Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2024

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: 001-40236

Edgewise Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**82-1725586**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

1715 38th St.
Boulder, CO 80301
(Address of principal executive offices) (Zip Code)

(720) 262-7002
(Registrant's telephone number, including area code)

Not Applicable
(Former name, former address and former fiscal year, if changed since last report)

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	**EWTX**	**Nasdaq Global Select Market**

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

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

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

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

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

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

As of October 31, 2024, there were 94,688,517 of the registrant's ordinary shares outstanding.

TABLE OF CONTENTS

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (Quarterly Report) contains forward-looking statements that involve risks and uncertainties. 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, you can identify forward-looking statements by terminology such as "may," "will," "could," "would," "should," "likely," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "projects," "potential," "continue" or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the safety and efficacy, and the ability of our preclinical studies and clinical trials to demonstrate the safety and efficacy, of our product candidates, and other positive results;
- our ability to utilize our proprietary drug discovery platform to develop a pipeline of product candidates to address muscle diseases;
- the timing, progress and results of preclinical studies and clinical trials for sevasemten (EDG-5506), EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other product candidates we may develop, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the studies or trials will become available, potential registrational studies or cohorts and our research and development programs;
- the timing, scope and likelihood of domestic and foreign regulatory filings and approvals, including timing of final U.S. Food & Drug Administration (FDA) approval of or Investigational New Drugs of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any other future product candidates;
- our ability to develop and advance our current product candidates and programs into, and successfully complete, clinical studies;
- our manufacturing, commercialization, operations and marketing capabilities, relationships with other businesses and other business strategies, systems and relationships;
- our plans relating to commercializing our product candidates, if approved, including the geographic areas of focus and sales strategy;
- the need to hire additional personnel and our ability to attract and retain such personnel;
- 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 and our expectations regarding the implementation of newborn screening for muscular dystrophy;
- our expectations regarding the approval and use of our product candidates in combination with other drugs;
- our competitive position and the success of competing product candidates and therapies that are or may become available;
- our estimates of the number of patients that we will enroll in our clinical trials;
- the beneficial characteristics, and the potential safety, efficacy and therapeutic effects, of our product candidates;

- our ability to obtain and maintain regulatory approval of our product candidates, and the timing or likelihood of regulatory filings and approvals, including our expectations to maintain the Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) for sevasemten and our expectation to seek special designations for our other product candidates;

- our plans relating to the further development of our product candidates, including additional indications we may pursue;

- existing regulations and regulatory developments in the United States, Europe and other jurisdictions;

- our expectations regarding the impact of public health pandemics, including the COVID-19 pandemic, on our business;

- our expectations regarding the upcoming change in the U.S. presidential administration;

- our expectations regarding the impact of Russia's war with Ukraine, and war and instability in Israel and the surrounding region on our business;

- our expectations regarding the impact of instability in the U.S. banking and financial services sector and other macroeconomic trends;

- our intellectual property position, including the scope of protection we are able to establish and maintain for intellectual property rights covering sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other product candidates we may develop, including the extensions of existing patent terms where available, the validity of intellectual property rights held by third parties, and our ability not to infringe, misappropriate or otherwise violate any third-party intellectual property rights;

- our continued reliance on third parties to conduct additional preclinical studies and planned clinical trials of our product candidates, and for the manufacture of our product candidates for preclinical studies and clinical trials;

- our relationships with patient advocacy groups, key opinion leaders, regulators, the research community and payors;

- our ability to obtain, and negotiate favorable terms of, any collaboration, licensing or other arrangements that may be necessary or desirable to develop, manufacture or commercialize our product candidates;

- the pricing and reimbursement of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other product candidates we may develop, if approved;

- the rate and degree of market acceptance and clinical utility of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other product candidates we may develop;

- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing which may be impacted by many factors including inflation;

- our financial performance;

- the period over which we estimate our existing cash, cash equivalents and marketable securities will be sufficient to fund our future operating expenses and capital expenditure requirements;

- the impact of laws and regulations; and

- our expectations regarding the period during which we will qualify as an emerging growth company under The Jumpstart Our Business Startups Act of 2012 and a smaller reporting company under the Securities Exchange Act of 1934, as amended.

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, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events or otherwise.

