

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549**

**FORM 10-Q**

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

**For the quarterly period ended September 30, 2021**

**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: 001-40656**

**TENAYA THERAPEUTICS, INC.**

**(Exact name of registrant as specified in its charter)**

**Delaware**

(State or other jurisdiction of  
incorporation or organization)

**171 Oyster Point Boulevard, 5th Floor**

**South San Francisco, CA**

(Address of principal executive offices)

**81-3789973**

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

**94080**

(Zip Code)

**(650) 825-6900**

(Registrant's telephone number, including area code)

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

<b>Title of each class</b>	<b>Trading Symbol(s)</b>	<b>Name of each exchange on which registered</b>
Common Stock, par value \$0.0001 per share	TNYA	Nasdaq Global Select Market

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

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

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

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

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 1, 2021, the registrant had 41,281,210 shares of common stock, \$0.0001 par value per share, outstanding.

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## SPECIAL NOTE REGARDING FORWARD LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or Quarterly Report, contains forward-looking statements. All statements other than statements of historical facts contained in this Quarterly Report, including statements regarding our future results of operations and financial position, business strategy, development plans, planned preclinical studies and clinical trials, future results of clinical trials, expected research and development costs, regulatory strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. In some cases, investors can identify forward-looking statements by terms such as “may,” “will,” “should,” “would,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “contemplate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions. These forward-looking statements include, but are not limited to, statements about:

- the ability of our preclinical studies and planned clinical trials to demonstrate safety and efficacy of our product candidates, and other positive results;
- the timing, progress and results of preclinical studies and planned clinical trials for our current product candidates and other product candidates we may develop;
- the timing, scope and likelihood of regulatory filings and approvals, including timing of investigational new drugs (“INDs”), clinical trial applications (“CTAs”), Food and Drug Administration (“FDA”) approvals, and final regulatory approval of our current product candidates and any other future product candidates;
- our ability to develop and advance our current product candidates and programs into, and successfully complete, clinical studies;
- our manufacturing, commercialization, and marketing capabilities and strategy and the timing of our facilities becoming operational;
- our plans relating to commercializing our product candidates, if approved;
- the need to hire additional personnel and our ability to attract and retain such personnel;
- our competitive position and the success of competing therapies that are or may become available;
- our plans relating to the further development of our product candidates, including additional indications and targets we may pursue;
- the impact of existing laws and regulations and regulatory developments in the United States, Europe and other jurisdictions;
- our expectations regarding the effects of the COVID-19 pandemic on our business, including our preclinical studies and clinical trials;
- our intellectual property position, including the scope of protection we are able to establish and maintain for intellectual property rights covering our current product candidates and other product candidates we may develop, including the extensions of existing patent terms where available, the validity of intellectual property rights held by third parties, and our ability not to infringe, misappropriate or otherwise violate any third-party intellectual property rights;
- our continued reliance on third parties to conduct additional preclinical studies and planned clinical trials of our product candidates, and for the development and manufacture of our product candidates for preclinical studies and clinical trials; our ability to obtain, and negotiate favorable terms of, any collaboration, partnership, licensing or other arrangements that may be necessary or desirable to develop, manufacture or commercialize our product candidates;
- the pricing and reimbursement of our current product candidates and other product candidates we may develop, if approved, including any increase in demand as a result of the availability of reimbursement from the government and third-party payors;
- the rate and degree of market acceptance and clinical utility of our current product candidates and other product candidates we may develop;
- our estimates regarding expenses, operating losses, future revenue, capital requirements and needs for additional financing;
- our financial performance;
- the period over which we estimate our existing cash, cash equivalents and investments in marketable securities will be sufficient to fund our future operating expenses and capital expenditure requirements; and
- our expectations regarding the period during which we will remain an emerging growth company under the JOBS Act.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this Quarterly Report and are subject to a number of risks, uncertainties and assumptions described in the section titled “Risk Factors” and elsewhere in this Quarterly Report. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, investors should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events or otherwise.

In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

## TENAYA THERAPEUTICS, INC.

Condensed Balance Sheets  
(In thousands)

	September 30, 2021 (Unaudited)	December 31, 2020
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 117,077	\$ 128,535
Investments in marketable securities	163,388	—
Prepaid expenses and other current assets	4,357	1,429
Total current assets	284,822	129,964
Property and equipment, net	30,891	17,185
Operating lease right-of-use assets	12,006	—
Restricted cash, non-current	547	547
Other non-current assets	3,528	465
Total assets	<u>\$ 331,794</u>	<u>\$ 148,161</u>
<b>LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
Current liabilities:		
Accounts payable	\$ 6,171	\$ 1,017
Accrued expenses and other current liabilities	5,607	3,161
Deferred rent and other lease liabilities, current	—	863
Operating lease liabilities, current	1,914	—
Total current liabilities	13,692	5,041
Deferred rent and other lease liabilities, non-current	—	3,662
Operating lease liabilities, non-current	14,307	—
Other non-current liabilities	96	19
Total liabilities	28,095	8,722
Commitments and contingencies (Note 6)		
Convertible preferred stock	—	220,754
Stockholders' equity (deficit):		
Common stock	4	—
Additional paid-in capital	433,112	1,584
Notes receivable from stockholders	(12)	(87)
Accumulated other comprehensive loss	(23)	—
Accumulated deficit	(129,382)	(82,812)
Total stockholders' equity (deficit)	303,699	(81,315)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 331,794</u>	<u>\$ 148,161</u>

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

TENAYA THERAPEUTICS, INC.

Condensed Statements of Operations and Comprehensive Loss  
(In thousands, except share and per share data)  
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	\$ 12,944	\$ 8,480	\$ 33,440	\$ 22,735
General and administrative	5,356	1,972	13,202	5,855
Total operating expenses	18,300	10,452	46,642	28,590
Loss from operations	(18,300)	(10,452)	(46,642)	(28,590)
Other income (expense), net:				
Interest income	23	7	41	82
Change in fair value of convertible preferred stock tranche liability	—	151	—	75
Other income (expense), net	15	(1)	31	356
Total other income (expense), net	38	157	72	513
Net loss before income tax expense	(18,262)	(10,295)	(46,570)	(28,077)
Income tax expense	—	—	—	—
Net loss	(18,262)	(10,295)	(46,570)	(28,077)
Other comprehensive loss:				
Unrealized loss on marketable securities	(23)	—	(23)	—
Comprehensive loss	\$ (18,285)	\$ (10,295)	\$ (46,593)	\$ (28,077)
Net loss per share, basic and diluted	\$ (0.68)	\$ (10.29)	\$ (4.75)	\$ (29.65)
Weighted-average shares used in computing net loss per share, basic and diluted	26,895,716	1,000,052	9,808,162	947,009

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

TENAYA THERAPEUTICS, INC.

Condensed Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)

(In thousands, except share data)

(Unaudited)

	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Notes Receivable from Stockholders	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount					
Balance as of January 1, 2021	24,493,528	\$ 220,754	1,210,306	\$ —	\$ 1,584	\$ (87)	\$ —	\$ (82,812)	\$ (81,315)
Issuance of Series C convertible preferred stock, net of issuance costs of \$20	1,608,750	19,981	—	—	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	12,508	—	29	—	—	—	29
Repurchase of common stock related to early exercise of options	—	—	(365)	—	—	—	—	—	—
Vesting of early exercised stock options	—	—	—	—	9	—	—	—	9
Stock-based compensation	—	—	—	—	432	—	—	—	432
Net loss	—	—	—	—	—	—	—	(13,098)	(13,098)
Balance as of March 31, 2021	26,102,278	\$ 240,735	1,222,449	\$ —	\$ 2,054	\$ (87)	\$ —	\$ (95,910)	\$ (93,943)
Issuance of common stock upon exercise of stock options	—	—	12,173	—	31	—	—	—	31
Repurchase of common stock related to early exercise of options	—	—	(11,980)	—	—	—	—	—	—
Vesting of early exercised stock options	—	—	—	—	9	—	—	—	9
Notes receivable from stockholders	—	—	—	—	—	75	—	—	75
Stock-based compensation	—	—	—	—	502	—	—	—	502
Net loss	—	—	—	—	—	—	—	(15,210)	(15,210)
Balance as of June 30, 2021	26,102,278	\$ 240,735	1,222,642	\$ —	\$ 2,596	\$ (12)	\$ —	\$ (111,120)	\$ (108,536)
Conversion of convertible preferred stock to common stock upon completion of initial public offering	(26,102,278)	(240,735)	26,102,278	3	240,732	—	—	—	240,735
Issuance of common stock upon initial public offering, net of issuance costs of \$18,459	—	—	13,800,000	1	188,540	—	—	—	188,541
Issuance of common stock upon exercise of stock options	—	—	160,096	—	268	—	—	—	268
Repurchase of common stock related to early exercise of options	—	—	(4,614)	—	—	—	—	—	—
Vesting of early exercised stock options	—	—	—	—	8	—	—	—	8
Stock-based compensation	—	—	—	—	968	—	—	—	968
Other comprehensive loss	—	—	—	—	—	—	(23)	—	(23)
Net loss	—	—	—	—	—	—	—	(18,262)	(18,262)
Balance as of September 30, 2021	—	\$ —	41,280,402	\$ 4	\$ 433,112	\$ (12)	\$ (23)	\$ (129,382)	\$ 303,699

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

TENAYA THERAPEUTICS, INC.

Condensed Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)  
(In thousands, except share data)  
(Unaudited)

	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Notes Receivable from Stockholders	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount					
Balance as of January 1, 2020	11,403,077	\$ 73,042	1,193,488	\$ —	\$ 764	\$ (86)	\$ —	\$ (44,417)	\$ (43,739)
Issuance of Series B convertible preferred stock, net of issuance costs of \$23 and partial settlement of convertible preferred stock tranche liability of \$27	3,086,415	30,671	—	—	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	3,333	—	3	—	—	—	3
Repurchase of common stock related to early exercise of options	—	—	(612)	—	—	—	—	—	—
Vesting of early exercised stock options	—	—	—	—	11	—	—	—	11
Notes receivable from stockholders	—	—	—	—	—	1	—	—	1
Stock-based compensation	—	—	—	—	154	—	—	—	154
Net loss	—	—	—	—	—	—	—	(9,051)	(9,051)
Balance as of March 31, 2020	14,489,492	\$ 103,713	1,196,209	\$ —	\$ 932	\$ (85)	\$ —	\$ (53,468)	\$ (52,621)
Issuance of common stock upon exercise of stock options	—	—	1,793	—	2	—	—	—	2
Vesting of early exercised stock options	—	—	—	—	11	—	—	—	11
Stock-based compensation	—	—	—	—	185	—	—	—	185
Net loss	—	—	—	—	—	—	—	(8,731)	(8,731)
Balance as of June 30, 2020	14,489,492	\$ 103,713	1,198,002	\$ —	\$ 1,130	\$ (85)	\$ —	\$ (62,199)	\$ (61,154)
Issuance of Series B convertible preferred stock, net of issuance costs of \$26 and settlement of convertible preferred stock tranche liability of \$684	3,086,415	31,324	—	—	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	4,583	—	12	—	—	—	12
Repurchase of common stock related to early exercise of options	—	—	(416)	—	—	—	—	—	—
Vesting of early exercised stock options	—	—	—	—	11	—	—	—	11
Stock-based compensation	—	—	—	—	198	—	—	—	198
Net loss	—	—	—	—	—	—	—	(10,295)	(10,295)
Balance as of September 30, 2020	17,575,907	\$ 135,037	1,202,169	\$ —	\$ 1,351	\$ (85)	\$ —	\$ (72,494)	\$ (71,228)

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



TENAYA THERAPEUTICS, INC.

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

	Nine Months Ended September 30,	
	2021	2020
<b>Cash flows from operating activities:</b>		
Net loss	\$ (46,570)	\$ (28,077)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	2,170	1,825
Amortization of premium on marketable securities	6	—
Stock-based compensation	1,902	537
Loss on disposal of property and equipment	4	35
Non-cash operating lease expense	743	—
Change in fair value of convertible preferred stock tranche liability	—	(75)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(3,029)	304
Other non-current assets	(3,063)	(168)
Accounts payable	2,518	527
Accrued expenses and other current liabilities	1,729	642
Deferred rent and other lease liabilities	—	(572)
Operating lease liabilities	(952)	—
Other non-current liabilities	77	(24)
Net cash used in operating activities	<u>(44,465)</u>	<u>(25,046)</u>
<b>Cash flows from investing activities:</b>		
Purchases of property and equipment	(12,567)	(1,586)
Purchases of marketable securities	(163,417)	—
Proceeds from maturities of marketable securities	—	2,753
Net cash (used in) provided by investing activities	<u>(175,984)</u>	<u>1,167</u>
<b>Cash flows from financing activities:</b>		
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	—	61,328
Proceeds from issuance of Series C convertible preferred stock, net of issuance costs	19,981	—
Proceeds from initial public offering, net of issuance costs	188,605	—
Proceeds from exercise of stock options	343	17
Repurchases of common stock	(13)	(1)
Proceeds from repayments on notes receivable from stockholders	75	—
Net cash provided by financing activities	<u>208,991</u>	<u>61,344</u>
Net change in cash, cash equivalents and restricted cash	(11,458)	37,465
Cash and cash equivalents and restricted cash at beginning of period	129,082	24,271
Cash and cash equivalents and restricted cash at end of period	<u>\$ 117,624</u>	<u>\$ 61,736</u>
<b>Components of cash, cash equivalents and restricted cash:</b>		
Cash and cash equivalents	117,077	61,337
Restricted cash, non-current	547	399
Cash, cash equivalents and restricted cash	<u>\$ 117,624</u>	<u>\$ 61,736</u>
<b>Supplemental disclosure of cash operating activities:</b>		
Cash paid for leases included in operating cash outflows	<u>\$ 4,423</u>	<u>\$ —</u>
<b>Supplemental disclosure of non-cash operating activities:</b>		
Lease liability obtained in exchange for right-of-use asset	<u>\$ 8,558</u>	<u>\$ —</u>
<b>Supplemental disclosure of non-cash investing and financing activities:</b>		
Conversion of convertible preferred stock to common stock upon completion of initial public offering	<u>\$ 220,754</u>	<u>\$ —</u>
Offering costs related to initial public offering included in accounts payable and accrued expenses and other current liabilities	<u>\$ 64</u>	<u>\$ —</u>
Property and equipment included in accounts payable and accrued expenses and other current liabilities	<u>\$ 3,677</u>	<u>\$ —</u>
Settlement of convertible preferred stock tranche liability in connection with the issuance of Series B convertible preferred stock	<u>\$ —</u>	<u>\$ 711</u>
Offering costs related to Series B convertible preferred stock in accrued expenses and other current liabilities	<u>\$ —</u>	<u>\$ 44</u>

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

## TENAYA THERAPEUTICS, INC.

### Notes to Unaudited Condensed Financial Statements

#### 1. Organization and Description of the Business

##### *Description of the Business*

Tenaya Therapeutics, Inc. (the “Company”) was incorporated in the state of Delaware in August 2016 and is headquartered in South San Francisco, California. The Company is a preclinical stage biotechnology company focused on discovering, developing and delivering curative therapies that address the underlying drivers of heart disease. The Company is advancing product candidates from three distinct but interrelated product platforms: gene therapy, cellular regeneration and precision medicine.

##### *Reverse Stock Split*

In July 2021, the Company’s board of directors approved an amended and restated certificate of incorporation to effect a reverse split of shares of the Company’s common stock, convertible preferred stock, and authorized shares on a 1-for-6 basis (the “Reverse Stock Split”) effective on July 23, 2021. The par value of the common stock and convertible preferred stock was not adjusted as a result of the Reverse Stock Split. All share data, per share data and related information for all periods presented in the accompanying condensed financial statements have been retrospectively adjusted to reflect the effect of the Reverse Stock Split.

##### *Initial Public Offering*

On August 3, 2021, the Company completed its initial public offering (“IPO”), at which time the Company issued an aggregate of 13,800,000 shares of its common stock (inclusive of 1,800,000 shares pursuant to the underwriters’ overallotment option) at a price of \$15.00 per share. The Company received net proceeds of \$188.5 million, after deducting underwriting discounts and commissions of \$14.5 million and other offering expenses of \$4.0 million. Immediately prior to the completion of the IPO, all of the Company’s outstanding shares of convertible preferred stock automatically converted into 26,102,278 shares of common stock.

##### *Liquidity*

The Company has incurred net losses since inception and expects such losses to continue in the future as it conducts research and development activities. As of September 30, 2021, the Company had an accumulated deficit of \$129.4 million. The Company incurred a net loss of \$46.6 million and \$28.1 million during the nine months ended September 30, 2021 and 2020. The Company had \$280.5 million of cash, cash equivalents and investments in marketable securities as of September 30, 2021.

Management recognizes the need to raise additional capital to fully implement its business plan. The Company may seek to raise capital through equity financings, debt financings, license agreements, collaborative agreements or other sources of financing. Management believes that its existing cash, cash equivalents and investments in marketable securities as of September 30, 2021 will be sufficient to fund the Company’s operations for at least the next twelve months following the date these condensed financial statements are filed with the Securities and Exchange Commission (“SEC”).

#### 2. Summary of Significant Accounting Policies

##### *Basis of Presentation*

The accompanying condensed financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (“GAAP”) and follow the requirements of the SEC for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP can be condensed or omitted.

The interim condensed balance sheet as of September 30, 2021, the interim condensed statements of operations and comprehensive loss, convertible preferred stock and stockholders’ equity (deficit) for the three and nine months ended September 30, 2021 and 2020 and the interim condensed statements of cash flows for the nine months ended September 30, 2021 and 2020 are unaudited. These unaudited interim condensed financial statements have been prepared on the same basis as the Company’s annual financial statements and reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for the fair statement of the Company’s financial position, results of operations and cash flows for the interim periods presented. The condensed results of operations for the three and nine months ended September 30, 2021 are not necessarily indicative of the results to be expected for the full year or for any other future annual or interim period. The condensed balance sheet as of December 31, 2020

included herein was derived from the audited financial statements as of that date. These condensed financial statements should be read in conjunction with the Company's audited financial statements included in the prospectus dated July 29, 2021, that forms a part of the Company's Registration Statements on Form S-1 (File Nos. 333-257820 and 333-258285), as filed with the SEC pursuant to Rule 424 promulgated under the Securities Act of 1933, as amended.

### ***Use of Estimates***

The preparation of condensed financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, and the disclosure of contingent assets and liabilities at the date of the condensed financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions made in the accompanying condensed financial statements include, but are not limited to, the fair value of common stock, the valuation of equity-based awards, the useful lives of property and equipment, the fair value of the convertible preferred stock tranche liability, accrued expenses related to research and development activities and the valuation allowance for deferred tax assets. The Company bases its estimates on historical experience, the current economic environment, and on various other assumptions that are believed to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

### ***Cash and Cash Equivalents***

The Company considers all highly liquid investments purchased with an original maturity of three months or less on the date of purchase to be cash equivalents. Cash equivalents primarily consist of money market funds, commercial paper and U.S. government agencies bonds that are stated at fair value.

### ***Marketable Securities***

The Company invests in marketable securities, primarily securities issued by the U.S. government and its agencies, commercial paper and corporate bonds. All marketable securities have been classified as available-for-sale and are carried at estimated fair value as determined based upon quoted market prices or pricing models for similar securities. Management determines the appropriate classification of its marketable debt securities at the time of purchase and reevaluates such designation at each balance sheet date. Unrealized gains and losses are excluded from earnings and are reported as a component of other comprehensive loss. Realized gains and losses and declines in fair value judged to be other than temporary, if any, on available-for-sale securities are included in other income (expense), net. There are no material realized gains or losses on marketable securities for all periods presented. The cost of securities sold is based on the specific-identification method. Interest earned on marketable securities is included in interest income.

### ***Leases***

The Company adopted Accounting Standards Codification ("ASC") Topic 842, *Leases* ("ASC 842") on January 1, 2021, as discussed below in the section titled "Recently Adopted Accounting Standards". Under ASC 842, the Company determines if an arrangement is a lease at inception.

Operating lease right-of-use ("ROU") assets represent the Company's right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized based on the present value of lease payments over the lease term at the commencement date of the lease. ROU assets also include any initial direct costs incurred and any lease payments made at or before the lease commencement date, less any lease incentive received. The Company uses the rate implicit in the lease in determining the present value of lease payments and, if that rate is not readily determinable, the Company uses its incremental borrowing rate based on the information available at the date of lease commencement. The incremental borrowing rate reflects the rate of interest that a lessee would have to pay to borrow, on a collateralized basis over a similar term, an amount equal to the lease payments in a similar economic environment. The Company's lease terms may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Lease expense for lease payments is recognized on a straight-line basis over the lease term.

The Company's non-lease components are primarily related to property taxes, insurance, and common area maintenance, which vary based on future outcomes, and are recognized as rent expense when incurred.

### ***Asset Retirement Obligation***

The Company records asset retirement obligations ("AROs") for the estimated cost of removing constructed leasehold improvement assets and restoring the leased premises back to their original condition, at the time when the contractual obligations are incurred. AROs represent the present value of the expected costs for the related restoration activities. The ARO assets and liabilities are recorded in property, plant and equipment and other long-term liabilities, respectively, in the Company's balance sheets. The Company records accretion expense, which represents the increase in the asset retirement obligations, over the remaining or operational life of the associated leasehold improvements. Accretion expense is recorded as operating expense in the statements of

operations using an accretion rate based on the credit adjusted risk-free interest rate. Changes resulting from revisions to the timing or amount of the original estimate of cash flows are recognized as an increase or a decrease in the asset retirement cost, or income when the asset retirement cost is depleted.

### **Net Loss Per Share**

Basic net loss per share is calculated by dividing the net loss by the weighted-average number of shares of the Company's common stock outstanding for the period, without consideration for potential dilutive shares of common stock. As the Company is in a loss position for the periods presented, diluted net loss per share is the same as basic net loss per share, since the effects of potentially dilutive securities are antidilutive. Shares related to early exercised stock options and restricted stock that are subject to repurchase are excluded from the basic and diluted net loss per share calculation until the Company's repurchase right lapses.

### **Recently Adopted Accounting Standards**

On January 1, 2021, the Company adopted ASC 842 using the modified retrospective transition method and elected the practical expedients to not reassess whether any expired or existing contracts are or contain leases, carry forward its historical lease classification and not reassess initial direct costs for existing leases. The Company elected to not separate non-lease components from the associated lease components and to not recognize ROU assets and lease liabilities for leases with a term of twelve months or less. Upon adoption of ASC 842, the Company recorded an operating right-of-use asset of \$4.6 million, operating lease liabilities of \$9.1 million and derecognized deferred rent and other lease liabilities of \$4.5 million. Results for the nine months ended September 30, 2021 are presented under ASC 842. Prior period amounts before January 1, 2021 have not been adjusted and continue to be reported in accordance with the Company's historical accounting under previous lease guidance, *ASC 840: Leases (Topic 840)*.

### **Recently Issued Accounting Pronouncements Not Yet Adopted**

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments – Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"), which replaces the existing incurred loss impairment model with an expected credit loss model. This standard will require companies to recognize an allowance for credit losses on available-for-sale debt securities rather than the current approach of recording a reduction to the carrying value of the asset. As an emerging growth company, ASU 2016-13 is effective for the Company beginning January 1, 2023. The Company is evaluating the impact of this standard on its financial statements.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU 2019-12"), which is intended to simplify the accounting for income taxes. This standard eliminates certain exceptions to the approach for intra period tax allocation, the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. As an emerging growth company, ASU 2019-12 is effective for the Company beginning January 1, 2022. The Company is evaluating the impact of this standard on its financial statements.

In August 2020, the FASB issued ASU No. 2020-06, *Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40)* ("ASU 2020-06"), which is intended to simplify the accounting for convertible debt instruments and convertible preferred stock. This standard removes the existing guidance in ASC 470-20 that requires companies to account for cash conversion features and beneficial conversion features in equity, separately from the host convertible debt or preferred stock. As an emerging growth company, ASU 2020-06 is effective for the Company beginning January 1, 2024. The Company currently does not expect the adoption of this standard to have a material impact on its financial statements.

## **3. Fair Value Measurements**

Financial assets and liabilities are recognized at fair value on a recurring basis. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability (an exit price) in an orderly transaction between market participants at the reporting date. The accounting guidance establishes a three-tiered hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

Level 1 - Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2 - Inputs other than quoted market prices included in Level 1 are either directly or indirectly observable for the asset or liability through correlation with market data at the measurement date and for the duration of the instrument's anticipated life.

Level 3 - Inputs reflect management's best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

The following tables summarize the Company's financial assets measured at fair value on a recurring basis by level within the fair value hierarchy:

Valuation Hierarchy	September 30, 2021				Fair Value
	Amortized Cost	Unrealized Gain	Unrealized Loss		
(In thousands)					
<b>Assets:</b>					
<b>Cash equivalents:</b>					
Money market funds	Level 1	\$ 68,815	\$ —	\$ —	\$ 68,815
Commercial paper	Level 2	44,246	—	—	44,246
Government agencies bonds	Level 2	3,015	—	—	3,015
<b>Marketable securities:</b>					
U.S. treasuries	Level 1	40,677	—	(9)	40,668
Commercial paper	Level 2	109,095	3	(12)	109,086
Corporate bonds	Level 2	9,036	—	(4)	9,032
Government agencies bonds	Level 2	4,603	—	(1)	4,602
<b>Total financial assets</b>		<b>\$ 279,487</b>	<b>\$ 3</b>	<b>\$ (26)</b>	<b>\$ 279,464</b>

Valuation Hierarchy	December 31, 2020				Fair Value
	Amortized Cost	Unrealized Gain	Unrealized Loss		
(In thousands)					
<b>Assets:</b>					
<b>Cash equivalents:</b>					
Money market funds	Level 1	\$ 127,535	\$ —	\$ —	\$ 127,535
<b>Total financial assets</b>		<b>\$ 127,535</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 127,535</b>

Money market funds are classified as Level 1 because they are valued using quoted market prices in active markets for identical assets. Financial instruments classified within Level 2 of the fair value hierarchy are valued based on observable inputs or can be derived from non-binding quotes from the Company's investment managers, which are based on proprietary valuation models of independent pricing services. These models generally use inputs such as observable market data, quoted market prices for similar instruments, or historical pricing trends of a security relative to its peers. To validate the fair value determination provided by its investment managers, the Company reviews the pricing movement in the context of overall market trends and trading information from its investment managers. In addition, the Company considers the inputs and methods used in determining the fair value in order to determine the classification of securities in the fair value hierarchy.

