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, 2026

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	**81-3789973**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
171 Oyster Point Boulevard, Suite 500 South San Francisco, CA	**94080**
(Address of principal executive offices)	**(Zip Code)**

(650) 825-6990
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	TNYA	Nasdaq Capital 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	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

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

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

As of October 29, 2026, the registrant had 227,928,240 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 (Quarterly Report) contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (Exchange Act). 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:

- our vision to change the treatment paradigm for heart disease;
- the ability of our ongoing preclinical studies and ongoing or planned clinical trials to demonstrate safety and efficacy of our product candidates, and other positive results;
- the timing, dosing, patient enrollment and populations, progress, and results of preclinical studies and ongoing or planned clinical trials for our current product candidates and other product candidates we may develop;
- the timing, scope and likelihood of regulatory filings, approvals, and alignment, including timing of investigational new drugs (INDs), clinical trial applications (CTAs), U.S. Food and Drug Administration (FDA) approvals, engagement with regulators, 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 trials;
- the size of the market opportunity for our product candidates, including our estimates of the number of patients who suffer from the diseases we are targeting;
- our manufacturing, commercialization, and marketing capabilities and strategy;
- our competitive position, potential advantages of our products compared to our competitors, 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, and our belief in the potential applications of our current product candidates to additional indications and targets;
- our collaboration with Alnylam, including potential targets, research and development strategy, the timing and duration of the collaboration, expected payments, and the achievement of milestones;
- the impact of existing laws and regulations and regulatory developments in the United States (U.S.), Europe and other jurisdictions;
- the impact of tariffs, trade restrictions, and other trade policy changes on our costs and supply chain;
- our intellectual property position, including the scope and length 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 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;
- our ability to regain or maintain compliance with Nasdaq's continued listing requirements;
- 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, cash outlays, capital requirements and needs for additional financing, including expenses arising as a result of being a public company;
- our financial performance;
- our facilities;
- 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;
- the impact of critical accounting policies on investors' ability to understand our financial performance; and
- our expectations regarding the period during which we will remain an emerging growth company under the Jumpstart Our Business Startups Act of 2012 (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 such as "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)
(Unaudited)

	September 30, 2026	December 31, 2025
ASSETS		
Current assets:		
Cash and cash equivalents	$ 66,797	$ 100,547
Accounts receivable	573	—
Prepaid expenses and other current assets	3,643	5,039
Total current assets	71,013	105,586
Property and equipment, net	1,136	27,672
Operating lease right-of-use assets	2,470	9,417
Other noncurrent assets	2,062	4,246
Total assets	$ 76,681	$ 146,921
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,660	$ 3,581
Accrued and other current liabilities	7,061	8,835
Contract liabilities, current	5,663	—
Operating lease liabilities, current	2,420	3,020
Total current liabilities	18,804	15,436
Contract liabilities, noncurrent	2,876	—
Operating lease liabilities, noncurrent	432	7,810
Other noncurrent liabilities	52	410
Total liabilities	22,164	23,656
Commitments and contingencies (Note 5)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 200,000,000 shares authorized as of September 30, 2026 and December 31, 2025; no shares issued and outstanding as of September 30, 2026 and December 31, 2025	—	—
Common stock, $0.0001 par value; 1,000,000,000 shares authorized as of September 30, 2026 and December 31, 2025; 227,928,240 and 216,760,283 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	22	21
Additional paid-in capital	741,106	728,252
Accumulated deficit	(686,611)	(605,008)
Total stockholders' equity	54,517	123,265
Total liabilities and stockholders' equity	$ 76,681	$ 146,921

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,	
	2026	**2025**	**2026**	**2025**
Revenue				
Collaboration revenue	$ 1,697	$ —	$ 3,032	$ —
Operating expenses:				
Research and development	14,866	15,363	46,313	53,809
General and administrative	5,648	5,573	16,501	18,747
Impairment loss	666	—	22,487	—
Loss on lease termination, net	—	—	1,368	—
Loss on disposal of property and equipment	89	—	89	—
Total operating expenses	21,269	20,936	86,758	72,556
Loss from operations	(19,572)	(20,936)	(83,726)	(72,556)
Other income, net:				
Interest income	635	658	2,126	2,107
Other (loss) income, net	(1)	3	(3)	27
Total other income, net	634	661	2,123	2,134
Net loss before income tax expense	(18,938)	(20,275)	(81,603)	(70,422)
Income tax expense	—	—	—	—
Net loss	(18,938)	(20,275)	(81,603)	(70,422)
Other comprehensive loss				
Net unrealized gain (loss) on marketable securities	—	1	—	(28)
Comprehensive loss	$ (18,938)	$ (20,274)	$ (81,603)	$ (70,450)
Net loss per share, basic and diluted	$ (0.08)	$ (0.12)	$ (0.37)	$ (0.48)
Weighted-average shares used in computing net loss per share, basic and diluted	226,006,534	163,345,972	220,188,197	145,531,495

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

TENAYA THERAPEUTICS, INC.

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

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of January 1, 2026	216,760,283	$ 21	$ 728,252	$ —	$ (605,008)	$ 123,265
Issuance of common stock upon vesting of restricted stock units	238,593	—	—	—	—	—
Stock-based compensation	—	—	2,196	—	—	2,196
Other comprehensive loss	—	—	—	—	—	—
Net loss	—	—	—	—	(19,272)	(19,272)
Balance as of March 31, 2026	216,998,876	$ 21	$ 730,448	$ —	$ (624,280)	$ 106,189
Issuance of common stock pursuant to employee stock purchase plan	74,780	—	43	—	—	43
Issuance of common stock upon exercise of warrants	6,955,000	1	4,868	—	—	4,869
Issuance of common stock upon vesting of restricted stock units	201,356	—	—	—	—	—
Stock-based compensation	—	—	1,701	—	—	1,701
Other comprehensive income	—	—	—	—	—	—
Net loss	—	—	—	—	(43,393)	(43,393)
Balance as of June 30, 2026	224,230,012	$ 22	$ 737,060	$ —	$ (667,673)	$ 69,409
Issuance of common stock upon vesting of restricted stock units	356,501	—	—	—	—	—
Issuance of common stock in connection with at-the market sales, net of issuance costs of $73	3,341,727	—	2,367	—	—	2,367
Stock-based compensation	—	—	1,679	—	—	1,679
Other comprehensive income	—	—	—	—	—	—
Net loss	—	—	—		(18,938)	(18,938)
Balance as of September 30, 2026	227,928,240	$ 22	$ 741,106	$ —	$ (686,611)	$ 54,517

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of January 1, 2025	86,542,340	$ 8	$ 607,229	$ 28	$ (514,411)	$ 92,854
Issuance of common stock and warrants in follow-on offering, net of issuance costs of $3,699	75,000,000	8	48,793	—	—	48,801
Issuance of common stock upon vesting of restricted stock units	295,191	—	—	—	—	—
Issuance of common stock in connection with at-the market sales, net of issuance costs of $280	822,566	—	912	—	—	912
Stock-based compensation	—	—	3,731	—	—	3,731
Other comprehensive loss	—	—	—	(38)	—	(38)
Net loss	—	—	—		(26,864)	(26,864)
Balance as of March 31, 2025	162,660,097	$ 16	$ 660,665	$ (10)	$ (541,275)	$ 119,396
Issuance of common stock pursuant to employee stock purchase plan	119,998	—	70	—	—	70
Issuance of common stock upon vesting of restricted stock units	196,007	—	—	—	—	—
Stock-based compensation	—	—	3,636	—	—	3,636
Other comprehensive income	—	—	—	9	—	9
Net loss	—	—	—	—	(23,283)	(23,283)
Balance as of June 30, 2025	162,976,102	$ 16	$ 664,371	$ (1)	$ (564,558)	$ 99,828
Issuance of common stock upon exercise of stock options and vesting of restricted stock units	260,933	—	24	—	—	24
Issuance of common stock upon exercise of warrants	507,500	—	356	—	—	356
Stock-based compensation	—	—	2,934	—	—	2,934
Other comprehensive income	—	—	—	1	—	1
Net loss	—	—	—	—	(20,275)	(20,275)
Balance as of September 30, 2025	163,744,535	$ 16	$ 667,685	$ —	$ (584,833)	$ 82,868

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,	
	2026	**2025**
Cash flows from operating activities:		
Net loss	$ (81,603)	$ (70,422)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	3,855	6,609
Amortization (accretion) of premium (discount) on marketable securities	—	411
Stock-based compensation	5,576	10,301
Non-cash operating lease expense	1,820	1,833
Impairment loss	22,487	—
Other	1,235	293
Changes in operating assets and liabilities:		
Accounts receivable	(388)	—
Prepaid expenses and other current assets	1,396	714
Other noncurrent assets	434	573
Accounts payable	286	(1,961)
Accrued and other current liabilities	(1,506)	(594)
Contract liabilities	8,539	—
Operating lease liabilities	(2,550)	(2,081)
Other noncurrent liabilities	(51)	—
Net cash used in operating activities	(40,470)	(54,324)
Cash flows from investing activities:		
Purchases of property and equipment	(170)	(551)
Proceeds from sales of property and equipment	26	—
Proceeds from sales of marketable securities	—	10,958
Proceeds from maturities of marketable securities	—	45,743
Net cash (used) provided by investing activities	(144)	56,150
Cash flows from financing activities:		
Proceeds from issuance of common stock, pre-funded warrants, and warrants in follow-on offering, net of issuance costs	—	48,801
Proceeds from exercise of stock options and employee stock purchase plan	43	94
Proceeds from exercise of warrants	4,869	356
Proceeds from at-the-market sales, net of issuance costs	2,367	912
Payment of accrued offering costs	(415)	—
Net cash provided by financing activities	6,864	50,163
Net change in cash, cash equivalents and restricted cash	(33,750)	51,989
Cash and cash equivalents and restricted cash at beginning of period	100,966	4,742
Cash and cash equivalents and restricted cash at end of period	$ 67,216	$ 56,731
Components of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 66,797	$ 56,312
Restricted cash included in other noncurrent assets	419	419
Cash, cash equivalents and restricted cash	$ 67,216	$ 56,731
Supplemental disclosure of non-cash investing and financing activities:		
Receivable from sale of property and equipment	$ 185	$ —

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 clinical-stage biotechnology company focused on discovering, developing and delivering curative therapies that address the underlying drivers of heart disease. The Company's lead product candidates include TN-201, a gene therapy for myosin binding protein C3-associated hypertrophic cardiomyopathy, TN-401, a gene therapy for plakophilin 2-associated arrhythmogenic right ventricular cardiomyopathy, and TN-301, a small molecule with potential clinical utility in cardiac, metabolic and muscular conditions, including heart failure with preserved ejection fraction and Duchenne's muscular dystrophy.

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, 2026, the Company had an accumulated deficit of $686.6 million. The Company incurred a net loss of $81.6 million and $70.4 million during the nine months ended September 30, 2026 and 2025, respectively. The Company had $66.8 million of cash and cash equivalents as of September 30, 2026.

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 and cash equivalents as of September 30, 2026, will be sufficient to fund the Company's operations for at least the next twelve months following the date these 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, 2026, the interim condensed statements of operations and comprehensive loss, stockholders' equity and cash flows for the nine months ended September 30, 2026 and 2025 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 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 nine months ended September 30, 2026, 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, 2025, 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 and the related notes thereto for the year ended December 31, 2025, included in the Company's Annual Report on Form 10-K, filed with the SEC on March 11, 2026.

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 financial statements include, but are not limited to, accrued expenses related to research and development activities and revenue recognition related to collaboration agreements. 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.

Significant Accounting Policies

There have been no material revisions to the Company's significant accounting policies described in Note 2, *Summary of Significant Accounting Policies*, to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2025, except for the accounts receivable, revenue recognition and contract liability policies related to the collaboration agreements entered into during the nine months ended September 30, 2026, as described below.

Accounts Receivable

The Company's accounts receivable relates to licensing and collaboration arrangements. These accounts receivable are short-term in nature. The Company estimates expected credit losses over the life of the financial assets as of the reporting date based on relevant information about past events, current conditions, and reasonable and supportable forecasts. For the nine months ended September 30, 2026 and 2025, the Company had no allowance for credit losses.

Revenue Recognition

Overview

The Company recognizes revenue in accordance with Accounting Standards Codification (ASC) Topic 606, *Revenue from Contracts with Customers* (ASC 606). Revenue is recognized when promised goods or services are transferred to a customer in an amount that reflects the consideration to which the Company expects to be entitled. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the promises and performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies the performance obligations. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Collaborative Frameworks and Assessment Under ASC 808 and ASC 606

The Company enters into arrangements that may include elements of collaboration with counterparties, such as joint steering committees or shared development activities. The Company evaluates each arrangement to determine whether the counterparty is acting in the capacity of a customer.

To the extent that the Company transfers goods or services to a counterparty in its capacity as a customer, the Company accounts for such transactions under ASC 606 and presents the related amounts as revenue. Amounts that do not represent transactions with a customer are accounted for under ASC Topic 808, *Collaborative Arrangements* (ASC 808), and presented within the appropriate operating expense line item.

Licensing and Collaboration Arrangements

The Company enters into licensing and collaboration arrangements that may include licenses to intellectual property, as well as research, development, or technical support services necessary to enable the licensee to utilize the underlying technology.

Identification of Performance Obligations

The Company evaluates the nature of its promises in these arrangements to determine whether they are distinct or should be combined. A license of intellectual property is combined with other promised services when the license is not distinct within the context of the contract, such as when the Company's ongoing research or

development activities significantly affect the utility of the licensed intellectual property. When a license and related services are not distinct, they are accounted for as a single combined performance obligation.

Recognition of Revenue

Revenue is recognized either at a point in time or over time, depending on the nature of the underlying performance obligations. Revenue is recognized at a point in time when control of a promised good or service transfers to the customer and the performance obligation is satisfied. Revenue is recognized over time when the Company satisfies a performance obligation over time, including when the Company's performance does not create an asset with an alternative use and the Company has an enforceable right to payment for performance completed to date, or when the customer simultaneously receives and consumes the benefits of the Company's performance.

For performance obligations satisfied over time, the Company measures progress using an appropriate method that faithfully depicts the transfer of control of the underlying services. Depending on the nature of the arrangement, the Company may use an input method, such as costs incurred relative to total estimated costs (cost-to-cost method), when such method best reflects the Company's performance.

For arrangements that provide access to intellectual property or other rights over a defined period without additional performance obligations, revenue is recognized ratably over the contractual term.

Upfront License Fees

If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company will recognize revenue from upfront license fees allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company determines whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments

Contingent milestones at contract inception are estimated at the amount which is not probable of a material reversal and included in the transaction price using the most likely amount method. Milestone payments that are not within the Company's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received and therefore the variable consideration is constrained. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each reporting period, the Company re-evaluates the probability of achieving development, regulatory or sales-based milestone payments that may not be subject to a material reversal and, if necessary, adjusts the estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect license and other revenue, as well as earnings, in the period of adjustment.

Sales-Based Royalties and Milestones

For arrangements that include sales-based royalties, including milestone payments based on the volume of sales, the Company will determine whether the license is deemed to be the predominant item to which the royalties or sales-based milestones relate and if such is the case, the Company will recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Contract Liabilities

Contract liabilities consist primarily of upfront non-refundable payments received under collaboration agreements for which the related performance obligations have not yet been fully satisfied. The Company classifies amounts expected to be recognized as revenue within the next twelve months as contract liabilities, current, with the remainder classified as contract liabilities, noncurrent based on the estimated timing of revenue recognition.

Recently Adopted Accounting Standards

In July 2025, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) No. 2025-05 (ASU 2025-05), *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets* (ASU 2025-05), which provides a practical expedient for entities to estimate expected credit losses on current accounts receivable and current contract assets arising from revenue transactions accounted for under ASC 606. ASU 2025-05 is effective for the Company for annual periods beginning after December 15, 2025, and interim periods within those annual periods. The Company adopted this ASU on a prospective basis during the first quarter of 2026. The adoption did not have a material impact on the Company's financial statements.

Recently Issued Accounting Pronouncements Not Yet Adopted

In December 2025, the FASB issued ASU No. 2025-11, *Interim Reporting (Topic 270): Narrow-Scope Improvements* (ASU 2025-11), which clarifies the applicability of the interim reporting guidance, the types of interim reporting, and the form and content of interim financial statements in accordance with GAAP. Per the FASB, the amendment does not intend to change the fundamental nature of interim reporting or expand or reduce current interim disclosure requirements but rather provide clarity and improve navigability of the existing interim reporting requirements. ASU 2025-11 is effective for interim periods within annual reporting periods beginning after December 15, 2027, with early adoption permitted. ASU 2025-11 may be applied either prospectively or retrospectively for all prior periods presented. The Company is evaluating the impact of this standard on its financial statements and related disclosures.