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

PART I —FINANCIAL INFORMATION

Item 1. Financial Statements

EDGEWISE THERAPEUTICS, INC.
Condensed Balance Sheets
(In thousands, except share and per share data)

	As of September 30, 2024	As of December 31, 2023
Assets	(unaudited)	
Current assets		
Cash and cash equivalents	$ 44,806	$ 86,097
Marketable securities, available for sale	447,730	232,296
Prepaid expenses and other assets	6,900	8,604
Total current assets	499,436	326,997
Property and equipment, net	9,966	10,443
Operating lease right-of-use asset	1,618	2,247
Other non-current assets	262	348
Total assets	$ 511,282	$ 340,035
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 5,649	$ 4,025
Accrued compensation	6,185	5,695
Accrued other expenses	6,131	6,071
Operating lease liability, current portion	992	980
Total current liabilities	18,957	16,771
Operating lease liability, net of current portion	3,448	4,434
Total liabilities	22,405	21,205
Commitments and contingencies (see note 5)		
Stockholders' equity:		
Preferred stock, $.0001 par value per share; 200,000,000 shares authorized and no shares issued or outstanding as of September 30, 2024 and December 31, 2023	—	—
Common stock, $.0001 par value per share; 1,000,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 94,408,911 shares and 70,453,342 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	9	7
Additional paid-in capital	826,731	563,487
Accumulated other comprehensive income	1,051	99
Accumulated deficit	(338,914)	(244,763)
Total stockholders' equity	488,877	318,830
Total liabilities and stockholders' equity	$ 511,282	$ 340,035

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

EDGEWISE 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,	
	2024	**2023**	**2024**	**2023**
Operating expenses				
Research and development	$ 32,222	$ 23,786	$ 90,596	$ 63,221
General and administrative	8,210	5,666	22,696	17,274
Total operating expenses	40,432	29,452	113,292	80,495
Loss from operations	(40,432)	(29,452)	(113,292)	(80,495)
Other income				
Interest income	6,303	3,739	19,141	10,475
Total other income	6,303	3,739	19,141	10,475
Net loss	(34,129)	(25,713)	(94,151)	(70,020)
Other comprehensive income (loss):				
Unrealized gain on available-for-sale securities	2,105	123	952	931
Total comprehensive loss	$ (32,024)	$ (25,590)	$ (93,199)	$ (69,089)
Net loss per share, basic and diluted	$ (0.36)	$ (0.41)	$ (1.03)	$ (1.10)
Weighted-average shares outstanding, basic and diluted	93,813,346	63,459,560	91,639,964	63,369,358

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

EDGEWISE THERAPEUTICS, INC.
Condensed Statements of Stockholders' Equity
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional	Accumulated Other Comprehensive	Accumulated	
	Shares	Amount	Paid-In Capital	Loss	Deficit	Total
Balance as of December 31, 2022	63,257,376	$ 6	$ 492,665	$ (1,355)	$ (144,600)	$ 346,716
Exercise of stock options	17,356	—	21	—	—	21
Stock-based compensation	—	—	3,837	—	—	3,837
Other comprehensive income	—	—	—	1,104	—	1,104
Net loss	—	—	—	—	(22,838)	(22,838)
Balance as of March 31, 2023	63,274,732	$ 6	$ 496,523	$ (251)	$ (167,438)	$ 328,840
Exercise of stock options and vesting of restricted stock	135,326	—	31	—	—	31
Purchase of common stock under employee stock purchase plan	48,874	—	338	—	—	338
Stock-based compensation	—	—	4,011	—	—	4,011
Other comprehensive loss	—	—	—	(296)	—	(296)
Net loss	—	—	—	—	(21,469)	(21,469)
Balance as of June 30, 2023	63,458,932	$ 6	$ 500,903	$ (547)	$ (188,907)	$ 311,455
Exercise of stock options	2,007	—	3	—	—	3
Stock-based compensation	—	—	4,559	—	—	4,559
Other comprehensive income	—	—	—	123	—	123
Net loss	—	—	—	—	(25,713)	(25,713)
Balance as of September 30, 2023	63,460,939	$ 6	$ 505,465	$ (424)	$ (214,620)	$ 290,427