The Company believes it is more likely than not that its marketable securities in an unrealized loss position will be held until maturity or the recovery of the cost basis of the investment. To date, the Company has not recorded any impairment charges on marketable securities related to other-than-temporary declines in market value. All available-for-sale marketable securities held as of September 30, 2021 are short-term investments with contractual maturities of less than one year.

#### **Convertible Preferred Stock Tranche Liability**

The Company's convertible preferred stock tranche liability (see Note 7) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. Fair value was calculated using an option pricing model that required significant unobservable inputs supported by little or no market activity. The convertible preferred stock tranche liability is considered a non-contingent forward and the standard forward pricing model was used with the following key assumptions: (a) calculation of an expected term and (b) a risk-free interest rate. On the second and third closings of the Company's Series B convertible preferred stock financings in March and August 2020, the convertible preferred stock tranche liability was settled and reclassified to Series B convertible preferred stock. Accordingly, there is no convertible preferred stock tranche liability as of September 30, 2021 and December 31, 2020.

The following table summarizes the significant unobservable assumptions used to value the convertible preferred stock tranche liability as of the settlement date of August 24, 2020:

	<u>August 24, 2020</u>
Term to valuation date (in years)	0.00
Discount rate	5.0%

The following table summarizes the changes in the estimated fair value of the Company's convertible preferred stock tranche liability measured on a recurring basis using significant Level 3 inputs:

	<u>Nine Months Ended September 30, 2020</u>	
	(In thousands)	
Beginning balance	\$	786
Change in fair value upon remeasurement		(75)
Settlement of convertible preferred stock tranche liability on second and third closings of the Series B convertible preferred stock		(711)
Ending balance	\$	—

#### 4. Balance Sheet Components

##### *Property and Equipment, Net*

Property and equipment, net consists of the following:

	<u>September 30, 2021</u>	<u>December 31, 2020</u>
	(In thousands)	
Construction in progress	\$ 21,895	\$ 7,678
Laboratory equipment	9,806	8,182
Leasehold improvements	7,242	7,237
Furniture and fixtures	534	534
Computer equipment and software	272	257
Total property and equipment	\$ 39,749	\$ 23,888
Less: accumulated depreciation and amortization	(8,858)	(6,703)
Total property and equipment, net	\$ 30,891	\$ 17,185

Depreciation and amortization expense for the three months ended September 30, 2021 and 2020 was \$0.7 million and \$0.6 million, respectively. Depreciation and amortization expense for the nine months ended September 30, 2021 and 2020 was \$2.2 million and \$1.8 million, respectively. Construction in progress primarily consists of capitalized machinery and equipment that is expected to be placed in service in the Company's manufacturing and office space located in Union City, California (see Note 6).

##### *Accrued Expenses and Other Current Liabilities*

Accrued expenses and other current liabilities consist of the following:

	<u>September 30, 2021</u>	<u>December 31, 2020</u>
	(In thousands)	
Accrued compensation and related expenses	\$ 2,663	\$ 2,090
Accrued research and development expenses	1,043	391
Accrued property and equipment	972	231
Other current liabilities	514	121
Accrued professional services	415	328
Total accrued expenses and other current liabilities	\$ 5,607	\$ 3,161

## 5. License Agreements

### *Gladstone License Agreement*

In October 2016, the Company entered into a license agreement with the J. David Gladstone Institute (Gladstone), pursuant to which Gladstone granted the Company a worldwide, royalty-bearing exclusive patent license and a non-exclusive technology license to develop and commercialize certain products for certain diseases (“Gladstone License Agreement”). Pursuant to the Gladstone License Agreement, the Company is obligated, among other things, to pay Gladstone (i) annual license maintenance fees ranging from \$25,000 up to \$0.1 million per year, which will be creditable against royalties paid in the following twelve month period, (ii) milestone payments up to \$4.1 million for royalty-bearing products directed to a particular target, which are contingent upon achieving specific clinical and commercialization milestone events, and (iii) tiered low-single digit royalties on future net sales of each royalty-bearing product. Under the agreement, the Company is subject to diligence requirements to develop and commercialize at least one royalty-bearing product. The Company may pay \$50,000 to \$100,000 to extend the deadline for its diligence milestone obligations for up to four additional one-year terms. As of September 30, 2021, the Company has not recognized any milestone and royalty payments under the Gladstone License Agreement.

During the three and nine months ended September 30, 2020 and 2021, there were no material amounts recorded related to annual license fees payable pursuant to the Gladstone License Agreement.

### *University of Texas Southwestern License Agreement*

In January 2020, the Company entered into a license agreement with the University of Texas Southwestern (“UTSW License”), pursuant to which UTSW granted the Company a royalty-bearing exclusive and sublicensable patent license and a non-exclusive, non-sublicensable license for mutually agreed upon development activities. Under the UTSW License, the Company is obligated to pay UTSW (i) a non-refundable upfront license fee of \$0.1 million, (ii) milestone payments up to a total of \$14.8 million in aggregate, which are contingent upon achieving specific development and commercialization milestone events, and (iii) royalties on future net sales of each royalty-bearing product ranging in the low-single digits. As of September 30, 2021, the Company has not recognized any milestone and royalty payments under the UTSW License.

During the three and nine months ended September 30, 2020, the Company recorded research and development expenses of \$nil and \$0.1 million related to the upfront license fee payable pursuant to the UTSW License.

### *Other License Agreements*

In addition to the agreements described above, the Company has also entered into other license agreements with various institutions and business entities, none of which are material individually or in the aggregate.

## 6. Commitments and Contingencies

### *Facility Leases*

In December 2016, the Company entered into a lease agreement for office and laboratory space in South San Francisco, California. The lease expires in May 2025 and the Company may renew the lease term for two additional five-year periods. Pursuant to the lease agreement, the Company received a tenant improvement allowance of \$5.8 million in aggregate for leasehold improvements to the facility.

In December 2020, the Company entered into a short-term sublease agreement for additional office and laboratory space in South San Francisco, California that expires on November 30, 2021.

In February 2021, the Company entered into a lease agreement for manufacturing and office space located in Union City, California. The lease commenced in May 2021 and has a ten-year term with one five-year renewal option. Upon the execution of the lease agreement, the Company provided the landlord with a refundable security deposit of \$3.3 million, which is included in other non-current assets on the condensed balance sheets.

On January 1, 2021, the Company adopted ASC 842 and the following disclosures as of and for the three and nine months ended September 30, 2021 are presented under ASC 842. As of September 30, 2021, the remaining weighted-average lease term was 6.8 years and the weighted-average incremental borrowing rate used to determine the operating lease liabilities was 9.5%.

For the three months ended September 30, 2021, the Company incurred \$1.6 million of lease costs, of which \$0.6 million is related to the Company's short-term lease and \$0.4 million is related to variable lease payments. For the three months ended September 30, 2020, the Company incurred \$0.6 million in rent expense.

For the nine months ended September 30, 2021, the Company incurred \$4.2 million of lease costs, of which \$1.8 million is related to the Company's short-term lease and \$1.2 million is related to variable lease payments. For the nine months ended September 30, 2020, the Company incurred \$1.9 million in rent expense.

As of September 30, 2021, the undiscounted future minimum lease payments due under the Company's non-cancelable operating leases are as follows:

	<u>Amount</u>
	<u>(In thousands)</u>
2021 (remaining 3 months)	\$ 902
2022	3,678
2023	3,792
2024	3,910
2025	2,445
Thereafter	8,291
Total undiscounted future minimum lease payments	<u>\$ 23,018</u>
Present value adjustment for minimum lease commitments	(6,515)
Tenant improvement receivable	(282)
Total operating lease liabilities	<u>\$ 16,221</u>

As of December 31, 2020, undiscounted future minimum lease payments due under the Company's non-cancelable operating lease are as follows:

	<u>Amount</u>
	<u>(In thousands)</u>
2021	\$ 3,752
2022	2,206
2023	2,283
2024	2,363
2025	999
Total future minimum lease payments	<u>\$ 11,603</u>

The Company has previously entered into agreements to sublease portions of the Company's facilities in South San Francisco to two different subtenants, both of which expired as of December 31, 2020. Pursuant to the sublease agreements, the Company received sublease income of \$nil and \$0.2 million during the three and nine months ended September 30, 2020, which is included in other income (expense), net on the condensed statements of operations and comprehensive loss.

#### ***Asset Retirement Obligation***

Under the lease agreement for the manufacturing and office facility in Union City, the Company is contractually obligated to remove constructed leasehold improvements related to capitalized machinery and equipment (see Note 4) and to restore the leased space to its original condition upon termination of the lease agreement. The Company applies the proportionate method to account for the buildup of the asset retirement obligation while leasehold improvements are in progress. As of September 30, 2021, the balance of the asset retirement obligation liability was not material. The Company elected to defer the commencement of accretion of the asset retirement obligation until the underlying construction is completed, on the basis that the financial statement impact from the deferral is immaterial.

#### ***Purchase Commitments***

The Company enters into contractual agreements with various suppliers in the normal course of its business, including vendors that provide machinery and equipment. All contracts are terminable, with varying provisions regarding termination. If a contract with a specific vendor were to be terminated, the Company would only be obligated for the products or services that the Company had received through the time of termination.



## Indemnification

In the normal course of business, the Company enters into agreements that may include indemnification provisions. Pursuant to such agreements, the Company may indemnify, hold harmless and defend an indemnified party for losses suffered or incurred by the indemnified party. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amounts of future payments the Company could be required to make under these provisions is not determinable. In addition, the Company has entered into indemnification agreements with its directors and certain officers that may 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. As of September 30, 2021 and December 31, 2020, the Company does not have any material indemnification claims that were probable or reasonably possible and, consequently, has not recorded any related liabilities.

## 7. Convertible Preferred Stock

On August 3, 2021, immediately prior to the completion of the Company's IPO, all outstanding shares of convertible preferred stock were converted into 26,102,278 shares of the Company's common stock.

Prior to the Company's IPO, convertible preferred stock as of December 31, 2020 consists of the following:

	December 31, 2020			
	Shares Authorized	Shares Issued and Outstanding	Net Carrying Value	Liquidation Preference
	(In thousands, except share data)			
<b>Convertible Preferred Stock</b>				
Series A	8,316,666	8,316,662	\$ 43,393	\$ 49,900
Series B	9,259,254	9,259,245	91,644	92,000
Series C	8,526,381	6,917,621	85,717	86,000
Total	<u>26,102,301</u>	<u>24,493,528</u>	<u>\$ 220,754</u>	<u>\$ 227,900</u>

The Company classifies its convertible preferred stock outside of total stockholders' equity (deficit) because, in the event of certain change of control events that are not solely within the control of the Company (including liquidation, sale or transfer of the Company), the shares would become redeemable at the option of the holders. As a result, the Company classified its convertible preferred stock as mezzanine equity on the balance sheet as the preferred stock is contingently redeemable. Subsequent adjustments to increase or decrease the carrying values to the ultimate liquidation values will be made only if and when it becomes probable that such liquidation event will occur.

### Series B Convertible Preferred Stock Financing

In August 2019, the Company entered into a Series B preferred stock purchase agreement ("Series B SPA") for the issuance of up to 9,259,254 shares of the Company's Series B convertible preferred stock at a purchase price of \$9.936 per share in multiple closings. The Company completed the initial closing in August 2019, whereby 3,086,415 shares of Series B convertible preferred stock were issued for gross proceeds of \$30.7 million. Pursuant to the Series B SPA, the Company was permitted to sell additional shares in subsequent closings contingent upon the approval of the Company's board of directors.

On issuance, the Company determined that its obligation to issue additional shares of Series B convertible preferred stock in future closings was a freestanding instrument that should be classified as a liability on the Company's balance sheets. The freestanding financial instrument, or convertible preferred stock tranche liability, was recorded at fair value on issuance of \$0.8 million with the remaining proceeds being allocated to the Series B convertible preferred stock. Any changes in fair value of the convertible preferred stock tranche liability in subsequent reporting periods are recognized as a component of other income (expense), net in the statements of operations and comprehensive loss (see Note 3).

In March 2020, the Company completed its second closing of Series B convertible preferred stock financing and issued 3,086,415 shares of Series B convertible preferred stock at the fixed purchase price of \$9.936 per share for gross proceeds of \$30.7 million, thereby settling a portion of the convertible preferred stock tranche liability. Immediately prior to the second closing, the Company measured the convertible preferred stock tranche liability to its then fair value. Upon the closing of the second tranche, the convertible preferred stock tranche liability was partially settled and the related balance of the liability of \$27,000 was reclassified to Series B convertible preferred stock.

In August 2020, the Company completed its third closing of Series B convertible preferred stock financing and issued 3,086,415 shares of Series B convertible preferred stock at the purchase price of \$9.936 per share for gross proceeds of \$30.7 million, thereby settling the remainder of the convertible preferred stock tranche liability. Immediately prior to the third closing, the Company measured the convertible preferred stock tranche liability to its then fair value. Upon the closing of the third tranche, the remaining convertible preferred stock tranche liability was settled and the balance of the liability of \$0.7 million was reclassified to Series B convertible preferred stock.

### ***Series C Convertible Preferred Stock Financing***

In December 2020, the Company entered into a Series C preferred stock purchase agreement (“Series C SPA”) for the issuance of up to 8,526,381 shares of the Company’s Series C convertible preferred stock at a purchase price of \$12.432 per share in two closings. The Company completed the initial closing in December 2020, whereby 6,917,621 shares of Series C convertible preferred stock were issued for gross proceeds of \$86.0 million.

In January 2021, the Company sold an additional 1,608,750 shares of Series C convertible preferred stock at a purchase price of \$12.432 per share for gross proceeds of \$20.0 million.

## **8. Common Stock**

The holders of common stock are entitled to one vote per share on all matters to be voted on by the stockholders of the Company and are entitled to dividends, if and when declared by the board of directors, subject to the prior rights of the preferred stockholders. Common stock issued and outstanding on the condensed statements of convertible preferred stock and stockholders’ equity (deficit) includes shares related to early exercised options and restricted stock that are subject to repurchase. Common stock issued and outstanding is reduced for any repurchases of early exercised stock options and restricted stock. As of September 30, 2021 and December 31, 2020, outstanding common stock included 40,915 and 138,127 shares related to early exercised stock options and restricted stock that are unvested and subject to repurchase.

Total shares of common stock reserved for issuance, on an as-if converted basis, is as follows:

	<b>September 30, 2021</b>	<b>December 31, 2020</b>
Conversion of outstanding shares of convertible preferred stock	—	24,493,528
Stock options issued and outstanding	2,530,121	1,160,808
Stock options available for future grant	<u>3,847,160</u>	<u>412,170</u>
Total	<u><u>6,377,281</u></u>	<u><u>26,066,506</u></u>

## **9. Stock-Based Compensation**

### ***2021 Equity Incentive Plan***

In July 2021, the Company adopted the 2021 Equity Incentive Plan (the “2021 Plan”), which became effective in connection with the IPO. The Company’s 2016 Equity Incentive Plan (the “2016 Plan”) was terminated in 2021. Shares subject to awards granted under the 2016 Plan that are repurchased by or forfeited to the Company will also be reserved for issuance under the 2021 Plan. The number of shares of common stock available for issuance under the 2021 Plan will automatically increase on the first day of January for a period of ten years, commencing on January 1, 2022, in an amount equal to the lesser of: 4,000,000 shares; 4% of the outstanding shares of the Company’s common stock as of the last day of the immediately preceding year; or such other amount as the Company’s board of directors may determine.

Total shares reserved and available for grant under the 2021 Plan as of September 30, 2021 are 3,847,160.

### Stock Option Activity

The following table summarizes stock option activity:

	Number of Options Outstanding	Weighted- Average Exercise Price Per Share	Weighted- Average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (In thousands)
Outstanding as of December 31, 2020	1,160,808	\$ 1.74	8.40	\$ 6,060
Options granted	1,595,128	9.36		
Options exercised	(184,777)	1.85		
Options cancelled	(41,038)	3.51		
Outstanding as of September 30, 2021	2,530,121	\$ 6.50	8.87	\$ 35,805
Exercisable as of September 30, 2021	590,000	\$ 2.18	7.71	\$ 10,898

### 2021 Employee Stock Purchase Plan

In July 2021, the Company adopted the 2021 Employee Stock Purchase Plan (the “ESPP”), which became effective in connection with the IPO. The Company initially reserved 800,000 shares for future issuance under the ESPP. The ESPP permits participants to purchase common stock through payroll deductions of up to 15% of their eligible compensation. The number of shares of common stock available for issuance under the ESPP will automatically increase on the first day of January following the year in which the first offering period under the ESPP commences, in an amount equal to the lesser of: 800,000 shares; 1% of the outstanding shares of the Company’s common stock as of the last day of the immediately preceding year; or such other amount as the board of directors may determine. The first offering period has not commenced as of September 30, 2021 and there is no stock-based compensation related to the ESPP for the three and nine months ended September 30, 2021.

### Stock-Based Compensation

The following table summarizes stock-based compensation recognized in the Company’s condensed statements of operations and comprehensive loss:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
	(In thousands)			
Research and development	\$ 326	\$ 97	\$ 654	\$ 273
General and administrative	642	101	1,248	264
Total stock-based compensation	<u>\$ 968</u>	<u>\$ 198</u>	<u>\$ 1,902</u>	<u>\$ 537</u>

As of September 30, 2021, there was approximately \$14.3 million of unrecognized stock-based compensation, which the Company expects to recognize over a weighted-average period of 3.5 years.

### Stock Option Valuation

The fair value of the Company’s stock option awards is estimated on the date of grant using the Black-Scholes option pricing model using the following assumptions:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Expected term (in years)	6.0 - 6.1	6.0	5.0 - 6.1	6.0 - 6.1
Expected volatility	95% - 96%	183%	95% - 103%	178% - 183%
Risk-free interest rate	0.9% - 1.2%	0.4%	0.6% - 1.2%	0.4% - 1.5%
Expected dividend yield	— %	— %	— %	— %

## Restricted Stock

A summary of restricted stock activity is as follows:

	<u>Number of Shares</u>	<u>Weighted Average Fair Value at Date of Grant per Share</u>
Unvested as of December 31, 2020	138,127	\$ 0.90
Granted	19,852	\$ 1.93
Vested	(100,105)	\$ 0.56
Repurchased	(16,959)	\$ 2.27
Unvested as of September 30, 2021	<u>40,915</u>	<u>\$ 1.67</u>

## Employee Recourse Notes

In 2017 and 2018, the Company entered into full recourse notes with certain employees upon the exercise of stock options that are treated as substantive exercises for accounting purposes. The Company has the right to repurchase unvested shares up to 90 days after employment is terminated. As of September 30, 2021, the principal and accrued interest amount of the notes was \$12,000. The notes are presented in the condensed statements of convertible preferred stock and stockholders' equity (deficit).

## 10. Income Taxes

For the three and nine months ended September 30, 2021 and 2020, the Company did not record any income tax expense. The Company has recorded a full valuation allowance against its U.S. federal and state deferred tax assets as the Company believes it is not more likely than not that the benefit will be realized.

## 11. Net Loss Per Share

The following potentially dilutive securities were not included in the calculation of diluted net loss per share as of the periods presented because the effect would have been anti-dilutive:

	<u>September 30,</u>	
	<u>2021</u>	<u>2020</u>
Convertible preferred stock	—	17,575,907
Outstanding stock options	2,530,121	1,099,224
Restricted stock subject to future vesting	40,915	179,062
Total	<u>2,571,036</u>	<u>18,854,193</u>

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

*You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our condensed financial statements and related notes included elsewhere in this Quarterly Report on Form 10-Q and our final prospectus, dated July 29, 2021, filed with the Securities and Exchange Commission pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended (the “Prospectus”).*

*In addition to historical financial information, this discussion and analysis and other parts of this report contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, based upon current expectations that involve risks, uncertainties and assumptions. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the section titled “Risk Factors” under Part II, Item 1A. below. You should carefully read the “Risk Factors” to gain an understanding of the factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section titled “Special Note Regarding Forward-Looking Statements.”*

### Overview

We are a biotechnology company headquartered in South San Francisco, California focused on discovering, designing, developing and delivering curative therapies that address the underlying drivers of heart disease. We are advancing a pipeline of disease-modifying therapies developed using our product platforms and core internal capabilities to target defined sub-populations of patients with rare or highly prevalent forms of heart disease.

We were incorporated in August 2016 and commenced operations thereafter. Our operations to date have included developing our gene therapy, cellular regeneration and precision medicine platforms, identifying and developing product candidates, conducting preclinical studies, acquiring technology, organizing and recruiting management and technical staff, business planning, establishing our intellectual property portfolio, raising capital, and providing general and administrative support for these operations. All of our programs are currently in the preclinical stage and we do not have any products approved for sale. We have not generated any revenue.

We are advancing a deep and diverse pipeline that includes both gene therapies and small molecules. In 2022, we intend to submit an investigational new drug application (“IND”) or a clinical trial application (“CTA”) to the U.S. Food and Drug Administration (“FDA”) or European Medicines Agency (“EMA”), respectively, for the most advanced product candidate from our Gene Therapy platform, TN-201, an adeno-associated virus (“AAV”)-based gene therapy to address genetic hypertrophic cardiomyopathy (“gHCM”) caused by Myosin Binding Protein C3 (“MYBPC3”) gene mutations. TN-201, currently in IND-enabling studies, is designed to deliver a fully functional MYBPC3 gene driven by our proprietary heart-specific promoter to restore normal levels of MYBPC3 protein. We initiated a global natural history study in November 2021 to improve our understanding of disease progression and unmet need in individuals carrying mutations in the MYBPC3 gene, with an initial focus on pediatric patients under the age of 18.

We also intend to submit an IND to the FDA in 2022 for the most advanced product candidate from our Precision Medicine platform, TYA-11631, a highly specific small molecule inhibitor of histone deacetylase 6 (“HDAC6i”). TYA-11631, currently in IND-enabling studies, has potentially broad utility in both heart failure (“HF”) with preserved ejection fraction (“HFpEF”) as well as genetic dilated cardiomyopathy (“gDCM”). We initiated cGMP activities to support the production of TYA-11631 at larger scales in October 2021. Our PKP2 program involves using an AAV-based gene therapy to address genetic arrhythmogenic right ventricular cardiomyopathy (“gARVC”) caused by plakophilin 2 (“PKP2”) gene mutations, and is currently at the candidate selection stage. Our DWORF program, an AAV-based gene therapy designed to express the Dwarf Open Reading Frame (“DWORF”) gene in the heart, has potentially broad utility in dilated cardiomyopathy (“DCM”) and HF with reduced ejection fraction (“HFrEF”), and is currently at the candidate selection stage. Our Reprogramming program for cardiac regeneration can potentially replace heart cells lost in patients experiencing HF due to prior myocardial infarction (“MI”), and is currently at the candidate selection stage. In addition, we have numerous earlier-stage programs emerging from our product platforms to address other forms of HF.

On August 3, 2021, we completed an initial public offering (“IPO”) of our common stock in which we issued an aggregate of 13,800,000 shares of our common stock (inclusive of 1,800,000 shares pursuant to the underwriters’ overallotment option) at a price of \$15.00 per share. We received net proceeds of \$188.5 million from the IPO, after deducting underwriting discounts and commissions of \$14.5 million and offering costs of \$4.0 million.

Since our inception, we have incurred net losses each year and we expect to continue to incur significant and increasing losses for the foreseeable future as we continue to advance our platforms, programs and product candidates, and as we transition to operating as a public company. Our net losses may fluctuate significantly from period to period, depending on the timing of expenditures on our research and development activities. Our net losses were \$46.6 million and \$28.1 million for the nine months ended September 30,

2021 and 2020, respectively. As of September 30, 2021, we had an accumulated deficit of \$129.4 million and cash, cash equivalents and investments in marketable securities of \$280.5 million. Our net losses resulted primarily from our research and development programs, and to a lesser extent, general and administrative costs associated with our operations. Historically, we have funded our operations primarily from the sale and issuance of our equity securities.

### ***COVID-19***

The global COVID-19 pandemic continues to evolve. Potential impacts to our business include disruptions or restrictions on our employees' ability to effectively conduct their work. In addition, some of our suppliers of certain lab materials, or service providers used in the performance of our research activities, are located in the areas impacted by COVID-19, which could limit our ability to achieve planned progress. COVID-19 could continue to adversely affect the economies and financial markets, resulting in a prolonged economic downturn or a period of high inflation rates that could affect our financing prospects. To date, we have experienced modest delays in the progress of our research and development activities, primarily due to extended lead times at certain suppliers and temporary and partial shutdowns at certain academic institutions as a result of statewide stay-at-home orders. However, these stay-at-home orders have largely terminated and operations have since resumed. We continue to monitor and assess the effects of the COVID-19 pandemic on our business, but the ultimate impact of the COVID-19 outbreak or a similar health epidemic is highly uncertain and subject to change. For additional details, see Part II, Item 1A. "Risk Factors."

## **Components of Operating Results**

### ***Research and Development***

Research and development activities account for a significant portion of our operating expenses. Research and development expenses relate primarily to discovery and development of our platforms, programs and product candidates, and are recognized as incurred. Internal research and development costs include, among others, personnel-related costs (including salaries, benefits and stock-based compensation for employees engaged in research and development functions), laboratory supplies and other non-capital equipment utilized for in-house research, allocated facilities and overhead costs. External research and development expenses include, among others, amounts incurred to clinical research organizations ("CROs") that conduct research and development activities on our behalf, consulting fees and amounts owed under licensing agreements. We do not allocate our costs by platform, program or product candidate, as a significant amount of research and development expenses include internal costs, which are deployed across multiple platforms, programs, product candidates and activities.