In December 2025, the FASB issued ASU No. 2025-10, *Government Grants (Topic 832): Accounting for Government Grants Received by Business Entities* (ASU 2025-10), which adds guidance to Accounting Standards Codification (ASC) 832 on the recognition, measurement, and presentation of government grants. The guidance establishes a framework for accounting for government grants, including grants related to assets and grants related to income. ASU 2025-10 is effective for the Company for annual periods beginning after December 15, 2028, and interim periods within those annual periods. Early adoption is permitted. The Company is evaluating the impact of this standard on its financial statements and related disclosures.

In November 2024, the FASB issued ASU No. 2024-03, *Income Statement (Topic 220): Reporting Comprehensive Income - Expense Disaggregation Disclosures, Disaggregation of Income Statement Expenses* (ASU 2024-03), which requires public companies to disclose, in interim and annual reporting periods, additional information about certain expenses in the financial statements. The amendments in this ASU will be effective for annual periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is evaluating the impact of this standard on its financial statements and related disclosures.

3. Collaboration Revenue

Alnylam Collaboration Agreement

In March 2026, the Company entered into a collaboration agreement with Alnylam Pharmaceuticals, Inc. (Alnylam), pursuant to which both parties agreed to a research collaboration to discover and validate novel gene targets for the potential treatment of cardiovascular disease.

Under the agreement, both parties nominated and aligned on a set of collaboration targets to move forward into the collaboration. Following such alignment, the parties will conduct in vitro and in vivo validation activities under a mutually agreed research plan and budget for a period of twenty-four (24) months (which may be extended for completion of the work). The Company expects to take primary responsibility for the performance of all in vitro and in vivo validation activities throughout the validation term; Alnylam will reimburse the Company for full-time employees and out-of-pocket costs and expenses incurred by the Company in accordance with the agreed-upon research budget. After completion of the validation activities, Alnylam will be solely responsible, at its own expense, for all development, manufacture, regulatory and commercialization activities for any products directed to a collaboration target. The Company was also entitled to a non-refundable upfront platform access fee of up to $10.0 million. The upfront platform access fee was initially subject to variability based on the number of collaboration targets selected and became fixed at $10.0 million upon finalization of the collaboration target list. Reimbursement of research costs represents variable consideration and is excluded from the transaction price at contract inception as such amounts are fully constrained. These amounts are recognized as revenue as the related costs are incurred.

The Company concluded that the license and research activities represent a single performance obligation that is satisfied over time. Revenue is recognized using a cost-to-cost input method, which measures progress based on costs incurred relative to total estimated costs under the research plan. For the three and nine months ended September 30, 2026, the Company recognized collaboration revenue of $1.5 million and $2.4 million, respectively, related to the upfront platform access fee and reimbursable research costs incurred during the respective periods.

The Company may also receive future milestone payments of up to an aggregate of $1.1 billion under the agreement. Development and regulatory milestones will be recognized when the underlying milestone event is achieved, and it becomes probable that a significant reversal of cumulative revenue recognized will not occur. Sales-based milestones will be recognized when the related sales occur. As of September 30, 2026, the Company is unable to predict or reasonably estimate the amount or timing of such milestone payments.

As of September 30, 2026, the aggregate amount of the transaction price allocated to the remaining performance obligation under the collaboration agreement was approximately $8.5 million, which the Company expects to recognize over the remaining validation term of approximately 18 months. The transaction price allocated to the remaining performance obligation does not include variable consideration related to reimbursable research costs as they are contingent on actual qualifying costs incurred. Milestone payments were considered variable consideration and were also not included in the transaction price as they are fully constrained. The Company will reassess the transaction price at each reporting period as uncertainties are resolved.

The following table summarizes activity in the Company's total contract liabilities for the three and nine months ended September 30, 2026. Additions to contract liabilities included $0.6 million of upfront payments from an additional immaterial collaboration arrangement.

	Contract Liabilities (In thousands)
Balance as of December 31, 2025	$ —
Addition	10,600
Less: revenue recognized from contract liabilities	(198)
Balance as of March 31, 2026	$ 10,402
Less: revenue recognized from contract liabilities	(739)
Balance as of June 30, 2026	$ 9,663
Less: revenue recognized from contract liabilities	(1,124)
Balance as of September 30, 2026	$ 8,539

4. Balance Sheet Components

Property and Equipment, Net

Property and equipment, net consists of the following:

	September 30, 2026	December 31, 2025
	(In thousands)	
Laboratory equipment	$ 12,060	$ 19,014
Leasehold improvements	7,787	26,244
Computer equipment and software	1,293	1,893
Furniture and fixtures	519	902
Manufacturing equipment	—	19,485
Construction in progress	—	67
Total property and equipment	$ 21,659	$ 67,605
Less: accumulated depreciation and amortization	(20,523)	(39,933)
Total property and equipment, net	$ 1,136	$ 27,672

Depreciation and amortization expense for the three months ended September 30, 2026 and 2025 was $0.4 million and $1.9 million, respectively. Depreciation and amortization expense for the nine months ended September 30, 2026 and 2025 was $3.9 million and $6.6 million, respectively.

In connection with the early termination of the Union City lease for the Company's Genetic Medicine Manufacturing Center (the GMMC) (see Note 5, *Commitments and Contingencies*), the Company recorded an impairment loss of $21.8 million during the second quarter of 2026. The impairment loss primarily reflects the write-down to zero of leasehold improvements and certain manufacturing and laboratory equipment that, pursuant to the lease termination agreement, are required to remain at the GMMC and be surrendered to the landlord without consideration.

During the third quarter of 2026, the Company committed to a plan to sell certain laboratory equipment previously expected to be retained for continued use or repurposing following the closure of the GMMC. Upon meeting the criteria for classification as held for sale, the Company measured the equipment at the lower of its carrying amount or fair value less costs to sell and recognized an impairment loss of $0.7 million during the three months ended September 30, 2026. The Company sold substantially all of this equipment through an auction and separate sales arrangements and recognized a net loss on disposal of property and equipment of $0.1 million, which also included losses from the retirement of certain equipment. The impairment charges and net loss on disposal are presented separately within operating expenses in the accompanying condensed statements of operations and comprehensive loss.

Accrued and Other Current Liabilities

Accrued and other current liabilities consist of the following:

	September 30, 2026	December 31, 2025
	(In thousands)	
Accrued compensation and related expenses	$ 3,991	$ 6,013
Accrued research and development expenses	2,524	1,651
Accrued professional services	341	718
Accrued taxes	45	277
Other current liabilities	160	176
Total accrued and other current liabilities	$ 7,061	$ 8,835

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	September 30, 2026	December 31, 2025
	(In thousands)	
Prepaid expenses	$ 3,458	$ 4,014
Other current assets	185	1,025
Total prepaid expenses and other current assets	$ 3,643	$ 5,039

5. 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 was initially set to expire in May 2025 with two five-year renewal options. In June 2024, the Company amended the lease to extend the term to November 2027. Pursuant to the terms of the amended lease, the Company has one remaining five-year renewal option.

In February 2021, the Company entered into a lease agreement for office and manufacturing space in Union City, California. The lease commenced in May 2021 and has a ten-year term with one five-year renewal option. On June 29, 2026, the Company entered into a lease termination agreement with the landlord. Pursuant to the agreement, the lease terminated on August 31, 2026, instead of its original expiration in July 2031. The Company is not required to remove tenant improvements or restore the premises and may surrender the facility in its existing condition. As consideration for the lease termination, the Company agreed to forfeit its $1.8 million security deposit and pay a one-time lease termination fee of $0.3 million. The Company accounted for the termination agreement as a lease modification under ASC 842 and remeasured the related operating lease liability and right-of-use asset

during the second quarter of 2026. As a result of the lease modification, the Company recognized a net loss on lease termination of $1.4 million during the nine months ended September 30, 2026, consisting of the security deposit forfeiture and a brokerage commission incurred in connection with the lease termination, partially offset by the gains recognized upon remeasurement of the operating lease liability and right-of-use asset and the derecognition of the asset retirement obligation liability.

Information related to operating lease activity during the three and nine months ended September 30, 2026 was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
	(In thousands)			
Operating lease cost	$ 556	$ 889	$ 2,334	$ 2,667
Variable lease cost	357	410	1,042	1,043
Total lease cost	$ 913	$ 1,299	$ 3,376	$ 3,710
Cash paid for amounts included in the measurement of lease liabilities			$ 3,063	$ 2,914

As of September 30, 2026, the Company had one operating lease with a remaining lease term of 1.2 years and a discount rate of 7.8%. As of December 31, 2025, the Company's operating leases had a weighted average remaining lease term of 4.1 years and a weighted average discount rate of 9.1%. Future minimum lease payments under the Company's operating lease as of September 30, 2026 were as follows:

	Amount
	(In thousands)
2026 (three months remaining)	$ 628
2027	2,347
2028	—
2029	—
2030	—
Thereafter	—
Total undiscounted future minimum lease payments	$ 2,975
Imputed interest	(123)
Total operating lease liabilities	$ 2,852

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. In general, 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 up to the time of termination.

Contingencies

From time to time, the Company may become involved in litigation and other legal actions. The Company estimates the range of liability related to any pending litigation where the amount and range of loss can be estimated. The Company records its best estimate of a loss when the loss is considered probable. Where a liability is probable and there is a range of estimated loss with no best estimate in the range, the Company records a charge equal to at least the minimum estimated liability for a loss contingency when both of the following conditions are met: (i) information available prior to issuance of the financial statements indicates that it is probable that a liability had been incurred at the date of the financial statements and (ii) the range of loss can be reasonably estimated. The Company was not involved in any material litigation as of September 30, 2026.

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, 2026, the Company did not have any material indemnification claims that were probable or reasonably possible and, consequently, has not recorded any related liabilities.

6. Stock-Based Compensation

2024 Inducement Equity Incentive Plan

In September 2024, the Board of Directors (the Board) adopted the Company's 2024 Inducement Equity Incentive Plan (the Inducement Plan), and subject to the adjustment provisions of the Inducement Plan, reserved 1,200,000 shares of the Company’s common stock for issuance pursuant to equity awards granted under the Inducement Plan. The Inducement Plan allows the Company to make equity awards to prospective employees of the Company as an inducement to such individual’s commencement of employment with the Company. On January 26, 2026, the Board approved an increase of 2,161,000 shares to the number of shares reserved under the Inducement Plan.

Total shares reserved and available for grant under the Inducement Plan as of September 30, 2026, were 848,800.

Repricing

On January 22, 2025, the Compensation Committee of the Company's Board approved a repricing of certain outstanding vested and unvested stock option awards under the Amended and Restated 2016 Equity Incentive Plan (the 2016 Plan) and 2021 Plan for eligible employees and certain other service providers (the Repricing Participants). The per share exercise price of eligible stock option awards was reduced to $1.21, the closing price of the Company's common stock on January 24, 2025 (the Repricing Effective Date). To receive the benefit of the repricing, Repricing Participants were required to remain a Service Provider (as such term is defined in the 2016 Plan or 2021 Plan) through the period (the Retention Period) that began on the Repricing Effective Date and ended on July 24, 2025 (the Retention Date) and not exercise any of their repriced stock options prior to the Retention Date. Option holders who exercised their repriced stock options prior to the Retention Date were required to pay the original exercise price per share of such repriced options. No other changes were made to the terms and conditions of the eligible stock option awards. The stock option repricing impacted 4.1 million stock option awards and affected 89 employees and service providers.

On February 6, 2025, the Company's Board approved an option repricing applicable to Faraz Ali, the Company's Chief Executive Officer, with terms mirroring the aforementioned repricing approved on January 22, 2025, except that for Mr. Ali, the options eligible for repricing were limited to options with exercise prices higher than $5.25 per share. The total number of shares underlying Mr. Ali’s repriced options was 915,875 shares.

The repricing resulted in a total incremental stock-based compensation expense of $1.3 million, which was calculated using the Black-Scholes option pricing model, of which $1.0 million is associated with vested repriced options as of the Retention Date and were recognized on a straight-line basis over the Retention Period. The remaining $0.3 million of the incremental stock-based compensation expense is associated with unvested repriced options beyond the Retention Period and will be recognized on a straight-line basis over the remaining original vesting periods. For the nine months ended September 30, 2026 and 2025, the Company recognized an immaterial amount and $1.0 million, respectively, of incremental stock-based compensation expense.

2021 Equity Incentive Plan

Under the Company’s 2021 Equity Incentive Plan (the 2021 Plan), 4,000,000 shares of the Company’s common stock were initially reserved for issuance of equity awards to employees, directors, and consultants, under terms and provisions established by the Board. Prior to May 27, 2026, the number of shares of common stock available for issuance under the 2021 Plan automatically increased 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 board of directors may determine.

On May 27, 2026, the Company's stockholders approved the Amended and Restated 2021 Equity Incentive Plan (the Restated 2021 Plan). The Restated 2021 Plan increased the number of shares of the Company's common stock reserved for issuance by 6,509,966 shares. In addition, beginning on January 1, 2027, the annual automatic increase provision was amended such that the number of shares available for issuance under the Restated 2021 Plan will increase on the first day of each fiscal year through 2031 in an amount equal to 4% of the outstanding shares of the Company's common stock as of the last day of the immediately preceding year, or such lesser amount as the Board or the Compensation Committee may determine, without the previous annual cap of 4,000,000 shares. The Restated 2021 Plan also provides that no more than 15,000,000 shares may be issued pursuant to incentive stock options.

The total number of shares reserved and available for grant under the Restated 2021 Plan as of September 30, 2026 was 4,215,105.

Stock Option Activity

The following table summarizes stock option activity:

	Shares	Weighted Average Exercise Price (in dollars)
Outstanding as of December 31, 2025	12,050,796	$ 1.90
Granted	8,581,923	$ 0.81
Exercised	—	$ —
Cancelled	(487,809)	$ 1.88
Outstanding as of September 30, 2026	20,144,910	$ 1.46

Stock option awards granted to employees generally vest over a four-year period. The contractual term of stock option awards is generally 10 years from the grant date.

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,	
	2026	2025	2026	2025
Expected term (in years)	6.0 – 6.1	6.1	5.5 – 6.1	5.5 – 6.1
Expected volatility	82% – 83%	90%	82% – 85%	87% – 91%
Risk-free interest rate	4.4% – 4.8%	3.7%	3.9% – 4.8%	3.7% – 4.5%
Expected dividend yield	—%	—%	—%	—%

Restricted Stock Units

Restricted stock units (RSUs) are awards that entitle the holder to receive freely tradable shares of the Company's common stock upon the completion of a specific period of continued service. RSUs generally vest over a two- to four-year period and are subject to forfeiture if employment terminates prior to the release of vesting restrictions. RSUs are valued at the market price of the underlying common stock on the date of grant. The Company recognizes noncash compensation expense for the fair value of RSUs on a straight-line basis over the

requisite service period of the awards. The following table summarizes activity of RSUs granted to employees with service-based vesting under the 2021 Plan.

	Shares	Weighted Average Grant Date Fair Value per Share (in dollars)
Unvested as of December 31, 2025	1,536,234	$ 2.39
Granted	2,162,667	$ 0.80
Vested	(796,450)	$ 2.30
Forfeited	(123,653)	$ 2.03
Unvested as of September 30, 2026	2,778,798	$ 1.20

2021 Employee Stock Purchase Plan

Under the Company's 2021 Employee Stock Purchase Plan (the ESPP), the Company initially reserved 800,000 shares for future issuance. The number of shares of common stock available for issuance under the ESPP automatically increases on the first day of each fiscal year for a period of ten years beginning with 2022 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 may determine. As of September 30, 2026, 2,999,814 shares were reserved for future issuance under the ESPP. Under the Company's ESPP, employees are generally eligible to participate and can purchase shares on each purchase date established semi-annually through payroll deductions at the lower of 85% of the fair market value of the Company's stock at the commencement of the offering period or each purchase date of the offering period. Each offering period spans six months. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 15% of qualified compensation, up to an annual limit of $25,000 per the Internal Revenue Service. For the nine months ended September 30, 2026 and 2025, the stock-based compensation expense for ESPP was not material.