	Common Stock		Additional	Accumulated Other Comprehensive	Accumulated	
	Shares	Amount	Paid-In Capital	Income	Deficit	Total
Balance as of December 31, 2023	70,453,342	$ 7	$ 563,487	$ 99	$ (244,763)	$ 318,830
Issuance of common stock, net of offering costs	22,450,206	2	238,797	—	—	238,799
Exercise of stock options	380,980	—	1,926	—	—	1,926
Stock-based compensation	—	—	4,871	—	—	4,871
Other comprehensive loss	—	—	—	(960)	—	(960)
Net loss	—	—	—	—	(28,525)	(28,525)
Balance as of March 31, 2024	93,284,528	$ 9	$ 809,081	$ (861)	$ (273,288)	$ 534,941
Exercise of stock options and vesting of restricted stock	283,039	—	1,577	—	—	1,577
Purchase of common stock under employee stock purchase plan	75,774	—	422	—	—	422
Stock-based compensation	—	—	5,224	—	—	5,224
Other comprehensive loss	—	—	—	(193)	—	(193)
Net loss	—	—	—	—	(31,497)	(31,497)
Balance as of June 30, 2024	93,643,341	$ 9	$ 816,304	$ (1,054)	$ (304,785)	$ 510,474
Exercise of stock options	765,570	—	3,547	—	—	3,547
Stock-based compensation	—	—	6,880	—	—	6,880
Other comprehensive income	—	—	—	2,105	—	2,105
Net loss	—	—	—	—	(34,129)	(34,129)
Balance as of September 30, 2024	94,408,911	$ 9	$ 826,731	$ 1,051	$ (338,914)	$ 488,877

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

EDGEWISE THERAPEUTICS, INC.
Condensed Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine months ended September 30,	
	2024	**2023**
Cash flows from operating activities		
Net loss	$ (94,151)	$ (70,020)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	1,492	1,068
Stock-based compensation	16,975	12,407
Amortization (accretion) of (discount) on marketable securities, net	(9,943)	(7,345)
Amortization of right-of-use asset	169	131
Changes in assets and liabilities:		
Prepaid expenses and other assets	1,705	(3,174)
Accounts payable	1,645	1,290
Accrued compensation	491	376
Accrued other expenses and other liabilities	60	136
Lease liability	(514)	13
Net cash used in operating activities	(82,071)	(65,118)
Cash flows from investing activities		
Purchases of marketable securities	(413,877)	(216,080)
Sales of marketable securities	12,720	19,189
Maturities of marketable securities	196,617	278,378
Purchases of property and equipment	(1,038)	(5,584)
Net cash (used in) provided by investing activities	(205,578)	75,903
Cash flows from financing activities		
Proceeds from issuance of common stock, net of underwriting discounts and commissions and offering costs	239,147	—
Exercise of stock options	7,050	55
Payment of deferred offering costs	(262)	(214)
Proceeds from Employee Stock Purchase Plan	423	338
Net cash provided by financing activities	246,358	179
Net change in cash and cash equivalents	(41,291)	10,964
Cash and cash equivalents at beginning of period	86,097	21,993
Cash and cash equivalents at end of period	$ 44,806	$ 32,957
Supplemental disclosures of non-cash investing and financing activities:		
Right-of-use asset obtained in exchange for new operating lease liability, net of tenant improvement receivable	$ —	$ 1,110
Reduction to right-of-use due to tenant improvement receivable	$ 460	$ —
Property and equipment purchases included in accounts payable and accrued other expenses	$ 204	$ 140

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

EDGEWISE THERAPEUTICS, INC.

Notes to Condensed Financial Statements
(Unaudited)

NOTE 1 DESCRIPTION OF BUSINESS

Organization and Description of Business

Edgewise Therapeutics, Inc. (the Company) was incorporated as a Delaware corporation in May 2017, and it is headquartered in Boulder, Colorado. The Company is a clinical stage biopharmaceutical company focused on the discovery, development and commercialization of innovative treatments for severe muscle diseases for which there is significant unmet medical need. The Company's lead product candidate, sevasemten (EDG-5506), is an orally administered small molecule designed to address the root cause of dystrophinopathies including Duchenne muscular dystrophy (Duchenne) and Becker muscular dystrophy (Becker). The Company is currently studying sevasemten in Phase 2 trials, which are being held in the U.S. and Israel and in certain countries in Europe and Australasia. In addition, the Company is enrolling a multipart Phase 2 trial with EDG-7500, for the potential treatment of hypertrophic cardiomyopathy (HCM). The Company is using its proprietary drug discovery platform to develop a pipeline of precision medicine product candidates that target key muscle proteins and modulators to address a broad array of serious muscle disorders.