Amounts recorded for external goods or services incurred for research and development activities that have not yet been invoiced often represent estimates. We do not currently have projects that require estimates for percentage of completion and we record estimates for amounts due but not yet invoiced based on input from external service providers.

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 platforms, programs and product candidates and progressing through preclinical and clinical product development stages. The process of conducting the necessary research to advance to the clinical stage and ultimately obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

### ***General and Administrative***

General and administrative expenses consist of personnel-related costs (including salaries, benefits and stock-based compensation for our employees in executive, finance and other administrative functions), legal fees, professional fees incurred for accounting, audit, and tax services, recruiting costs, and facility costs not otherwise included in research and development expenses. Legal fees include those related to corporate and intellectual property related matters.

We expect that our general and administrative expenses will increase for the foreseeable future to support our continued research and development activities, general operations, future business development opportunities and professional fees. In addition, we expect to incur additional expenses associated with operating as a public company, including legal, accounting, insurance, exchange listing, SEC compliance, and investor relations costs.

### ***Interest Income***

Interest income primarily consists of interest earned on our cash, cash equivalents and investment balances.

### Change in Fair Value of Convertible Preferred Stock Tranche Liability

The change in fair value of our convertible preferred stock tranche liability fluctuates based on remeasurements at each reporting period. Our convertible preferred stock tranche liability stemmed from our obligation to issue additional shares to investors upon the second and third closings of our Series B convertible preferred stock. Until settlement, fluctuations in the fair value of a convertible preferred stock tranche liability are based on the remeasurement at each reporting period. Our convertible preferred stock tranche liability was settled on the second and third closings of our Series B convertible preferred stock financing in March and August 2020, respectively.

### Other Income (Expense), Net

Other income (expense), net primarily consists of sublease income for a portion of our facilities in South San Francisco during 2020.

## Results of Operations

### Comparison of the Three Months Ended September 30, 2021 and 2020

The following table summarizes our results of operations for the periods presented:

	Three Months Ended September 30,		\$ Change	% Change
	2021	2020		
	(In thousands)			
Operating expenses:				
Research and development	\$ 12,944	\$ 8,480	\$ 4,464	53%
General and administrative	5,356	1,972	3,384	172%
Total operating expenses	18,300	10,452	7,848	75%
Loss from operations	(18,300)	(10,452)	(7,848)	75%
Other income (expense), net:				
Interest income	23	7	16	229%
Change in fair value of convertible preferred stock tranche liability	—	151	(151)	100%
Other income (expense), net	15	(1)	16	1,600%
Total other income (expense), net	38	157	(119)	76%
Net loss	<u>\$ (18,262)</u>	<u>\$ (10,295)</u>	<u>\$ (7,967)</u>	<u>77%</u>

### Research and Development Expenses

The following table summarizes our research and development expenses for the periods presented:

	Three Months Ended September 30,		\$ Change	% Change
	2021	2020		
	(In thousands)			
Facility and laboratory costs	\$ 4,779	\$ 3,357	\$ 1,422	42%
Personnel-related costs	4,764	2,844	1,920	68%
External costs	3,301	2,140	1,161	54%
Other research and development expenses	100	139	(39)	28%
Total research and development expenses	<u>\$ 12,944</u>	<u>\$ 8,480</u>	<u>\$ 4,464</u>	<u>53%</u>

Research and development expenses were \$12.9 million and \$8.5 million for the three months ended September 30, 2021 and 2020, respectively. The change of \$4.5 million, or 53%, was primarily due to:

- an increase of \$1.4 million in facility and laboratory costs, including laboratory supplies and materials and other allocated costs, as we ramped up our research and development operations;
- an increase of \$1.9 million in personnel-related costs, including stock-based compensation, primarily due to growth in the number of our research and development employees as we expanded our research and development capabilities; and

- an increase of \$1.2 million in external costs, including amounts paid to CROs for research and development activities, consulting fees, preclinical studies and other external research expenses as we progressed development of our programs.

### General and Administrative

General and administrative expenses were \$5.4 million and \$1.9 million for the three months ended September 30, 2021 and 2020, respectively. The change of \$3.4 million, or 172%, was primarily due to a \$1.4 million increase in personnel-related expenses, including stock-based compensation, as a result of higher headcount as we grew our operations, a \$0.8 million increase in professional service expenses, as we prepared to become a public company and completed our IPO of our common stock, and a \$0.6 million increase in facility and related costs, as we leased additional space in 2021.

### Interest Income

Interest income was \$23,000 and \$7,000 for the three months ended September 30, 2021 and 2020, respectively. The increase of \$16,000, or 229%, was primarily due to a change in our investment portfolio mix.

### Change in Fair Value of Convertible Preferred Stock Tranche Liability

The change in fair value of our convertible preferred stock tranche liability for the three months ended September 30, 2020 was attributable to changes in the fair value of the underlying Series B convertible preferred stock. There was no similar expense for the three months ended September 30, 2021 as our convertible preferred stock tranche liability was settled in 2020.

### Comparison of the Nine Months Ended September 30, 2021 and 2020

The following table summarizes our results of operations for the periods presented:

	Nine Months Ended September 30,		\$ Change	% Change
	2021	2020		
	(In thousands)			
Operating expenses:				
Research and development	\$ 33,440	\$ 22,735	\$ 10,705	47%
General and administrative	13,202	5,855	7,347	125%
Total operating expenses	46,642	28,590	18,052	63%
Loss from operations	(46,642)	(28,590)	(18,052)	63%
Other income (expense), net:				
Interest income	41	82	(41)	50%
Change in fair value of convertible preferred stock tranche liability	—	75	(75)	100%
Other income (expense), net	31	356	(325)	91%
Total other income (expense), net	72	513	(441)	86%
Net loss	\$ (46,570)	\$ (28,077)	\$ (18,493)	66%

### Research and Development Expenses

The following table summarizes our research and development expenses for the periods presented:

	Nine Months Ended September 30,		\$ Change	% Change
	2021	2020		
	(In thousands)			
Facility and laboratory costs	\$ 13,495	\$ 8,487	\$ 5,008	59%
Personnel-related costs	11,649	7,750	3,899	50%
External costs	8,032	6,204	1,828	29%
Other research and development expenses	264	294	(30)	10%
Total research and development expenses	\$ 33,440	\$ 22,735	\$ 10,705	47%



Research and development expenses were \$33.4 million and \$22.7 million for the nine months ended September 30, 2021 and 2020, respectively. The change of \$10.7 million, or 47%, was primarily due to:

- an increase of \$5.0 million in facility and laboratory costs, including laboratory supplies and materials and other allocated costs, as we ramped up our research and development operations;
- an increase of \$3.9 million in personnel-related costs, including stock-based compensation, primarily due to growth in the number of our research and development employees as we expanded our research and development capabilities; and
- an increase of \$1.8 million in external costs, including amounts paid to CROs for research and development activities, consulting fees, preclinical studies and other external research expenses as we progressed development of our programs.

#### ***General and Administrative***

General and administrative expenses were \$13.2 million and \$5.9 million for the nine months ended September 30, 2021 and 2020, respectively. The change of \$7.3 million, or 125%, was primarily due to a \$2.9 million increase in personnel-related expenses, including stock-based compensation, as a result of higher headcount as we grew our operations, a \$2.6 million increase in professional service expenses as we prepared to become a public company and completed our IPO of our common stock and a \$0.9 million increase in facility and related costs, as we leased additional space in 2021.

#### ***Interest Income***

Interest income was \$41,000 and \$82,000 for the nine months ended September 30, 2021 and 2020, respectively. The decrease of \$41,000, or 50%, was primarily due to a change in our investment portfolio mix.

#### ***Change in Fair Value of Convertible Preferred Stock Tranche Liability***

The change in fair value of our convertible preferred stock tranche liability for the nine months ended September 30, 2020 was attributable to changes in the fair value of the underlying Series B convertible preferred stock. There was no similar expense for the nine months ended September 30, 2021 as our convertible preferred stock tranche liability was settled in 2020.

#### ***Other Income (Expense), Net***

Other income (expense), net of \$0.4 million for the nine months ended September 30, 2020 was primarily related to sublease income. Our sublease agreements expired during 2020.

### **Liquidity and Capital Resources**

#### ***Sources of Liquidity***

Since our inception, we have not generated any revenue and we have incurred significant net losses and negative cash flows from operations. From our inception through September 30, 2021, we have funded our operations primarily from the sale and issuance of our equity securities. In connection with our IPO, we issued and sold 13,800,000 shares of our common stock at a price of \$15.00 per share for net proceeds of approximately \$188.5 million. As of September 30, 2021, we had cash, cash equivalents and investments in marketable securities of \$280.5 million and an accumulated deficit of \$129.4 million.

#### ***Funding Requirements***

We expect our expenses and operating losses will increase substantially over the foreseeable future. The expected increase in expenses will be driven in large part by our ongoing activities, if and as we:

- continue to advance our Gene Therapy, Cellular Regeneration and Precision Medicine platforms;
- continue preclinical development of our product candidates and initiate additional preclinical studies;
- commence clinical trials of our product candidates;
- build out our manufacturing facilities and establish our manufacturing capabilities, including developing our contract development and manufacturing relationships;
- acquire and license technologies aligned with our Gene Therapy, Cellular Regeneration and Precision Medicine platforms;

- seek regulatory approval of our product candidates that successfully complete clinical trials;
- expand our operational, financial, and management systems and increase personnel, including personnel to support our preclinical and clinical development, manufacturing, and future commercialization efforts;
- continue to develop, grow, perfect, enforce and defend our intellectual property portfolio; and
- incur additional legal, accounting, and other expenses in operating our business, including the additional costs associated with operating as a public company.

Based on our current operating plan, we believe that our existing cash, cash equivalents and investments in marketable securities will be sufficient to meet our working capital and capital expenditure needs through at least the next twelve months following the date of this Quarterly Report on Form 10-Q.

In order to complete the development of our product candidates and commercialize our product candidates, if approved, we will require substantial additional funding. Until such time as we can generate significant revenue from sales of our product candidates, if ever, we expect to finance our operations through public or private equity offerings or debt financings or other capital sources, which may include strategic collaborations or other arrangements with third parties, or other sources of financing. We may not be able to raise additional capital on terms acceptable to us or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, including restricting our operations and limiting our ability to incur liens, issue additional debt, pay dividends, repurchase our common stock, make certain investments or engage in merger, consolidation, licensing or asset sale transactions. If we raise funds through strategic collaborations, partnerships and other similar arrangements with third parties, we may be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. If we are unable to raise additional capital on acceptable terms when needed, our business, results of operations, and financial condition would be adversely affected.

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. 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, future commercialization efforts or other operations. Because of the numerous risks and uncertainties associated with research, product development and commercialization of product candidates, we are unable to predict the timing or amount of our working capital requirements or when or if we will be able to achieve or maintain profitability.

### **Cash Flows**

The following table summarizes our cash flows for each of the periods indicated:

	Nine Months Ended September 30,	
	2021	2020
	(In thousands)	
Net cash (used in) provided by:		
Operating activities	\$ (44,465)	\$ (25,046)
Investing activities	(175,984)	1,167
Financing activities	208,991	61,344
Net change in cash, cash equivalents and restricted cash	<u>\$ (11,458)</u>	<u>\$ 37,465</u>

### **Operating Activities**

Net cash used in operating activities for the nine months ended September 30, 2021 was \$44.5 million, which consisted primarily of a net loss of \$46.6 million and a net change in net operating assets and liabilities of \$2.7 million, partially offset by \$4.8 million in non-cash charges. The non-cash charges primarily consisted of depreciation and amortization of \$2.2 million, stock-based compensation of \$1.9 million and non-cash operating lease expense of \$0.7 million. The change in net operating assets and liabilities was primarily due to an increase in other non-current assets of \$3.1 million related to a security deposit for a lease entered into in February 2021, an increase in prepaid expenses and other current assets of \$3.0 million, and a decrease in operating lease liabilities of \$1.0 million, partially offset by an increase in accounts payable and accrued expenses and other current liabilities of \$4.2 million.

Net cash used in operating activities for the nine months ended September 30, 2020 was \$25.0 million, which consisted primarily of a net loss of \$28.1 million, partially offset by non-cash charges of \$2.3 million and a net change in net operating assets and liabilities of \$0.7 million. The non-cash charges primarily consisted of depreciation and amortization of \$1.8 million and stock-based compensation of \$0.5 million. The change in net operating assets and liabilities was primarily due to an increase in accounts payable and accrued expenses and other current liabilities of \$1.2 million, a decrease in prepaid expenses and other current assets of \$0.3 million, partially offset by a decrease in deferred rent and other lease liabilities of \$0.6 million and an increase in other non-current assets of \$0.2 million.

### ***Investing Activities***

Net cash used in investing activities for the nine months ended September 30, 2021 was \$176.0 million, which consisted of purchases of marketable securities of \$163.4 million and purchases of property and equipment of \$12.6 million primarily related to our manufacturing and office space in the San Francisco Bay Area.

Net cash provided by investing activities for the nine months ended September 30, 2020 was \$1.2 million, which consisted of proceeds from maturities of marketable securities of \$2.8 million, partially offset by purchases of property and equipment of \$1.6 million.

### ***Financing Activities***

Net cash provided by financing activities for the nine months ended September 30, 2021 was \$209.0 million, which primarily consisted of proceeds from our initial public offering, net of issuance costs, of \$188.6 million, net proceeds received from the sale and issuance of our Series C convertible preferred stock of \$20.0 million, and proceeds from the exercise of stock options of \$0.3 million.

Net cash provided by financing activities for the nine months ended September 30, 2020 was \$61.3 million, which consisted primarily of net proceeds received from the sale and issuance of our Series B convertible preferred stock.

### **Contractual Obligations and Other Commitments**

As of September 30, 2021, there have been no material changes from the contractual obligations and commitments previously disclosed in our Prospectus.

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

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

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

Our management's discussion and analysis of the financial condition and results of operations is based on our financial statements, which have been prepared in accordance with the U.S. generally accepted accounting principles, or GAAP. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, expenses and related disclosures. Our estimates are based on historical experience and on various other factors that 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 nine months ended September 30, 2021, there were no material changes to our critical accounting policies or in the methodology used for estimates from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in the Prospectus, except as follows. The determination of the fair value of our common stock is used in estimating the fair value of stock-based awards at grant date. Prior to the IPO, our common stock was not publicly traded, therefore we estimated the fair value of our common stock as discussed in our Prospectus. Following our IPO, the closing sale price per share of our common stock as reported on the Nasdaq Global Select Market on the date of grant will be used to determine the exercise price per share of our share-based awards to purchase common stock.

### **Recent Accounting Pronouncements**

See Note 2 to our unaudited interim condensed financial statements for more information about recent accounting pronouncements, the timing of their adoption, and our assessment, to the extent we have made one yet, of their potential impact on our financial condition or results of operations.

### **Emerging Growth Company and Smaller Reporting Company Status**

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (“JOBS Act”). We will remain an emerging growth company until the earliest to occur of: (i) the last day of the fiscal year in which we have more than \$1.07 billion in annual revenue; (ii) the date we qualify as a “large accelerated filer,” with at least \$700 million of equity securities held by non-affiliates; (iii) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period; and (iv) December 31, 2026.

The JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards, delaying the adoption of these accounting standards until they would apply to private companies. We have elected to use the extended transition period to enable us to comply with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date on which we (i) are no longer an emerging growth company and (ii) affirmatively and irrevocably opt out of the extended transition period provided by the JOBS Act. 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 are also a smaller reporting company, meaning that the market value of our stock held by non-affiliates is less than \$700 million and our annual revenue was less than \$100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company if either (i) the market value of our stock held by non-affiliates is less than \$250 million or (ii) our annual revenue is less than \$100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than \$700 million.

If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth company’s smaller reporting companies have reduced disclosure obligations regarding executive compensation.

### **Item 3. Quantitative and Qualitative Disclosures About Market Risk**

We are a smaller reporting company, as defined by Rule 12b-2 under the Securities and Exchange Act of 1934 and in Item 10(f)(1) of Regulation S-K, and are not required to provide the information under this item.

### **Item 4. Controls and Procedures**

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

Our management, with the participation and supervision of our Chief Executive Officer and our Chief Financial Officer, have evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that, as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls and procedures are effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. We believe that a control system, no matter how well designed and operated, cannot provide absolute assurance that the objectives of the control system are met, and no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within a company have been detected.

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

There were no changes in our internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) during the quarter ended September 30, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**Item 1. Legal Proceedings**

From time to time, we may become involved in various legal proceedings that arise in the ordinary course of our business. We are not currently a party to any litigation or legal proceedings that are likely to have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources, and other factors.

**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 quarterly report and in our other public filings in evaluating our business. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline, and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations and the market price of our common stock.*

**Risk Factors Summary**

*Our ability to execute on our business strategy is subject to a number of risks and uncertainties, including those outside of our control, that could cause our actual results to be harmed, including risks regarding the following:*

- We are early in our development efforts, with a limited operating history, have not initiated or completed any clinical trials, and have no products approved for commercial sale, which may make it difficult for you to evaluate our current business and likelihood of success and future viability.
- We have not generated any product revenue to date, have incurred significant net losses since our inception, and expect to continue to incur significant net losses for the foreseeable future.
- Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve several objectives relating to the discovery, development and commercialization of our product candidates, if approved.
- 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.
- Our operations and financial results could be adversely impacted by the effects of the COVID-19 pandemic in the United States and the rest of the world.
- Our product candidates are in the early stages of development and we have no products approved for commercial sale. If we are unable to successfully develop, receive regulatory approval for, manufacture and commercialize our product candidates, or successfully develop any other product candidates, or experience significant delays in doing so, our business will be harmed.
- We intend to identify and develop gene therapy product candidates based on novel technology, and because the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.
- The mechanisms of action of our product candidates are unproven, and we do not know whether we will be able to develop any drug of commercial value.
- Preclinical and clinical drug development involves a lengthy and expensive process with an uncertain outcome. The clinical trials of our product candidates may not demonstrate safety and efficacy to the satisfaction of the FDA, EMA or other comparable foreign regulatory authorities or otherwise produce positive results and the results of preclinical studies and early clinical trials may not be predictive of future results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.
- Our product candidates may cause serious adverse events, toxicities or other undesirable side effects when used alone or in combination with other approved products or investigational new drugs that may result in a safety profile that could prevent regulatory approval, prevent market acceptance, limit their commercial potential or result in significant negative consequences.

- Due to the significant resources required for the development of product candidates, and depending on our ability to access capital, we must prioritize development of certain programs and product candidates. Moreover, we may expend our limited resources on programs or 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.
- We are in the process of building out a manufacturing facility to support future production of certain of our product candidates. We have no experience in manufacturing, and there can be no assurance that we will be able to complete our manufacturing facility or, if completed, we will be able to successfully manufacture product candidates.
- The regulatory approval processes of the FDA, EMA and other comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable. If we are ultimately unable to obtain regulatory approval of our product candidates, we will be unable to generate product revenue and our business will be substantially harmed.
- If we are unable to obtain, maintain, protect, defend and enforce patent and other intellectual property coverage for our technology and product candidates, our competitors could develop and commercialize technology and product candidates similar or identical to ours, and our ability to commercialize our technology and product candidates may be adversely affected.
- Our commercial success depends significantly on our ability to operate without infringing, misappropriating or otherwise violating the patents and other intellectual property and proprietary rights of third parties. Claims by third parties that we infringe, misappropriate or otherwise violate their intellectual property or proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts, and could have a material adverse effect on the success of our business.
- We rely on third parties to conduct our preclinical studies, and plan to rely on third parties to conduct clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research and studies or to comply with applicable regulatory requirements, which may harm our business.

### **Risks Related to Our Financial Position, Need for Additional Capital and Limited Operating History**

***We are early in our development efforts, with a limited operating history, have not initiated or completed any clinical trials, and have no products approved for commercial sale, which may make it difficult for you to evaluate our current business and likelihood of success and future viability.***

We are a preclinical stage biotechnology company with a limited operating history upon which you can evaluate our business and prospects. We commenced operations in 2016, have not initiated or completed any clinical trials, have no products approved for commercial sale and have not generated any revenue. We are developing therapies that address the underlying drivers of heart disease, which is an unproven and highly uncertain undertaking and involves a substantial degree of risk. All of our product candidates are still in preclinical development and have never been tested in humans. Since our inception in 2016, we have devoted substantially all of our focus and financial resources to developing our gene therapy, cellular regeneration and precision medicine platforms, identifying and developing product candidates, conducting preclinical studies, acquiring technology, organizing and recruiting management and technical staff, business planning, establishing our intellectual property portfolio, raising capital, and providing general and administrative support for these operations.

We have not yet demonstrated our ability to successfully initiate and complete any clinical trials, obtain marketing approvals, manufacture a clinical- or commercial-scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. As a result, it may be more difficult for investors to accurately predict our likelihood of success and viability than it could be if we had a longer operating history.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors and risks frequently experienced by early-stage biotechnology companies in rapidly evolving fields. We also may need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We have not yet demonstrated an ability to successfully overcome such risks and difficulties, or to make such a transition. If we do not adequately address these risks and difficulties or successfully make such a transition, our business will suffer.

***We have not generated any product revenue to date, have incurred significant net losses since our inception, and expect to continue to incur significant net losses for the foreseeable future.***

We have incurred significant net losses since our inception, have not generated any product revenue to date and, up until the date of our IPO in July 2021, have financed our operations through private placements of our convertible preferred stock. Our net loss was \$46.6 million for the nine months ended September 30, 2021. As of September 30, 2021, we had an accumulated deficit of \$129.4

million. Substantially all of our losses have resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We are still in the early stages of development of our product candidates and have not yet initiated or completed any clinical trials. Our product candidates will require substantial additional development time and resources before we will be able to apply for regulatory approvals and, if approved, begin generating revenue from product sales. As a result, we expect that it will be several years, if ever, before we receive approval to commercialize a product and generate revenue from product sales. Even if we succeed in receiving marketing approval for and commercializing one or more of our product candidates, we expect that we will continue to incur substantial research and development and other expenses in order to discover, develop and market additional potential products.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter such that a period-to-period comparison of our results of operations may not be a good indication of our future performance, particularly since we expect our expenses to increase if and when our product candidates progress through clinical development as product candidates in later stages of clinical development generally have higher development costs than those in earlier stages, primarily due to the increased size and duration of later-stage clinical trials. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our working capital, our ability to fund the development of our product candidates and our ability to achieve and maintain profitability and the performance of our stock.

***Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve several objectives relating to the discovery, development and commercialization of our product candidates, if approved.***

We rely on our multi-modality drug discovery platforms to identify and develop product candidates. Our business depends entirely on the success of these platforms and the successful development, regulatory approval, manufacturing and commercialization of product candidates that we discover with these platforms. Our ability to generate revenue and achieve profitability depends significantly on our ability, or any future collaborator's ability, to achieve several objectives, including:

- successful and timely completion of preclinical and clinical development of product candidates and programs in our Gene Therapy, Cellular Regeneration and Precision Medicine platforms, and our other future product candidates and programs;
- obtaining regulatory approval to commence clinical trials of our product candidates;
- establishing and maintaining relationships with contract research organizations (“CROs”) and clinical sites for the clinical development of our product candidates and any other future product candidates;
- the initiation and successful patient enrollment and completion of clinical trials on a timely basis;
- acceptable frequency and severity of adverse events in the clinical trials;
- the efficacy and safety profiles that are satisfactory to the U.S. Food and Drug Administration (“FDA”) or any comparable foreign regulatory authority for marketing approval;
- timely receipt of marketing approvals from applicable regulatory authorities for any product candidates for which we successfully complete clinical development;
- complying with any required post-marketing approval commitments to applicable regulatory authorities;
- establishing and operating a manufacturing facility and developing an efficient and scalable manufacturing process for our product candidates;
- establishing and maintaining commercially viable supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and meet the market demand for our product candidates, if approved;
- successful commercial launch following any marketing approval, including the development of a commercial infrastructure, whether in-house or with one or more collaborators;
- successful outputs from our capsid engineering and promotor and regulator elements efforts;
- a continued acceptable safety profile following any marketing approval of our product candidates;
- commercial acceptance of our product candidates by patients, the medical community and third-party payors;
- satisfying any required post-marketing approval commitments to applicable regulatory authorities;
- identifying, assessing and developing new product candidates;

- obtaining, maintaining, and expanding patent and other intellectual property protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting and enforcing our rights in our intellectual property portfolio;
- defending against third-party infringement, misappropriation, or other claims, if any;
- entering into, on favorable terms, any collaboration, licensing or other arrangements that may be necessary or desirable to develop, manufacture or commercialize our product candidates;
- obtaining coverage and adequate reimbursement by third-party payors for our products and patients' willingness to pay in the absence of such coverage and adequate reimbursement;
- obtaining additional funding to develop, manufacture and commercialize our product candidates;
- addressing any competing therapies and technological and market developments;
- managing costs, including any unforeseen costs, that we may incur as a result of nonclinical study or clinical trial delays due to the effects of the COVID-19 pandemic or other causes; and
- attracting, hiring and retaining qualified and key personnel including clinical, scientific, management and administrative personnel.

We may never be successful in achieving our objectives and, even if we are, may never generate revenue that is significant or large enough to achieve profitability. 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 maintain or further our research and development efforts, raise additional necessary capital, grow our business and continue our operations.

We may also experience delays in establishing our manufacturing facility, developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from initiating and completing our planned clinical trials or commercializing our product candidates on a timely or profitable basis, if at all.