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,	
	2026	2025	2026	2025
Research and development	$ 768	$ 1,485	$ 2,853	$ 5,405
General and administrative	911	1,449	2,723	4,896
Total stock-based compensation	$ 1,679	$ 2,934	$ 5,576	$ 10,301

7. Stockholders' Equity

Lender Warrant

On August 6, 2024, the Company entered into a Loan and Security Agreement (the Loan Agreement) with Silicon Valley Bank (SVB). In connection with the Loan Agreement, the Company issued to SVB a warrant which is exercisable for up to 73,649 shares of common stock at an exercise price of $2.55 per share (the Lender Warrant). The Lender Warrant was classified as equity and its fair value was recorded in the stockholders' equity section of the balance sheet. The Lender Warrant expires on August 6, 2034. As of September 30, 2026, all borrowing commitments under the Loan Agreement had expired, and no term loans were outstanding.

"At-the-Market" Equity Offering

On August 10, 2022, the Company entered into a sales agreement (the Sales Agreement) with Leerink Partners LLC to establish an "at-the-market" (ATM) offering defined in Rule 415 under the Securities Act. Pursuant to the Sales Agreement, the Company is permitted to offer and sell, from time to time, shares of its common stock

having a maximum aggregate offering price of up to $75.0 million. In January 2025, the Company sold 822,566 shares of common stock under the ATM offering for net proceeds of $0.9 million, after deducting commissions and offering costs of $0.3 million. During the third quarter of 2026, the Company sold 3,341,727 shares of common stock under the ATM offering for net proceeds of $2.4 million, after deducting commissions of $0.1 million. As of September 30, 2026, the Company may issue and sell up to approximately $67.4 million of common stock under the ATM offering.

Follow-On Offering

On December 15, 2025, the Company completed an underwritten public offering of 50,000,000 units, priced at a public offering price of $1.20 per unit, with each unit consisting of one share of its common stock and a warrant to purchase one share of its common stock at an exercise price of $1.50 per share, which were immediately exercisable and expire five years from the date of issuance (December 2025 Warrants), under its registration statement on Form S-3 (File No. 333-286005). The Company received net proceeds of $55.8 million, after deducting underwriting discounts and commissions of $3.6 million and other offering expenses of $0.6 million.

On March 5, 2025, the Company completed an underwritten public offering of 75,000,000 units, priced at a public offering price of $0.70 per unit, with each unit consisting of one share of its common stock, a warrant to purchase one share of its common stock at an exercise price of $0.80 per share, which were immediately exercisable and expire five years from the date of issuance (a Series A Warrant) and a warrant to purchase one-half of a share of its common stock at an exercise price of $0.70 per share, which were immediately exercisable and expired on June 30, 2026 (a Series B Warrant), under its registration statement on Form S-3 (File No. 333-266741). The Company received net proceeds of approximately $48.8 million, after deducting underwriting discounts and commissions of approximately $3.2 million and other offering expenses of approximately $0.5 million. During the second quarter of 2026, certain holders exercised Series B Warrants to purchase 6,955,000 shares of common stock for an aggregate exercise price of $4.9 million. From issuance through their expiration on June 30, 2026, Series B Warrants to purchase 10,211,250 shares of our common stock were exercised, and the remaining Series B Warrants to purchase 27,288,750 shares of our common stock expired.

The Company analyzed the December 2025, Series A, and Series B Warrants under ASC 480, *Distinguishing Liabilities from Equity*, and ASC 815-40, *Derivatives and Hedging - Contracts in Entity's Own Equity*, and concluded that they were not liabilities, were indexed to its own stock and met all other conditions for equity classification. Accordingly, the Company has classified them as permanent equity.

As of September 30, 2026, total shares of common stock reserved for issuance, on an as-if converted basis, are as follows:

	September 30, 2026
Outstanding stock options and restricted stock units	22,923,708
Outstanding Lender Warrant	73,649
Outstanding Series A Warrants	74,987,500
Outstanding December 2025 Warrants	50,000,000
Shares available for further issuance under the 2024 Inducement Equity Incentive Plan	848,800
Shares available for further issuance under the 2021 Equity Incentive Plan	4,215,105
Shares available for further issuance under the 2021 Employee Stock Purchase Plan	2,999,814
Total	156,048,576

8. Fair Value Measurements

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:

		September 30, 2026			
	Valuation Hierarchy	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
		(In thousands)			
Assets:					
Cash equivalents:					
Money market funds	Level 1	$ 63,071	$ —	$ —	$ 63,071
Total financial assets		$ 63,071	$ —	$ —	$ 63,071

		December 31, 2025			
	Valuation Hierarchy	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
		(In thousands)			
Assets:					
Cash equivalents:					
Money market funds	Level 1	$ 98,424	$ —	$ —	$ 98,424
Total financial assets		$ 98,424	$ —	$ —	$ 98,424

Money market funds are classified as Level 1 because they are valued using quoted market prices in active markets for identical assets.

The carrying amount of the Company's remaining financial assets and liabilities, which include cash, receivables and payables, approximate their fair values due to their short-term nature.

Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company maintains a significant majority of its cash balances with a single financial institution, with the remainder held at other financial institutions. The Company's cash balances exceed federally insured limits. The Company has not experienced any losses on its cash balances and management monitors the creditworthiness of these institutions and believes the Company is not exposed to significant credit risk related to these balances.

9. Income Taxes

For the three and nine months ended September 30, 2026 and 2025, the Company did not record any income tax expense or benefit. The Company has recorded a full valuation allowance against its U.S. federal and state deferred tax assets as the Company believes it is more likely than not that the benefit will not be realized.

10. Net Loss Per Share

Basic and diluted loss per share are computed by dividing net loss by the weighted-average number of common shares outstanding during the reporting period. 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:

	September 30,	
	2026	2025
Outstanding stock options and restricted stock units	22,923,708	13,986,181
Outstanding Lender Warrant	73,649	122,749
Outstanding Series A and Series B Warrants	74,987,500	111,992,500
Outstanding December 2025 Warrants	50,000,000	—
Total	147,984,857	126,101,430

11. Segment Reporting

The Company is a clinical-stage biotechnology company focused on discovering, developing and delivering curative therapies that address the underlying drivers of heart disease and has one operating and reportable segment. The Company's chief operating decision maker (CODM) is the chief executive officer.

The statement of operations includes research and development expenses, general and administrative costs, interest income, and income taxes; the Company has not generated any product revenue. In addition to reviewing the expenses in the Company's statement of operations, the CODM is regularly provided with operating expenses by function. The CODM does not review assets at a different asset level or category than the amounts disclosed in the Company's balance sheet. The Company's long-lived assets are located in the United States.

The following table provides information about the Company's operating expenses by function and includes a reconciliation to net loss.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue				
Collaboration revenue	$ 1,697	$ —	$ 3,032	$ —
Operating expenses:				
Research and development				
Clinical	6,174	4,065	16,752	15,410
Manufacturing (pre-commercial)	1,487	4,107	8,804	13,631
Research	5,034	3,579	11,812	13,681
Other	2,171	3,612	8,945	11,087
Total research and development	$ 14,866	$ 15,363	$ 46,313	$ 53,809
General and administrative	5,648	5,573	16,501	18,747
Impairment loss	666	—	22,487	—
Loss on lease termination, net	—	—	1,368	—
Loss on disposal of property and equipment	89	—	89	—
Total operating expenses	21,269	20,936	86,758	72,556
Loss from operations	(19,572)	(20,936)	(83,726)	(72,556)
Other income, net:				
Interest income	635	658	2,126	2,107
Other (loss) income, net	(1)	3	(3)	27
Total other income, net	634	661	2,123	2,134
Net loss before income tax expense	(18,938)	(20,275)	(81,603)	(70,422)
Income tax expense	—	—	—	—
Net loss	$ (18,938)	$ (20,275)	$ (81,603)	$ (70,422)

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 audited financial statements and related notes thereto for the year ended December 31, 2025, included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 11, 2026.

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 (the Securities Act), 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 clinical-stage biotechnology company focused on discovering, developing and delivering potentially curative therapies that address the underlying drivers of heart disease. Heart disease remains the leading cause of death in the world. We believe the emerging insights into the genetic causes of cardiovascular conditions and increased recognition for precision medicine approaches has created an opportunity to change the treatment paradigm for heart disease, and in doing so improve and extend the lives of patients.

We were founded with a long-term goal of building a fully integrated biopharmaceutical company focused on discovering, developing and ultimately commercializing first-/best-in-class precision medicines for heart disease. Early on in our company history, we invested in differentiated capabilities to enable modality-agnostic target identification and validation, anchored in human genetics and the use of human disease models. These highly productive platform drug discovery capabilities directly contributed to the development of our three clinical-stage programs, as well as to several earlier-stage pipeline programs.

Today, we are focused on advancing our clinical-stage gene therapy candidates, TN-201 for *MYBPC3*-associated hypertrophic cardiomyopathy (HCM), and TN-401 for *PKP2*-associated arrhythmogenic right ventricular cardiomyopathy (ARVC). Each candidate is currently in Phase 1b/2 trials to establish the safety profile of two different doses in adults with disease due to pathogenic/likely pathogenic mutations. We have presented promising interim results for both product candidates and are engaging with regulators to discuss pivotal trial plans. We plan to share additional follow-up data in the fourth quarter of 2026 and to provide an update on our interactions with regulators. We are also advancing a third internally discovered clinical-stage candidate, TN-301, a highly specific small molecule inhibitor of histone deacetylase 6 (HDAC6) with potentially broad utility in heart failure with preserved ejection fraction (HFpEF) and other cardiac, metabolic, muscular and pulmonary diseases, including but not limited to Duchenne muscular dystrophy (DMD), genetic dilated cardiomyopathy (DCM) and pulmonary arterial hypertension (PAH).

For programs arising out of our modality agnostic drug discovery platform that address relatively rare conditions our strategy is to develop and commercialize at least some of these programs on our own, although we may selectively consider partnerships to access technology, accelerate our progress, or improve our global reach to patients. Where our discovery efforts lead to product candidates intended for relatively prevalent indications, our strategy is to out-license or partner such programs. Our core capabilities and expertise offer additional opportunities for collaboration. In March 2026, we entered into a multi-target research collaboration with Alnylam Pharmaceuticals, Inc. (Alnylam), to identify and validate novel gene targets for the potential treatment of cardiovascular disease in exchange for a $10 million upfront payment, reimbursement for research efforts and up to $1.1 billion in development and commercial milestones payments. Our genetic medicines manufacturing know-how provides the basis for licensing, collaboration, and/or other agreements with global contract development and manufacturing and/or life science reagent companies.

TN-201 is our investigational gene therapy for individuals with HCM due to *MYBPC3* gene mutations. These mutations result in a deficiency of myosin-binding protein C (MyBP-C), which in turn can cause the heart walls of affected individuals to become significantly thickened, leading to fibrosis, abnormal heart rhythms, cardiac

dysfunction, heart failure and death. HCM is a chronic, progressive condition, and those diagnosed with the disease often experience significant impairment in overall quality of life and may be at higher risk for serious complications and co-morbidities. TN-201 utilizes a recombinant AAV9 capsid and is designed to deliver a working *MYBPC3* gene to specific cells of the heart in order to produce MyBP-C and thereby potentially slow or even reverse the course of *MYBPC3*-associated HCM following a single infusion.

MyPEAK™-1 is our Phase 1b/2 multi-center, open-label clinical trial, designed to assess the safety, tolerability and efficacy of a one-time intravenous infusion of TN-201. We have completed enrollment in MyPEAK-1 necessary to characterize dose response and inform dose selection for late-stage clinical trials, including three patients at the 3E13 vg/kg dose (Cohort 1), three at the 6E13 vg/kg dose (Cohort 2) and additional patients in the 6E13 vg/kg dose expansion cohort.

In June 2026, we presented new interim safety and efficacy data from MyPEAK-1 as of a May 2026 data cut off. Data shared included 78-104 weeks of follow-up for the three Cohort 1 patients and 26-52 weeks of follow-up for four patients dosed with TN-201 at 6E13 vg/kg. Other than Patient 5, all patients have completed every scheduled visit and remain in the trial. Among the latest findings reported, six patients evaluable for efficacy endpoints demonstrated improvements in multiple clinical parameters of disease including echocardiographic (ECG) measures of hypertrophy, and one or more measures of symptom burden. Cardiac troponin, a known risk factor for cardiac-related morbidities or mortality, improved or remained stable in five of six patients and NT-pro BNP, a measure of heart muscle strain, improved in three patients. ECG measures of left ventricular structure showed decreases in multiple measures of left ventricular hypertrophy, a defining characteristic of HCM. All six evaluable patients achieved reductions in left ventricular mass index (LVMI) and five of six demonstrated reductions in one or more measures of wall thickness. Decreases in LVMI were durable through two years for the first two Cohort 1 patients and decreases among Cohort 2 patients were observed at an earlier timepoint post-dose compared to Cohort 1 patients.

Among all six patients, treatment with TN-201 resulted in a lessening of symptom burden as assessed by either New York Heart Association (NYHA) classification, a physician assessment of the impact of heart failure symptoms on activities of daily living, and/or the Kansas City Cardiomyopathy Questionnaire (KCCQ), a validated tool for measuring health status, symptom burden and quality of life among HCM patients. All patients were symptomatic at baseline (NYHA class II-III) and five of six improved by at least one class following treatment with TN-201. All five were assessed as Class I and no longer have heart failure symptoms that interfere with activities of daily living, while the sixth patient remained stable. Four of six patients demonstrated meaningful benefit in symptoms and quality of life, with KCCQ Clinical Summary Scores (KCCQ-CSS) improving by between 12 to 56 points from baseline, while the three evaluable Cohort 2 patients who received TN-201 at a dose of 6E13 vg/kg had improved KCCQ-CSS with a mean increase of 36 points. An improvement of 5 points or greater is considered clinically meaningful.

Functional capacity was also assessed by six-minute walk test (6MWT) and cardiopulmonary exercise testing (CPET) and improved by at least one of the two measures in three patients, with Cohort 2 patients showing greater benefit. Two patients from Cohort 2 and one Cohort 1 patient achieved meaningful improvements in 6MWT with changes from baseline ranging from 50- to 255-meters. An increase of 30 meters is considered clinically meaningful. Patient 4 from Cohort 2 also demonstrated clinically meaningful improvement in peak oxygen consumption (pVO2). CPET were not available as of the data cut off for the remaining Cohort 2 patients. Two patients from Cohort 1 experienced a decline in 6MWT, and no Cohort 1 patients saw improvements in CPET. Cohort 1 performance may be confounded by preexisting functional limitations and/or relatively longer duration and higher dose of immunosuppression.

TN-201 continued to be generally well tolerated across both MyPEAK-1 dose cohorts for the seven patients for whom safety data was reported. No dose-limiting toxicities were observed and all patients have tapered off immunosuppressive medicines. Adjustments to monitoring and immunosuppression during Cohort 1 resulted in faster tapers and lower cumulative corticosteroid doses in Cohort 2, despite the higher TN-201 dose. Results from serial cardiac biopsies taken at baseline or Week 8 and again post-dose or Week 52 showed that TN-201 DNA transduction and mRNA expression were robust, and MyBP-C levels increased by an average of 4% over time. Protein level changes were measured using liquid chromatography mass spectrometry (LCMS) normalized to myosin heavy chain (MYHC). As of the most recent assessment, MyBP-C levels were shown to increase in four of six patients, while variation in expression between individual biopsies is believed to have contributed to smaller

differences in the remaining two patients. While additional follow-up is needed, the totality of results suggest that the higher dose of 6E13 vg/kg may result in equivalent or greater clinical benefit at an earlier timepoint post-dose.

We expect to present one-year Cohort 2 data and two-year durability data for Cohort 1 from MyPEAK-1 by year end. We are also in ongoing discussions with regulatory authorities, with an additional interaction planned to explore late-stage pivotal trial plans for TN-201, and intend to provide an update by year end.

Despite advances in the treatment of the obstructive HCM in recent years with the approval of cardiac myosin inhibitors, there are no approved treatments for those with the non-obstructive form of disease and limited options for those diagnosed before the age of 18. Recognizing the urgent medical need among pediatric patients, we initiated MyClimb, a retrospective and prospective natural history study of pediatric patients to characterize the outcomes, burden of illness, risk factors, quality of life, and biomarkers associated with disease progression in pediatric patients. MyClimb complements existing disease registries focused primarily on adult patient HCM populations and may support and expedite the development of TN-201 in the pediatric patient population. MyClimb completed enrollment of more than 200 individuals and is believed to be the largest study of pediatric individuals with *MYBPC3*-associated HCM ever conducted. Initial data indicated that 93% of participants had the nonobstructive HCM phenotype, for which there are currently no approved treatment options and that genotype was a significant predictor of risk. The data also revealed that LVMI may serve as a surrogate marker for poor long-term outcomes and as an appropriate marker to evaluate the early effectiveness of TN-201's potential in a future pivotal trial.