Risks and Uncertainties

The board of directors of the Company discusses with management macroeconomic and geopolitical developments, including inflation, instability in the banking and financial services sector, tightening of the credit markets, the impact of the upcoming change in the U.S. presidential administration, international conflicts, public health pandemics, cybersecurity and sanctions so that the Company can be prepared to react to new developments as they arise. The board of directors and the management of the Company are carefully monitoring these developments and the resulting economic impact on its financial condition and results of operations.

Liquidity and Capital Resources

The Company has an accumulated deficit of $338.9 million and cash, cash equivalents and marketable securities of $492.5 million as of September 30, 2024. The Company's ability to fund ongoing operations is highly dependent upon raising additional capital through the issuance of equity securities and issuing debt or other financing vehicles.

On June 16, 2023, the Company entered into a Sales Agreement (Sales Agreement) with BofA Securities, Inc. (BofA Securities) under which the Company could offer and sell shares of common stock, having aggregate sales proceeds of up to $125,000,000 from time to time, through an "at the market offering" program (ATM Program) under which BofA Securities acted as sales agent. Effective January 19, 2024, the Company suspended and terminated the prospectus related to the Company's common stock (the ATM Prospectus) issuable pursuant to the terms of the Sales Agreement. As of the date of the suspension of the ATM Prospectus, the Company had sold 7,560,068 shares of our common stock at a weighted average price of $7.93 per share. The gross proceeds were $59.9 million, and the net proceeds were $59.4 million after deducting underwriting discounts and commissions of $0.2 million and offering expenses of $0.3 million.

On January 23, 2024, the Company closed an underwritten registered direct offering of 21,818,182 shares of common stock at a public offering price of $11.00 per share (the January 2024 Offering). The aggregate gross proceeds from the January 2024 Offering were $240.0 million, and the net proceeds were $231.9 million after deducting underwriting discounts and commissions of $7.5 million and offering expenses of $0.6 million.

On May 10, 2024, the Company entered into a sales agreement with Leerink Partners LLC (Leerink Sales Agreement) under which the Company may offer and sell shares of common stock, having aggregate sales proceeds of up to $175,000,000 from time to time, through an "at the market offering" program (Leerink ATM) under which Leerink Partners LLC will act as sales agent. The Company has not yet offered or sold any shares of common stock related to the Leerink ATM.

The Company's ability to secure capital is dependent upon success in developing its technology and product candidates. The Company cannot provide assurance that additional capital will be available on acceptable terms, if at all. The issuance of additional equity or debt securities will likely result in substantial dilution to the Company's stockholders. Should additional capital not be available to the Company in the near term, or not be available on acceptable terms, the Company may be unable to realize value from the Company's assets or discharge liabilities in the normal course of business, which may, among other alternatives, cause the Company to delay, substantially reduce, or discontinue operational activities to conserve cash balances, which could have a material adverse effect on the Company's ability to achieve its intended business objectives.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. The financial statements do not reflect any adjustments relating to the recoverability and reclassification of assets and liabilities that might be necessary if the Company is unable to continue as a going concern. The Company believes that the $492.5 million of cash, cash equivalents and marketable securities on hand as of September 30, 2024 will be sufficient to fund its operations in the normal course of business and meet its liquidity needs through at least the next 12 months from the issuance of these financial statements.

NOTE 2 SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (U.S. GAAP).

Segment Information

The Company operates in one operating segment and, accordingly, no segment disclosures have been presented herein. All equipment and other fixed assets are physically located within the United States.

Use of Estimates

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

Cash Equivalents

The Company considers all liquid investments with a maturity of three months or less when purchased to be cash equivalents. Cash equivalents as of September 30, 2024 and December 31, 2023 primarily consist of money market funds and cash.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to significant concentration of credit risk consist primarily of cash, cash equivalents and marketable securities. Periodically, the Company may maintain deposits in financial institutions in excess of government insured limits. The Company believes that it is not exposed to significant credit risk as its deposits are held at financial institutions that management believes to be of high credit quality. The Company has not experienced any losses on deposits since inception. The Company regularly invests excess cash with major financial institutions in money market funds, corporate debt securities, and commercial paper, all of which can be readily purchased and sold using established markets. The Company believes that the market risk arising from our holdings of these financial instruments is mitigated based on the fact that many of these securities are of high credit rating.

