4DMT. "With over 70 patients

dosed to-date with R100-based product candidates in wet age-related macular degeneration and rare ophthalmic diseases, this collaboration also demonstrates the modularity of the Therapeutic Vector Evolution platform resulting in efficient design and development of new intravitreal products. 4DMT retains rights to large market non-hereditary ophthalmic diseases."

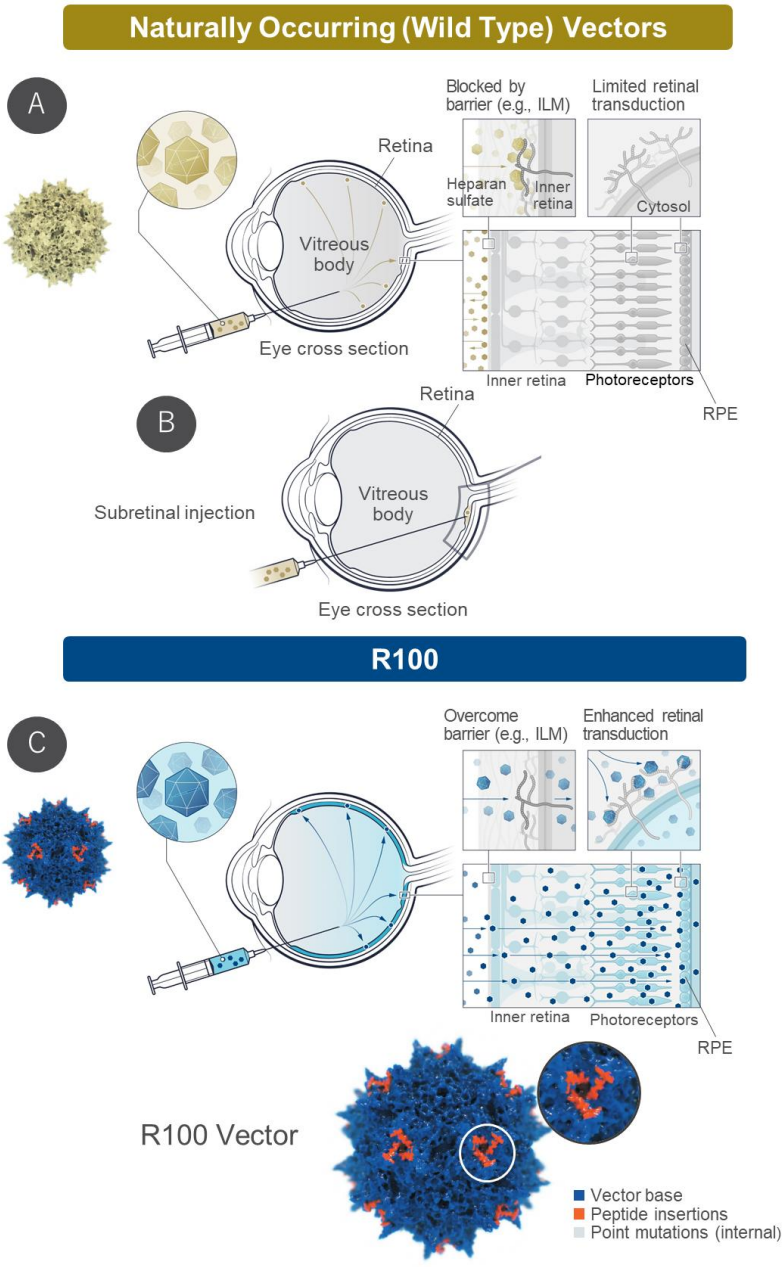
Adam Pearson, Chief Strategy Officer (CStO) at Astellas said, "At Astellas, we have a strong commitment to developing novel treatments for ophthalmic diseases, and have positioned Blindness & Regeneration as one of the Primary Focuses of our R&D strategy. Staying at the forefront of gene therapy technology is a key part of our strategy. We believe that this collaboration will bring synergies between the two companies' cutting-edge research, and will ultimately lead to the development of new therapeutics for patients with ophthalmic diseases at high risk of blindness."

***R100 Structure & Target Vector Profile ~Intravitreal Delivery for Retinal Diseases~**

A) Naturally occurring vectors cannot effectively reach the retina from the vitreous due to the inner limiting membrane (ILM)

B) Therefore in many cases, genetic medicines utilizing naturally occuring vectors require subretinal injection via vitrectomy surgery

C) R100 was customized and evolved to overcome the ILM barrier and transduce the retina with a routine intravitreal injection



About Astellas
Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions that combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into VALUE for patients. For more information, please visit our website at https://www.astellas.com/en.