***We 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.***

As of September 30, 2021, we had \$280.5 million in cash, cash equivalents and investments in marketable securities. We expect our current cash, cash equivalents and investments in marketable securities will be sufficient to fund our current operating plan for operations through at least the next twelve months from the date of this Quarterly Report on Form 10-Q. Our estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

Developing pharmaceutical 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 consumed substantial amounts of cash since inception, and we expect our expenses to increase in connection with our ongoing activities, particularly as we initiate and conduct clinical trials of, and seek marketing approval for, our programs as well as develop our proprietary drug discovery platforms. Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with sales, marketing, manufacturing and distribution activities. Our expenses could increase beyond expectations if we are required by the FDA, EMA or other regulatory agencies to perform clinical trials or preclinical studies in addition to those that we currently anticipate. Other unanticipated costs may also arise. Because the design and outcome of our planned and anticipated preclinical studies and clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of resources and funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop. We are not permitted to market or promote any product candidate before we receive marketing approval from the FDA. We also expect to incur costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in order to continue our operations.

Our future capital requirements will depend on many factors, including, but not limited to:

- the scope, progress, results and costs of researching, developing and testing our product candidates including conducting preclinical studies and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates or any future candidates;



- the number and characteristics of other product candidates that we pursue or acquire;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of establishing and operating our own manufacturing facility;
- the costs of manufacturing commercial-grade products and sufficient inventory to support commercial launch;
- the cost of commercialization activities, include the cost of building a sales force in anticipation of product commercialization and distribution costs;
- the revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the cost and timing of hiring new employees to support our continued growth;
- the costs of preparing, filing and prosecuting patent applications, maintaining, protecting and enforcing our intellectual property rights and defending intellectual property-related claims;
- the effect of competing products that may limit market penetration of our products;
- the ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies;
- the timing, receipt and amount of sales of, or milestone payments related to or royalties on, our current or future product candidates, if any;
- our need to implement additional internal systems and infrastructure, including financial and reporting systems;
- the compliance and administrative costs associated with being a public company; and
- the extent to which we acquire or invest in businesses, products, or technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

A change in the outcome of any of these or other factors with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate, and many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

We may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. Attempting to secure additional financing may divert our management from our day-to-day activities, which may adversely affect our ability to develop our product candidates. Our failure to raise capital as and when needed or on acceptable terms would have a negative impact on our financial condition and our ability to pursue our business strategy, and we may have to delay, reduce the scope of, suspend or eliminate one or more of our platforms, programs, planned clinical trials or future commercialization efforts.

***Our operations and financial results could be adversely impacted by the effects of the COVID-19 pandemic in the United States and the rest of the world.***

In March 2020, the World Health Organization declared the novel coronavirus disease (“COVID-19”) outbreak a global pandemic. To limit the spread of COVID-19, governments have taken various actions including the issuance of stay-at-home orders and physical distancing guidelines. Accordingly, businesses have adjusted, reduced or suspended operating activities. Beginning in March 2020, the majority of our workforce began working from home. Disruptions caused by the COVID-19 pandemic, including the effects of the stay-at-home orders and work-from-home policies, have impacted productivity, have resulted in increased operational expenses, certain adjustments to our operations, delays in our development efforts, and delays in certain supply chain activities. We may experience further disruptions as a result of COVID-19 that could severely impact our business, including:

- interruptions, difficulties or delays arising in our existing operations and company culture as a result of all of our employees working remotely, including those hired during the COVID-19 pandemic;
- delays in the build out of our manufacturing facility;

- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;
- delays or difficulties in enrolling and retaining patients in clinical trials and incurrence of additional costs as a result of preclinical study and clinical trial delays and adjustments;
- challenges related to ongoing and increased operational expenses related to the COVID-19 pandemic;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays, difficulties or increased costs to comply with COVID-19 protocols at our leased facilities and clinical sites;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals that may serve as our potential clinical trial sites and hospital staff supporting the conduct of clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others;
- limitations in resources that would otherwise be focused on the conduct of our business or our clinical trials, including because of sickness or the desire to avoid contact with large groups of people or as a result of government-imposed “Stay-at-Home” orders or similar working restrictions;
- delays in preclinical and clinical sites receiving the supplies and materials needed to conduct our planned clinical trials;
- interruption in global shipping that may affect the transport of clinical trial materials, such as investigational drug product to be used in our clinical trials;
- changes in regulations as part of a response to the COVID-19 pandemic which may require us to change the ways in which our clinical trials are conducted, or to discontinue clinical trials altogether, or which may result in unexpected costs;
- delays in necessary interactions with regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government or contractor personnel;
- refusal of the FDA to accept data from clinical trials in affected geographies outside the United States;
- increased competition for CROs, contract development and manufacturing organizations (“CDMOs”), suppliers and vendors; and
- delays in collecting, receiving and analyzing data from patients enrolled in our clinical trials due to limited staff at potential clinical trial sites, limitation or suspension of on-site visits by patients, or patients’ reluctance to visit the clinical trial sites during the pandemic.

We will continue to assess the impact that the COVID-19 pandemic may have on our ability to effectively conduct our business operations as planned and there can be no assurance that we will be able to avoid a material impact on our business from the spread of COVID-19 or its consequences, including disruption to our business and downturns in business sentiment generally or in our industry.

Additionally, certain third parties with whom we engage or may engage, including our collaborators, CROs, third-party manufacturers, suppliers, clinical trial sites, regulators and other third parties with whom we conduct business have similarly adjusted their operations and are assessing their capacity in light of the COVID-19 pandemic. If these third parties experience shutdowns or continued business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. For example, as a result of the COVID-19 pandemic, we experienced delays in the procurement of materials needed to conduct our preclinical studies. Additionally, certain preclinical studies are conducted by CROs, some of which were delayed as a result of the COVID-19 pandemic and which could be discontinued or further delayed as the pandemic continues. Research and development expenses and general and administrative expenses may vary significantly if there is an increased impact from COVID-19 on the costs and timing associated with the conduct of our business. As we continue to actively advance our programs, we are assessing the impact of the COVID-19 pandemic, our expected timelines and costs on an ongoing basis.

Three vaccines for COVID-19 have been granted Emergency Use Authorization (“EUA”) by the FDA, and more are likely to be authorized in the coming months. On August 23, 2021, the FDA approved the first COVID-19 vaccine, which will continue to be available under an EUA as well. The resultant demand for vaccines and potential for manufacturing facilities and materials to be commandeered under the Defense Production Act of 1950, or equivalent foreign legislation, has made it more difficult to obtain materials and manufacturing slots for our product candidates needed for our preclinical studies and clinical trials, which could lead to delays in these studies and trials.

The extent to which the COVID-19 pandemic impacts our business will depend on future developments such as the rate of the spread of the disease, new travel restrictions and social distancing in the United States and other countries, business closures or business disruptions, supply of and continued demand for vaccines, the continued effectiveness of the vaccines, and the effectiveness of actions taken in the United States and other countries to contain and treat the disease and to address its impact, including on financial markets or otherwise. Further, a lack of coordinated response on risk mitigation and continued vaccination deployment with respect to the COVID-19 pandemic could result in significant increases to the duration and severity of the pandemic and could have a corresponding negative impact on our business. While the extent of the impact of the current COVID-19 pandemic on our business and financial results is uncertain, a continued and prolonged public health crisis such as the COVID-19 pandemic could have a material negative impact on our business, financial condition and operating results.

To the extent the COVID-19 pandemic adversely affects our business, financial condition and operating results, it may also have the effect of heightening many of the risks described in this “Risk Factors” section.

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

Until such time, if ever, as we can generate substantial revenues, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources, which may dilute our stockholders or restrict our operating activities. We do not have any committed external source of funds. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, maintaining certain leverage ratios, making acquisitions, engaging in acquisition, merger or collaboration transactions, selling or licensing our assets, making capital expenditures, redeeming our stock, making certain investments, declaring dividends or encumbering our assets to secure future indebtedness. Such restrictions could adversely impact our ability to conduct our operations and execute our business plan.

If we raise additional funds through upfront payments or milestone payments pursuant to strategic collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or intellectual property, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

***Our ability to utilize our net operating loss carryforwards and certain other tax attributes to offset future taxable income may be limited.***

Our net operating loss (“NOL”) carryforwards may be unavailable to offset future taxable income because of restrictions under U.S. tax law. Under current law, our federal NOLs generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but is limited to 80% of our current year taxable income. Federal NOLs generated in tax years ending on or prior to December 31, 2017 expire after 20 taxable years. Different restrictions apply under state law. As of December 31, 2020, we had available federal NOL carry forwards of approximately \$78.5 million, of which \$75.4 million do not expire.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (“Code”), if a corporation undergoes an “ownership change” (generally defined as a cumulative change in the corporation’s ownership by “5-percent shareholders” that exceeds 50 percentage points over a rolling three-year period), the corporation’s ability to use its pre-change NOLs and certain other pre-change tax attributes to offset its post-change taxable income may be limited. Similar rules may apply under state tax laws. We have experienced such ownership changes in the past, and we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which are outside our control. As of December 31, 2020, a formal study was conducted and concluded that we experienced an ownership change during 2020. As a result, we have removed \$3.1 million of deferred tax assets related to NOLs and research tax credit carryforwards due to Section 382 limitations. Our ability to utilize our remaining NOLs and certain other tax attributes could be further limited by a future “ownership change” as described above and consequently, we may not be able to utilize a material portion of our NOLs and certain other tax attributes, which could have a material adverse effect on our cash flows and results of operations.

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

*Our product candidates are in the early stages of development and we have no products approved for commercial sale. If we are unable to successfully develop, receive regulatory approval for, manufacture and commercialize our product candidates, or successfully develop any other product candidates, or experience significant delays in doing so, our business will be harmed.*

We are very early in our development efforts. We have not initiated clinical trials for any of our programs. We do not have any products that are approved for commercial sale, and we may never be able to develop or commercialize marketable products. Before we generate any revenue from product sales, each of our programs and product candidates will require additional preclinical and clinical development, expansion of manufacturing capabilities and expertise, regulatory approval, building a commercial organization or successfully outsourcing commercialization, substantial investment and significant marketing efforts. We are not permitted to market or promote any product candidates before we receive approval from the FDA or comparable foreign regulatory authorities, and we may never receive such approval. Because our product candidates are in an early stage of development, there is a high risk of failure and we may never succeed in developing marketable products.

If we do not successfully initiate and complete our planned clinical trials in a timely manner or fail to achieve favorable results from the trial, we may experience significant delays or other issues in advancing our programs. We cannot be certain that our clinical trials will be initiated and completed on time, if at all, or whether our planned clinical strategy will be acceptable to the FDA or comparable foreign regulatory authorities. There is a high failure rate for biopharmaceutical products proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical trials even after achieving promising results in earlier stage clinical trials. Data obtained from preclinical studies and clinical trials are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

Because of the early stage of development of our programs, our ability to eventually generate significant revenues from our product candidates, which we do not expect will occur for several years, if ever, will depend on a number of factors, including:

- the successful and timely completion of our ongoing preclinical studies;
- generating sufficient data to support the initiation or continuation of clinical trials;
- addressing any delays, necessary adjustments and additional costs in preclinical studies and clinical trials resulting from factors related to the COVID-19 pandemic;
- submission of INDs or other regulatory applications for our planned clinical trials and authorizations from regulators to initiate clinical trials;
- contracting with the necessary parties to conduct clinical trials;
- successful enrollment in, and completion of, clinical trials on a timely basis;
- achieving favorable results from clinical trials;
- the timely manufacture of sufficient quantities of a product candidate for use in clinical trials and, if approved, commercialization;
- successful outputs from our capsid engineering and promotor and regulator elements efforts;
- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials and commercialization activities;
- the frequency and severity of adverse events in clinical trials;
- demonstrating efficacy, safety and tolerability profiles that are satisfactory to the FDA, EMA or any comparable foreign regulatory authority for marketing approval;
- the timely receipt of marketing approvals from applicable regulatory authorities;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- operational, technical and clinical development challenges associated with pediatric indications that we may pursue, including challenges associated with recruiting and enrolling eligible pediatric patients in clinical trials;
- additional clinical trials and other requirements imposed by regulatory authorities for expanding drug labeling to include pediatric populations or for approval a pediatric product candidate, including formulation changes, additional bridging studies, manufacturing changes, dosage and administration changes, among others;

- the maintenance of existing or the establishment of new supply arrangements with third-party drug product suppliers and manufacturers for clinical development and, if approved, commercialization;
- maintaining consistent quality, purity, and potency across clinical supplies and commercial supplies for any approved products;
- obtaining and maintaining patent and other intellectual property protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting, enforcing and defending our rights in our intellectual property portfolio;
- our ability to expand into multiple indications;
- the successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile and sustained therapeutic effect of our therapies following any marketing approval;
- the actual market-size, ability to identify patients and the demographics of patients eligible for our product candidates, which may be different than expected;
- commercial acceptance by patients, the medical community and third-party payors, particularly since the product candidates we develop may be novel; and
- our ability to compete with other therapies.

We do not have control over many of these factors, including certain aspects of preclinical and clinical development and the regulatory submission process and potential threats to our intellectual property rights. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

To become and remain profitable, we must develop, obtain approval for and eventually commercialize products candidates, if approved, that generate significant revenue. We do not expect to receive approval of any product candidates for many years and may never succeed in these activities. In addition, it is not uncommon for product candidates to exhibit unforeseen safety issues or inadequate efficacy when tested in humans despite promising results in preclinical animal models, and we may ultimately be unable to demonstrate adequate safety and efficacy of our product candidates to obtain marketing approval. Even if we obtain approval and begin commercializing one or more of our product candidates, we may never generate revenue that is significant or large enough to achieve profitability.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development, manufacturing and other expenditures to develop and market additional product candidates. Our failure to become or remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

Even if we successfully discover and advance product candidates into clinical development, their success will be subject to all of the clinical, regulatory and commercial risks described elsewhere in this “Risk Factors” section. Accordingly, we cannot assure you that we will ever be able to discover, develop, obtain regulatory approval of, manufacture, commercialize or generate significant revenue from any product candidates.

***We intend to identify and develop gene therapy product candidates based on novel technology, and because the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.***

We intend to discover, develop, manufacture, and commercialize gene therapy product candidates for the heart. Our product candidates may use both known capsids, such as AAV9, as well as proprietary capsids developed in-house through our own capsid engineering efforts or licensed from third parties. Furthermore, our product candidates may also use novel heart-specific promoters and we may explore different routes-of-administration involving infusion- or injection-based catheters to support targeted delivery and efficient uptake of gene therapies for the heart. We are also establishing proprietary manufacturing processes for our product candidates. Our future success depends on the successful development of these novel therapeutic approaches.

To date, only three products that utilize AAV-mediated gene transfer have been approved in the United States or Europe, including Novartis Pharmaceuticals’ Zolgensma (developed by AveXis), Roche’s Luxturna (developed by Spark Therapeutics), and uniQure’s Glybera. No AAV-based gene therapies have yet been approved for the heart, much less therapies for the heart using novel

capsids or promoters or delivery methods. It is therefore 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.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear, have changed over time and are subject to further change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the FDA, EMA or comparable foreign regulatory authorities. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Changes in the regulatory authorities' data requirements and risk mitigation methods, including requirements resulting from safety concerns raised by regulatory authorities in clinical programs of unrelated companies in the gene therapy and cardiovascular fields in general, could have a material impact on our clinical development, increase our costs, and delay regulatory approval of our product candidates. Moreover, there is substantial overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research ("CBER") to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review.

Our product candidates will need to meet safety and efficacy standards applicable to any new biologic under the regulatory framework administered by the FDA. In addition to FDA oversight and oversight by institutional review boards ("IRBs"), under guidelines promulgated by the National Institutes of Health ("NIH"), gene therapy clinical trials are also subject to review and oversight 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 public health or the environment. 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. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The same applies in the European Union. The EMA's Committee for Advanced Therapies ("CAT") is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

Approvals by the EMA may not be indicative of what the FDA may require for approval. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approvals necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue and our business, financial condition, results of operations and prospects could be materially harmed.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, EMA, and other regulatory bodies to revise the requirements for the conduct of the clinical studies and approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, EMA, and other regulatory authorities 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 product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, there is heightened risk that the FDA, EMA or other 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. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may conduct or rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

The regulatory review committees and advisory groups described above and the new 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. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. 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.

***The mechanisms of action of our product candidates are unproven, and we do not know whether we will be able to develop any drug of commercial value.***

We have discovered and are developing product candidates that have what we believe are novel mechanisms of action. Because no currently-approved drugs appear to operate via the same biochemical mechanisms as our compounds, we cannot be certain that our product candidates will result in commercially viable drugs that safely and effectively treat the indications for which we intend to develop them. The results we have seen for our compounds in preclinical models may not translate into similar results in humans, and results of early clinical trials in humans may not be predictive of the results of larger clinical trials that may later be conducted with our product candidates. As an example, patients may develop antibodies against the product candidates, or the product candidates may otherwise have a more limited duration of therapeutic effect than anticipated, resulting in decreased efficacy over time, which could delay approval and, if approved, limit the ultimate commercial value. Even if we are successful in developing and receiving regulatory approval for a product candidate for the treatment of a particular disease, we cannot be certain that it will be accepted by prescribers or be reimbursed by insurers or that we will also be able to develop and receive regulatory approval for that or other product candidates for the treatment of other diseases. If we are unable to successfully develop and commercialize our product candidates, our business will be materially harmed.

Moreover, in the event any of our competitors were to develop their own product candidates that have a similar mechanism of action to any of our product candidates, any efficacy or safety concerns identified during the development of such similar product candidates may have an adverse impact on the development of our product candidates. For example, if our competitors' product candidate having a similar mechanism of action as any of our product candidates is shown in clinical trials to give rise to serious safety concerns or have poor efficacy when administered to the target patient population, the FDA or other regulatory bodies may subject our product candidates to increased scrutiny, leading to additional delays in development and potentially decreasing the chance of ultimate approval of our product candidates.

***Preclinical and clinical drug development involves a lengthy and expensive process with an uncertain outcome. The clinical trials of our product candidates may not demonstrate safety and efficacy to the satisfaction of the FDA, EMA or other comparable foreign regulatory authorities or otherwise produce positive results and the results of preclinical studies and early clinical trials may not be predictive of future results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.***

It is impossible to predict when or if any product candidate that we develop will prove effective or safe in humans or will receive marketing approval. Before obtaining marketing approval from the FDA, EMA or other comparable foreign regulatory authorities for the sale of our product candidates, we must complete preclinical development and extensive clinical trials to demonstrate with substantial evidence the safety and efficacy of such product candidates.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and its ultimate outcome is uncertain. We cannot guarantee that any of our clinical trials will be conducted as planned or completed on schedule, or at all. Clinical trials can fail at any stage of testing and failure may result from a multitude of factors, including, among other things, flaws in study design, dose selection issues, placebo effects, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits. The outcome of preclinical studies and early-stage clinical trials may not be predictive of the success of later clinical trials. For example, our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. As a result, we cannot assure you that any clinical trials that we conduct will demonstrate consistent or adequate efficacy and safety to support marketing approval.

To the extent we pursue any pediatric indications or expand any approved drug product labeling to include pediatric populations, we may face additional challenges associated with clinical testing in pediatric populations, which can increase our operational costs, delay regulatory approval and commercialization, or expose us to additional liability. For example, finding qualified clinical sites that have access to sufficient pediatric populations and that are interested in participating in our clinical trials may take additional time than adult indications. There may be fewer eligible patients with the target genetic disorder or heart disease or condition applicable to our product candidate for our planned clinical trials. This may increase the time needed to enroll patients for our planned pediatric clinical trials, increase our clinical development timelines, delay approval for such pediatric indications, and increase our operational costs. We may also be required to modify the formulation or other aspects of the product candidate, as compared to the comparable product candidate intended for adult patient populations, make manufacturing changes, modify route of administration, and conduct additional clinical studies, such as bridging studies and additional safety studies, before we can commence our clinical trials in pediatric populations. Any delays in our planned clinical development activities for pediatric patients could have an adverse effect on our business operations.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials, and we cannot be certain that we will not face similar setbacks. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs. This is particularly true for clinical trials in very rare diseases, such as with certain indications we are pursuing, where the very small patient population makes it difficult or impossible to conduct two traditional, adequate and well-controlled studies, and therefore the FDA or comparable foreign regulatory authorities are often permitted to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or comparable foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Furthermore, the failure of any of our product candidates to demonstrate safety and efficacy in any clinical trial could negatively impact the perception of our other product candidates and/or cause the FDA or comparable regulatory authorities to require additional testing before approving any of our product candidates.

To date, we have not initiated or completed any clinical trials required for the approval of our product candidates. We may experience numerous unforeseen events during, or as a result of, preclinical studies or clinical trials that could delay or prevent receipt of marketing approval or our ability to commercialize our product candidates, including:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials;
- receipt of feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trial observations or results that require us to modify the design of our clinical trials;
- negative or inconclusive clinical trial results that may require us to conduct additional clinical trials or abandon certain drug development programs;
- obtaining approval from one or more IRBs;
- the number of patients required for clinical trials being larger than anticipated, enrollment in these clinical trials being slower than anticipated or participants dropping out of these clinical trials at a higher rate than anticipated;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the suspension or termination of our clinical trials for various reasons, including non-compliance with regulatory requirements or a finding that our product candidates have undesirable side effects or other unexpected characteristics or risks;
- changes to clinical trial protocol;
- clinical sites deviating from trial protocol or dropping out of a trial;
- the cost of clinical trials of our product candidates being greater than anticipated;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates being insufficient or inadequate;



- subjects experiencing severe or unexpected drug-related adverse effects;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice (“cGMPs”), regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practices (“GCP”) or other regulatory requirements;
- third-party contractors not performing data collection or analysis in a timely or accurate manner;
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications;
- regulators revising the requirements for approving our product candidates;
- absence in some countries of established groups with sufficient regulatory expertise for review of AAV gene therapy protocols; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing in a timely manner, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may incur unplanned costs, be delayed in seeking and obtaining marketing approval, if we receive such approval at all, receive more limited or restrictive marketing approval, be subject to additional post-marketing testing requirements or have the drug removed from the market after obtaining marketing approval.

Moreover, in the future, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenues from any of these product candidates will be delayed. Moreover, our product development costs will also increase if we experience delays in preclinical studies or clinical trials or in obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, which could result in increased costs and expenses and/or delays. Any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues.

In addition, many of the factors that cause, or lead to, termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate. Any delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize our product candidates and our competitors may be able to bring products to market before we do, and the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition and prospects significantly.

***Our product candidates may cause serious adverse events, toxicities or other undesirable side effects when used alone or in combination with other approved products or investigational new drugs that may result in a safety profile that could prevent regulatory approval, prevent market acceptance, limit their commercial potential or result in significant negative consequences.***

We are developing novel therapies for the treatment of heart disease. As a result, there is uncertainty as to the safety profile of product candidates we may develop. If our product candidates are associated with undesirable side effects or have unexpected characteristics in preclinical studies or clinical trials we may need to interrupt, delay or abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Treatment-related side effects could also affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. There is no guarantee that our product candidates will not have side effects similar to those seen in other gene therapies or that we will be able to prevent side effects from escalating to an unsafe level for our patients. Any of these occurrences may prevent us from achieving or maintaining market acceptance of the affected product candidate and may harm our business, financial condition and prospects significantly.

Patients in our planned clinical trials may in the future suffer other serious adverse events or other side effects not observed in our preclinical studies or previous clinical trials. Patients treated with our other product candidates may also be undergoing other therapies which can cause side effects or adverse events that are unrelated to our product candidate but may still impact the success of our clinical trials. The inclusion of critically ill patients in our clinical trials may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using or due to the gravity of such patients' illnesses. For example, it is expected that some of the patients enrolled in our clinical trials will die or experience major clinical events either during the course of our clinical trials or after participating in such trials.

If further serious adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to the clinical trials, patients may drop out of our trials, or we may be required to abandon the trials or our development efforts of that product candidate altogether. We, the FDA, EMA, other comparable regulatory authorities or an IRB may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the biotechnology industry that initially showed therapeutic promise in early-stage trials have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the product candidate from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects. Further, if any of our product candidates obtains marketing approval, toxicities associated with such product candidates previously not seen during clinical testing may also develop after such approval and lead to a requirement to conduct additional clinical safety trials, additional contraindications, warnings and precautions being added to the drug label, significant restrictions on the use of the product or the withdrawal of the product from the market. We cannot predict whether our product candidates will cause toxicities in humans that would preclude or lead to the revocation of regulatory approval based on preclinical studies or early stage clinical trials.

***The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and the results of our clinical trials may not satisfy the requirements of the FDA, EMA or other comparable foreign regulatory authorities.***

We will be required to demonstrate with substantial evidence through well-controlled clinical trials that our product candidates are safe and effective for use in a diverse population before we can seek marketing approvals for their commercial sale. Success in preclinical studies and early-stage clinical trials does not mean that future clinical trials will be successful. For instance, we do not know whether any of our product candidates will perform in our current or future preclinical studies or future clinical trials as it has in prior preclinical studies or earlier clinical trials. Product candidates in clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of the FDA, EMA and other comparable foreign regulatory authorities despite having progressed through preclinical studies. Regulatory authorities may also limit the scope of later-stage trials until we have demonstrated satisfactory safety, which could delay regulatory approval, limit the size of the patient population to which we may market our product candidates, or prevent regulatory approval.

In some instances, there can be significant variability in safety and efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial protocols, differences in size and type of the patient populations, differences in and adherence to the dose and dosing regimen and other trial protocols and the rate of dropout among clinical trial participants. Patients treated with our product candidates may also be undergoing other therapies and may be using other approved products or investigational new drugs, which can cause side effects or adverse events that are unrelated to our product candidates. As a result, assessments of efficacy can vary widely for a particular patient, and from patient to patient and site to site within a clinical trial. This subjectivity can increase the uncertainty of, and adversely impact, our clinical trial outcomes.

We do not know whether any clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain approval to market any of our product candidates.

***If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our regulatory submissions or receipt of necessary marketing approvals could be delayed or prevented.***

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials to such trial's conclusion as required by the FDA, EMA or other comparable foreign regulatory authorities. Patient enrollment is a significant factor in the timing of clinical trials. Our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate.