The FDA has granted TN-201 Fast Track, Orphan Drug and Rare Pediatric Drug Designations. TN-201 has also been accepted into the agency's Rare Disease Evidence Principles (RDEP) process for biallelic children with *MYBPC3*-associated HCM. TN-201 received orphan medicinal product designation from the European Commission (EC) and was granted PRIority MEdicine (PRIME) designation by the European Medicines Agency (EMA).

TN-401 is our AAV9-based gene therapy for the treatment of ARVC due to disease-causing variants in the *PKP2* gene. ARVC, also known as arrhythmogenic cardiomyopathy or ACM, is a chronic, progressive disease characterized by frequent, severe, and potentially life-threatening ventricular arrhythmias. The disease is associated with adverse heart remodeling, fibrosis, cardiac dysfunction, significant impairment to patients' overall quality of life, as well as an elevated risk of sudden cardiac death. *PKP2* mutations are the most common genetic cause of ARVC and result in insufficient expression of a protein needed for proper functioning of the desmosomal complex that maintains physical connections and electrical signaling between heart muscle cells. TN-401 utilizes a recombinant AAV9 capsid and is designed to deliver a working *PKP2* gene to heart muscle cells in order to produce plakophilin protein and potentially slow or even reverse the course of *PKP2*-associated ARVC following a single infusion.

RIDGE™-1 is our Phase 1b/2 multi-center, open-label clinical trial, designed to assess the safety, tolerability and efficacy of a one-time intravenous infusion of TN-401. We have completed enrollment in RIDGE-1 necessary to characterize dose response and inform dose selection for late-stage clinical trials, including three patients at the 3E13 vg/kg dose (Cohort 1), three at the 6E13 vg/kg dose (Cohort 2) and additional patients in the 6E13 vg/kg dose expansion cohort.

In May 2026, we presented interim data from the first six patients dosed in RIDGE-1 at the American Society of Gene and Cell Therapies 2026 Annual Meeting. Data reported included electrophysiology, biopsy and safety results 20-52 weeks post-dose following a single infusion of TN-401. As of the April 2026 data cut off, all patients have completed all assessments and remain on study.

Treatment with TN-401 resulted in dramatic improvements in electrical stability, as measured by premature ventricular contractions (PVCs) and non-sustained ventricular tachycardias (NSVTs). PVCs and NSVTs are each important prognosticators of more serious, potentially life-ending ventricular arrythmias and their frequency is utilized as an important component in calculating patient risk and the need for an implantable cardiac defibrillator (ICD). All six patients had PVC counts of greater than 500 per day at baseline and demonstrated meaningful reductions in arrhythmia burden with a mean reduction in average daily PVC count of 60% for Cohort 1 and 67% for Cohort 2. Two patients with a high rate of NSVTs at baseline experienced substantial decreases, apparent as early as Week 20. Patient 2 had an NSVT burden of 78 counts per 24-hours, which dropped to zero and remained stable at Week 52. At baseline, Patient 5 had an NSVT count of 43 per 24 hours, which dropped to 4 per 24 hours at Week 20. Four patients had low NSVT burden at baseline and remained low. Other measures of clinical response including ECG changes (QRS duration and T wave inversions), heart structure and function and NYHA class were in the normal range at baseline and/or remained stable during the post-dose follow-up period.

Post-dose biopsies provided evidence of robust transduction and TN-401-specific expression for the first five patients for whom data was available at the time of data cut off. Biopsy samples were collected from the right ventricular septum at Week 8 for the first four patients and at Week 22 for Patient 5, with results from a second post-dose biopsy at Week 52 available for Cohort 1 patients. Robust evidence of dose-dependent TN-401 DNA transduction and mRNA expression were apparent, while changes in PKP2 protein levels in heart muscle cells from baseline to the most recent timepoints ranged from -4% to +15% as measured by LCMS normalized to MYHC. Measurements of PKP2 protein levels were impacted by the varied composition of cardiac tissue in an ARVC heart.

TN-401 was well tolerated across both RIDGE-1 dose cohorts and no dose-limiting toxicities had occurred as of the April 2026 data cut off. The prophylactic immune suppression regimen of sirolimus and prednisone successfully managed immune responses and all patients discontinued immunosuppressive medicines. Adverse events associated with TN-401 treatment were asymptomatic and self-resolved or responded to treatment. There was no clinical thrombotic microangiopathy, no ventricular arrhythmias related to TN-401 and no other cardiotoxicities associated with TN-401.

We expect to present interim data by year end, including one-year data for Cohort 2 and durability data for Cohort 1. We are also engaging with regulators and have upcoming meetings scheduled to discuss late-stage pivotal trial planning for TN-401. We plan to provide an update on the feedback received from our discussions by year end.

In February 2025, we were awarded a Clinical Grant (Clin2) of $8.0 million from California Institute for Regenerative Medicine (CIRM), a state of California Agency that funds regenerative medicine, stem cell, and gene therapy research. Proceeds from the grant help fund clinical trial costs for our ongoing Phase 1b/2 RIDGE-1 clinical trial of TN-401 gene therapy. RIDGE-1 is being conducted at multiple clinical trial sites with ARVC expertise at leading cardiology centers in the U.S. and United Kingdom.

To support our development efforts for TN-401, we have initiated RIDGE, a global noninterventional natural history and seroprevalence study of nearly 200 patients with ARVC due to *PKP2* gene mutations. RIDGE is believed to be the largest natural history study of adults with *PKP2*-associated ARVC. Interim data from RIDGE was presented at Heart Rhythm Society's annual meeting in April 2025 and showed that patients with *PKP2*-associated ARVC experience a high burden of arrhythmias despite treatments with approved anti-arrhythmic medications including flecainide, beta blockers and surgical interventions such as ablation and ICD placement. Further, current treatments appeared to do little to halt or prevent progressive structural changes to the heart that occur as a result of *PKP2* mutations. A large majority of adults with *PKP2*-associated ARVC would be eligible to participate in RIDGE-1 based on low levels of pre-existing antibodies to AAV9.

TN-401 has received Orphan Drug, Fast Track and Regenerative Medicine Advanced Therapy designation from the FDA. TN-401 has also received orphan medicinal product designation from the EC and was granted PRIME designation by the EMA.

We are also advancing TN-301, a highly specific HDAC6 inhibitor that has potential utility in HFpEF and other cardiac, metabolic, muscular and pulmonary diseases. TN-301 was initially discovered and validated as having cardioprotective qualities in preclinical studies of a rapidly worsening mouse model of *BAG3* mutant DCM. HDAC6 is a cytoplasmic enzyme known to regulate diverse cellular processes. Based on TN-301's multi-modal mechanism of action, that includes reductions in inflammation, oxidative stress, fibrosis, and metabolic dysregulation, as well as improvements in autophagy, protein quality control, mitochondrial metabolism, and lipid metabolism, TN-301 may be well suited to the treatment of HFpEF, as well as other cardiac, metabolic, muscular and pulmonary disorders where there is strong alignment between TN-301's mechanism and the pathophysiology of disease.

We shared positive data from our Phase 1 clinical trial of TN-301 in healthy participants at the 2023 Heart Failure Society of America Annual Scientific Meeting. TN-301 was generally well tolerated across the broad range of doses studied. Pharmacokinetic results showed overall dose proportionality with a half-life supportive of once-daily dosing. Increasing doses and exposures with TN-301 correlated with increased pharmacodynamic effects. There were no changes in histone acetylation with TN-301 underscoring the selectivity of TN-301 for HDAC6 and potentially reducing the risk of off target effects.

Extensive *in vitro* and *in vivo* studies in HFpEF disease models have also shown that TN-301 addresses diverse pathological processes with direct and systemic benefits in models of HFpEF. In comparative studies, selective HDAC6 inhibition as a single agent has been shown to have similar efficacy to empagliflozin, an SGLT2 inhibitor which is approved for the treatment of HFpEF and co-administration of our HDAC6 inhibition with a SGLT2 inhibitor demonstrated additive benefit. Taken together, these data support continued development

of TN-301 as a potential treatment for patients with HFpEF and other severe diseases including those outside of cardiology in which inflammation, fibrosis and metabolic dysregulation may be implicated.

Based on our observations of TN-301's mechanism and evidence of efficacy for an approved pan-HDAC agent, we are also exploring the development of TN-301 for DMD, a condition caused by genetic mutations in the *dystrophin* gene, leading to absence of functional dystrophin protein in the heart and skeletal muscle. The muscle pathologies that underlie muscle wasting in the absence of dystrophin include inflammation, fibrosis, altered regeneration, mitochondrial dysfunction and disrupted autophagic flux – all processes that can be improved by HDAC6 inhibition.

At the Muscular Dystrophy Association Clinical & Scientific Congress 2026, we presented results from preclinical studies comparing TN-301 with the FDA-approved pan HDAC inhibitor, givinostat, in a well-established mouse model of DMD, and in human induced Pluripotent Stem Cell (iPSC)-derived cardiomyocytes from DMD patients. After five weeks of once-daily oral dosing, TN-301 showed a statistically significant increase in forelimb grip strength in *mdx* mice at both 3 mg/kg and 30 mg/kg compared to vehicle with both doses of TN-301 achieving wildtype levels of grip strength after five weeks. Further, TN-301 demonstrated greater efficacy at both doses compared to the 10 mg/kg dose of givinostat, which corresponds to the clinically relevant dose used in DMD patients. Notably, the effects of TN-301 at both doses approached those observed with the 30 mg/kg dose of givinostat, a level that is not tolerated in humans.

In engineered heart tissues derived from human DMD-induced iPSCs, TN-301 corrected calcium handling abnormalities, a key driver of DMD cardiomyopathy, including beat-to-beat fluctuations in calcium amplitude. In contrast, givinostat exacerbated calcium handling irregularities. In an experiment of DMD patient-derived iPSC cardiomyocytes designed to measure oxygen consumption and mitochondrial stress, both known contributors to DMD cardiomyopathy, TN-301 corrected basal and maximal respiration whereas givinostat worsened both measures. Taken together, these data support the potential advancement of TN-301 in DMD therapy, suggesting that HDAC6 inhibition may have benefits for both skeletal and cardiac muscle and reduced liabilities compared to pan-HDAC inhibitors.

We plan to advance TN-301 toward clinical trials in patients in order to generate proof-of-activity data, with HFpEF and DMD being among the most promising potential indications identified to date. We are currently conducting enabling toxicology work to support such advancement and plan to initiate at least one company-sponsored Phase 2 clinical trial in the second half of 2027. Consistent with our strategy, we believe that TN-301's late-stage development and commercialization in large indications would best be led by a strategic pharmaceutical partner with global resources to explore the full potential of the molecule.

The FDA granted both Orphan Drug and Rare Pediatric Drug Designations to TN-301 as a treatment for DMD.

In addition to our clinical-stage candidates, we have multiple early-stage programs using various therapeutic approaches, including gene addition, gene editing, gene silencing, and cellular regeneration to address other forms of rare and/or prevalent forms of heart disease. We do not have any products approved for sale and have not generated any product revenue to date.

Results of Operations

Comparison of the Three Months Ended September 30, 2026 and 2025:

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

(in thousands, except percentages)	Three Months Ended September 30, 2026	2025	$ Change	% Change
Revenue				
Collaboration revenue	$ 1,697	$ —	$ 1,697	NM
Operating expenses:				
Research and development	14,866	15,363	(497)	(3%)
General and administrative	5,648	5,573	75	1%
Impairment loss	666	—	666	NM
Loss on disposal of property and equipment	89	—	89	NM
Total operating expenses	21,269	20,936	333	2%
Loss from operations	(19,572)	(20,936)	1,364	(7%)
Other income, net:				
Interest income	635	658	(23)	(3%)
Other (loss) income, net	(1)	3	(4)	NM
Total other income, net	634	661	(27)	(4%)
Net loss	$ (18,938)	$ (20,275)	$ 1,337	(7%)

NM - Not Meaningful

Collaboration Revenue

Collaboration revenue for the three months ended September 30, 2026 was $1.7 million, compared to none in the prior year period. The increase consisted of $1.1 million from the recognition of revenue associated with upfront fees and $0.6 million from reimbursements for research and development activities under collaboration arrangements entered into during the first quarter of 2026.

Research and Development Expenses

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 research programs, product candidates and proprietary platform technology, and are recognized as incurred. Internal research and development costs include, among others, employee-related costs (including salaries, benefits and stock-based compensation for employees engaged in research and development functions), laboratory supplies, other non-capital equipment utilized for in-house research, and allocated overhead costs. External research and development expenses include, among others, fees paid to contract research organizations to execute preclinical studies and clinical trials on our behalf, and consulting fees. In connection with the closure of our Genetic Medicines Manufacturing Center (GMMC), our external research and development expenses may include fees paid to third-party contract development manufacturing organizations (CDMOs) for future process development and manufacturing activities. We do not allocate our costs by research program, product candidate or proprietary platform technology, as a significant amount of research and development expenses represent internal costs, which are deployed across our programs, product candidates, proprietary platform technology, and other activities.

We expense all research and development costs in the periods in which they are incurred. Costs of certain research and development activities are recognized based on estimates derived from a number of factors, including an evaluation of the progress of the activities, as well as input from external service providers.

The process of conducting the necessary research to advance through the clinical stages 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 cannot reasonably estimate or know the nature, timing or estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates. The level of our research and development expenses over the next twelve months will be subject to operational decisions

made following data generated from our MyPEAK-1 and RIDGE-1 clinical trials and our ability to achieve regulatory alignment on our pivotal trial plans for our TN-201 and TN-401 programs, as well as our Phase 2 development plans for TN-301.

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

(in thousands, except percentages)	Three Months Ended September 30, 2026	2025	$ Change	% Change
Clinical	$ 6,174	$ 4,065	$ 2,109	52%
Research	5,034	3,579	1,455	41%
Manufacturing (pre-commercial)	1,487	4,107	(2,620)	(64%)
Other	2,171	3,612	(1,441)	(40%)
Total research and development expenses	$ 14,866	$ 15,363	$ (497)	(3%)

Research and development expenses were $14.9 million and $15.4 million for the three months ended September 30, 2026 and 2025, respectively. The decrease of $0.5 million, or 3%, was primarily due to:

- a $2.6 million decrease in manufacturing costs, reflecting primarily reduced manufacturing-related expenses following the closure of our GMMC, including lower depreciation, facility-related and personnel costs; and
- a $1.4 million decrease in other research and development costs, reflecting primarily lower stock-based compensation, consulting expenses and personnel-related costs, partially offset by
- a $2.1 million increase in clinical costs, reflecting primarily higher clinical trial expenses associated with our TN-201 and TN-401 programs, as well as the impact of grant funding recognized as a reduction of clinical expenses in the prior-year period; and
- a $1.5 million increase in research cost, reflecting primarily increased research and preclinical development activities associated with our TN-301 program.

General and Administrative

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

We will continue to incur legal, accounting, insurance and other expenses in operating our business as a public company, including costs associated with regulatory and compliance activities. As with our research and development, the level of our general and administrative expenses over the next twelve months will be subject to operational decisions made following data generated from our MyPEAK-1 and RIDGE-1 clinical trials and our ability to achieve regulatory alignment on our pivotal trial plans for our TN-201 and TN-401 programs, as well as our Phase 2 development plans for TN-301.

General and administrative expenses remained relatively consistent at $5.6 million for the three months ended September 30, 2026 and 2025.

Impairment Loss

Impairment loss was $0.7 million for the three months ended September 30, 2026, compared to no impairment loss for the same period in 2025. The impairment loss was primarily related to the write-down of property and equipment classified as held for sale to its estimated fair value less costs to sell.

Interest Income

Interest income primarily consists of interest earned on our cash and cash equivalents balances. Interest income remained relatively consistent at $0.6 million and $0.7 million for the three months ended September 30, 2026 and 2025, respectively.

Net Loss

Net loss for the three months ended September 30, 2026, was $18.9 million, compared to a net loss of $20.3 million for the three months ended September 30, 2025.