About 4DMT
4DMT is a clinical-stage biotherapeutics company harnessing the power of directed evolution for genetic medicines targeting large market diseases. 4DMT seeks to unlock the full potential of genetic medicines using its proprietary invention platform, Therapeutic Vector Evolution, which combines the power of the Nobel Prize-winning technology, directed evolution, with approximately one billion synthetic AAV capsid-derived sequences to invent customized and evolved vectors for use in our product candidates. All of our vectors are proprietary to 4DMT and were invented at 4DMT, including the vectors utilized in our clinical-stage and preclinical pipeline product candidates: R100, A101, and C102. The Company is initially focused on five clinical-stage product candidates in three therapeutic areas for both rare and large market diseases: ophthalmology, pulmonology, and cardiology. The 4DMT customized and evolved vectors were invented with the goal of being delivered at relatively low doses through clinically routine, well-tolerated, and minimally invasive routes of administration, transducing diseased cells in target tissues efficiently, having reduced immunogenicity and, where relevant, having resistance to pre-existing antibodies. 4DMT is currently advancing five product candidates in clinical development: 4D-150 for wet AMD and DME, 4D-710 for cystic fibrosis lung disease, 4D-310 for Fabry disease cardiomyopathy, 4D-125 for XLRP, and 4D-110 for choroideremia. The 4D preclinical product candidates in development are: 4D-175 for geographic atrophy and 4D-725 for AATLD.

4D-150, 4D-710, 4D-310, 4D-125, and 4D-110 are our product candidates in clinical development and have not yet been approved for marketing by the US FDA or any other regulatory authority. No representation is made as to the safety or effectiveness of 4D-150, 4D-710, 4D-310, 4D-125, or 4D-110 for the therapeutic uses for which they are being studied.

Cautionary Notes (Astellas)
In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties.
Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.

4D Molecular Therapeutics' Forward Looking Statements:

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, implied and express statements regarding the therapeutic potential, abilities, safety and efficacy of 4D Molecular Therapeutics' proprietary intravitreal R100 vector, including in connection with Astellas' use thereof pursuant to the License Agreement, the potential benefits or applications of 4D Molecular Therapeutics' Vector Evolution platform, including any other vectors developed through the Vector Evolution platform, and the amount of potential milestone and option payments payable under the License Agreement. The words "may," "might," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "expect," "estimate," "seek," "predict," "future," "project," "potential," "continue," "target" and similar words or expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including risks and uncertainties that are described in greater detail in the section entitled "Risk Factors" in 4D Molecular Therapeutics' most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q, as well as any subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent 4D Molecular Therapeutics' views only as of today and should not be relied upon as representing its views as of any subsequent date. 4D Molecular Therapeutics explicitly disclaims any obligation to update any forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward looking statements.

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Contacts for inquiries or additional information:

Astellas Pharma Inc.
Corporate Communications
+81-3-3244-3201

4D Molecular Therapeutics, Inc.
Julian Pei

Head of Investor Relations and Corporate Communications

Investor.Relations@4DMT.com

267-644-5097

Schedule 11.2.1

Existing Licensor Patents

Country	Application Number	Filing Date	Title	Patent Number	Grant Date	Status
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Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO RULES 13a-14(a) AND
15d-14(a) UNDER THE EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, David Kirn, certify that:

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

Dated: November 9, 2023

By: /s/ David Kirn
David Kirn
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO EXCHANGE ACT RULES 13a-14(a) AND
15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Uneek Mehra, certify that:

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

Dated: November 9, 2023

By: /s/ Uneek Mehra
Uneek Mehra
Chief Financial and Business Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q (the "Report") of 4D Molecular Therapeutics, Inc. (the "Company") for the period ended September 30, 2023, David Kirn, as Chief Executive Officer of the Company, and Uneek Mehra, as Chief Financial Officer of the Company, each hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of his knowledge:

a) the Company's Report for the period ended September 30, 2023 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

b) the information contained in such Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 9, 2023

By: /s/ David Kirn
David Kirn
Chief Executive Officer
(Principal Executive Officer)

Dated: November 9, 2023

By: /s/ Uneek Mehra
Uneek Mehra
Chief Financial and Business Officer
(Principal Financial and Accounting Officer)