We may experience delays if our clinical trial sites limit their onsite staff or temporarily close as a result of the COVID-19 pandemic. In addition, patients may not be able to visit clinical trial sites for dosing or data collection purposes due to limitations on travel and physical distancing imposed or recommended by federal or state governments or patients' reluctance to visit the clinical trial sites during the pandemic. These factors resulting from the COVID-19 pandemic could delay the anticipated readouts from our clinical trials and our regulatory submissions.

We are developing product candidates for the treatment of heart disease, including for certain indications, such as rare genetic diseases, with limited patient pools from which to draw for clinical trials. We also may encounter difficulties in identifying and enrolling patients with a stage of disease appropriate for our planned clinical trials and monitoring such subjects adequately during and after treatment. The process of finding and diagnosing patients may prove costly. Further, the treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies.

We expect patient enrollment to be affected because our competitors have ongoing clinical trials for programs that are under development for the same indications as our product candidates, and patients who would otherwise be eligible for our clinical trials could instead enroll in clinical trials of our competitors' programs. Patient enrollment for our current or any future clinical trials may be affected by other factors, including:

- size and nature of the patient population;
- challenges associated with recruiting pediatric patient population, including, but not limited to, identifying clinical sites that are qualified to participate in pediatric clinical trials, smaller number of eligible pediatric patients who are interested in participating within the target age groups, or under- diagnosis or challenges associated with diagnosing pediatric populations with the applicable target disease or condition;
- perceived risks and benefits of novel, unproven approaches;
- severity of the disease under investigation;
- availability and efficacy of approved drugs for the disease under investigation;
- patient eligibility criteria for the trial in question as defined in the protocol;
- perceived risks and benefits of the product candidate under study;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved or other product candidates being investigated for the indications we are investigating;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- the activities of key opinion leaders ("KOLs") and patient advocacy groups;
- proximity and availability of clinical trial sites for prospective patients; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion or, because they may have an advanced disease, will not survive the full terms of the clinical trials.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates and jeopardize our ability to obtain marketing approval for the sale of our product candidates. Furthermore,

even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining participation in our clinical trials through the treatment and any follow-up periods.

***Due to the significant resources required for the development of product candidates, and depending on our ability to access capital, we must prioritize development of certain programs and product candidates. Moreover, we may expend our limited resources on programs or 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 product candidates, in particular our product candidates in IND-enabling studies and those that begin clinical trials, we must decide which programs, 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 programs, 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 platforms, programs or 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 programs or product candidates or misread trends in the biotechnology industry, in particular in the field of cardiology, 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 programs, 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 our platforms or 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.

***We face significant competition and if our competitors develop and market technologies or products more rapidly than we do or that are more effective, safer or less expensive than the products we develop, our commercial opportunities will be negatively impacted.***

The biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates. Our competitors have developed, are developing or may develop products, product candidates and processes competitive with our product candidates. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may attempt to develop product candidates.

We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, emerging and start-up companies, universities and other research institutions. We also compete with other organizations to recruit management, scientists and clinical development personnel, which could negatively affect our level of expertise and our ability to execute our business plan. We will also face competition in establishing clinical trial sites, enrolling subjects for clinical trials and in identifying and in-licensing new product candidates.

We expect to face competition from existing products and products in development for each of our programs. We anticipate substantial direct competition from a variety of competitors, including:

- General cardiovascular drug development: Companies known to have approved product and active drug development efforts for cardiovascular disease include but are not limited to AstraZeneca, Bayer, Bristol Myers Squibb, Cytokinetics, Eli Lilly, Johnson & Johnson/Janssen, Maze Therapeutics, Merck, Novartis, and Novo Nordisk;
- Gene Therapy platform: Companies known to be pursuing gene therapy approaches for the heart include but are not limited to 4D Molecular Therapeutics, Bayer, Bristol Myers Squibb, BioMarin Pharmaceutical, DiNAQOR, Nuevoco, Precigen, Renova Therapeutics, Renovacor, Rocket Pharmaceuticals, Sardicor, Stride Bio, and uniQure;
- Cellular Regeneration platform: Companies known to be pursuing approaches to cellular regeneration for the heart include but are not limited to AstraZeneca, Bayer, BioCardia, Cardior Pharmaceuticals, Jaan Biotherapeutics, Khloris Biosciences, Mesoblast, Mogrify, Sana Biotechnologies and Xylocor Therapeutics; and
- Precision Medicine platform: Companies known to be pursuing approaches to drug discovery for the heart using disease models based on iPSC-CMs include but are not limited to DiNAQOR and Tara Biosystems.

Many of these current and potential competitors have significantly greater financial, manufacturing, marketing, drug development, technical and human resources and commercial expertise than we do. Large pharmaceutical and biotechnology

companies, in particular, have extensive experience in clinical testing, obtaining regulatory approvals, recruiting patients and manufacturing biotechnology products. These companies also have significantly greater research and marketing capabilities than we do and may also have products that have been approved or are in late stages of development, and collaborative arrangements in our target markets with leading companies and research institutions. Established pharmaceutical and biotechnology companies may also invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make the product candidates that we develop obsolete. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies, as well as in acquiring technologies complementary to, or necessary for, our programs. As a result of all of these factors, our competitors may succeed in obtaining approval from the FDA, EMA or other comparable foreign regulatory authorities or in discovering, developing and commercializing products in our field before we do.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient, have a broader label, are marketed more effectively, are more widely reimbursed or are less expensive than any products that we may develop. Our competitors also may obtain marketing approval from the FDA, EMA or other comparable foreign regulatory authorities for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Even if the product candidates we develop achieve marketing approval, they may be priced at a significant premium over competitive products if any have been approved by then, resulting in reduced competitiveness. Technological advances or products developed by our competitors may render our technologies or product candidates obsolete, less competitive or not economical. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

***Interim, topline and preliminary data from our clinical trials that we announce or publish may change as more patient 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 may publicly disclose preliminary, interim or topline data from our clinical trials. These interim updates 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 topline 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. Topline 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, topline data should be viewed with caution until the final data is available. In addition, we may report interim analyses of only certain endpoints rather than all endpoints. 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 changes between interim data and final data could significantly harm our business and prospects. Further, additional disclosure of interim data by us or by our competitors in the future could result in volatility in the price of our common stock.

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 is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the preliminary or topline data that we report differ from late, final or actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, financial condition, results of operations and prospects.

***We may not be successful in our efforts to utilize our proprietary drug discovery platforms to develop a pipeline of product candidates.***

A key element of our strategy is to leverage our proprietary drug discovery platforms to develop a pipeline of product candidates to treat heart disease. In order to do so, we must continue to invest in our proprietary drug discovery platforms and development capabilities, including our internal disease modeling and capsid engineering efforts, our in-house cassette engineering capabilities to create novel promoters and regulatory elements to support our programs, and targeted drug delivery approaches for efficient uptake of gene therapies for the heart. Although our research and development efforts to date have resulted in a pipeline of product candidates, these product candidates may not be safe and effective. Our capsid engineering, promoter and regulatory elements may not be successful. In addition, although we expect that our proprietary drug discovery platforms and development capabilities will allow us to develop a diverse pipeline of product candidates, we may not prove to be successful at doing so. Furthermore, we may

also find that the uses of our proprietary drug discovery platforms are limited because alternative uses of our therapeutics prove not to be safe or effective.

Even if we are successful in building our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval or achieve market acceptance. Further, because our product candidates and programs are based on our proprietary drug discovery platforms, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs.

In addition, the biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies. Our future success will depend in part on our ability to maintain a competitive position with our approach. If we fail to stay at the forefront of technological change in utilizing our proprietary drug discovery platforms to create and develop programs and product candidates, we may be unable to compete effectively. Our competitors may render our approach obsolete or limit the commercial value of our product candidates, by advances in existing technological approaches or the development of new or different approaches, potentially eliminating the advantages in our drug discovery process that we believe we derive from our research approach and proprietary technologies. By contrast, adverse developments with respect to other companies that attempt to use a similar approach to our approach may adversely impact the actual or perceived value of our proprietary drug discovery platforms and potential of our programs and product candidates. If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations.

***The manufacture of drugs is complex, and we or our third-party manufacturers may encounter difficulties in production. If we or any of our third-party manufacturers encounter such difficulties, our ability to provide adequate supply of our product candidates for preclinical studies or clinical trials or our products for patients, if approved, could be delayed or prevented.***

Manufacturing drugs, especially in large quantities, is complex and may require the use of innovative technologies. Each lot of an approved drug product must undergo thorough testing for safety identity, strength, quality, purity and potency. Manufacturing drugs requires key materials and facilities specifically designed for and validated for this purpose, as well as sophisticated QA and QC procedures. Slight deviations anywhere in the manufacturing process, including manufacturing drug substance, drug product filling, labeling, packaging, storage and shipping and quality control and testing, may result in lot failures or product recalls. When changes are made to the manufacturing process, we may be required to provide preclinical and clinical data showing the comparable quality and efficacy of the products before and after such changes. If we or our third-party manufacturers are unable to produce sufficient quantities for preclinical studies or clinical trials or for commercialization as a result of these challenges, or otherwise, our development and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and growth prospects.

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

Our gene therapy 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 need to 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 regulatory authorities' standards or specifications with consistent and acceptable production yields and costs.

In addition, the 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 facilities 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 manufacturing process or facilities also could restrict our ability to meet market demand for our products, if approved. Additionally, should our agreement 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.

***We are in the process of building out a manufacturing facility to support future production of certain of our product candidates. We have no experience in manufacturing, and there can be no assurance that we will be able to complete our manufacturing facility or, if completed, we will be able to successfully manufacture product candidates.***

We have historically relied on third parties to manufacture supplies of our product candidates. We plan to fully integrate and internalize AAV manufacturing capabilities to support our Gene Therapy and Cellular Regeneration platforms. We have established an in-house Pilot Plant Operation facility that operates at the 200L scale to support all non-clinical studies including IND-enabling pharmacology (efficacy) and toxicology (safety) studies. We have initiated construction of a dedicated cGMP facility for drug product manufacturing in the San Francisco Bay Area that we expect will be operational in the first half of 2022. The facility will initially produce drug product at the 1000L scale to support FIH studies for TN-201, the most advanced product candidate from our MYBPC3 program. To optimize our use of resources and utilize extensive third-party experience in small molecule manufacturing, we intend to work with CDMOs for our small molecule programs. We initiated cGMP manufacturing activities for our HDAC6 inhibitor program, TYA-11631, in October 2021.

Although some of our employees have experience in the manufacturing of biopharmaceutical products from prior employment at other companies, we as a company have no prior experience in manufacturing. We may face delays or increased costs in the build out of our manufacturing facility or the production of clinical supply at our manufacturing facility, once operational, and cannot guarantee when our facility will be able to produce sufficient quantities of product candidates needed to support our preclinical studies and planned clinical trials. In addition, government approvals will be required for us to operate our manufacturing facility and can be time-consuming to obtain, and there can be no assurance that such approval will be obtained. As a manufacturer of pharmaceutical products, we also will be required to demonstrate and maintain compliance with cGMP requirements related to production processes, quality control and assurance and recordkeeping. Furthermore, establishing and maintaining manufacturing operations may require a reallocation of other resources and management time, as well as potentially significant capital expenditures, 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 timelines or result in difficulties in maintaining compliance with applicable regulatory requirements.

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 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.

Any failure or delay in the development of our manufacturing facility or capabilities may hamper our ability to further process improvement, maintain quality control, limit our reliance on contract manufacturers and protect our trade secrets and other intellectual property, and could adversely impact the development or commercialization of our product candidates.

***Our manufacturing facilities will be subject to significant government regulations and approvals, which are often costly and could result in adverse consequences to our business if we fail to comply with the regulations or maintain the approvals.***

We will 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. We may encounter difficulties in achieving QC and QA and may experience shortages in qualified personnel. We will be subject to inspections by the FDA and comparable agencies in other jurisdictions to confirm compliance with applicable regulatory requirements. These requirements include the qualification and validation of our manufacturing equipment and processes. Any failure to follow cGMP or other regulatory requirements or delay,

interruption or other issues that arise in the manufacture of our product candidates as a result of a failure of our facilities or the facilities or operations of our third-party suppliers to comply with regulatory requirements or pass any regulatory authority inspection could significantly impair our ability to develop and commercialize our product candidates, including leading to significant delays in the availability of our product candidates for our clinical trials or the termination or hold on a clinical trial, or the delay or prevention of a filing or approval of marketing applications for our product candidates. Significant noncompliance could also result in the imposition of sanctions, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approvals for our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could damage our reputation. If we are not able to maintain regulatory compliance, we may not be permitted to market our product candidates and/or may be subject to product recalls, seizures, injunctions, or criminal prosecution.

***We may not be able to successfully manufacture our product candidates in sufficient quality and quantity, which would delay or prevent us from developing our product candidates and commercializing resulting approved products, if any.***

To date, our product candidates have been manufactured in quantities adequate for preclinical studies. In order to conduct clinical trials for a product candidate and for commercialization of the resulting product if that product candidate is approved for sale, we will need to manufacture product candidates in larger quantities. We may not be able to successfully repeat or increase the manufacturing capacity for any of our product candidates in a timely or cost-effective manner or at all. Significant changes or scale-up of manufacturing may require additional validation studies, which are costly and which regulatory authorities must review and approve. In addition, quality issues may arise during those changes or scale-up activities. If we are unable to successfully manufacture of any of our product candidates in sufficient quality and quantity, the development of that product candidate and regulatory approval or commercial launch for any resulting products may be delayed or there may be a shortage in supply, which could significantly harm our business.

***Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.***

As product candidates progress through preclinical studies and clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize yield and manufacturing batch size, minimize costs and achieve consistent quality and results. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commercialize our product candidates, if approved, and generate revenue.

***Even if approved, we may not successfully commercialize our product candidates.***

Our product candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe, commercially viable products would severely limit our ability to become profitable or to achieve significant revenues. Even if one or more of our product candidates is approved, we may be unable to successfully commercialize our product candidates for several reasons, including:

- some or all of our product candidates may be found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances; our product candidates, if safe and effective, may nonetheless not be able to be developed into commercially viable products;
- it may be difficult to manufacture or market our product candidates on a scale that is necessary to ultimately deliver our products to end-users;
- intellectual property and proprietary rights of third parties may preclude us from marketing our product candidates;
- the nature of our indications as rare diseases means that the potential market size may be limited; and
- third parties may market superior or equivalent drugs which could adversely affect the commercial viability and success of our product candidates.



***Our product candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community necessary for commercial success.***

Even if our product candidates receive regulatory approval, they may not gain adequate market acceptance among physicians, patients, third-party payors and others in the medical community. If we are unable to demonstrate sufficient safety to permit a broader use of our product candidates, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of any of our approved product candidates will depend on a number of factors, including:

- the efficacy and safety profile as demonstrated in clinical trials compared to alternative treatments;
- the size of the relevant pediatric patient population if approved for a pediatric indication, including challenges associated with diagnosing or identifying pediatric populations with the applicable target disease or condition;
- perceived safety and efficacy profile and ease of use for pediatric patient population if approved for a pediatric indication;
- the timing of market introduction of the product candidate as well as competitive products;
- the clinical indications for which a product candidate is approved;
- restrictions on the use of product candidates in the labeling approved by regulatory authorities, such as boxed warnings or contraindications in labeling, or a risk evaluation and mitigation strategy, if any, which may not be required of alternative treatments and competitor products;
- physicians, hospitals, treatment centers and patients considering our product candidates as a safe, pure and effective treatment;
- the perceived prevalence and severity of any side effects for our product candidates compared to the prevalence and severity of any side effects for conventional products and other gene therapies;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- relative convenience and ease of administration;
- the willingness of the target patient population or their caregivers to try new therapies and of physicians to prescribe these therapies;
- the availability of coverage and adequate reimbursement by third-party payors, including government authorities;
- patients' willingness to pay for these therapies in the absence of such coverage and adequate reimbursement;
- the effectiveness of sales and marketing efforts;
- support from KOLs and patient advocacy groups;
- unfavorable publicity relating to our product candidates; and
- the approval of other new therapies for the same indications.

If any of our product candidates are approved but do not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors and patients, we may not generate or derive sufficient revenue from that product candidate and our financial results could be negatively impacted.

***Adverse public perception or regulatory scrutiny of gene therapy technology or precision medicine for the treatment of heart diseases may negatively impact the developmental progress or commercial success of product candidates that we develop.***

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

Gene therapy and precision medicine remain novel technologies. The commercial success of our products, if successfully developed and approved, may be adversely affected by claims that gene therapy or precision 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 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 or precision medicine, whether related to our technology or our 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.

***The limited number of patients who have the diseases for which our product candidates are being developed 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. Even if such product candidates achieve marketing approval, because such target patient populations are small and the addressable patient population may be even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth.***

Some of the indications for which we plan to evaluate our 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 planned 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. Moreover, the effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. We may not be able to initiate or continue clinical trials on a timely basis or at all for our product candidates if we 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 some 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.

Additionally, the potentially addressable patient population for our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

***Any product candidates we develop may become subject to unfavorable third-party coverage and reimbursement practices, as well as pricing regulations.***

The availability and extent of coverage and adequate reimbursement by third-party payors including government health administration authorities, private health coverage insurers, managed care organizations and other third-party payors is essential for most patients to be able to afford expensive treatments. The indications we are initially pursuing have small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size.

Sales of any of our product candidates that receive marketing approval will depend substantially, both in the United States and internationally, on the extent to which the costs of such product candidates will be covered and reimbursed by third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize an adequate return on our investment. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products. In the United States, for example, principal decisions about reimbursement for new products are typically made by the Centers for Medicare & Medicaid Services ("CMS"), an agency within the U.S. Department of Health and Human Services ("HHS"). CMS decides whether

and to what extent a new product will be covered and reimbursed under Medicare, and private third-party payors often follow CMS's decisions regarding coverage and reimbursement to a substantial degree. However, one third-party payor's determination to provide coverage for a product candidate does not assure that other payors will also provide coverage for the product candidate or at the same level of reimbursement. As a result, the coverage determination process is often time-consuming and costly. This process will require us to provide scientific and clinical support for the use of our products to each third-party payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Increasingly, 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. Further, such payors are increasingly challenging the price, examining the medical necessity and reviewing the cost effectiveness of medical product candidates. There may be especially significant delays in obtaining coverage and reimbursement for newly approved drugs. Third-party payors may limit coverage to specific product candidates on an approved list, known as a formulary, which might not include all FDA-approved drugs for a particular indication. We may need to conduct expensive pharmaco-economic studies to demonstrate the medical necessity and cost effectiveness of our products. Nonetheless, our product candidates may not be considered medically necessary or cost effective. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be.

Outside the United States, the commercialization of therapeutics is generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the European Union, medical product prices are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after a product receives marketing approval. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, product prices under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

If we are unable to establish or sustain coverage and adequate reimbursement for any product candidates from third-party payors, the adoption of those products and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved. Coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

***Our business entails a significant risk of product liability and if we are unable to obtain sufficient insurance coverage, such inability could have an adverse effect on our business and financial condition. If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.***

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing and marketing of therapeutic treatments. We currently have no product liability insurance. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have an adverse effect on our business and financial condition. Also, 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 collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

We may be sued if any of our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing, or sale post-approval. 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 laws. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit testing and commercialization of our products. Even successful defense would require significant financial and management resources.

Regardless of the merits or eventual outcome, liability claims may result in:

- delays in the development of our product candidates;
- FDA, EMA or other regulatory authority investigation of the safety and effectiveness of our products, our manufacturing processes and facilities or our marketing programs;
- decreased or interrupted demand for our products;
- 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;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing, or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources; and
- the inability to commercialize any products.

### **Risks Related to Regulatory Approval and Other Legal Compliance Matters**

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

Our product candidates are and will continue to be subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, safety, efficacy, approval, recordkeeping, reporting, labeling, storage, packaging, advertising and promotion, pricing, marketing and distribution of drugs. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process must be successfully completed in the United States and in many foreign jurisdictions before a new drug can be approved for marketing. Obtaining approval by the FDA, EMA and other comparable foreign regulatory authorities is costly, 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 data. Even if we eventually complete clinical testing and receive approval for our product candidates, the FDA, EMA and other comparable foreign regulatory authorities may approve our product candidates for a more limited indication or a narrower patient population than we originally requested or may impose other prescribing limitations or warnings that limit the product's commercial potential. We have not submitted for, or obtained, regulatory approval for any product candidate, and it is possible that none of our product candidates will ever obtain regulatory approval. Further, development of our product candidates and/or regulatory approval may be delayed for reasons beyond our control. We cannot provide any assurance that any product candidate we may develop will progress through required clinical testing and obtain the regulatory approvals necessary for us to begin selling them.

We have not initiated, conducted, managed or completed any clinical trials nor managed the regulatory approval process with the FDA or any other regulatory authority. Applications for our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, EMA or other comparable foreign regulatory authorities may disagree with the design, implementation or results of our clinical trials;
- the FDA, EMA or other comparable foreign regulatory authorities may determine that our product candidates are not safe and effective, are 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;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;

- the FDA, EMA or other comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- we may be unable to demonstrate to the FDA, EMA or other comparable foreign regulatory authorities that our product candidate’s risk-benefit ratio for its proposed indication is acceptable;
- the FDA, EMA or other 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, EMA or other comparable foreign regulatory authorities may significantly change in a manner rendering our 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 significantly harm our business, results of operations and prospects. Any delay or failure in seeking or obtaining required approvals would have a material and adverse effect on our ability to generate revenue from any particular product candidates we are developing and for which we are seeking approval. Furthermore, any regulatory approval to market a drug may be subject to significant limitations on the approved uses or indications for which we may market, promote and advertise the drug or the labeling or other restrictions. In addition, the FDA has the authority to require a Risk Evaluation and Mitigation Strategy (“REMS”) plan as part of approving an NDA, or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug. These requirements or restrictions might include limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. These limitations and restrictions may significantly limit the size of the market for the drug and affect reimbursement by third-party payors.

We are also subject to numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing and third-party reimbursement. The foreign regulatory approval process varies among countries, and generally includes all of the risks associated with FDA and EMA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval.

***The FDA, EMA and other comparable foreign regulatory authorities may not accept data from trials conducted in locations outside of their jurisdiction.***

We are planning on undertaking clinical trials in the United States and additional clinical trials internationally. The acceptance of study data by the FDA, EMA or other comparable foreign regulatory authority from clinical trials conducted outside of their respective jurisdictions may be subject to certain conditions. In cases where data from U.S. clinical trials are intended to serve as the basis for marketing approval in the foreign countries outside the United States, the standards for clinical trials and approval may be different. There can be no assurance that any U.S. or foreign regulatory authority would accept data from trials conducted outside of its applicable jurisdiction. If the FDA, EMA or any applicable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction.

***Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.***

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction. For example, even if the FDA or EMA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion and reimbursement of the product candidate in those countries. However, a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Obtaining foreign regulatory approvals and establishing and maintaining compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries.

If we or any future collaborator fail to comply with the regulatory requirements in international markets or fail to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our potential product candidates will be harmed.

***Even if we successfully complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.***

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. We have not received approval from regulatory authorities in any jurisdiction to market any of our product candidates. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, issue a complete response letter, or ultimately, we may not be able to obtain regulatory approval. In addition, we may experience delays or rejections if an FDA Advisory Committee recommends disapproval or restrictions on use.

In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative actions, or changes in regulatory authority policy during the period of product development, clinical trials and the review process. 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. In addition, varying interpretations of data obtained from preclinical and clinical testing could delay, limit or prevent the receipt of marketing approval for a product candidate.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or other labeling changes. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. Regulatory authorities may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS or equivalent requirement. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects.

***Even if our product candidates receive regulatory approval, they will be subject to significant post- marketing regulatory requirements and oversight.***

Any regulatory approvals that we may receive for our product candidates will require the submission of reports to regulatory authorities and on-going surveillance to monitor the safety and efficacy of the product candidate, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements and regulatory inspection. For example, the FDA may require a REMS in order to approve our product candidates, which could entail requirements for a medication guide, physician training and communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or foreign regulatory authorities approve our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post- marketing information and reports, registration, as well as on-going compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. In addition, failure to comply with FDA, EMA and other comparable foreign regulatory requirements may subject our company to administrative or judicially imposed sanctions, including:

- delays in or the rejection of product approvals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on the products, manufacturers or manufacturing process;
- warning or untitled letters;
- civil and criminal penalties;

- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions or import bans;
- voluntary or mandatory product recalls and publicity requirements;
- total or partial suspension of production; and
- imposition of restrictions on operations, including costly new manufacturing requirements.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates, if approved, and generate revenue. Furthermore, non-compliance by us or any future collaborator with regulatory requirements, including safety monitoring and with requirements related to the development of products for the pediatric population can also result in significant financial penalties.

***We may not be able to obtain orphan drug designation or obtain or maintain orphan drug exclusivity for our product candidates and, even if we do, that exclusivity may not prevent the FDA, EMA or other comparable foreign regulatory authorities, from approving competing products.***

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually 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. Similarly, in Europe, the European Commission, upon the recommendation of the EMA's Committee for Orphan Medicinal Products, grants orphan drug designation to promote the development of drugs that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in Europe and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for drugs intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in Europe would be sufficient to justify the necessary investment in developing the drug. In Europe, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers. However, there can be no assurances that we will be able to obtain orphan designations for our product candidates.

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 user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the United States provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances. The applicable exclusivity period is ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

The FDA has granted orphan drug designation for TN-201, the most advanced product candidate from our MYBPC3 program, and we may seek orphan drug designation for other product candidates in the United States, Europe and other jurisdictions. Even if we obtain orphan drug designation for a product candidate, we may not be able to obtain or maintain orphan drug exclusivity for that product candidate. We may not be the first to obtain marketing approval of any product candidate for which we have obtained orphan drug designation for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to ensure that we will be able to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the product candidate any advantage in the regulatory review or approval process or entitles the product candidate to priority review.