Comparison of the Nine Months Ended September 30, 2026 and 2025:

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

(in thousands, except percentages)	Nine Months Ended September 30, 2026	2025	$ Change	% Change
Revenue				
Collaboration revenue	$ 3,032	$ —	$ 3,032	NM
Operating expenses:				
Research and development	46,313	53,809	(7,496)	(14%)
General and administrative	16,501	18,747	(2,246)	(12%)
Impairment loss	22,487	—	22,487	NM
Loss on lease termination, net	1,368	—	1,368	NM
Loss on disposal of property and equipment	89	—	89	NM
Total operating expenses	86,758	72,556	14,202	20%
Loss from operations	(83,726)	(72,556)	(11,170)	15%
Other income, net:				
Interest income	2,126	2,107	19	1%
Other (loss) income, net	(3)	27	(30)	NM
Total other income, net	2,123	2,134	(11)	(1%)
Net loss	$ (81,603)	$ (70,422)	$ (11,181)	16%

NM - Not Meaningful

Research and Development Expenses

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

(in thousands, except percentages)	Nine Months Ended September 30, 2026	2025	$ Change	% Change
Clinical	$ 16,752	$ 15,410	$ 1,342	9%
Manufacturing (pre-commercial)	8,804	13,631	(4,827)	(35%)
Research	11,812	13,681	(1,869)	(14%)
Other	8,945	11,087	(2,142)	(19%)
Total research and development expenses	$ 46,313	$ 53,809	$ (7,496)	(14%)

Research and development expenses were $46.3 million and $53.8 million for the nine months ended September 30, 2026 and 2025, respectively. The decrease of $7.5 million, or 14%, was primarily due to:

- a decrease of $4.8 million in manufacturing costs, primarily reflecting lower depreciation and facility-related expenses associated with the closure of our GMMC, as well as lower employee-related costs resulting from the workforce reduction implemented in March 2025 (the 2025 Workforce Reduction);
- a $2.1 million decrease in other research and development expenses, primarily reflecting lower stock-based compensation and employee-related costs; and
- a $1.9 million decrease in research expenses, primarily reflecting lower employee-related costs resulting from the 2025 Workforce Reduction and reduced laboratory supplies as well as facility-related expenses, partially offset by
- a $1.3 million increase in clinical cost, primarily reflecting increased clinical study expenses and the impact of grant funding recognized as a reduction of clinical expenses in the prior-year period, partially offset by lower clinical consulting and temporary labor costs.

General and Administrative

General and administrative expenses were $16.5 million and $18.7 million for the nine months ended September 30, 2026 and 2025, respectively. The decrease of $2.2 million, or 12%, was primarily due to a $2.2 million decrease in stock-based compensation expense.

Impairment Loss

Impairment loss was $22.5 million for the nine months ended September 30, 2026, compared to no impairment loss for the same period in 2025. The impairment loss primarily related to the $21.8 million write-down of long-lived assets associated with the closure of our GMMC, as well as additional impairment charges on property and equipment classified as held for sale.

Loss On Lease Termination, Net

During the nine months ended September 30, 2026, we recognized a net loss on lease termination of $1.4 million in connection with the termination of the lease for our Union City manufacturing facility. The loss primarily reflected the forfeiture of the facility's security deposit and a brokerage commission incurred in connection with the lease termination, partially offset by gains associated with the remeasurement of the operating lease right-of-use asset and lease liability and the derecognition of the related asset retirement obligation liability. No comparable loss was recognized in the prior-year periods.

Interest Income

Interest income primarily consists of interest earned on our cash and cash equivalents balances. Interest income remained relatively consistent at $2.1 million for each of the nine months ended September 30, 2026 and 2025.

Net Loss

Net loss for the nine months ended September 30, 2026, was $81.6 million, compared to a net loss of $70.4 million for the nine months ended September 30, 2025.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have generated limited collaboration revenue and no product revenue, and we have incurred significant net losses and negative cash flows from operations. From our inception through September 30, 2026, we have funded our operations primarily from the sale and issuance of our equity securities. As of September 30, 2026, we had cash and cash equivalents of $66.8 million and an accumulated deficit of $686.6 million.

CIRM Grant

In February 2025, we announced we were awarded an $8.0 million grant from the CIRM to support RIDGE-1. The award is payable to us upon achievement of certain clinical milestones. We expect this grant to provide funding for a portion of our research and development activities, subject to the achievement of applicable milestones and compliance with the grant terms.

Funding Requirements

We expect that we will continue to incur operating losses over the foreseeable future. Our operating expenses may increase in the future, if and as we:

- continue to advance our lead product candidates, TN-201, TN-401 and TN-301;
- expand the scope of our existing clinical trials and transition into late-stage clinical development;
- seek regulatory and marketing approvals of any of our product candidates that successfully complete clinical trials;
- establish relationships with CDMOs to support commercial-scale manufacturing;

- expand our operational, financial, and information systems and personnel to support our future product development and commercialization efforts;
- seek to identify additional research programs and additional product candidates;
- initiate preclinical studies and clinical trials for any additional product candidates we identify;
- advance our future product candidates into clinical development;
- maintain, develop, expand, enforce, defend and protect our intellectual property portfolio; and
- continue to operate as a public company.

Based on our current operating plan, we believe that our existing cash and cash equivalents 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, 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 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 global economic conditions or disruptions to, and volatility in, the credit and financial markets in the United States and worldwide. 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 presented:

	Nine Months Ended September 30,	
	2026	2025
	(In thousands)	
Net cash provided by (used in):		
Operating activities	$ (40,470)	$ (54,324)
Investing activities	(144)	56,150
Financing activities	6,864	50,163
Net change in cash, cash equivalents and restricted cash	$ (33,750)	$ 51,989

Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2026 was $40.5 million, which consisted primarily of a net loss of $81.6 million, partially offset by $35.0 million in non-cash charges and a net change in operating assets and liabilities of $6.2 million. The change in net operating assets and liabilities was primarily due to an increase of contract liabilities of $8.5 million, partially offset by a decrease in operating lease

liabilities of $2.6 million. Cash flows from operations are generally impacted by the timing of payments to vendors and vendor payment terms. The non-cash charges primarily consisted of loss on impairment of $22.5 million, stock-based compensation of $5.6 million and depreciation and amortization of $3.9 million.

Net cash used in operating activities for the nine months ended September 30, 2025 was $54.3 million, which consisted primarily of a net loss of $70.4 million and a net change in operating assets and liabilities of $3.3 million, partially offset by $18.6 million in non-cash charges. The change in net operating assets and liabilities was primarily due to a decrease in accounts payable and accrued expenses and other current liabilities of $2.6 million. Cash flows from operations are generally impacted by the timing of payments to vendors and vendor payment terms. The non-cash charges primarily consisted of stock-based compensation of $10.3 million and depreciation and amortization of $6.6 million.

Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2026 was $0.1 million, which consisted of purchases of property and equipment, partially offset by proceeds from sales of property and equipment.

Net cash provided by investing activities for the nine months ended September 30, 2025 was $56.2 million, which consisted primarily of proceeds from maturities of marketable securities of $45.7 million and sales of marketable securities of $11.0 million.

Financing Activities

Net cash provided in financing activities for the nine months ended September 30, 2026 was approximately $6.9 million, which consisted primarily of $4.9 million proceeds from exercise of warrants and $2.4 million net proceeds from at-the-market sales, partially offset by payments of accrued offering costs.

Net cash provided by financing activities for the nine months ended September 30, 2025 was $50.2 million, which primarily consisted of net proceeds from our March 2025 follow-on offering of $48.8 million.

Contractual Obligations and Other Commitments

Other than the early termination of the Union City lease in connection with the closure of our GMMC, as discussed in Note 5, *Commitments and Contingencies*, to our condensed financial statements, there have been no material changes from the contractual obligations and commitments previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2025.

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

A summary of our critical accounting policies and estimates is presented in Part II, Item 7 of our Annual Report on Form 10-K for the year ended December 31, 2025. There were no material changes to our critical accounting policies and estimates during the three and nine months ended September 30, 2026.

Recent Accounting Pronouncements

See Note 2, *Summary of Significant Accounting Policies*, 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 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 $1.235 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 companies, 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 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, has 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 (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, 2026, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on Effectiveness of Controls

A control system, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the benefits of possible controls and procedures relative to their costs. In addition, the design of any system of controls is based in part upon certain assumptions about the

likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we 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, 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 require substantial additional capital to finance our operations, which if available, may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. 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 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.
- Drug development involves a lengthy and expensive process with an uncertain outcome. The preclinical studies, clinical trials and post-marketing studies of our product candidates may not demonstrate safety and efficacy to the satisfaction of the FDA, European Medicines Agency (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 delay or prevent regulatory approval, or market acceptance, or even if approval is received, require them to be taken off the market, include new safety warnings, contraindications or precautions, or otherwise 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.
- 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.
- 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.
- We rely on third parties to conduct our preclinical studies and our clinical trials, and plan to rely on third parties to conduct such future drug development activities. These third parties may not perform satisfactorily, including failing to meet completion deadlines, or to comply with applicable regulatory requirements, which may harm our business.
- If we do not regain compliance with or continue to satisfy the Nasdaq continued listing requirements, our common stock could be delisted from Nasdaq. Our ability to publicly or privately sell equity securities and the liquidity of our common stock could be adversely affected if our common stock is delisted.

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 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 limited experience conducting clinical trials, have no products approved for commercial sale and have not generated any product 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. Since our inception, we have devoted substantially all of our focus and financial resources to identifying and developing product candidates, conducting preclinical studies and clinical trials, developing our internal capabilities, 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 complete any late-stage clinical trials, obtain marketing approvals, manufacture a late stage 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 would 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. 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 have financed our operations principally through issuances of our stock. As of September 30, 2026, we had an accumulated deficit of $686.6 million. Substantially all of our losses have resulted from expenses incurred in connection with our research and development programs, manufacturing activities and from general and administrative costs associated with our operations. 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 late-stage clinical development, where costs may increase significantly. 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.

Our business depends on the successful research, development, manufacturing, regulatory approval and commercialization of product candidates that we discover. 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, including, but not limited to, generating sufficient data to support the initiation or continuation of clinical trials;
- submission of INDs or other regulatory applications for our planned clinical trials, obtaining regulatory approval to commence clinical trials of our product candidates, and achieving favorable results from clinical trials;
- establishing and maintaining relationships with contract research organizations (CROs) and clinical sites for the clinical development of our 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;
- efficacy and safety profiles that are satisfactory to the 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;
- developing an efficient and scalable manufacturing process for our product candidates, and the timely manufacture of sufficient quantities of a product candidate for use in clinical trials and, if approved, commercialization;
- 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;
- the achievement of development, regulatory and sales-based milestones under our collaboration agreement with Alnylam;
- successful outputs from our capsid engineering and promoter and regulatory elements efforts;
- a continued acceptable safety profile following any marketing approval of our product candidates;
- 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 of our product candidates by patients, the medical community and third-party payors;
- our ability to distribute our products to certain segments of the patient population only accessible through restricted or closed distribution channels;
- satisfying any required post-marketing approval commitments to applicable regulatory authorities;
- maintaining consistent quality, purity, and potency across clinical supplies and commercial supplies for any approved products;
- identifying, assessing and developing new product candidates, and our ability to expand into multiple indications;
- obtaining, maintaining, and expanding patent and other intellectual property protection, trade secret protection and regulatory exclusivity, both in the U.S. 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 and to meet our obligations set forth under such arrangements;
- 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; 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 our

value 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 require substantial additional capital to finance our operations, which, if available, may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs or future commercialization efforts.

Developing 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 over the long-term in connection with our ongoing activities, particularly as we initiate and conduct clinical trials of, and seek marketing approval for, our product candidates. 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 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 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.

As of September 30, 2026, we had $66.8 million in cash and cash equivalents. 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 preclinical development programs, platforms, manufacturing activities, ongoing or planned clinical trials or future commercialization efforts.

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. For example, under our collaboration agreement with Alnylam, we are not permitted to conduct any research or development activities with respect to certain collaboration targets or any therapeutic products designed to be directed to such targets, for as long as the target remains a collaboration target. If we are unable to raise additional funds through equity or debt financings, 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 on their use under U.S. tax law. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, 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. In addition, the use of our NOLs and other tax attributes may be subject to other limitations under applicable law. For example, California has enacted a temporary suspension on the use of state NOLs in taxable years beginning in 2024, 2025 and 2026, which would adversely affect our company if we earn taxable income in 2026. Consequently, our ability to use our NOLs and certain other tax attributes may be limited.

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.

Before we are able to generate any revenue from product sales, each of our programs and product candidates will require additional preclinical and/or clinical development, expansion of manufacturing capabilities and expertise or successfully outsourcing manufacturing, regulatory approval, building a commercial organization or successfully outsourcing commercialization, substantial investment and significant marketing efforts. Consequently, because of the substantial operational and financial investment required to further develop and commercialize our product candidates, 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 clinical trials in a timely manner, including the successful manufacturing of the relevant product candidate, or fail to achieve favorable results from our trials, we may experience significant delays or be unable to advance 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. Furthermore, any changes to our development programs may cause our product candidates to perform differently and affect the results of planned clinical trials, which 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.

There is a high failure rate for biopharmaceutical products proceeding through clinical trials. 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 or earlier clinical studies. In addition, 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 and we may experience the same. We may also encounter regulatory delays or rejections as a result of many factors, including varying interpretations of data or 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 those described in the Risk Factor entitled "*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 do not have control over many of these factors, including certain aspects of the manufacturing process, preclinical and clinical development, the regulatory review process and potential threats to our intellectual property rights. If we are not successful with respect to one or more of these factors, 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 product candidates 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. Even if we obtain approval and begin commercializing one or more of our product candidates, we may never generate revenue that is significant enough to achieve profitability, as we will continue to incur substantial research and development, manufacturing and other expenditures to develop

and market additional product candidates. Even as 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.

Within the broader genetic medicine field, very few therapeutic products, including those that utilize AAV-mediated gene transfer, have received marketing authorization from the FDA, EMA or comparable foreign regulatory authorities. 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, how much it will cost, or how likely it will be to obtain regulatory approvals for our product candidates in the U.S., EU or other jurisdictions.

The regulatory requirements that will govern any novel gene therapy product candidate we develop are not entirely clear, have changed over time and are subject to further change. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. 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 or preclude 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 U.S., 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 investigational 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 clinical study sites receive NIH funding and 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 EU. 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 EU, 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 in the EU may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point. Furthermore, 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 gene therapy 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 trials and approval of our product candidates or limit the use of products utilizing gene regulation technologies, either of which could harm our business. For example, the FDA has imposed clinical holds on various clinical trials of gene therapy product candidates being developed by other companies. 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 our product candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop our 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 our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our 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 see for our compounds in preclinical models may not be replicated in subsequent preclinical studies or translate into similar results in humans in clinical trials, and results of early clinical trials in humans may not be predictive of the results of larger clinical trials or post-marketing studies 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.

Drug development involves a lengthy and expensive process with an uncertain outcome. The preclinical studies, clinical trials and post-marketing studies 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.

Preclinical and 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 preclinical studies or clinical trials will be initiated, conducted or completed on schedule or as planned, or at all. Failure can occur at any stage of testing. Such failure may result from a multitude of factors, including, among other things, flaws in study design, dose selection issues, placebo effects, patient enrollment criteria, novel assay design and failure to demonstrate favorable safety or efficacy traits, which could delay or prevent the submission of an IND or clinical trial application, initiation of a clinical trial, receipt of marketing approval or our ability to commercialize our product candidates, or require us to suspend or terminate further development of our product candidates. Moreover, 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 preclinical studies, clinical trials or post-marketing studies that we conduct will demonstrate consistent or adequate efficacy and safety to support marketing approval.

Further, FDA and other regulatory authorities may implement new policies and regulations on clinical trials. For example, the EU Clinical Trials Regulation (CTR), which repealed the EU Clinical Trials Directive, became applicable on January 31, 2022, and provided a three-year transition period. The CTR streamlined the processes for applying for authorization and supervision of clinical trials in the EU. From January 31, 2025, any trials approved under the Clinical Trials Directive that continue running will need to comply with the CTR, and their sponsors must enter information on the trials in the Clinical Trials Information System. Trials we initiate in the United Kingdom are also subject to regulatory requirements and policies of the MHRA. Compliance with the CTR and/or MHRA requirements by us, our collaborators and third-party service providers, such as CROs, may increase our clinical trial costs and impact the timeline of our development plans. If we are slow or unable to adapt to changes in clinical trial requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be negatively impacted.

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.

We may experience numerous unforeseen events during, or as a result of, preclinical studies, clinical trials or post-marketing studies 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 clinical trials;
- receipt of feedback from regulatory authorities that requires us to modify the design of our preclinical or clinical trials;
- preclinical study or clinical trial observations or results that require us to modify the design of our clinical trials;
- negative or inconclusive preclinical study or clinical trial results that may require us to conduct additional preclinical studies or clinical trials or abandon certain research and/or drug development programs;
- extended IRB, IBC and/or EC review process, or inability to obtain approval from one or more of these committees;
- the number of patients required for clinical trials being larger than anticipated, enrollment in these clinical trials being slower than anticipated, participants dropping out of these clinical trials at a higher rate than anticipated, or more patients failing to meet eligibility criteria than anticipated;
- any failure or delay in reaching an agreement with CROs and clinical trial sites;
- the suspension or termination of our clinical trials, as a result of a clinical hold by regulatory authorities or a voluntary pause, for various reasons, such as we experienced with MyPEAK-1;
- changes to clinical trial protocol;
- clinical sites deviating from trial protocol or dropping out of a trial;
- the costs and/or duration of preclinical studies or clinical trials 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 or slower than anticipated;
- subjects experiencing serious, severe, unexpected or otherwise important drug-related or study-related adverse effects;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- inaccurate or untimely clinical data collection, entry, analysis or reporting by clinical sites, third-party contractors and/or CROs;
- variability of efficacy assessments;
- 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 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, GCP or other regulatory requirements;
- 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;
- an unsuccessful post-marketing study or failure to complete such a study;
- 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.