***We may face difficulties from changes to current FDA and healthcare regulations and future legislation.***

Existing regulatory policies 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 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 lose any marketing approval that we may have obtained, and we may not achieve or sustain profitability.

We also 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. For example, certain policies of the current U.S. administration may impact our business and industry, which could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how current and future legislation, executive actions, and litigation, including the executive orders referenced below, will be implemented, and the extent to which they will impact our business, our clinical development, and the FDA's and other agencies' ability to exercise their regulatory authority, including FDA's pre-approval inspection and timely review of any regulatory filings or applications we submit to the FDA. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course or constraints on our business operations, including operations of our contractors, our business may be negatively impacted.

For example, in March 2010, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "ACA"), was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and continues to significantly impact the U.S. pharmaceutical industry. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA. For example, various portions of the ACA have been the subject of legal and constitutional challenges in the U.S. Supreme Court. In June 2021, the U.S. Supreme Court held that Texas and other challengers had no legal standing to challenge the ACA, dismissing the case without specifically ruling on the constitutionality of the ACA. On January 28, 2021, President Biden issued an executive order to initiate a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructs 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. We cannot predict how this Supreme Court decision or future litigation will impact our business, or what other healthcare measures and regulations will ultimately be implemented at the federal or state level or the effect of any future legislation or regulation may have on our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, effective April 1, 2013, which will remain in effect through 2030, with the exception of a temporary suspension implemented under various COVID-19 relief legislation from May 1, 2020 through the end of 2021, unless additional congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and accordingly, our financial operations.

Moreover, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019. At the federal level, the Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, in 2020, HHS and CMS issued various rules that are expected to impact, among others, price reductions from pharmaceutical manufacturers to plan sponsors under Part D, fee arrangements between pharmacy benefit managers and manufacturers, manufacturer price reporting requirements under the Medicaid Drug Rebate Program, including regulations that affect manufacturer-sponsored patient assistance programs subject to pharmacy benefit manager accumulator programs and Best Price reporting related to certain value-based purchasing arrangements. Multiple lawsuits have been brought against the HHS challenging various aspects of the rules. The impact of these lawsuits as well as legislative, executive, and administrative actions of the Biden administration on us and the pharmaceutical industry as a whole is unclear.

In January 2021, the Biden administration issued a "regulatory freeze" memorandum that directs department and agency heads to review new or pending rules of the prior administration. Under the American Rescue Plan Act of 2021, effective January 1, 2024,



the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs will be eliminated. Elimination of this cap may require pharmaceutical manufacturers to pay more in rebates than it receives on the sale of products, which could have a material impact on our business. In July 2021, the Biden administration released an executive order, “Promoting Competition in the American Economy,” with multiple provisions aimed at increasing competition for prescription drugs. In response to this executive order, the HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and potential legislative policies that Congress could pursue to advance these principles. In addition, Congress is considering legislation that, if passed, could have significant impact on prices of prescription drugs covered by Medicare, including limitations on drug price increases. The impact of these legislative, executive, and administrative actions and any future healthcare measures and agency rules implemented by the Biden administration on us and the pharmaceutical industry as a whole is unclear. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates if approved.

At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical 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. A number of states are considering or have recently enacted state drug price transparency and reporting laws that could substantially increase our compliance burdens and expose us to greater liability under such state laws once we begin commercialization after obtaining regulatory approval for any of our products. We are unable to predict the future course of federal or state healthcare legislation in the United States directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. These and any further changes in the law or regulatory framework that reduce our revenue or increase our costs could also have a material and adverse effect on our business, financial condition and results of operations.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our product candidates. It is also possible that additional governmental action is taken to address the COVID-19 pandemic.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for biotechnology products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

***We are subject to stringent laws, rules, regulations, policies, industry standards and contractual obligations regarding data privacy and security and may be subject to additional related laws and regulations in jurisdictions into which we expand. Many of these laws and regulations are subject to change and reinterpretation and could result in claims, changes to our business practices, monetary penalties, increased cost of operations or other harm to our business.***

The regulatory framework for privacy and personal information security issues worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. The U.S. federal and various state, local and foreign government bodies and agencies have adopted or are considering adopting laws, rules, regulations and standards limiting, or laws, rules, regulations and standards regarding, the collection, distribution, use, disclosure, storage, security and other processing of personal information.

Outside of the United States, legal requirements relating to the collection, storage, processing and transfer of personal data continue to evolve. For example, the collection and use of health data and other personal data is governed in the European Union by the General Data Protection Regulation (“GDPR”), which extends the geographical scope of EU data protection law to entities and operations outside of the European Union under certain conditions and imposes substantial obligations upon companies and new rights for individuals, and by certain EU member state-level legislation. For example, the GDPR requires data controllers to implement stringent operational requirements for processors and controllers of personal data, including transparent and expanded disclosure to data subjects about how their personal data is to be used, limitations on retention of information, mandatory data breach notification requirements, and higher standards for data controllers to demonstrate that they have obtained valid consent for certain data processing activities. Failure to comply with the GDPR may result in fines up to €20,000,000 or 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties. The GDPR may increase our responsibility and liability in relation to personal data that we may process, and we may be required to implement additional measures in an effort to comply with the GDPR and with other laws, rules, regulations and standards in the European Union, including those of EU member states, relating to privacy and data protection. This may be onerous and if our efforts to comply with GDPR or other

applicable EU laws, rules, regulations and standards are not successful, or are perceived to be unsuccessful, it could adversely affect our business. Further, in July 2020, the European Court of Justice (“ECJ”) invalidated the EU-U.S. Privacy Shield, which had enabled the transfer of personal data from the European Union to the United States for companies that had self-certified to the Privacy Shield. The ECJ decision also raised questions about the continued validity of one of the primary alternatives to the EU-U.S. Privacy Shield, namely the European Commission’s Standard Contractual Clauses, and EU regulators have issued additional guidance regarding considerations and requirements that we and other companies must consider and undertake when using the Standard Contractual Clauses. Although the European Commission has presented a new set of Standard Contractual Clauses, which are required to be implemented, there are few, if any, viable alternatives to the EU-U.S. Privacy Shield and the Standard Contractual Clauses and it remains to be seen whether additional means for lawful data transfers will become available. To the extent that we were to rely on the EU-U.S. or Swiss-U.S. Privacy Shield programs, we will not be able to do so in the future, and the ECJ’s decision and other regulatory guidance or developments may impose additional obligations with respect to the transfer of personal data from the European Union and Switzerland to the United States, all of which could restrict our activities in those jurisdictions, limit our ability to provide our products and services in those jurisdictions, require us to modify our policies and practices, and to engage in additional contractual negotiations, or increase our costs and obligations and impose limitations upon our ability to efficiently transfer personal data from the European Union and Switzerland to the United States.

Further, the exit of the United Kingdom from the European Union, often referred to as Brexit, has created uncertainty with regard to data protection regulation in the United Kingdom. Specifically, the United Kingdom exited the European Union on January 1, 2020, subject to a transition period that ended December 31, 2020. The United Kingdom has implemented legislation similar to the GDPR, referred to as the UK GDPR, which provides for significant fines of up to the greater of £17.5 million or 4% of global turnover and exposes us to two parallel regimes with potentially divergent enforcement actions for certain violations. With respect to transfers of personal data, on June 28, 2021, the European Commission issued an adequacy decision in respect of the United Kingdom’s data protection framework, enabling data transfers from EU member states to the United Kingdom to continue without requiring organizations to put in place contractual or other measures in order to lawfully transfer personal data between the territories. While it is intended to last for at least four years, the European Commission may unilaterally revoke the adequacy decision at any point, and if this occurs it could lead to additional costs and increase our overall risk exposure. The relationship between the United Kingdom and European Union in relation to aspects of data protection law in the medium and longer term remains unclear, including with respect to cross-border data transfers and the role of the UK Information Commissioner’s Office with respect to the European Union, which exposes us to further compliance risk. We may incur liabilities, expenses, costs, and other operational losses relating to the GDPR, the UK GDPR, and other laws and regulations in the European Union and United Kingdom relating to privacy and data protection, including those of applicable EU member states in connection with any measures we take to comply with them.

Finally, federal, state and foreign laws, rules, regulations and standards may apply generally to the privacy and security of information we maintain, and may differ from each other in significant ways, thus complicating compliance efforts and potentially requiring us to undertake additional measures to comply with them. In the United States, there are a broad variety of data privacy, protection and security laws, rules, regulations and standards that may apply to our activities, such as state data breach notification laws, state personal data privacy laws (for example, the California Consumer Privacy Act of 2018 (“CCPA”)), state health information privacy laws, and federal and state consumer protection laws (for example, Section 5(c) of the Federal Trade Commission Act). A range of enforcement agencies exist at both the state and federal levels that can enforce these laws, rules, regulations and standards. For example, the CCPA, which took effect on January 1, 2020, requires covered businesses that process personal information of California residents to disclose their data collection, use and sharing practices. Further, the CCPA provides California residents with new data privacy rights (including the ability to opt out of certain disclosures of personal information), imposes new operational requirements for covered businesses, provides for significant civil penalties for violations as well as a private right of action for certain data breaches and statutory damages (that is expected to increase data breach class action litigation and result in significant exposure to costly legal judgements and settlements). Aspects of the CCPA and its interpretation and enforcement remain uncertain. In addition, California voters passed the California Privacy Rights Act of 2020 (“CPRA”) in November 2020, which becomes effective in most material respects on January 1, 2023. The CPRA will, among other things, give California residents the ability to limit use of certain sensitive personal information, further restrict the use of cross-contextual advertising, establish restrictions on the retention of personal information, expand the types of data breaches subject to the CCPA’s private right of action, provide for increased penalties for CPRA violations concerning California residents under the age of 16, and establish a new California Privacy Protection Agency to implement and enforce the new CCPA and CPRA. Although there are limited exemptions for clinical trial data under the CCPA, the CCPA and other similar laws could impact our business activities, depending on their interpretation. Further, laws in all 50 states require businesses to provide notice to customers whose personal data has been disclosed as a result of a data breach. Additionally, other state legislatures have enacted or are currently contemplating, and may pass, their own comprehensive data privacy and security laws, with potentially greater penalties and more rigorous compliance requirements relevant to our business. For example, in March 2021, Virginia enacted the Virginia Consumer Data Protection Act, or CDPA, which becomes effective on January 1, 2023, and in June 2021, Colorado enacted the Colorado Privacy Act, or CPA, which takes effect on July 1, 2023. The CPA and CDPA share similarities with the CCPA, the CPRA, and legislation proposed in other states.

With the GDPR, CCPA, CRPA and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and making necessary changes to our policies and practices, and may incur significant costs and expenses in an effort to do so. We are currently in the process of developing and updating our policies and procedures in accordance with requirements under the CCPA and the GDPR, as well as other applicable data privacy and protection laws and regulations. Additionally, the CPRA and the CCPA may lead other states to pass comparable legislation, with potentially greater penalties and more rigorous compliance requirements relevant to our business. Additionally, if third parties we work with, such as vendors or service providers, violate applicable laws, rules or regulations or our policies, such violations may also put our or our customers' data, including personal data, at risk, which could in turn have an adverse effect on our business.

We make public statements about our use, collection, disclosure and other processing of personal data through our privacy policies, information provided on our website and press statements. Although we endeavor to comply with our public statements and documentation, we may at times fail to do so or be alleged to have failed to do so. The publication of our privacy policies and other statements that provide promises and assurances about data privacy and security can subject us to potential government or legal action if they are found to be deceptive, unfair or misrepresentative of our actual practices. Any failure or perceived failure by us or our vendors or service providers to comply with our applicable policies or notices relating to privacy or data protection, our contractual or other obligations to third parties, or any of our other legal obligations, laws, rules, regulations and standards relating to privacy or data protection, may result in governmental investigations or enforcement actions, litigation, claims and other proceedings, harm our reputation, and could result in significant liability.

***Inadequate funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.***

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the U.S. Securities and Exchange Commission ("SEC") and other government agencies on which our operations may rely, including those 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 to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in recent years, including in 2018 and 2019, the U.S. government shut down several times and certain regulatory agencies, such as the FDA and the SEC, had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

***Our relationships with healthcare professionals, clinical investigators, CROs and third-party payors in connection with our current and future business activities may be subject to federal and state healthcare fraud and abuse laws, false claims laws, transparency laws, government price reporting, and health information privacy and security laws, which could expose us to significant losses, including, among other things, criminal sanctions, civil penalties, contractual damages, exclusion from governmental healthcare programs, reputational harm, administrative burdens and diminished profits and future earnings.***

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, clinical investigators, CROs, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, as well as market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations may include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

- the federal false claims laws, including the civil False Claims Act, which can be enforced by private citizens through civil whistleblower or qui tam actions, and civil monetary penalties laws, prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996 (“HIPAA”), prohibits, among other things, executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Civil Monetary Penalty Act of 1981 and implementing regulations, which impose penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal healthcare program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent, or offered or transferred remuneration to a federal healthcare beneficiary that a person knows or should know is likely to influence the beneficiary’s decision to order or receive items or services reimbursable by the government from a particular provider or supplier;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (“HITECH”) and their implementing regulations, also imposes obligations, including mandatory contractual terms, on covered entities, which are health plans, healthcare clearinghouses, and certain health care providers, as those terms are defined by HIPAA, and their respective business associates and their subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the federal Physician Payments Sunshine Act requires applicable manufacturers of covered drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to annually report to CMS information regarding payments and other transfers of value to physicians, defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals as well as information regarding ownership and investment interests held by physicians and their immediate family members. Beginning with data reporting in 2022, reporting obligations with respect to covered recipients have been expanded to include physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and anesthesiologist assistants, and certified nurse midwives for payments and transfers of value made during the previous year; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance regulations promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures, or drug pricing; state and local laws that require the registration of pharmaceutical sales and medical representatives; state laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare and data privacy and security laws and regulations will involve substantial ongoing costs and may require us to undertake or implement additional policies or measures. We may face claims and proceedings by private parties, and claims, investigations and other proceedings by governmental authorities, relating to allegations that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations, and it is possible that courts or governmental authorities may conclude that we have not complied with them, or that we may find it necessary or appropriate to settle any such claims or other proceedings. In connection with any such claims, proceedings, or settlements, we may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and 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. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

***Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, CDMOs, suppliers and vendors may engage in misconduct or other improper activities. Misconduct by these parties could include failures to comply with FDA regulations, provide accurate information to the FDA, comply with federal and state health care fraud and abuse laws and regulations, accurately report financial information or data or disclose unauthorized activities to us. In particular, research, sales, marketing and business arrangements in the health care industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct, but it is not always possible to identify and deter misconduct by these 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 comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We 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. We also could incur significant costs associated with civil or criminal fines and penalties.

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 maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of hazardous and flammable materials, including chemicals and biological materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***Our business activities may be subject to the U.S. Foreign Corrupt Practices Act and similar anti-bribery and anti-corruption laws of other countries in which we operate, as well as U.S. and certain foreign export controls, trade sanctions, and import laws and regulations. Compliance with these legal requirements could limit our ability to compete in foreign markets and subject us to liability if we violate them.***

Our business activities are subject to the U.S. Foreign Corrupt Practices Act of 1977, as amended ("FCPA"), the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we operate. These laws generally prohibit companies and their employees, agents, representatives, business partners, and third-party intermediaries from, directly or indirectly, offering, promising, giving or authorizing others to give anything of value, either directly or indirectly, to recipients in the public or private sector in order to influence official action or otherwise obtain or retain business. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-U.S. governments. Additionally, in many other countries, hospitals are owned and operated by the government, and doctors and other hospital employees would be considered foreign officials under the FCPA. Recently, the SEC and Department of Justice ("DOJ") have increased their FCPA enforcement activities with respect to biotechnology and pharmaceutical companies.

We sometimes leverage third parties to assist with the conduct of our business abroad. We, our employees, agents, representatives, business partners and our third-party intermediaries may have direct or indirect interactions with officials and employees of government agencies or state-owned or affiliated entities and may be held liable for the corrupt or other illegal activities of these employees, agents, representatives, business partners or third-party intermediaries even if we do not explicitly authorize such activities. We cannot assure you that all of our employees, agents, representatives, business partners and third-party intermediaries will not take actions in violation of applicable law for which we may be ultimately held responsible. As we increase our international sales and business, our risks under these laws may increase.

These laws also require that we make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls and compliance procedures designed to prevent violations of anti-corruption laws. While we have policies and procedures to address compliance with such laws, there is no certainty that all of our employees, agents, representatives, business partners and third-party intermediaries, or those of our affiliates, will comply with our policies or applicable laws and regulations, for which we may be ultimately held responsible.

Allegations or violations of these laws and regulations could result in whistleblower complaints, fines, severe civil or criminal sanctions, settlements, prosecution, enforcement actions, damages, adverse media coverage, investigations, loss of export privileges, disgorgement, and other remedial measures, suspension or debarment from government contracts and prohibitions on the conduct of our business including our ability to offer our products in one or more countries. Responding to any investigation or action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees. As a general matter, investigations, enforcement actions and sanctions could damage our reputation, our brand, our international activities, our ability to attract and retain employees and our business, prospects, operating results and financial condition.

In addition, our products may be subject to U.S. and foreign export controls, trade sanctions and import laws and regulations. Governmental regulation of the import or export of our products, or our failure to obtain any required import or export authorization for our products, when applicable, could harm our international sales and adversely affect our revenue. Compliance with applicable regulatory requirements regarding the export of our products may create delays in the introduction of our products in international markets or, in some cases, prevent the export of our products to some countries altogether. Furthermore, U.S. export control laws and economic sanctions prohibit the shipment of certain products and services to countries, governments, and persons targeted by U.S. sanctions. If we fail to comply with export and import regulations and such economic sanctions, penalties could be imposed, including fines and/or denial of certain export privileges. Moreover, any new export or import restrictions, new legislation or shifting approaches in the enforcement or scope of existing regulations, or in the countries, persons, or products targeted by such regulations, could result in decreased use of our products by, or in our decreased ability to export our products to, existing or potential customers with international operations. Any decreased use of our products or limitation on our ability to export or sell our products would likely adversely affect our business.

***Changes in tax law could increase the tax burden on our orphan drug programs and adversely affect our business and financial condition.***

In 2017, the U.S. government enacted the Tax Act, which as modified by the CARES Act, includes significant changes to the taxation of business entities. These changes include, among others, a reduction in the orphan drug tax credit from 50% to 25% of qualifying expenditures. When and if we become profitable, this reduction in tax credits may result in an increased federal income tax burden on our orphan drug programs as it may cause us to pay federal income taxes earlier under the revised tax law than under the prior law and may increase our total federal tax liability attributable to such programs. Additionally, the current administration has proposed an increase of the corporate tax rate and other changes, including a global minimum tax, which, if enacted would further increase our total federal tax liability when and if we become profitable.

In addition, it is uncertain if and to what extent various states will conform to the Tax Act and proposed tax legislation, and states may enact suspensions or limitations on the use of NOLs and tax credits (including, without limitation, California legislation enacted in 2020 that suspends the use of California NOLs and limits the use of certain California tax credits for certain periods). The impact of the Tax Act and any proposed tax legislation on holders of our common stock is also uncertain and could be adverse. Investors should consult with their legal and tax advisors with respect to the Tax Act and any proposed tax legislation and the potential tax consequences of investing in or holding our common stock.

## **Risks Related to Employee Matters, Managing Our Growth and Other Risks Related to Our Business**

***Our success is highly dependent on our ability to attract and retain highly skilled executive officers and employees.***

To succeed, we must recruit, retain, manage and motivate qualified clinical, scientific, technical and management personnel, and we face significant competition for experienced personnel. We are highly dependent on the principal members of our management and scientific and medical staff, particularly Faraz Ali, M.B.A., our Chief Executive Officer. Additionally, the effects of the COVID-19 pandemic may interfere with our ability to hire or retain qualified and key personnel. If we do not succeed in attracting and retaining such personnel, particularly at the management level, it could adversely affect our ability to execute our business plan and harm our operating results. In particular, the loss of one or more of our executive officers could be detrimental to us if we cannot recruit suitable replacements in a timely manner. We do not maintain “key person” insurance for any of our executives or other employees. We could in the future have difficulty attracting and retaining experienced personnel and may be required to expend significant financial resources in our employee recruitment and retention efforts.

Many of the other biotechnology companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide higher compensation, more diverse opportunities and better prospects for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can discover, develop and commercialize our product candidates will be limited and the potential for successfully growing our business will be harmed.

Additionally, we rely on our scientific founders and other scientific and clinical advisors and consultants to assist us in formulating our research, development and clinical strategies. These advisors and consultants are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, these advisors and consultants typically will not enter into non-compete agreements with us. If a conflict of interest arises between their work for us and their work for another entity, we may lose their services. Furthermore, our advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with ours. In particular, if we are unable to maintain consulting relationships with our scientific founders or if they provide services to our competitors, our development and commercialization efforts will be impaired and our business will be significantly harmed.

***In order to successfully implement our plans and strategies, we will need to grow the size of our organization, and we may experience difficulties in managing this growth.***

As of September 30, 2021, we had more than 100 full-time employees. In order to successfully implement our development and commercialization plans and strategies, and as we begin to operate as a public company, we expect to need additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA, EMA and other comparable foreign regulatory agencies’ review process for our product candidates, while complying with any contractual obligations to contractors and other third parties we may have; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to successfully develop and, if approved, commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing 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, including key aspects of our research and development, clinical development, manufacturing and operations. We cannot assure you that the services of 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 third-party service providers is compromised for any reason, our preclinical studies and the initiation and conduct of our planned clinical trials may be extended, delayed or terminated, and we may not be able to obtain marketing approval of our product candidates or otherwise advance our programs and business. We cannot assure you that we will be able to manage our existing third-party service providers or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and/or engaging additional third-party service providers, we may not be able to successfully implement the tasks necessary to develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

***If we are unable to establish sales or marketing capabilities or enter into agreements with third parties to sell or market our product candidates, we may not be able to successfully sell or market our product candidates that obtain regulatory approval.***

We currently do not have and have never had a marketing or sales team. In order to commercialize any product candidates, if approved, we must build marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services for each of the territories in which we may have approval to sell or market our product candidates. We may not be successful in accomplishing these required tasks.

Establishing an internal sales or marketing team with technical expertise and supporting distribution capabilities to commercialize our product candidates will be expensive and time-consuming and will require significant attention of our executive officers to manage. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could adversely impact the commercialization of any of our product candidates that we obtain approval to market, if we do not have arrangements in place with third parties to provide such services on our behalf. Alternatively, if we choose to collaborate, either globally or on a territory-by-territory basis, 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, we will be required to negotiate and enter into arrangements with such third parties relating to the proposed collaboration and such arrangements may prove to be less profitable than commercializing the product on our own. If we are unable to enter into such arrangements when needed, on acceptable terms, or at all, we may not be able to successfully commercialize any of our product candidates that receive regulatory approval, or any such commercialization may experience delays or limitations. If we are unable to successfully commercialize our approved product candidates, either on our own or through collaborations with one or more third parties, our future product revenue will suffer, and we may incur significant additional losses.

***Our computer systems, or those of any of our CROs, manufacturers, contractors, consultants or other third parties or potential future collaborators, may fail or suffer security incidents or data privacy breaches or other unauthorized or improper access to, use of, or destruction of our proprietary or confidential data, employee data, or personal data, which could result in additional costs, loss of revenue, significant liabilities, harm to our brand and material disruption of our operations.***

Despite the implementation of security measures in an effort to protect systems that store our information, given their size and complexity and the increasing amounts of information maintained on our internal information technology systems, and those of our third-party CROs, other contractors (including sites performing our clinical trials), consultants and other third parties, such systems are vulnerable to breakdown or other damage or interruption from, among other things, service interruptions, system malfunctions, natural disasters, terrorism, war, telecommunication and electrical failures, security breaches and incidents from inadvertent or intentional actions by our employees, contractors, consultants, business partners, and other third parties, cyber-attacks and other hacking attempts by malicious third parties (including supply chain cyber-attacks or attacks by nation-state or nation-state supported actors, or the deployment of harmful malware, ransomware, distributed denial-of-service (“DDoS”) attacks, phishing attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure or lead to the loss, destruction, alteration, prevention of access to, disclosure, dissemination of, or damage or unauthorized access to, our data (including trade secrets or other confidential information, intellectual property, proprietary business information and personal data) or other data that we process or maintain or that is processed or maintained on our behalf, or other assets, which could result in financial, legal, business and reputational harm to us.

We have in the past and may in the future experience phishing attempts, and companies have, in general, experienced an increase in phishing and social engineering attacks from third parties in connection with the COVID-19 pandemic, and the increase in remote working further increases security threats. To the extent that any data breach, disruption or security incident were to result in any loss, destruction, unavailability, alteration, disclosure, dissemination of, or damage or unauthorized access to, our personal data, applications, assets or any other data processed or maintained on our behalf, or for it to be believed or reported that any of the foregoing occurred, we could incur significant liability, including consequential damages, financial harm and reputational damage and the development and commercialization of our product candidates could be delayed. We cannot ensure that our data protection efforts and our investment in information technology, or the efforts or investments of CROs, consultants or other third parties, will prevent breakdowns or breaches in our or their systems or other cybersecurity incidents that cause loss, destruction, unavailability, alteration, dissemination of, or damage or unauthorized access to, our data, including personal data, assets and other data processed or maintained on our behalf, that could have a material adverse effect upon our reputation, business, operations or financial condition. We also rely on third parties to manufacture our product candidates, and any data breaches or other security events relating to their



computer systems could also have a material adverse effect on our business. Controls employed by our information technology department and our CROs, consultants and other third parties could prove inadequate, and our ability to monitor such third parties' data security practices is limited. Due to applicable laws, rules, regulations and standards or contractual obligations, we may be held responsible for any information security failure or cyber-attack attributed to our third-party service providers as they relate to the information we share with them.