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 more 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 trials, such as bridging studies and additional safety studies before we can commence our clinical trials in pediatric populations. The FDA or other health authorities may require us to complete studies in adults prior to initiating testing in children. Any delays in our planned clinical development activities for pediatric patients could have an adverse effect on our business operations.

If we are required to conduct additional preclinical studies or clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete preclinical studies or clinical trials of our product candidates or other testing in a timely manner and if the results of these studies, trials or tests are not positive or are only modestly positive or if there are safety concerns, we may incur unplanned costs and be delayed in submitting an IND, initiating clinical trials or seeking and obtaining marketing approval. We may also decide to change the design or protocol of one or more of our planned clinical trials, which could result in increased costs and expenses and/or delays. Any delays in initiating or completing our preclinical studies or clinical trials will increase our costs, slow down our development and approval process and jeopardize our ability to commence product sales and generate revenues, including by shortening any period during which we may have the exclusive right to commercialize our product candidates and permitting our competitors to bring products to market before we do. If we receive approval, it is possible that we may receive 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, which 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 delay or prevent regulatory approval or market acceptance, or even if approval is received, require them to be taken off the market, include new safety warnings, contraindications or precautions, or otherwise 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. Patients who received our product candidates have suffered and may continue to suffer adverse events, including serious adverse events or other side effects, including those not observed in our preclinical studies or previous clinical trials. Patients treated with our product candidates may also be undergoing other therapies or procedures which can cause side effects or adverse events that are unrelated to our product candidates but may still impact the success of our clinical trials. The inclusion of critically ill patients in our clinical trials and other programs may result in deaths or other adverse medical events, either during the course of or after receiving our product candidates. These events may be due to one or more factors, including, without limitation, the underlying heart disease, other therapies or medications that such patients may be using, the drug product formulation of our product candidates, complications arising from protocol regimens, the method of delivery of our product candidates or other diseases the patients have. In some cases, it may not be clear if an adverse event is due to the product candidate, another therapy, the underlying disease, or another cause, and causality may be incorrectly attributed to the product candidate.

To the extent we supply any of our product candidates to independent third parties, such as investigator-initiated clinical studies or physicians who initiate and sponsor treatment protocols under expanded access, or compassionate use, outside of well-controlled studies, where we are not the sponsor and thus do not control the conduct of such treatment protocols or clinical studies, unexpected or serious adverse events, even if not directly related to our product candidate, or non-compliance by such third parties could have a material adverse effect on our clinical development plans, result in negative press, or raise questions regarding the safety profile or effectiveness of our product candidates. By supplying our product candidates for such limited uses outside of well-controlled studies sponsored or conducted by us could expose us to additional regulatory obligations and risks, including reporting obligations to the FDA and other regulatory authorities under expanded access regulations and exposure to potential product liability.

Serious adverse events or other side effects observed in any of our clinical trials, through our expanded access program, or in similar trials by other sponsors, may result in difficulty recruiting patients to the clinical trials, cause patients to drop out of our trials, or require that we 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 or that the expected benefit does not justify the risk. For example, in November 2025, we announced that the FDA placed MyPEAK-1 on clinical hold to request a protocol amendment, primarily to standardize activities related to patient monitoring and management of the immunosuppressive regimen across trial sites. While the hold was lifted after swift and collaborative engagement with the FDA, there is no assurance that it or any future hold on our clinical trials would not have a material adverse effect on our business or our data milestones or development timelines for TN-201 or other product candidates.

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. 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 such side effects from escalating to an unsafe level for our patients. 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, result in marketing approval with restrictive label warnings or for limited patient populations, or result in potential product liability claims. 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. No regulatory agency has made any determination that any of our product candidates or discovery programs is safe or effective for use by the

general public for any indication. We cannot predict whether our product candidates will cause toxicities in humans that would preclude regulatory approval, or if approved, 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 and success in one trial does not ensure success in the next.

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. Patient enrollment and retention are significant factors in the timing of clinical trials and our ability to enroll eligible patients may be limited or slower than we anticipate.

We are developing product candidates for the treatment of heart disease, including for certain indications, such as rare genetic diseases, that have 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. Patients also have the right to withdraw from our clinical trials for any reason. Enrollment may also be impacted by an IRB or ethics committee decision to pause or stop enrollment at a trial site, a DSMB recommendation to pause or stop trial enrollment, or a decision by a regulatory authority to pause or stop trial enrollment in a particular country. Additionally, the FDA, EMA or other comparable foreign regulatory authorities may require long-term follow-up assessments for a certain number of patients, which could delay marketing approval.

We also expect patient enrollment to be affected because our competitors have ongoing clinical trials for programs that are under development or are approved 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 or choose to take an approved medication. Patient enrollment for our clinical trials has been and may continue to be affected by other factors, including:

- size and nature of the patient population;

- the 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;
- ongoing clinical trials evaluating other product candidates in the same 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;
- regulatory actions, ongoing IRB and/or ethics committee decisions and DSMB recommendations;
- 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;
- public perception about the use of genetic medicines in human therapeutics or precision medicine;
- patient referral practices of physicians;
- challenges associated with recruiting eligible patients;
- the ability to monitor patients adequately during and after treatment;
- limited staff and resources at clinical trial sites, including support for clinical trial enrollment and the availability of hospital beds;
- the activities of key opinion leaders (KOLs) and patient advocacy groups;
- proximity and availability of clinical trial sites for prospective patients and the ability of patients to travel to these sites;
- the practical and financial burden of the trial protocol on patients, including conflicts with their work, family and personal activities, as well as travel costs, lodging, lost wages and insufficient reimbursement or support;
- delays in site activation, contracting and budget approvals;
- protocol amendments, reviews and approvals;
- 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; and
- limitations on the rate of patient enrollment required by the clinical trial protocol, including those that may be requested by health authorities.

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 clinical trials, we must decide which programs, product candidates and indications to pursue and advance 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 result in the diversion of 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 assumptions and/or determinations regarding data emerging from our preclinical studies or clinical trials, 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 U.S. and internationally, including major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, emerging and start-up companies, universities and other research institutions. We face competition in recruiting personnel, 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 and anticipate substantial direct competition from a variety of competitors. 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. Due to the nature of gene therapy products, use of a competitor gene therapy product by a prospective patient may preclude use of our gene therapy product candidate at a later point in time. 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.

Initial, interim and topline 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 initial, interim or topline data from our clinical trials. These updates are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following availability of additional data and 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 initial, interim and/or topline results that we report may differ from future results of the same trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Initial, interim and/or 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, initial, interim and 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. Initial and 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 initial and/or interim data and final data could significantly harm our business and prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses, may do their own 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 initial, interim 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.

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 ensure 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, including during the manufacture of drug substance, drug product, filling, labeling, packaging, storage, shipping, QC and testing and/or in connection with release assays, including potency assays, could result in product defects, lot failures, product recalls, product liability claims or insufficient inventory, and may ultimately disrupt or delay the supply of our product candidates.

Although some of our employees have experience in the manufacturing of biopharmaceutical products from prior employment at other companies, we as a company have limited experience in manufacturing and oversight of CDMOs conducting manufacturing activities on our behalf. We may also encounter problems hiring and retaining the experienced personnel needed to manage our complex manufacturing operations, including those outsourced to CDMOs. If we experience unanticipated employee shortage or turnover in any of these areas, we may not be able to maintain oversight of our QC, conduct further process improvements or meet our product development timelines; we may also experience difficulties in maintaining compliance with applicable regulatory requirements, which would impair our product development and commercialization efforts.

To date, our product candidates have been manufactured in quantities adequate for preclinical studies and our Phase 1b/2 clinical trials for our lead product candidates. We will need to manufacture product candidates in larger

quantities for commercialization of the resulting product, if that product candidate is approved for sale, and we may need to manufacture more product to conduct later-stage clinical trials. 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.

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. Moreover, as a result of the closure of our GMMC, we may be required to conduct additional studies to ensure comparability of TN-201 and TN-401, notify and submit additional filings to regulatory authorities, and obtain regulatory approval for any new facilities used to produce these gene therapy clinical product candidates, which approval may be delayed or never received. While the manufacturing process alone is complex, quality issues may also arise during drug product filling, labeling, packaging, storage, shipping and ongoing QC and testing activities.

If we are unable to successfully manufacture 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.

The manufacture of our product candidates will be subject to significant government regulations and approvals, which are often costly and could result in adverse consequences to our business if we or our CDMO's 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 and our CDMOs 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 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 CDMOs or 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 non-compliance 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. Furthermore, regulatory requirements for the manufacturing of genetic medicines may change over time. Our failure to comply with such changes could have a material impact on the manufacturing costs for our product candidates, delay our planned preclinical and clinical trial timelines and/or preclude regulatory approval 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 or efficacy 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;
- 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 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 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;
- 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;
- negative public perception about the use of genetic medicines, whether related to our technology or our competitor's technology;
- unfavorable publicity relating to our product candidates;
- the approval of other new therapies for the same indications; and
- the acceptance and use of genetic testing required to diagnose the disease and identify patients who qualify for treatment with our product candidates.

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.

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 for our gene therapy product candidates 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 U.S. 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 U.S., for example, principal decisions about reimbursement for new products are typically made by CMS, an agency within the U.S. Department of Health and Human Services. 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 U.S., 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 EU, 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 U.S. 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 U.S., the reimbursement for our products may be reduced compared with the U.S. 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 have limited product liability insurance. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. As clinical trial and product liability insurance becomes increasingly expensive, 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 or any medications, procedures or activities associated with our clinical trial protocols or our expanded access program cause or are perceived to cause injury, or if our product candidates 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 protocol or 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 a 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 the facilities used to manufacture our products 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;
- 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;
- inability to raise capital or enter into strategic agreements for the development of our product candidates;
- 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, distribution and orphan exclusivity of drugs. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process must be successfully completed in the U.S. 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 and the availability of alternative therapies. 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.

We cannot provide assurance that any of the product candidates we develop will progress through required clinical testing and obtain the regulatory approvals necessary for us to begin selling them. Applications for our product candidates could be delayed or 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 refuse to accept an application or decide not to accept data from our clinical trials conducted in locations outside of their jurisdiction;
- 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;
- the FDA, EMA or other comparable foreign regulatory authorities may require that we conduct additional 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 CDMOs and/or third-party manufacturers with which we contract for clinical and commercial supplies;
- the FDA, EMA or other comparable foreign regulatory authorities may fail to approve companion diagnostic tests required for commercialization of our product candidates; 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.

Further, the FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines. It is unclear how our industry and our clinical programs will be impacted by policies, regulations and initiatives implemented under the current administration and FDA commissioner, or other executive orders. There is significant uncertainty in the industry and how federal agencies like the FDA will operate under the current administration, which may delay or negatively impact our regulatory review processes with the FDA.

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.

If the FDA or EMA grants marketing approval of a product candidate, other comparable regulatory authorities in foreign jurisdictions must still approve the manufacturing, marketing and promotion and reimbursement of the product candidate in those countries. 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 U.S., 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 U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction.

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 our product candidates receive regulatory approval, such approval may be for a narrower indication than we seek, and our product candidates will be subject to significant post-marketing regulatory requirements and oversight.

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. 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. The 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. In addition, the FDA has the authority to require a Risk Evaluation and Mitigation Strategy plan as part of approving an NDA or biologics license application, 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. 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, and may significantly limit the size of the market for the drug and affect reimbursement by third-party payors.

In addition, if the FDA, EMA or other 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 cGMP and GCP for any clinical trials that we conduct post-approval. Manufacturers of drug products and the facilities where the products are manufactured are also 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 pediatric populations can also result in significant financial penalties.

To the extent we obtain orphan drug and other designations from the FDA for our product candidates, we may not realize the full benefits of such designations. Further, orphan drug exclusivity may not prevent the FDA, EMA or other comparable foreign regulatory authorities, from approving competing products.

Regulatory authorities in some jurisdictions, including the U.S. and the EU, 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 U.S., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. Similarly, in the EU, the EC, 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. In the EU, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 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 the U.S., 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 U.S. 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. In view of the court decision in the *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021) case, in a January 2023 notice, the FDA clarified that while the agency complies with the court's order in *Catalyst*, the FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the *Catalyst* order. The Consolidated Appropriations Act of 2026, signed into law in February 2026, codified this longstanding FDA interpretation of the Orphan Drug Act, allowing the FDA to approve multiple versions of the same orphan drug for different subindications and subpopulations. The applicable exclusivity period for an orphan drug is ten years in the EU. The EU orphan 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.

Under the Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval for a drug or biological product for a rare pediatric disease may qualify for a voucher that can be redeemed to receive priority review for a different product. The sponsor may also transfer or sell the voucher to another sponsor. FDA awards rare pediatric disease priority review vouchers to sponsors of rare pediatric disease products that are approved and meet certain criteria, including a product candidate intended to treat a manifestation of a serious or life-threatening disease or condition in children aged 0 through 18 years of age. The rare pediatric disease priority review program has been extended by Congress through September 2029 under the Consolidated Appropriations Act, 2026, which means the FDA may not award any priority review vouchers under this program after September 30, 2029. There is no guarantee that any of our product candidates will be approved by that date, or at all. We may not obtain a priority review voucher prior to expiration of the program, unless Congress further reauthorizes the program.

All of our clinical product candidates have been granted orphan drug designation by the FDA and/or the EC, and we may seek orphan drug designation for other product candidates in the U.S., Europe and other jurisdictions. Both TN-201 and TN-301 have also received rare pediatric disease designation from the FDA for MYBPC3-associated HCM and DMD, respectively. We may not be able to maintain orphan drug exclusivity for our product candidates and may not realize all the benefits of the orphan drug designation and the rare pediatric disease designation. Receiving these designations does not change FDA's standards for regulatory approval of our product candidates and may not lead to faster regulatory review of any product candidate or increase the likelihood that any product candidate will receive marketing approval, if at all. We may seek orphan drug designation for other product candidates in the U.S., Europe and other jurisdictions, however, there can be no assurances that we will be able to obtain orphan drug designation for our other product candidates.

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, in which case we could be precluded from receiving marketing approval for our product candidate for the applicable exclusivity period. In addition, exclusive marketing rights in the U.S. 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. 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 U.S. 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 the 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.

In June 2024, the U.S. Supreme Court overruled the Chevron doctrine, which gives deference to regulatory agencies' statutory interpretations in litigation against federal government agencies, such as the FDA, where the law is ambiguous. This landmark Supreme Court decision may invite more companies and other stakeholders to bring lawsuits against the FDA to challenge longstanding decisions and policies of the FDA, including FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, any of which could delay the FDA's review of our regulatory submissions. Further, decreases in FDA staff and resources under the current administration could impact continuity of our interactions with the agency and increase delays in our correspondence and regulatory review processes.