If any data breach or other security incident were to occur and cause interruptions in our operations, it could result in a material disruption of our programs and the development of our product candidates could be delayed. In addition, the loss of clinical trial data for our product candidates could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. Further, any such event that leads to loss, damage, or unauthorized access to, or use, alteration, disclosure or dissemination of, personal data, including personal data regarding our clinical trial subjects or employees, could harm our reputation directly, compel us to comply with federal and state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to substantial liability under laws, rules, regulations and standards that protect the privacy and security of personal data, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business. Additionally, we do not currently maintain cybersecurity insurance and therefore the successful assertion of one or more large claims against us in connection with a breach or other cybersecurity-related matter could adversely affect our business, financial condition, results of operations and prospects.

Notifications and follow-up actions related to a data breach or other security incident could impact our reputation and cause us to incur significant costs, including significant legal expenses and remediation costs. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the lost data. We expect to incur significant costs in an effort to detect and prevent security incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security incident. However, we cannot guarantee that we will be able to detect or prevent any such incidents, or that we can remediate any such incidents in an effective or timely manner. Our efforts to improve security and protect data from compromise may also identify previously undiscovered instances of data breaches or other cybersecurity incidents. To the extent that any data breach, disruption or security incident were to result in any loss, destruction, or alteration of, damage, unauthorized access to or inappropriate or unauthorized disclosure of or dissemination of, our data, including personal data, or other information that is processed or maintained on our behalf, we could be exposed to litigation and governmental investigations and inquiries, the further development and commercialization of our product candidates could be delayed, and we could be subject to significant fines or penalties for any noncompliance with certain state, federal and international privacy and security laws, rules, regulations and standards.

***Our operations are vulnerable to interruption by fire, earthquakes, power loss, telecommunications failure, terrorist activity, pandemics and other events beyond our control, which could harm our business.***

Our facilities are located in the San Francisco Bay Area. We have not undertaken a systematic analysis of the potential consequences to our business and financial results from a major flood, blizzard, wildfire, earthquake, power loss, terrorist activity, pandemics or other disasters and do not have a recovery plan for such disasters. In addition, we do not carry sufficient insurance to compensate us for actual losses from interruption of our business that may occur, and any losses or damages incurred by us could harm our business. Also, our CDMOs and suppliers' facilities are located in multiple locations where other natural disasters or similar events which could severely disrupt our operations, could expose us to liability and could have a material adverse effect on our business. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

***A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.***

We may seek regulatory approval of our product candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements and reimbursement regimes in foreign countries;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;

- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the FCPA or comparable foreign regulations;
- challenges obtaining, maintaining, protecting, defending and enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

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

***We have in-licensed one issued U.S. patent, own one issued U.S. patent, and own one allowed U.S. patent application relating to our technology and product candidates. If we are unable to obtain, maintain, protect, defend and enforce patent and other intellectual property coverage for our technology and product candidates, our competitors could develop and commercialize technology and product candidates similar or identical to ours, and our ability to commercialize our technology and product candidates may be adversely affected.***

Our commercial success depends in large part on our ability to obtain, maintain, protect, defend and enforce patents, trade secrets and other intellectual property for our product candidates and proprietary technologies and their uses, as well as our ability to operate without infringing, misappropriating or otherwise violating the intellectual property and proprietary rights of others. We rely on patent, copyright, trade secret and trademark laws in the United States and certain other countries to protect our proprietary technology, and 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. We also seek to protect our proprietary position by acquiring or in-licensing relevant issued patents or pending applications from third parties, but the efforts we and our licensors take to protect our intellectual property may provide only limited protection. In particular, the development of our product candidates and technology is at an early stage and consequently, our patent portfolio is also at an early stage. Presently, we own one issued U.S. patent, one allowed U.S. patent application and one other pending non-provisional U.S. patent application, seven non- expired Patent Cooperation Treaty (“PCT”) applications, nine pending foreign patent applications and at least five pending provisional U.S. patent applications. There can be no assurance that we or our licensors will obtain any additional issued patents or that any issued patents we or our licensors obtain will provide us with any competitive advantage. Any failure to obtain adequate patent protection for our product candidates and technology would have a material adverse effect on our business, financial condition, results of operations and prospects.

Pending 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 the patent applications of our licensors will result in additional patents being issued or that any such 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, narrowed in scope, or revoked in proceedings instituted by third parties before various patent offices or in courts in the United States and abroad. The degree of future protection for our and our licensor’s intellectual property and 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. These uncertainties and limitations in our ability to properly protect the intellectual property rights relating to our product candidates could have a material adverse effect on our financial condition and results of operations.

We cannot be certain that the claims in our U.S. pending patent applications, corresponding international patent applications and patent applications in certain foreign jurisdictions, or those of our licensors, will be considered patentable by the United States Patent and Trademark Office (“USPTO”), courts in the United States or by the patent offices and courts in foreign countries, nor can we be certain that any issued claims will not be found invalid or unenforceable if challenged. Additionally, our provisional applications may never result in issued patents. A U.S. provisional patent application expires twelve months from its filing date, and its subject matter can only be claimed in an issued patent if, among other things, we file a non-provisional patent application making a valid priority claim to that provisional patent application before it expires. If we do not timely file a non-provisional patent application, we may lose

the benefit of the priority dates of our provisional patent application, and intervening prior art may jeopardize patent protection on the inventions disclosed in such a provisional patent application. While we intend to timely file non-provisional patent applications claiming the benefit of the priority dates of our provisional patent applications, and otherwise diligently prosecute our patent rights, we cannot predict whether any of our future patent applications for our technology and product candidates will result in the issuance of patents that effectively protect our technology and product candidates. Additionally, our owned pending PCT patent applications are not eligible to become issued patents until, among other things, we file a national stage patent application within 30 months in the countries in which we seek patent protection. If we do not timely file any national stage patent applications, we may lose our priority date with respect to our PCT patent applications and any patent protection on the inventions disclosed in such PCT patent applications. If we or our licensors do not successfully obtain patent protection, or if the scope of the patent protection we or our licensors obtain is not sufficiently broad, valid, and enforceable, we may be unable to prevent others from using our technology, developing or commercializing similar or identical technology and products, or marketing competing products and technologies. Any failure to obtain or maintain patent protection with respect to our technology and product candidates would have a material adverse effect on our business, financial condition, results of operations and prospects.

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

- 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, 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 may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable, narrowed in scope 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;
- 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 and limiting the scope of our protection in countries outside the United States.

The patent prosecution process is also expensive and time-consuming, and we and our licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner or in all jurisdictions where protection may be commercially advantageous. It is also possible that we or our licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not be able to obtain or maintain patent applications and patents due to the subject matter claimed in such patent applications and patents being in disclosures in the public domain.

In addition, although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors 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. Consequently, we may not be able to prevent any third parties, including our competitors, from using any of our technology that is in the public domain to compete with our technology and 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.

***If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical product candidates would be adversely affected.***

The patent position of biotechnology companies generally is highly uncertain, involves complex legal and factual questions, and has been the subject of much litigation in recent years. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications and those of our licensors may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive product candidates or otherwise provide any commercial advantage.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own or in-license currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Our competitors or other third parties may avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (“Hatch-Waxman Amendments”) to conduct research and clinical trials and may be able to circumvent our patent rights by developing similar or alternative technologies or products in a non-infringing manner. Any patents that we may own or in-license may be challenged or circumvented by third parties or may be narrowed, rendered unenforceable, or invalidated as a result of challenges by third parties. Consequently, we do not know whether our product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our potential future patents or the patents of our licensors by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

While we believe our intellectual property allows us to pursue our current development programs, several companies and academic institutions are pursuing alternate approaches to gene therapy and have built intellectual property around these approaches and methods. In addition, we may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patent applications may not issue as patents and even if issued, may be challenged and invalidated or rendered unenforceable. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States may remain confidential until a patent issues.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and the inventorship, scope, validity or enforceability of our potential future patents or the patents of our licensors may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation, reexamination, post-grant review (“PGR”) and inter partes review (“IPR”), or other similar proceedings challenging any patents that we may own or in-license. Such submissions may also be made prior to a patent’s issuance, precluding the granting of a patent based on one of our owned or licensed pending patent applications. A third party may also claim that our potential future owned patents or licensed patent rights are invalid or unenforceable in a litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our potential future owned patents or licensed patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, our potential future patents or the patents of our licensors may become subject to post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our priority of invention or other features of patentability with respect to our potential future patents and patent applications and those of our licensors. Such challenges may result in loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our potential future patents and patent applications or the patents and patent applications of our licensors is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

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

The degree of current and 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 develop products that are similar to our product candidates but that are not covered by the claims of the patent applications that we own or license;
- we or our current or future licensors or collaborators might not have been the first to make the inventions covered by the patent applications that we own or license now or may own or license in the future;
- we or our current or future licensors or 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 the pending patent applications we own or license will not lead to issued patents;
- issued patents that we may hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors or other third parties;
- others may have access to the same intellectual property rights licensed to us in the future on a nonexclusive basis;
- 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;
- the patents of others may have an adverse effect on our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third-party may subsequently file a patent covering such intellectual property.

Should any of these events occur, it could significantly harm our business, results of operations and prospects.

***Our commercial success depends significantly on our ability to operate without infringing, misappropriating or otherwise violating the patents and other intellectual property and proprietary rights of third parties. Claims by third parties that we infringe, misappropriate or otherwise violate their intellectual property or proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts, and could have a material adverse effect on the success of our business.***

Our commercial success depends in part on avoiding infringement, misappropriation or other violation of the patents, intellectual property and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe, misappropriate or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and products that may be approved in the future, 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 industry, including patent infringement lawsuits, oppositions, reexaminations, IPR proceedings and PGR proceedings before the USPTO and 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 are and in the future 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. Given the vast number of patents in our field of technology, we cannot be certain or guarantee that we do not infringe existing patents or that we will not infringe patents that may be granted in the future.

For example, we are aware of third-party patent rights that could be construed to cover the use of our TN-201 product candidate. We believe that if these third-party patent rights were to be asserted against us, we would have valid defenses against such assertions, including that such patent rights are invalid and not infringed. However, if such third-party patent rights were asserted against us and found to be valid, enforceable and infringed, we could be liable for damages and be required to obtain a license to such patent rights prior to commercializing TN-201 in the United States, and such license may not be available on commercially reasonable terms or at all. Additionally, we are aware of third-party patent rights related to the use of certain AAV vectors, which have been asserted against others, including in at least one instance against a company for pre-approval activities. If these patent rights were to be asserted against us, we believe we would have valid defenses to such assertions, however such defenses may not be successful and we could be liable for damages and need to secure a license to such patent rights, which may not be available on commercially reasonable terms or

at all. In the event any of the foregoing were to occur, we may be prevented from further developing and commercializing any affected product candidates, including TN-201.

As the biotechnology industry expands and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement, misappropriation or other violation of the patent or other intellectual property rights of third parties. If any third-party claims that we infringe any of the above-referenced patent rights or any other patent rights, such claims would be time consuming and could:

- result in costly litigation that may cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing any of 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;
- subject us to significant liability and damages to third parties, including treble damages if we are found to willfully infringe third-party intellectual property; or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

Although no third party has asserted a claim of patent infringement against us as of the date of this periodic report, there can be no assurance that we will not be subject to claims of patent or other intellectual property infringement in the future that could prevent our product candidates from being marketed. Furthermore, we may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technology and product candidates. Because patent applications are maintained as confidential for a certain period of time, until the relevant application is published, we may be unaware of third-party patents that may be infringed by commercialization of any of our product candidates, and we 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. There is also no assurance that there is not prior art of which we are aware, but which we do not believe is relevant to our business, which may, nonetheless, ultimately be found to limit our ability to make, use, sell, offer for sale or import our products that may be approved in the future, or impair our competitive position. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

Third parties may assert patent infringement claims against us directed at any of our product candidates based on our existing patent applications or patents that may be granted in the future, regardless of their merit. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our products, treatment indications, or processes could subject us to significant 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. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be 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. In addition, even if we or our future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Moreover, we cannot be certain that we could redesign our product candidates, treatment indications, or processes to avoid infringement, if necessary, and we could encounter delays in our product introductions while we attempt to develop alternative technology and product candidates to avoid infringing third-party intellectual property rights. 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, which could harm our business, financial condition and operating results. 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.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection

with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Because of the inevitable uncertainty in intellectual property litigation, we could lose a patent infringement or other action asserted against us regardless of our perception of the merits of the case. Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. There is no assurance that a court would find in our favor on questions of infringement, validity, enforceability, or priority. A court of competent jurisdiction could hold that these third-party patents are valid, enforceable, and infringed, which could materially and adversely affect our ability to commercialize any future products we may develop and any other future products or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent or find that our technology did not infringe any such claims. Further, even if we were successful in defending against any such claims, such claims could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

We may in the future pursue invalidity proceedings with respect to third-party patents. The outcome following legal assertions of invalidity is unpredictable. Even if resolved in our favor, these legal proceedings 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 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 proceedings adequately. Some of these third parties may be able to sustain the costs of such proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent proceedings could compromise our ability to compete in the marketplace. If we do not prevail in the patent proceedings the third parties may assert a claim of patent infringement directed at our product candidates.

In addition, our agreements with some of our suppliers or other entities with whom we do business require us to defend or indemnify these parties to the extent they become involved in infringement claims, including the types of claims described above. We could also voluntarily agree to defend or indemnify third parties in instances where we are not obligated to do so if we determine it would be important to our business relationships. If we are required or agree to defend or indemnify third parties in connection with any infringement claims, we could incur significant costs and expenses that could adversely affect our business, operating results or financial condition.

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

Many pharmaceutical companies, biotechnology companies, and academic institutions may have patents and patent applications potentially relevant to our business. We may find it necessary or prudent to obtain licenses to such patents from such third-party intellectual property holders, for example, in order to avoid infringing these third-party patents. We may also require licenses from third parties for certain technologies for use with future product candidates. We may be unable to acquire or in-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 may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital 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 expect that competition for the in-licensing or acquisition of third-party intellectual property rights for future products that are attractive to us may increase in the future, which may mean fewer suitable opportunities for us as well as higher acquisition or licensing costs. We 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 or at all. 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 the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***We may be involved in lawsuits to protect or enforce our potential future patents or our licensors' patents, which could be expensive, time consuming and unsuccessful. Further, our potential future patents or our licensors' patents could be found invalid or unenforceable if challenged in court.***

Competitors and other third parties may infringe, misappropriate or otherwise violate our intellectual property rights. To prevent infringement, misappropriation, unauthorized use or other violation, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, our potential future patents also may become involved in inventorship, priority or validity disputes. In a patent infringement proceeding, a court may decide that a patent we may own or in-license is not valid, is unenforceable or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our potential future patents do not cover such technology. 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 potential future patent or the patent of our licensor is invalid or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity 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, lack of sufficient written description, non-enablement, or obviousness-type double patenting. 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 invalidity claims before the USPTO or patent offices abroad, even outside the context of litigation. Such mechanisms include re-examination, PGR, IPR, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in the revocation or cancellation of or amendment to our potential future patents in such a way that they no longer cover our technology or product candidates or prevent third parties from competing with our technology or product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our licensor, and the patent examiners are unaware during prosecution. There is also no assurance that there is not prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim in our potential future patents and patent applications or the patents and patent applications of our licensors, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or proprietary drug discovery platform, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

In addition, if the breadth or strength of protection provided by our patent applications or the patents and patent applications of our licensors is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

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.

In addition, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our own potential future patented product and practicing our own potential future patented technology.

***Intellectual property litigation may lead to unfavorable publicity that harms our reputation and causes the market price of our common shares to decline.***

During the course of any intellectual property litigation, there could be public announcements of the initiation of the litigation as well as results of hearings, rulings on motions, and other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our existing products, programs or intellectual property could be diminished. Accordingly, the market price of shares of our common stock may decline. Such announcements could also harm our reputation or the market for our future products, which could have a material adverse effect on our business.



***Derivation proceedings may be necessary to determine priority of inventions, and an unfavorable outcome may require us to cease using the related technology or to attempt to license rights from the prevailing party.***

Derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patent applications or the patents or patent applications 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 harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all. Our defense of derivation 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 such proceedings 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.

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

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and pharmaceutical industries involve a high degree of technological and legal complexity. Therefore, obtaining and enforcing biotechnology and pharmaceutical patents is costly, time consuming and inherently uncertain. Changes in either the patent laws or in the interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property and may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. We cannot predict the breadth of claims that may be allowed or enforced in our potential future patents or in third-party patents. In addition, the U.S. Congress or other foreign legislative bodies may pass patent reform legislation that is unfavorable to us.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. In 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 U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a first-to-invent system to a first inventor-to-file system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings, including post-grant review, *inter partes* review and derivation proceedings. Under a first-inventor-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor was the first to invent the claimed invention. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, became effective in 2013. The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition and results of operations. In addition, patent reform legislation may pass in the future that could lead to additional uncertainties and increased costs surrounding the prosecution, enforcement and defense of our patent applications.

Furthermore, the U.S. Supreme Court and the U.S. Court of Appeals for the Federal Circuit have made, and will likely continue to make, changes in how patent laws in the United States are interpreted. The U.S. 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. Similarly, foreign courts have made, and will likely continue to make, changes in how the patent laws in their respective jurisdictions are interpreted. We cannot predict future changes in the interpretation of patent laws or changes to patent laws that might be enacted into law by United States and foreign legislative bodies. Depending on decisions by the U.S. Congress, the U.S. federal courts, the USPTO, or similar authorities in foreign jurisdictions, 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 patent and the patents we might obtain or license in the future.

***We may be subject to claims challenging the inventorship or ownership of our owned patent applications or in-licensed patent rights and other intellectual property.***

We or our licensors may be subject to claims that former employees or other third parties have an ownership interest in our owned patent applications or in-licensed patents, trade secrets or other intellectual property rights as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or other third parties who are involved in developing our current or future products. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership of our patent applications or our licensors’ owned or in-licensed patents, trade

secrets or other intellectual property rights. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property rights that are important to our current or future product candidates. It may be necessary or we may desire to enter into a license to settle any such claim; however, there can be no assurance that we would be able to obtain a license on commercially reasonable terms, if at all. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and distraction to management and other employees, and any litigation or the threat of litigation may adversely affect our reputation or affect our ability to hire employees or contract with independent contractors.

In addition, while it is our policy to require our employees, consultants, advisors, contractors and other third parties who may be involved in the conception or development of intellectual property rights to execute agreements assigning such intellectual property rights to us, we or our licensors may be unsuccessful in executing such agreements with each party who, in fact, conceives or develops intellectual property rights that we regard as our own. The assignment of intellectual property rights may not be self-executing or sufficient in scope, or the assignment agreements may be breached, and we or our licensors may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property rights. Furthermore, individuals executing agreements with us may have preexisting or competing obligations to a third party, such as an academic institution, and thus an agreement with us or our licensors may be ineffective in perfecting ownership of inventions developed by that individual. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

***Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.***

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products. 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 patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Depending upon the timing, duration and specifics of FDA marketing approval of our product candidates, one or more of our U.S. patents or those of our licensors may be eligible for limited patent term restoration under 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. A maximum of one patent may 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.

We may not be granted any extensions for which we apply in the United States or any other jurisdiction because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, 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. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or restoration, or the foreign equivalent, or if the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

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

Filing, prosecuting and defending 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, even in jurisdiction where we do pursue patent protection. Consequently, we may not be able to prevent third parties from practicing our or our licensors' inventions in all countries outside the United States, even in jurisdictions where we or our

licensors due pursue patent protection, 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 potential future patents, the patents of our licensors, or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many foreign countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, which could make it difficult for us to stop the infringement of our potential future patents or our licensor's patents, if pursued and obtained, or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our or our licensors' 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 potential future patents or the patents of our licensors at risk of being invalidated or interpreted narrowly and our patent applications or the patent applications of our licensors at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors 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.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment and other requirements imposed by regulations and governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to the USPTO and various foreign patent offices outside of the United States at various points over the lifetime of our potential future patents and patent applications and those of our licensors. We have systems in place to remind us to pay these fees, and we rely on our outside patent annuity service to pay these fees when due. Additionally, the USPTO and various foreign patent offices require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. An inadvertent lapse or non-compliance with such requirements can sometimes be cured by payment of a late fee or by other means in accordance with rules applicable to the particular jurisdiction. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If such an event were to occur, it could have a material adverse effect on our business, financial condition and results of operations.

***If our trademarks and trade names 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.***

We intend to use registered or unregistered trademarks or trade names to brand and market ourselves and our products, but we do not yet own a U.S. registered trademark for our corporate name, "Tenaya". Our future trademark applications in the United States and in foreign jurisdictions may not be allowed or may subsequently be opposed. Once filed and registered, our potential future 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 potential future 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. As a means to enforce our potential future trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings, which can be expensive and time-consuming. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our potential future 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.

Additionally, our potential future registered trademarks may not be maintained or enforced. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our potential future trademark applications and registrations, and our potential future trademarks may not survive such proceedings. If we do not secure registrations for our potential future trademarks, we may encounter more difficulty in enforcing them against third parties than we otherwise would. Our efforts to enforce or protect our proprietary rights related to trademarks, domain names or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

***If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.***

In addition to seeking patent protection to protect the intellectual property underlying our technology and product candidates, we also rely on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information to maintain our competitive position. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties who have access to such information, and confidential information and invention assignment agreements with employees, consultants, advisors and other third parties involved in the development of intellectual property, we cannot guarantee that we and our licensors have entered into such agreements with each party that may have had access to our trade secrets or proprietary information or that has been involved in the development of intellectual property. Additionally, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach such agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. We may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. 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. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts 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. Furthermore, we expect these trade secrets, know-how and proprietary information to over time be disseminated within the industry through independent development, the publication of journal articles describing the methodology and the movement of personnel from academic to industry scientific positions. Consequently, we may be unable to prevent our proprietary technology from being exploited in the United States and abroad, which could affect our ability to expand in domestic and international markets or require costly efforts to protect our technology. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems, but such security measures may be breached, and we may not have adequate remedies for any such breach. 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.

***We may be subject to claims that we or our employees, consultants, advisors or contractors have wrongfully used or disclosed alleged confidential information or trade secrets.***

We have entered into and may enter in the future into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, lessees of shared multi-company property and other third parties. We may become subject to litigation where a third party asserts that we or our employees or other third parties inadvertently or otherwise breached the agreements and used or disclosed trade secrets or other information proprietary to the third parties. Defense of such matters, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions. Moreover, 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. Failure to defend against any such claim could subject us to significant liability for monetary damages or prevent or delay our developmental and commercialization efforts, which could adversely affect 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 team and other employees.

Parties making claims against us may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. 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. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, operating results, financial condition and prospects.

***We may be subject to claims that we have wrongfully hired an employee from our competitors or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.***

As is common in the biotechnology and pharmaceutical industry, 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 and pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or consultants inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. 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 or personnel, which could adversely affect 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 team and other employees.

***Our rights to develop and commercialize our technology and product candidates may be subject, in part, to the terms and conditions of licenses granted to us by others.***

We have entered into a license agreement with UTSW pursuant to which we have acquired the exclusive right to certain patents and patent applications relating to therapeutics overexpressing the peptide named Dwarf Open Reading Frame, and have entered into various other license agreements with other third parties. We may enter into additional license agreements in the future with other third parties to advance our research or allow commercialization of product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our licensors fail to prosecute, maintain, enforce, and defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our products that are subject of such licensed rights could be adversely affected.

Our licensors may have relied on third-party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Additionally, our licenses may be subject to certain rights of third parties, and, as a result, our current and future licenses may not provide us with exclusive rights to use the licensed intellectual property and technology. For example, the intellectual property we license from UTSW is subject to certain non-commercial rights reserved by UTSW and certain rights retained by the U.S. government, including march-in rights. Patents licensed to us could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against our licensors or another licensee or in administrative proceedings brought by or against our licensors or another licensee in response to such litigation or for other reasons. As a result, we may not be able to prevent competitors or other third parties from developing and commercializing competitive products, including in territories covered by our licenses.

It is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, manufacturing methods, product candidates,

or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

Our current licenses impose, and our future licenses likely will impose, various diligence, royalty payment, milestone payment, insurance and other obligations on us. If we fail to comply with any of these or other obligations in our license agreements, we may be required to pay damages and the licensor may have the right to terminate the licenses. 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. If our license agreements terminate, or we experience a reduction or elimination of licensed rights under these agreements, we may have to negotiate new or reinstated licenses with less favorable terms or we may not have sufficient intellectual property rights to operate our business.

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 we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

Disputes may arise between us and our current or future licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- whether third parties are entitled to compensation or equitable relief, such as an injunction, for our use of the intellectual property rights without their authorization;
- our involvement or lack of involvement in the prosecution, defense, and enforcement of licensed patents and our licensors' overall patent enforcement strategy;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the amounts of royalties, milestones or other payments due under the license agreement;
- our right to transfer or assign the license;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, or are insufficient to provide us the necessary rights to use the intellectual property rights, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

In spite of our efforts, our current and future licensors might conclude that we have materially breached our obligations under our license agreements and might therefore terminate such license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

***The patent protection and patent prosecution for some of our product candidates may be dependent on third parties.***

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to our product candidates, there may be times when the filing and prosecution activities for patents relating to our product candidates are controlled by our licensor, potential licensors or collaboration partners. If any of our licensor, potential licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering our product candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensees, our licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

***Intellectual property discovered through government funded programs may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers.***

We have in-licensed certain patents and patent applications that were generated through the use of U.S. government funding or grants, and we may acquire or license in the future intellectual property rights that have been generated through the use of U.S. government funding or grants. Pursuant to the Bayh-Dole Act of 1980, the U.S. government has certain rights in inventions developed with government funding. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right, under certain limited circumstances, to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third-party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as march-in rights). For example, the intellectual property we license from UTSW is subject to certain rights retained by the U.S. government, including march-in rights. If the U.S. government exercises its march-in rights in our current or future intellectual property rights that are generated through the use of U.S. government funding or grants, we could be forced to license or sublicense intellectual property developed by us or that we license on terms unfavorable to us, and there can be no assurance that we would receive compensation from the U.S. government for the exercise of such rights. The U.S. government also has the right to take title to these inventions if the grant recipient fails to disclose the invention to the government or fails to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying any of these inventions or produced through the use of any of these inventions be manufactured substantially in the United States. This preference for U.S. industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. industry may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. Any failure by us to comply with federal regulations regarding intellectual property rights that were developed through the use of U.S. government funding could have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

***We rely on third parties to conduct our preclinical studies, and plan to rely on third parties to conduct clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research and studies or to comply with applicable regulatory requirements, which may harm our business.***

We utilize and depend upon independent investigators and collaborators, such as medical institutions, CROs and strategic partners to conduct and support our preclinical studies and plan to continue to do so for our future clinical trials. These third parties have had and will continue to have a significant role in the conduct of our preclinical studies and planned clinical trials and the subsequent collection and analysis of data.