The pharmaceutical industry in the U.S. has also been significantly impacted by other major legislative initiatives. Under the American Rescue Plan Act of 2021, the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs was 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 August 2022, Congress passed the IRA, which includes prescription drug provisions that have significant implications for the pharmaceutical industry and Medicare beneficiaries, including allowing the federal government to negotiate a maximum fair price for certain high-priced single source Medicare drugs, imposing penalties and excise tax for manufacturers that fail to comply with the drug price negotiation requirements, requiring inflation rebates for all Medicare Part B and Part D drugs, with limited exceptions, if their drug prices increase faster than inflation, and redesigning Medicare Part D to reduce out-of-pocket prescription drug costs for beneficiaries, among other changes. Various stakeholders, including pharmaceutical companies and the

Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of the IRA are unconstitutional. Further, the current administration has issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of Health and Human Services to establish a mechanism through which American patients can buy drugs directly from manufacturers who sell at a most-favored-nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the United States. In September and October 2025, the government announced the first agreements with two major pharmaceutical companies to bring American drug prices in line with the lowest paid by other developed nations, requiring the companies to offer medicines at a deep discount off the list price when selling directly to American patients. In January 2026, CMS launched the GENEROUS Model (GENErating cost Reductions fOr U.S. Medicaid Model), which aims to ensure fair and reasonable drug prices for Medicaid through CMS-led negotiations with drug manufacturers. Under the GENEROUS Model, manufacturers will provide supplemental rebates to participating states for drugs included in the model to align Medicaid net prices with what certain other countries pay. The GENEROUS Model is voluntary for manufacturers and states. In October 2026, CMS published the Global Benchmark for Efficient Drug Pricing (GLOBE) Model Final Rule, which will implement a mandatory Medicare Part B five-year demonstration payment model that will require manufacturers of certain high-spend Part B drugs and biologicals to pay rebates to CMS tied to international drug prices for those products. The Final Rule added exclusions for orphan-only drugs, cellular and gene therapy products, and plasma-derived products, and delayed the start of the first performance year to April 1, 2027. CMS also noted that it intends to waive GLOBE's mandatory participation requirements for manufacturers in any calendar quarter during which they are participating in the GENEROUS Model. The GLOBE Model final rule is subject of ongoing litigation, which can impact its implementation. These and other government measures that use most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the United States, or increases generic and biosimilar drug entry sooner than expected, that can have a material adverse effect on our industry, ability to set adequate pricing for new drugs to recover R&D costs, ability to attract potential investors and potential buyers in the future. We cannot predict the full impact of the executive orders focused on reducing prescription drug prices or increasing domestic drug manufacturing capacity, or other measures that may be implemented by the current administration related to drug pricing, drug supply chain and manufacturing in the United States. The impact of ongoing and future judicial challenges as well as future legislative, executive, and administrative actions and any future healthcare measures and agency rules implemented by the current administration on us and the pharmaceutical industry as a whole is unclear. Further, changes to the leadership of federal agencies under the current administration, as well as new policies, executive orders and actions, such as a freeze on hiring, return-to-office policy, and a freeze on implementing new regulations and on external communications, may impact normal operations of the FDA and other agencies or result in a material impact on our clinical development plans and timelines. The implementation of cost containment measures, including the prescription drug provisions under the IRA, as well as 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 U.S. 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.

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, transfer, security and other processing of personal information.

Outside of the U.S., we are subject to extensive legal requirements relating to privacy, data protection, security and the collection, distribution, use, disclosure, storage, transfer and other processing of personal data. For example, the collection and use of health data and other personal data, including personal data collected in clinical trials, is governed in the EU by the GDPR, which imposes substantial obligations upon companies and provides rights for individuals, and by certain EU member state-level legislation. The GDPR also forms part of the law of England and Wales, Scotland and Northern Ireland by virtue of section 3 of the European Union (Withdrawal) Act 2018 and as amended by the Data Protection, Privacy and Electronic Communications (Amendments etc.) (EU Exit) Regulations 2019 (SI 2019/419), known as UK GDPR. Failure to comply with the GDPR or UK GDPR may result in fines up to €20,000,000 (£17.5 million in the UK) or 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties. The GDPR or UK GDPR impose significant responsibility and expose us to potential 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 UK GDPR or with other laws, rules, regulations and standards in the EEA, UK and Switzerland relating to privacy and data protection. This may be onerous and if our efforts to comply with GDPR and UK GDPR or other applicable laws, rules, regulations and standards are not successful, or are perceived to be unsuccessful, it could adversely affect our business. Further, restrictions on the transfer of personal data from the EEA, UK, Switzerland or other jurisdictions to the U.S. or other regions, 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 to the U.S. In Canada, the Personal Information Protection and Electronic Documents Act (PIPEDA) and similar provincial laws impose obligations on companies with respect to processing personal information, including health-related information, regarding Canadian data subjects and provides individuals certain rights with respect to such information, including the right to access and challenge the accuracy of their personal information held by an organization. Failure to comply with PIPEDA, where applicable, could result in fines and penalties.

In the U.S., a variety of laws, rules, regulations and standards relating to privacy, data protection, security, and the distribution, use, disclosure, storage, transfer and other processing of data potentially may apply to our activities, such as state data breach notification laws, state personal data privacy laws, for example, the CCPA, state health information privacy laws, and federal and state consumer protection laws. The CCPA requires covered businesses that process personal information of California residents to disclose their data collection, use, sharing and retention practices, provides California residents with data privacy rights (including the ability to opt out of certain disclosures of personal information including for certain advertising purposes), imposes 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 judgments and settlements). Although there are limited exemptions for clinical trial data under the CCPA and certain other state laws, the CCPA and other new and evolving state laws could impact our business activities, depending on their interpretation. Numerous other states have enacted laws relating to privacy and data security that either are in operation or slated to go into operation over the next several years. In

many cases, these laws are comprehensive privacy laws similar to the CCPA, with potentially greater penalties and more rigorous compliance requirements.

States also are enacting laws addressing specific subject matter, such as Washington's My Health, My Data Act, which includes a private right of action. Laws in all 50 states may require businesses to provide notice to individuals whose personal data has been disclosed as a result of a data breach. The U.S. government also has instituted rules, effective April 8, 2025, that prohibit or restrict transactions involving certain types and amounts of sensitive data between U.S. persons and foreign persons associated with specific countries of concern. Among other things, these new rules require U.S. businesses to seek assurances from certain foreign parties with which they share sensitive data (under certain types of agreements) that those parties will not further share that data with parties in countries of concern. 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 significantly, thus complicating compliance efforts and potentially requiring us to undertake additional measures to comply with them.

With the GDPR, PIPEDA, CCPA, and other laws, regulations and other obligations relating to privacy, data protection and security imposing new and relatively burdensome obligations, and with substantial uncertainty over the interpretation and application of these laws, regulations 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 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. 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.

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 which 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), which 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;
- the HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act 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), certain non-physician healthcare professionals (such as nurse practitioners and physician assistants, among others), and teaching hospitals as well as information regarding ownership and investment interests held by physicians and their immediate family members; 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 non-compliance 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 and implemented a compliance program, 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 are 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 and agents from offering or providing improper payments or benefits to recipients in the public or private sector. 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.

We sometimes leverage third parties to assist with the conduct of our business abroad. As we increase our international business activities, our risks under these laws may increase. We, our employees and agents 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 and agents even if we do not explicitly authorize such activities.

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, we cannot assure you that all of our employees and agents will not take actions in violation of applicable law 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. 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.

There is currently significant uncertainty about the future relationship between the U.S. and various other countries, most significantly China, with respect to trade restrictions, treaties, foreign investment laws, data transfer restrictions, and other limitations on cross-border operations. The U.S. government has made and continues to make significant additional changes in U.S. trade policy and may continue to take future actions that could introduce additional restrictions and negatively impact our business. For example, legislation in Congress known as the BIOSECURE Act was passed as part of the 2026 National Defense Authorization Act, which limits certain U.S. biotechnology companies from using equipment or services produced or provided by select Chinese biotechnology companies, and others in Congress have advocated for the use of existing executive branch authorities to limit those Chinese service providers' ability to engage in business in the U.S. Other new regulations could affect the transfer of certain types of data abroad, including to China, and may add expenses or unforeseen burdens to the process of contracting with service providers. These regulations, or similar laws and regulations in the future, could adversely impact our current or future third-party arrangements with certain companies, including those in China or Chinese-owned U.S. companies, which could delay or impact our clinical trials and consequently delay or obstruct successful commercialization of our product candidates. We cannot predict what actions may ultimately be taken with respect to trade relations between the United States and China or other countries, what products and services may be subject to such actions or what actions may be taken by the other countries in retaliation. If we are unable to obtain or use services from existing service providers or become unable to export or sell our products to any of our customers or service providers, our business, liquidity, financial condition, and/or results of operations would be materially and adversely affected.

Increased tariffs on imports, trade sanctions, other trade restrictions, or a global trade war could increase our costs and materially and adversely affect our business operations and financial condition.

Our business could be negatively affected by tariffs, trade restrictions, and other governmental actions, any of which can be imposed suddenly and unpredictably. For example, there is currently significant uncertainty about the future relationship between the U.S. and various other countries with respect to trade policies, treaties, trade regulations, and tariffs. Beginning in 2025, the U.S. government imposed or announced various new tariffs on certain imports, including commodity-specific, reciprocal, and country-specific tariffs, and additional tariffs may be forthcoming. For example, in April 2025, the U.S. Department of Commerce initiated an investigation into potential tariffs on pharmaceuticals, pharmaceutical ingredients, and derivative products; when these investigations are complete, the U.S. government may decide to levy additional tariffs on these products. These or additional tariffs (and the uncertainty around their implementation) could affect inputs to our products, as well as equipment, materials, or components that we import into the U.S. from our suppliers, which could significantly impact the cost of these items. Retaliatory tariffs could also negatively affect our ability to export and sell our potential products into those countries. If these tariffs are implemented, reinstated or adjusted, if additional tariffs are placed, or if any related countermeasures are taken by the U.S. or other countries, our business, financial condition, and results of operations may be materially harmed.

Trade restrictions, tariffs, and other general economic or political conditions may limit our ability to obtain key materials, components, or equipment for our products or significantly increase supply chain costs and other expenses associated with our business, which could further materially and adversely affect our results of operations, financial condition, and prospects. The ultimate impact of any tariffs or trade restrictions will depend on various factors, including the timing of implementation and the duration, amount, scope, and nature of the tariffs and trade restrictions and reactions from other countries.

Tariffs, trade restrictions or any resulting trade war could negatively affect the global market, including for pharmaceuticals, and could have a significant adverse effect on our business, financial condition, and results of operations.

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

Changes in tax law, including to the orphan drug tax credit and other changes to U.S. and non-U.S. taxation, could increase our tax liability and adversely affect our operating results. For example, starting from January 1, 2022, the Tax Cuts and Jobs Act of 2017 requires taxpayers to capitalize domestic research and development costs in the year incurred and amortize such costs rather than deduct such costs in the year incurred. On July 4, 2025, the U.S. federal tax legislation commonly referred to as the One Big Beautiful Bill Act (the OBBB Act) was enacted, which makes a number of changes to U.S. federal income tax law, including permanently suspending the requirement to capitalize and amortize domestic research and development expenditures and permitting such deductions on a current basis. While we have reflected the effects of the enactment of OBBB Act for the fiscal year ended December 31, 2025, we will continue to evaluate the full impact of the legislation on us. These changes could affect our total U.S. federal tax liability when and if we become profitable.

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.

We are highly dependent on the principal members of our management, our scientific and clinical advisors and consultants, and our scientific and medical staff. 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 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 advisors and consultants 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 long-term plans and strategies, we will need to grow the size of our organization, and we may experience difficulties in managing this growth.

In order to successfully implement our development and commercialization plans and strategies, we expect to need additional managerial, operational, sales, marketing, financial and other personnel in the future.

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, subject to the successful clinical development of our product candidates, 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.

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.

As part of our business, we and our CROs, manufacturers, contractors (including sites performing our clinical trials), consultants and other third parties, collect, maintain and transmit sensitive data on our networks and systems, including our intellectual property and proprietary and confidential business information (such as research data and personal information). 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, manufacturers, 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, inadvertent or intentional actions by our employees, contractors, consultants, business partners, and other third parties and cyber-attacks and other hacking attempts by malicious third parties, which may disrupt or 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 or other data that we process or maintain or that is processed or maintained on our behalf, or other assets. Evolving technologies, including artificial intelligence (AI) technologies that facilitate the identification and exploitation of security vulnerabilities, may increase the risks of security breaches and incidents and the impacts of security breaches and incidents that occur. Although we have not observed material impacts of cyber-attacks on our operations and financial condition to date, we and our third-party service providers have frequently been the target of threats of this nature and we expect these threats and attacks to continue.

Any disruption or security breach or incident resulting in any loss, destruction, unavailability, alteration, disclosure or dissemination of, or damage or unauthorized access to, our data, or any other data that we maintain or otherwise process or that is maintained or otherwise processed on our behalf, or other assets, or for it to be believed or reported that any of the foregoing occurred, could cause us to incur significant liability, including consequential damages, financial harm and reputational damage and the delay of the development and commercialization of our product candidates. The loss, corruption, or unavailability of clinical trial data for our product candidates also could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. We cannot ensure that our data protection or cybersecurity efforts and our investment in information technology, or the efforts or investments of CROs, consultants or other third parties, will prevent breakdowns in our or their systems or have prevented, or will prevent, security breaches or incidents, including those that cause loss, destruction, unavailability, alteration, dissemination of, or damage or unauthorized access to, our data, including personal data, or other assets or other data processed or maintained on our behalf. Any such breakdowns, breaches, or incidents, and any resulting impacts, could have a material adverse effect upon our reputation, business, operations and financial condition.

We also rely on third parties to support the development and manufacture of our product candidates, and any security breaches or other incidents 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' cybersecurity practices is limited. Due to applicable laws, rules, regulations and standards or contractual obligations, we may be held responsible for any cyber-attack, security breach or incident or cybersecurity failure attributed to our third-party service providers as relevant to the information they maintain or otherwise process for us. While we maintain cybersecurity insurance that we believe to be reasonable for our business, our current cybersecurity insurance may not fully cover the damages arising from the assertion of one or more large claims against us in connection with a breach or other cybersecurity-related matter, which 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. We expect to incur significant costs in an effort to detect and prevent security breaches and other incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security breach or other incident. However, we cannot guarantee that we will be able to detect or prevent any such breaches or incidents, or that we can identify, remediate or otherwise address any such breaches or 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. Any disruption or security breach or incident resulting in any loss, destruction, or alteration of, or damage, unauthorized access to or inappropriate or unauthorized disclosure, dissemination or other processing of, our data, including personal data, or other information maintained or otherwise processed on our behalf, or any belief or reporting of any of these matters having occurred, could expose us to litigation and governmental investigations and inquiries, could lead to delays in the further development and commercialization of our product candidates, and could result in significant fines or penalties for any noncompliance with certain state, federal and international privacy and security laws, rules, regulations and standards.

We use AI technologies in various aspects of our business operations, and the use of these technologies may not produce the anticipated benefits and could expose us to additional risks.

We utilize AI technologies in various aspects of our business operations, including drug discovery, clinical trial design, data analysis, and general and administrative functions. We expect that our use of AI technologies may increase as we continue to develop our product portfolio and advance our business. However, these technologies are

rapidly evolving, and there can be no assurance that their use will improve the efficiency, accuracy or success of our research and development activities or business plans.

AI models depend on the quality, quantity and appropriateness of the data and methodologies used to develop, train and operate them, and their outputs may be inaccurate, incomplete, biased or otherwise unreliable. Results generated or assisted by AI models may not be reproducible or predictive of results obtained in preclinical studies, clinical trials or other development activities. If we rely on inaccurate or misleading outputs, we could make incorrect research or development decisions, spend resources on product candidates that ultimately prove unsuccessful, or fail to identify potentially promising product candidates.

Our use of external AI providers may require us to provide or permit access to proprietary data, confidential information or intellectual property. Although we seek to protect such information through contractual, technical, and other safeguards, these measures may not prevent unauthorized access, use, or disclosure. We may also be subject to disputes concerning ownership or rights in data, models, inventions or other intellectual property developed or generated through the use of AI technologies, and third-party AI technologies may incorporate data or intellectual property for which sufficient rights have not been obtained.

The legal and regulatory frameworks applicable to AI technologies are rapidly evolving in the US and other jurisdictions and may impose additional requirements relating to data protection, intellectual property, validation, transparency, cybersecurity or the use of AI in drug development. Compliance with new or evolving requirements could increase our costs, restrict our use of certain technologies or require changes to our research and development or general business practices. Any failure of AI technologies to perform as anticipated, loss or unauthorized disclosure of proprietary information, intellectual property disputes, cybersecurity incidents, or failure to comply with applicable legal or regulatory requirements could adversely affect our research and development activities, competitive position, business, financial condition and results of operations.

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 earthquake, flood, blizzard, wildfire, 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 development and marketing our product candidates internationally, subject to regulatory approval in applicable jurisdictions, could materially adversely affect our business.

We may seek regulatory approval of our product candidates outside of the U.S. and/or work with contractors or partners in foreign jurisdictions, and we expect that we will be subject to additional risks and requirements related to our operations in foreign countries, including:

- differing regulatory requirements and reimbursement regimes;
- 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 U.S.;
- 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 U.S.;
- 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

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 relating to our product candidates and platforms and to operate without infringing, misappropriating or otherwise violating the intellectual property of others. Additionally, recent reforms and changes at government agencies of the U.S. and those of non-U.S. jurisdictions could increase the uncertainties, timing and costs surrounding the prosecution or maintenance of our patent applications, and the maintenance, enforcement, or defense of our issued patents. We rely on patent, copyright, trade secret and trademark laws in the U.S. and certain other countries to protect our technology, and we generally seek to protect our position by filing patent applications in the U.S. and abroad and by acquiring or in-licensing relevant issued patents or pending applications from third parties. However, these efforts may provide only limited protection. 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.

Pending patent applications cannot be enforced until issued, and then only to the extent the issued claims cover the product candidate or relevant 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 designed around or invalidated by third parties.

Even issued patents may later be found invalid or unenforceable, or they may be modified, narrowed in scope, or revoked in proceedings instituted by third parties before various patent offices or in courts in the U.S. 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. 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 and platforms 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

non-compliance 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.

We may be unable 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. 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. Furthermore, 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. 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 and platforms or which effectively prevent others from commercializing competitive product candidates and technologies 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.

While we believe our intellectual property allows us to pursue our current development programs, we may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. 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. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. 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.

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 patents, potential future patents or the patents of our licensors may be challenged in the courts or patent offices in the U.S. and abroad. For example, we may be subject to a third-party pre-issuance submission of prior art, post-grant review or inter partes review at the USPTO, or other similar proceedings including, opposition, derivation, revocation or reexamination proceedings in the U.S. or abroad. A third party may also claim that our 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 patents, potential future patents or licensed patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us. Such proceedings also may result in substantial cost and require significant time from our scientists, manufacturing staff and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our patents, 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 our product candidates.

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.

Our research, development and commercialization activities may be subject to claims that we infringe, misappropriate or otherwise violate patents or other intellectual property rights of others. Additionally, other entities may have, develop or obtain patents that could impair our competitive position or limit our ability to make, use, sell, offer for sale or import our product candidates. There is a substantial amount of litigation, both within and outside the U.S., involving patent and other intellectual property rights in the biotechnology industry. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. Third-party patents or patent applications may include 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/or 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 both within and outside the U.S., 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 against such assertions, including that such patent rights are invalid and/or not infringed. 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.

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. 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. We may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. 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. 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. 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 ambiguity in the meaning of patent claims. Generative artificial intelligence resources that are publicly available also present a risk that a company may inadvertently obtain, incorporate or use a third party's intellectual property.

Third parties may assert patent infringement claims against us directed at any of our product candidates based on 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 would involve substantial litigation expense and would be a substantial diversion of management and employee resources from our business. 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. 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.

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 our 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 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.

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.

Changes in either the patent laws or in the interpretations of patent laws in the U.S. 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.

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.

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 U.S. 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. For example, the U.S. Supreme Court held in Amgen v. Sanofi (2023) that a functionally claimed genus was invalid for failing to comply with the enablement requirement of the Patent Act. In addition, the U.S. Court of Appeals for the Federal Circuit recently issued a decision involving the interaction of a patent term adjustment, terminal disclaimers, and obvious-type double patenting. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Similarly, foreign courts have made and will 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 U.S. and foreign legislative bodies. For example, the IRA passed by Congress authorizes the Secretary of the Department of HHS to negotiate prices directly with participating manufacturers for selected medicines covered by Medicare even if these medicines are protected by an existing patent. While we do not believe that the IRA or its effects will impact our ability to obtain patents in the near future, we cannot be certain whether it will affect our patent strategy in the long run. 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 patents, 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 patents, patent applications or in-licensed patents, trade secrets or other intellectual property rights as an inventor or co-inventor. For example, although we are not aware of any inventorship disputes as of the date of this periodic report, 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 product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership of our patents, 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 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 impact 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 U.S., 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. 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 U.S. 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 project or 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 project or request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

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 U.S. can be less extensive than those in the U.S. 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 U.S., even in jurisdictions 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 U.S., even in jurisdictions where we or our licensors do pursue patent protection. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own competing 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 U.S.

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.

European patent applications now have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unified Patent Court (UPC). This is a significant change in European patent practice. As the UPC is a relatively new court system, there is limited precedent for the court, increasing the uncertainty of any litigation. As a single court system can invalidate a European patent, we, where applicable, have opted out of the UPC and as such, but for proceedings such as an opposition, each European patent would need to be challenged in each individual country.

Geo-political actions in the U.S. and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. Government actions may also prevent filing, prosecution and maintenance of issued patents in various jurisdictions. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in such jurisdictions. If such an event were to occur, it could have a material adverse effect on our business. In addition, jurisdictions outside of the U.S. could also permit our patents to be exploited without consent or compensation. In such circumstances we would not be able to prevent third parties from practicing our inventions or from selling or importing products made using our inventions in and into such jurisdictions. Accordingly, 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 U.S. at various points over the lifetime of our current, potential future patents and patent applications and those of our licensors. 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". 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 on 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, that these parties will not breach such agreements and disclose our proprietary information, including our trade secrets, or that we would be able to obtain adequate remedies for such breaches should they occur. We may not be able to prevent the unauthorized disclosure or use of our trade secrets. 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 U.S. are less willing or unwilling to protect trade secrets. 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.

Moreover, third parties may still derive similar information independently, and we would have no right to prevent them from using that information to compete with us. We expect know-how and information to be disseminated over time within the industry through independent development, publication of journal articles, and movement of personnel between companies and from academic to industry scientific positions. We 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.

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.

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 confidential or other information proprietary of their former employers or their former or current clients.

In addition, we have entered into and may in the future enter into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as collaborators, CROs, third-party manufacturers, consultants, potential partners 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 any such claims, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of resources from our business. We cannot predict whether we would prevail in any such claims. 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 development and commercialization efforts, including the loss of valuable intellectual property rights or personnel, all of 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 us obtaining licenses from others and the terms and conditions of such licenses. If we fail to comply with our obligations in any agreement under which we license intellectual property rights from third parties, we could lose licensed rights that are important to our business.

We have entered into and may in the future enter into additional license agreements with third parties to advance our research or allow commercialization of product candidates. These licenses may not provide us with exclusive rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products.

In addition, 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. If our licensors fail to prosecute, maintain, enforce, and defend, or lose rights to such patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any product that is the subject of such licensed rights could be adversely affected. Even where we have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions taken by or on behalf of our licensors prior to the date upon which we assumed control over patent prosecution.

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, our rights to use the licensed intellectual property would not be exclusive and they may be able to license such patents to our competitors, permitting our competitors to market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

For example, the intellectual property we licensed from the University of Texas, Southwestern (UTSW) is subject to certain non-commercial rights reserved by UTSW. 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.

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. If any such event occurs, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours. Further, 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. This could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects.

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. Disputes may arise between us and our current and future licensors. 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. 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.

Our licensors may also 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 our 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.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical studies and our clinical trials, and plan to rely on third parties to conduct such future drug development activities. These third parties may not perform satisfactorily, including failing to meet completion deadlines, or to comply with applicable regulatory requirements, which may harm our business.

The third parties upon which we rely to conduct our preclinical studies and clinical trials have a significant role in the conduct of such drug development activities 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 devotes to our preclinical studies or our clinical trials. 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. If we need to enter into alternative arrangements with, or replace or add any third parties, it would involve a transition period, and may require substantial cost and extensive management time and focus. Any of these events may delay our drug development activities, increase costs, and materially impact our ability to meet our desired clinical development timelines.

Our heavy reliance on these third parties for such drug development activities reduces our control over these activities. As a result, we have less direct control over the conduct, timing and completion of preclinical studies and clinical trials and the management of data developed through such drug development activities than if we were relying entirely upon our own staff. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with applicable protocol, legal and regulatory requirements and scientific standards, such as GCP and cGMP, and our reliance on third parties does not relieve us of these responsibilities. Regulatory authorities enforce these requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable requirements, such as GCP or cGMP, 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 applicable regulations. 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, or may be delayed in, obtaining marketing approvals for our product candidates or otherwise successfully commercializing our product candidates.

We rely on third parties to produce and maintain certain of our product candidates. This increases the risk that we will not have sufficient quality and quantities of product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our business.

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 may have, our supplier could cease supplying to us or change the terms on which it is willing to continue supplying to us at any time. In particular, we decommissioned the GMMC in 2025 to reduce costs. Although we have retained our internal process and assay development know-how and plan to transfer our AAV manufacturing process to a contract development manufacturing organization with global capabilities in anticipation of future late-stage development and/or commercial launch of TN-201 and/or TN-401, any delay, cGMP or quality issues, or difficulties in negotiating favorable terms with the contract manufacturing organization could result in a material adverse effect on our business operations and our clinical development plans. If we 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. Furthermore, any decision by us to change a third-party manufacturer

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 may be unable to maintain or establish required agreements with third-party manufacturers on acceptable terms. Even if we are able to do so, 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 manufacture our product candidates according to our specifications or comply with applicable regulatory requirements, including cGMP;
- 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.

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 API, drug substance and finished drug products. We are in the process of developing our supply chain for certain of our product candidates and intend to put in place framework agreements under which CDMOs will provide us with necessary quantities of API, drug substance and drug product based on our development needs. As we advance our product candidates through development, we will consider our lack of redundant supply 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.

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 dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of raw materials involve several risks, including limited control over pricing, availability, quality and delivery schedules. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our competitors who are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements. Any 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 dependence upon others for the manufacture and ongoing storage and ongoing testing 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.

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;
- 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 partnership or acquisition;
- unauthorized use or disclosure of our confidential information accessed in connection with partnership activities;
- 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.

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

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. The collaboration negotiation process is time-consuming, costly and complex. 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. 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 regarding our business, the applicable product candidate or technology subject to the collaboration negotiation and the related market potential.

If we are unable to reach a definitive agreement, 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 or technical capabilities, 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 reduce our planned capital expenditures across other areas of our business or 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.

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. For example, under our collaboration agreement with Alnylam, following the completion of the validation activities, Alnylam will have complete control of all development, manufacture, regulatory and commercialization activities for any products directed to a collaboration target. 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 or technical capabilities 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;
- the relationship 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;
- 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 an agreement with a collaborator 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.

There are risks involved with both establishing our own commercial capabilities and entering into arrangements with third parties to perform these services. 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, 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.

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

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

The trading price of our common stock is 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 of achievement of our research, clinical, regulatory and other milestones for our product candidates;
- the 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 product candidates or those of our competitors;
- actual or anticipated changes in our growth rate relative to our competitors;
- regulatory or legal developments in the U.S. and other countries;
- application of new standards and practices applied by the FDA for review of product candidates;

- litigation, including 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, licensing arrangements or capital commitments;
- the decision to terminate or dispose of any preclinical or clinical programs;
- the level of research and development expenses incurred for our product candidates and programs;
- actual or anticipated changes in estimates as to financial results, development timelines or coverage and/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;
- 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.

If we do not regain compliance with or continue to satisfy the Nasdaq continued listing requirements, our common stock could be delisted from Nasdaq. Our ability to publicly or privately sell equity securities and the liquidity of our common stock could be adversely affected if our common stock is delisted.

The continued listing standards of the Nasdaq Global Select Market require, among other things, that the minimum price of a listed company's stock be at or above $1.00. On January 28, 2026, we received a letter from the Staff of Nasdaq indicating that, based upon the closing bid price of shares of our common stock for the 30 consecutive business day period between December 12, 2025, through January 27, 2026, we did not meet the minimum bid price of $1.00 per share required for continued listing on the Nasdaq Global Select Market pursuant to Nasdaq Listing Rule 5450(a)(1). In accordance with Nasdaq's listing rules, we have been afforded 180 calendar days to regain compliance with the bid price requirement. In order to regain compliance, the bid price of our common stock must close at a price of at least $1.00 per share for a minimum of 10 consecutive trading days within the 180-day grace period. While we did not regain compliance by the end of the Compliance Period, we were eligible for additional time to regain compliance (the Second Compliance Period) pursuant to Nasdaq Listing Rule 5810(c)(3)(A)(i) by transferring to the Nasdaq Capital Market. To qualify for the Second Compliance Period, we submitted a transfer application and paid an application fee. Our common stock was transferred to the Nasdaq Capital Market effective as of the opening of business on July 31, 2026 and continues to trade under the symbol "TNYA". In addition, we are required to meet the continued listing requirement for the market value of our publicly held shares and all other initial listing standards for Nasdaq, with the exception of the bid price requirement, and provided written notice of our intention to cure the deficiency during the Second Compliance Period, by effecting a reverse stock split, if necessary. On October 23, 2026, we filed our definitive proxy statement relating to a special meeting of our stockholders to approve an amendment to our certificate of incorporation to effect, at the discretion of our board of directors, a reverse stock split. Even if we effect this reverse stock split, there can be no assurance that we will be able to regain or maintain compliance with the minimum bid price requirement or any other Nasdaq listing standards, if applicable.

If we do not regain compliance with or continue to satisfy the Nasdaq continued listing requirements, our common stock will be subject to delisting. Delisting from Nasdaq could adversely affect our ability to raise additional financing through the public or private sale of equity securities, would significantly affect the ability of investors to trade our securities and would negatively affect the value and liquidity of our common stock. Delisting could also have other negative results, including the potential loss of confidence by employees, the loss of institutional investor interest and fewer business development opportunities.

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 due to a variety of factors, many of which are outside of our control and may be difficult to predict. 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.

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, 2026, our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 27% 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 or other major corporate transactions. This concentration of ownership 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 a stockholder, entrench our management and board of directors or delay or prevent a merger, 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 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. For example, we filed (i) on March 4, 2025, a prospectus supplement to our shelf registration statement on Form S-3 that became effective on August 10, 2022 that covered the offering, issuance and sale of 75,000,000 shares of our common stock, Series A Warrants to purchase 75,000,000 shares of our common stock, and Series B warrants to purchase 37,500,000 shares of our common stock, (ii) a new shelf registration statement on Form S-3 that became effective on March 31, 2025, which will allow us to undertake various equity and debt offerings up to $300.0 million (the 2025 Shelf Registration), and (iii) on December 12, 2025, a prospectus supplement to our 2025 Shelf Registration that covered the offering, issuance and sale of 50,000,000 shares of our common stock and warrants to purchase 50,000,000 shares of our common stock. From issuance through their expiration on June 30, 2026, Series B Warrants to purchase 10,211,250 shares of our common stock were exercised, and the remaining Series B Warrants to purchase 27,288,750 shares of our common stock expired on June 30, 2026.

If the unexpired Series A Warrants and December 2025 Warrants are exercised and shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

We will incur 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 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. As a result of our initiatives to comply with such regulatory requirements, we incur significant legal, accounting and other expenses which may increase after we are no longer an "emerging growth company." Moreover, our management and other personnel need to devote a substantial amount of time to these compliance initiatives.

In particular, as a public company we are required to comply with SEC rules that implement Section 404 of the Sarbanes-Oxley Act. Under these rules, we are 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, unless we continue to qualify as a "smaller reporting company" at such time. To achieve compliance with Section 404 within the prescribed periods, we engage 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 deadlines 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.

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

The market price of our common stock has been and may continue to 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.

Provisions in our certificate of incorporation and 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 certificate of incorporation and 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";
- 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 certificate of incorporation and 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 or certain other conditions are met.

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

Our 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 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 certificate of incorporation or our 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 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. 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 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

We had no sales of unregistered equity securities during the period covered by this report that were not previously reported in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

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

None.

(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

Rule 10b5-1 Trading Arrangements

During the quarter ended September 30, 2026, no director or officer, as defined in Rule 16a-1(f), adopted or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement," each as defined in Regulation S-K Item 408.

Item 6. Exhibits

Exhibit Number	Description	Form	File No.	Exhibit	Filing Date
3.1	Composite Amended and Restated Certificate of Incorporation of Tenaya Therapeutics, Inc., as amended	10-Q	001-40656	3.1	8-9-2023
3.2	Amended and Restated Bylaws of Tenaya Therapeutics, Inc.	8-K	001-40656	3.1	3-21-2023
31.1*	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.				
31.2*	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.				
32.1†	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
101.INS*	Inline XBRL Instance Document–the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document.				
101.SCH*	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents.				
104*	Cover page formatted as Inline XBRL and contained in Exhibit 101.				

+ Management contract, compensatory plan or agreement.

* 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 4, 2026

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

Date: November 4, 2026

By: /s/ Eric Hyllengren, M.B.A.
Eric Hyllengren, M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: November 4, 2026

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)

Exhibit 31.2

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, Eric Hyllengren, 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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: November 4, 2026

TENAYA THERAPEUTICS, INC.

By: /s/ Eric Hyllengren, M.B.A.
Name: Eric Hyllengren, M.B.A.
Title: Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

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

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. (the "Company") for the quarterly period ended September 30, 2026, 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 4, 2026

I, Eric Hyllengren, 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. (the "Company") for the quarterly period ended September 30, 2026, 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/ Eric Hyllengren, M.B.A.
Eric Hyllengren, M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Date: November 4, 2026