These third parties are not our employees, and except for remedies available to us under our agreements with such third parties, we have limited ability to control the amount or timing of resources that any such third party will devote to our preclinical studies or our planned clinical trials. Furthermore, the competition for third parties has increased as a result of the COVID-19 pandemic. The third parties we rely on for these services may also have relationships with other entities, some of which may be our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. Some of these third parties may terminate their engagements with us at any time. We also expect to have to negotiate budgets and contracts with CROs and clinical trial sites and we may not be able to do so on favorable terms, which may result in delays to our development timelines and increased costs. If we need to enter into alternative arrangements with, or replace or add any third parties, it would involve substantial cost and require extensive management time and focus, or involve a transition period, and may delay our drug development activities, as well as materially impact our ability to meet our desired clinical development timelines.

Our heavy reliance on these third parties for such drug development activities will reduce our control over these activities. As a result, we will have less direct control over the conduct, timing and completion of preclinical studies and clinical trials and the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Nevertheless, we are responsible for ensuring that each of our studies and trials is conducted in accordance with applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. For example, we will 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 standards, regulations for conducting, recording and reporting the results of clinical trials to assure that data and reported results are reliable and accurate and that the rights, integrity and confidentiality of trial participants are protected. EMA also requires us to comply with similar standards. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. There can be no assurance that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials substantially comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under current cGMP regulations and will require a large number of test patients. Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients, may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

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 our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

***We contract with third parties for the production of our product candidates for preclinical studies and expect to continue to do so for additional preclinical studies, clinical trials and ultimately for commercialization for certain of our product candidates. This reliance on third parties increases the risk that we will not have sufficient quality and quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.***

We do not currently have the infrastructure or internal capability to manufacture supplies of our product candidates for use in development and commercialization. We plan to fully integrate and internalize AAV manufacturing capabilities to support our Gene Therapy and Cellular Regeneration platforms and have initiated construction of a cGMP manufacturing facility. Until our manufacturing facility is complete and operational, we will continue to rely on third-party manufacturers for our Gene Therapy and Cellular Regeneration platforms. Moreover, to optimize our use of resources and utilize extensive third-party experience in small molecule manufacturing, we intend to continue to rely on third-party manufacturers for our small molecule programs.

Competition for third-party manufacturers and supplies has increased as a result of the COVID-19 pandemic. Changing third-party manufacturers could result in delays in our manufacturing supply chain which could delay or otherwise impact development of our programs and result in increased costs.

We do not have long-term supply agreements, and we purchase our required drug product on a purchase order basis, which means that aside from any binding purchase orders we have from time to time, our supplier could cease supplying to us or change the terms on which it is willing to continue supplying to us at any time. If we were to experience an unexpected loss of supply of our product candidates for any reason, whether as a result of manufacturing, supply or storage issues or otherwise, we could experience



delays, disruptions, suspensions or terminations of, or be required to restart or repeat, any pending or ongoing preclinical studies or clinical trials.

We may be unable to maintain or establish required agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the failure of the third party to manufacture our product candidates according to our schedule and specifications, or at all, including if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the termination or nonrenewal of arrangements or agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements, including cGMPs;
- the failure of the third party to manufacture our product candidates according to our specifications;
- the mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the infringement, misappropriation or other violation of our intellectual property or proprietary information, including our trade secrets and know-how.

We do not have complete control over all aspects of the manufacturing process of our CDMOs and are dependent on these CDMOs for compliance with cGMP regulations for manufacturing active pharmaceutical ingredients (“API”), drug substance and finished drug products. We are in the process of developing our supply chain for each of our product candidates and intend to put in place framework agreements under which CDMOs will generally provide us with necessary quantities of API, drug substance and drug product on a project-by-project basis based on our development needs. As we advance our product candidates through development, we will consider our lack of redundant supply for the API, drug substance and drug product for each of our product candidates to protect against any potential supply disruptions. However, we may be unsuccessful in putting in place such framework agreements or protecting against potential supply disruptions.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside of the United States. If our CDMOs cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA, EMA or others, they will not be able to secure and/or maintain marketing approval for their manufacturing facilities. In addition, we do not have control over the ability of our CDMOs to maintain adequate QC, QA, facilities, equipment and qualified personnel. If the FDA, EMA 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 will need to find alternative manufacturing facilities, and those new facilities would need to be inspected and approved by FDA, EMA or comparable regulatory authority prior to commencing manufacturing, which would significantly impact our ability to develop, obtain marketing approval for or market our product candidates, if approved. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates or drugs and harm our business and results of operations.

We rely on third-party suppliers for the raw materials required for the production of our product candidates for all of our programs. Our reliance on third-party supplies will continue even after we operationalize our cGMP manufacturing facility to support our Gene Therapy and Cellular Regeneration platforms. 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, especially in light of the supply chain issues caused by the effects of the COVID-19 pandemic, 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.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

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

Because we currently rely on third parties in the course of our business, we may share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements, and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements, invention assignment or other similar agreements with our collaborators, advisors, employees, consultants and other third parties prior to beginning research or disclosing trade secrets or proprietary information. These agreements typically limit the rights of the third parties to use or disclose our trade secrets and confidential information. 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 intentionally or 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 and despite our efforts to protect our trade secrets, our competitors' discovery of our proprietary technology, trade secrets or confidential information or other unauthorized use or disclosure of such information would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

***If we engage in future 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.***

From time to time, we evaluate various acquisition opportunities and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing programs and initiatives in pursuing such a strategic merger or acquisition;
- 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 marketing approvals;
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs; and
- our inability to realize anticipated efficiencies and strategic benefits from such acquisitions or strategic partnerships.

In addition, if we undertake acquisitions or pursue partnerships in the future, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

***If we decide to establish collaborations, but are not able to establish those collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.***

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We strategically evaluate collaborations and partnerships with biopharmaceutical companies that may have more robust and complementary capabilities and resources to accelerate the development and maximize the availability and

potential of our product candidates, particularly for more prevalent indications. The relationships may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business.

We would face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, EMA or comparable foreign regulatory authorities, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of intellectual property and industry and market conditions generally. The potential collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. Further, we may not be successful in our efforts to establish a collaboration or other alternative arrangements for product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view them as having the requisite potential to demonstrate safety and efficacy.

In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Even if we are successful in entering into a collaboration, the terms and conditions of that collaboration may restrict us from entering into future agreements on certain terms with potential collaborators.

If and when we seek to enter into collaborations, 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 a product candidate, reduce or delay its development program or one or more of our other development programs, 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 our product candidates or bring them to market and generate product revenue.

***We may enter into collaborations with third parties for the development and commercialization of product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.***

If we enter into any collaboration arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations involving our product candidates would pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to, and the manner in which they perform their obligations under, these collaborations and may not
- perform their obligations as expected;
- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, including as a result of a business combination or sale or disposition of a business unit or development function, 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 program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could 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;
- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product relative to other products;
- we may grant exclusive rights to our collaborators that would prevent us from collaborating with others;

- collaborators may not properly obtain, maintain, defend, protect or enforce our intellectual property rights or may use our proprietary information and intellectual property in such a way as to invite litigation or other intellectual property-related proceedings that could jeopardize or invalidate our proprietary information and intellectual property or expose us to potential litigation or other intellectual property related proceedings;
- 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;
- 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;
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all;
- collaborators may not provide us with timely and accurate information regarding development progress and activities under the collaboration or may limit our ability to share such information, which could adversely impact our ability to report progress to our investors and otherwise plan our own development of our product candidates;
- collaborators may own or co-own intellectual property covering our products 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.

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.

***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 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 due to the impacts of the COVID-19 pandemic;
- 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.

### **Risks Related to the Securities Market and Ownership of Our Common Stock**

***An active, liquid and orderly trading market may not continue to be developed or sustained for our common stock and as a result it may be difficult for you to sell your shares of our common stock.***

Prior to our initial public offering, no market for shares of our common stock existed. The trading market for our common stock on the Nasdaq Global Select Market has been limited and an active trading market for our shares may not be sustained. The lack of an active market may also reduce the fair market value of your shares of common stock. Furthermore, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic collaborations or acquire companies, technologies or other assets by using our shares of common stock as consideration.

***The price of our stock may be volatile, and you could lose all or part of your investment.***

The trading price of our common stock is likely to be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. The stock market in general, and pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies.

Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In addition to the factors discussed in this “Risk Factors” section and elsewhere in this periodic report, these factors include:

- the timing and results of preclinical studies and clinical trials of our product candidates, those conducted by third parties or those of our competitors;
- the success of competitive products or announcements by potential competitors of their product development efforts;
- regulatory actions with respect to our products or our competitors’ products;
- actual or anticipated changes in our growth rate relative to our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, collaborations or capital commitments;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;

- market conditions in the pharmaceutical and biotechnology sector;
- changes in the structure of healthcare payment systems;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- expiration of market stand-off or lock-up agreements;
- the impact of any natural disasters or public health emergencies, such as the COVID-19 pandemic;
- the availability of fiscal and monetary stimulus measures to counteract the impact of natural disasters or public health emergencies, such as the COVID-19 pandemic;
- fluctuations in interest rates and inflation rates; and
- general economic, political, industry and market conditions.

The realization of any of the above risks or any of a broad range of other risks, including those described in this “Risk Factors” section, could have a dramatic and adverse impact on the market price of our common stock.

***Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.***

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. From time to time, we may enter into license or collaboration agreements or strategic partnerships with other companies that include development funding and significant upfront and milestone payments and/or royalties, which may become an important source of our revenue. These upfront and milestone payments may vary significantly from period to period and any such variance could cause a significant fluctuation in our operating results from one period to the next.

In addition, we measure compensation cost for stock-based awards made to employees at the grant date of the award, based on the fair value of the award as determined by our board of directors, and recognize the cost as an expense over the employee’s requisite service period. As the variables that we use as a basis for valuing these awards change over time, including, our underlying stock price and stock price volatility, the magnitude of the expense that we must recognize may vary significantly.

Furthermore, our operating results may fluctuate due to a variety of other factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing and cost of, and level of investment in, research and development activities relating to our programs, product candidates and any future programs and product candidates, which will change from time to time;
- our ability to enroll patients in clinical trials and the timing of enrollment;
- the cost of establishing and operating a manufacturing facility and manufacturing our product candidates and any future product candidates, which may vary depending on FDA, EMA or other comparable foreign regulatory authority guidelines and requirements, the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies or other assets;
- the timing and outcomes of clinical trials for our product candidates, or competing product candidates;
- the need to conduct unanticipated clinical trials or trials that are larger or more complex than anticipated;
- competition from existing and potential future products that compete with our programs and product candidates, and changes in the competitive landscape of our industry, including consolidation among our competitors or partners;
- any delays in regulatory review or approval of our product candidates;
- the level of demand for our product candidates, if approved, which may fluctuate significantly and be difficult to predict;
- the risk/benefit profile, cost and reimbursement policies with respect to our product candidates, if approved, and existing and potential future products that compete with any of our product candidates;
- our ability to commercialize any of our product candidates, if approved, inside and outside of the United States, either independently or working with third parties;

- our ability to establish and maintain collaborations, licensing or other arrangements;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies;
- the changing and volatile global economic and political environment; and
- increased impact from the COVID-19 pandemic on the costs and timing associated with the conduct of our clinical trial and other related business activities.

The cumulative effect 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 industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below 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 guidance we may provide.

***Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

As of September 30, 2021, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 50.0% of our common stock. These stockholders, acting together, may be able to control matters requiring stockholder approval. For example, they may be able to control elections of directors, amendments of our organizational documents or approval of any merger, sale of assets or other major corporate transactions. This concentration of ownership control may delay, discourage or prevent a change of control, including unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders, entrench our management and board of directors or delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

***Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.***

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 intend to sell shares, could reduce the market price of our common stock.

As of September 30, 2021, we had 41,280,402 outstanding shares of common stock on an as converted basis. Of these shares, 13,800,000 shares are freely tradable and substantially all of the remaining shares of common stock will be available for sale in the public market beginning in January 2022 following the scheduled expiration of lock-up agreements that certain of our stockholders and the underwriters entered into in connection with our IPO. Morgan Stanley & Co. LLC, Piper Sandler & Co. and Cowen and Company, LLC may release these stockholders from their lock-up agreements with the underwriters at any time and without notice, which would allow for earlier sales of shares in the public market.

In addition, we have filed a registration statement on Form S-8 under the Securities Act registering the issuance of 7,177,883 shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under the registration statement on Form S-8 can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described above.

Moreover, as of September 30, 2021, holders of an aggregate of 26,102,278 shares of our common stock had rights, subject to certain 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. If we were to register the resale of these shares, they could be freely sold in the public market beginning in February 2022 following the scheduled expiration of lock-up agreements that certain of our stockholders and the underwriters entered into in connection with our initial public offering.

Sales of a substantial number of such shares upon expiration of the lock-up and market stand-off agreements, the perception that such sales may occur, or early release of these agreements, could cause our market price to fall or make it more difficult for you to sell your common stock at a time and price that you deem appropriate.

***We are an “emerging growth company” and a “smaller reporting company” and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies will make our common stock less attractive to investors.***

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012 (“JOBS Act”). For as long as we continue to be an emerging growth company, we intend to take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure in our periodic reports;
- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended (“Sarbanes-Oxley Act”);
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements; and
- exemptions from the requirements of holding nonbinding advisory stockholder votes on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards 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, will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, our financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

We will remain an emerging growth company until the earliest to occur of: (1) the last day of the fiscal year in which we have more than \$1.07 billion in annual revenue; (2) the date we qualify as a “large accelerated filer,” with at least \$700.0 million of equity securities held by non-affiliates; (3) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period; and (4) the last day of the fiscal year ending after the fifth anniversary of our IPO.

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company,” as defined in the Securities Exchange Act of 1934, as amended (“Exchange Act”), which would allow us to continue to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

We cannot predict if investors will find our common stock less attractive because we may 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.

***If securities or industry analysts do not publish research or reports, or if they publish adverse or misleading research or reports, regarding us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish about us, our business or our market. We currently have research coverage from a limited number of securities or industry analysts. If no or few new securities or industry analysts commence coverage of us, the stock price may be negatively impacted. If any of the analysts who cover us issue adverse or misleading research or reports regarding us, our business model, our intellectual property, our stock performance or our market, or if our operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.



***We will incur increased costs as a result of operating as a public company, and our management will devote substantial time to related compliance initiatives. Additionally, if we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.***

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an “emerging growth company.” We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and the Nasdaq Stock Market LLC (“Nasdaq”), including the Nasdaq Board Diversity Rule, which was approved by the SEC on August 6, 2021, regarding the diversity of our board of directors. We are also subject to more stringent state law requirements. For example, under California law, we will be required to have at least three female directors on our board of directors and one director from an “underrepresented community” by December 31, 2021, and two additional directors from an “underrepresented community” by December 31, 2022. A director from an “underrepresented community” means a director who self-identifies as Black, African American, Hispanic, Latino, Asian, Pacific Islander, Native American, Native Hawaiian, Alaska Native, gay, lesbian, bisexual or transgender. We cannot guarantee that we can recruit, attract and/or retain qualified members of the board of directors and meet gender and diversity requirements under California law or the Nasdaq rule, which may expose us to financial penalties and adversely affect our reputation.

Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules, regulations and standards substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly, which will increase our operating expenses. For example, we expect these rules, regulations and standards to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage, particularly in light of recent cost increases related to coverage. We cannot accurately predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

In addition, as a public company we are required to incur additional costs and obligations in order to comply with SEC rules that implement Section 404 of the Sarbanes-Oxley Act. Under these rules, beginning with our second annual report on Form 10-K after we become a public company, we will be required to make a formal assessment of the effectiveness of our internal control over financial reporting, and once we cease to be an emerging growth company, we will be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaging in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially continue to engage outside consultants and adopt a detailed work plan to assess and document the adequacy of our internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are designed and operating effectively, and implement a continuous reporting and improvement process for internal control over financial reporting.

The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation to meet the detailed standards under the rules. During the course of its testing, our management may identify material weaknesses or deficiencies which may not be remedied in time to meet the deadline imposed by the Sarbanes-Oxley Act.

Our internal control over financial reporting will not prevent or detect all errors and all fraud or prevent material weaknesses from being identified in such reporting. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system’s objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

***Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.***

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and

forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the facts that judgments in decision-making can be faulty and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements due to error or fraud may occur and not be detected.

***We may be subject to securities litigation, which is expensive and could divert management attention.***

The market price of our common stock may be volatile and, in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. This risk is especially relevant for us because biotechnology companies have experienced significant stock price volatility in recent years and we may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

***We do not intend to pay dividends on our common stock in the foreseeable future, so any returns will be limited to the value of our common stock.***

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to any appreciation in the value of their stock.

***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. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. These provisions, among other things:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit only the board of directors to establish the number of directors and fill vacancies on the board;
- provide that directors may only be removed "for cause" and only with the approval of two-thirds of our stockholders;
- authorize the issuance of "blank check" preferred stock that our board could use to implement a stockholder rights plan (also known as a poison pill);
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting;
- authorize our board of directors to amend the bylaws;
- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings; and
- require a super-majority vote of stockholders to amend or repeal specified provisions of our amended and restated certificate of incorporation and amended and restated bylaws.

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 amended and restated certificate of incorporation, amended and restated 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.

***Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America are the exclusive forums for substantially all 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 bylaws provide that the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, another State court in Delaware or the federal district court for the District of Delaware) is the exclusive forum for the following (except for any claim as to which such court determines that there is an indispensable party not subject to the jurisdiction of such court (and the indispensable party does not consent to the personal jurisdiction of such court within 10 days following such determination), which is vested in the exclusive jurisdiction of a court or forum other than such court or for which such court does not have subject matter jurisdiction):

- 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 under the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction.

Our amended and restated bylaws further provide that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage lawsuits against us and our directors, officers and other employees. In addition, these exclusive-forum provisions may impose additional litigation costs for stockholders who determine to pursue any such lawsuits against us.

Any person or entity purchasing or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and consented to these provisions. There is uncertainty as to whether a court would enforce such provisions, and the enforceability of similar choice of forum provisions in other companies' charter documents has been challenged in legal proceedings. It is possible that a court could find these types of provisions to be inapplicable or unenforceable, and if a court were to find either exclusive-forum provision in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

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

### **(a) Sales of Unregistered Securities**

From July 1, 2021 through July 30, 2021 (the date of the filing of our registration statement on Form S-8, File No. 333-258337), we granted to our employees stock options to purchase an aggregate of an aggregate of 446,998 shares of our common stock under the 2016 Plan at an exercise price of \$10.56 per share.

The sales of the above securities were deemed to be exempt from registration under the Securities Act in reliance upon Section 4 (a)(2) of the Securities Act (or Regulation D or Regulation S promulgated thereunder), or Rule 701 promulgated under Section 3(b) of the Securities Act as transactions by an issuer not involving any public offering or pursuant to benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed upon the stock certificates issued in these transactions.

**(b) Use of Proceeds from Public Offering of Common Stock**

On August 3, 2021, we completed our IPO and issued an aggregate of 13,800,000 shares of its common stock (inclusive of 1,800,000 shares pursuant to the underwriters' over-allotment option) at a price of \$15.00 per share. The gross proceeds from the offering for shares sold in our IPO was \$207.0 million. We received net proceeds from the IPO of approximately \$188.5 million, after deducting underwriting discounts and commissions of approximately \$14.5 million and offering expenses of approximately \$4.0 million. None of the expenses associated with the IPO were paid to directors, officers, persons owning 10% or more of any class of equity securities, or to their associates. Morgan Stanley & Co. LLC, Piper Sandler & Co. and Cowen and Company, LLC acted as book-running managers for the IPO. Shares of our common stock began trading on the Nasdaq Global Select Market on July 30, 2021. The offer and sale of the shares were registered under the Securities Act on a registration statement on Form S-1 (Registration No. 333-257820), which was declared effective on July 29, 2021. There has been no material change in the planned use of proceeds from our IPO as described in the registration statement on Form S-1.

**(c) Issuer Purchases of Equity Securities**

None.

**Item 3. Defaults Upon Senior Securities**

Not applicable.

**Item 4. Mine Safety Disclosures**

Not applicable.

**Item 5. Other Information**

None.

**Item 6. Exhibits**

<b>Exhibit Number</b>	<b>Description</b>	<b>Form</b>	<b>File No.</b>	<b>Exhibit</b>	<b>Filing Date</b>
3.1	<a href="#">Amended and Restated Certificate of Incorporation of Tenaya Therapeutics, Inc.</a>	8-K	001-40656	3.1	8-3-2021
3.2	<a href="#">Amended and Restated Bylaws of Tenaya Therapeutics, Inc.</a>	8-K	001-40656	3.2	8-3-2021
4.1	<a href="#">Specimen common stock certificate of the Registrant</a>	S-1/A	333-257820	4.2	7-26-2021
10.1	<a href="#">Form of Indemnification Agreement between the Registrant and each of its directors and executive officers.</a>	S-1/A	333-257820	10.1	7-26-2021
10.2	<a href="#">2021 Equity Incentive Plan and forms of agreements thereunder.</a>	S-1/A	333-257820	10.3	7-26-2021
10.3	<a href="#">2021 Employee Stock Purchase Plan and forms of agreements thereunder.</a>	S-1/A	333-257820	10.4	7-26-2021
10.4	<a href="#">Employment Letter between the Registrant and Faraz Ali, M.B.A.</a>	S-1/A	333-257820	10.5	7-26-2021
10.5	<a href="#">Employment Letter between the Registrant and Timothy Hoey, Ph.D.</a>	S-1/A	333-257820	10.6	7-26-2021
10.6	<a href="#">Employment Letter between the Registrant and Leone D. Patterson, M.B.A.</a>	S-1/A	333-257820	10.7	7-26-2021
10.7	<a href="#">Employment Letter between the Registrant and Whittemore (Whit) Tingley, M.D., Ph.D.</a>	S-1/A	333-257820	10.8	7-26-2021
10.8	<a href="#">Executive Change in Control and Severance Plan.</a>	S-1/A	333-257820	10.12	7-26-2021
10.9	<a href="#">Executive Incentive Compensation Plan.</a>	S-1/A	333-257820	10.13	7-26-2021
10.10	<a href="#">Outside Director Compensation Policy.</a>	S-1/A	333-257820	10.14	7-26-2021
31.1*	<a href="#">Certification of 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.</a>				
31.2*	<a href="#">Certification of 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.</a>				
32.1†	<a href="#">Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</a>				
101.INS*	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.				
101.SCH*	Inline XBRL Taxonomy Extension Schema Document				
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document				
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document				

101.LAB\* Inline XBRL Taxonomy Extension Label Linkbase Document  
101.PRE\* Inline XBRL Taxonomy Extension Presentation Linkbase Document  
104\* Cover Page Interactive Data File (embedded within the Inline XBRL document)

\* Filed herewith.

† The certifications attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of the Registrant 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, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Tenaya Therapeutics, Inc.

Date: November 10, 2021

By: \_\_\_\_\_  
/s/ Faraz Ali, M.B.A.  
**Faraz Ali, M.B.A.**  
**Chief Executive Officer and Director**  
**(Principal Executive Officer)**

Date: November 10, 2021

By: \_\_\_\_\_  
/s/ Leone D. Patterson, M.B.A.  
**Leone D. Patterson, M.B.A.**  
**Chief Financial and Business Officer**  
**(Principal Financial and Accounting Officer)**

**CERTIFICATION OF 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**

I, Faraz Ali, M.B.A., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Tenaya 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)) 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) 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
  - (c) 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.

Date: November 10, 2021

TENAYA THERAPEUTICS, INC.

By: /s/ Faraz Ali, M.B.A.  
Name: Faraz Ali, M.B.A.  
Title: Chief Executive Officer and Director (*Principal Executive Officer*)



**CERTIFICATION OF 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**

I, Leone D. Patterson, M.B.A., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Tenaya 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)) 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) 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
  - (c) 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.

Date: November 10, 2021

TENAYA THERAPEUTICS, INC.

By: /s/ Leone D. Patterson, M.B.A.  
Name: Leone D. Patterson, M.B.A.  
Title: Chief Financial and Business Officer  
(Principal Financial and Accounting Officer)

**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**

I, Faraz Ali, M.B.A., certify, pursuant to 18 U.S.C. Section 1350 as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, (1) the Quarterly Report on Form 10-Q of Tenaya Therapeutics, Inc. for the quarterly period ended September 30, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Faraz Ali, M.B.A.

\_\_\_\_\_  
Faraz Ali, M.B.A.

Chief Executive Officer and Director

*(Principal Executive Officer)*

Date: November 10, 2021

I, Leone D. Patterson, M.B.A., certify, pursuant to 18 U.S.C. Section 1350 as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, (1) the Quarterly Report on Form 10-Q of Tenaya Therapeutics, Inc. for the quarterly period ended September 30, 2021, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Leone D. Patterson, M.B.A.

\_\_\_\_\_  
Leone D. Patterson, M.B.A.

Chief Financial and Business Officer

*(Principal Financial and Accounting Officer)*

Date: November 10, 2021