Deferred Offering Costs

The Company capitalizes certain legal, professional, accounting and other third-party fees that are directly associated with in-process equity issuances as deferred offering costs until such equity issuances are consummated. After consummation of the equity issuance, these costs are recorded as a reduction in the capitalized amount associated with

the equity issuance. Should the equity issuance be abandoned, the deferred offering costs are expensed immediately as a charge to operating expenses in the statement of operations. Deferred offering costs were $0.3 million as of September 30, 2024 and December 31, 2023. Such costs are classified in other non-current assets in the accompanying balance sheets.

Property and Equipment

Property and equipment is stated at cost less accumulated depreciation and amortization. Depreciation of property and equipment is computed using the straight-line method over the estimated useful life of the related asset, which is generally three to seven years, and in the case of leasehold improvements, the shorter of the estimated useful lives of the assets or the term of the lease.

Leases

The Company accounts for its leases under Accounting Standards Codification (ASC) Topic 842, *Leases* (ASC 842). At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. Leases with a term greater than 12 months are recognized on the balance sheet as Right-of-Use (ROU) assets and current and non-current lease liabilities, as applicable. The Company has elected not to recognize on the balance sheet leases with terms of 12 months or less. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew. The Company monitors its material leases on a quarterly basis.

Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of future lease payments over the expected remaining lease term. Lease cost for operating leases is recognized on a straight-line basis over the lease term as an operating expense. Certain adjustments to the ROU asset may be required for items such as lease prepayments or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment.

For all asset classes of its leases, the Company has elected to account for the lease and non-lease components together for existing classes of underlying asset. Costs determined to be variable and not based on an index or rate are not include in the measurement of the lease liability.

Impairment of Long-Lived Assets

The Company reviews long-lived assets for impairment whenever events or circumstances indicate that the carrying value of such assets may not be fully recoverable. Impairment is evaluated based on the sum of undiscounted estimated future cash flows expected to result from use of the related asset compared to its carrying value. If impairment is recognized, the carrying value of the impaired asset is reduced to its fair value. There were no impairment charges or long-lived assets disposed of during three and nine months ended September 30, 2024 and 2023.

Income Taxes

Deferred income taxes are provided on temporary differences between financial statement and income tax reporting. Temporary differences are differences between the amounts of assets and liabilities reported for financial statement purposes and their tax bases.

Deferred tax assets are recognized for temporary differences that will be deductible in future years' tax returns and for operating loss and tax credit carryforwards. Deferred tax assets are reduced by a valuation allowance if such deferred tax assets are deemed more likely than not that some or all of the deferred tax assets will not be realized. Historically, the Company has not recognized these potential benefits in its financial statements and has fully reserved for such net deferred tax assets, as it believes it is more likely than not that the full benefit of these net deferred tax assets will not be realized. Deferred tax liabilities are recognized for temporary differences that will be taxable in future years. The Company evaluated its tax positions and determined it has no uncertain tax positions as of September 30, 2024.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. The Financial Accounting Standards Board (FASB) ASC Topic 820, *Fair Value Measurements and Disclosures* (ASC 820), establishes a hierarchy of inputs used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are those that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of financial instruments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

Level 1—quoted prices in active markets for identical assets and liabilities.

Level 2—other significant observable inputs (including quoted prices for similar assets and liabilities, interest rates, credit risk, etc.).

Level 3—significant unobservable inputs (including the Company's own assumptions in determining the fair value of assets and liabilities).

Marketable Securities, Available for Sale

All marketable securities have been classified as "available-for-sale" and are carried at fair value, based upon quoted market prices. The Company considers its available-for-sale portfolio as available for use in current operations. Accordingly, the Company classifies its investments as short-term marketable securities, even though the stated maturity date may be one year or more beyond the current balance sheet date. Unrealized gains and losses, net of any related tax effects, are excluded from earnings and are included in other comprehensive income (loss) and reported as a separate component of stockholders' equity until realized. Interest income, realized gains and losses, and declines in value judged to be other than temporary, if any, on available-for-sale securities are included in other income. The cost of securities sold is based on the specific-identification method. The amortized cost of securities is adjusted for amortization of premiums and accretion of discounts to maturity. In accordance with the Company's investment policy, management invests in money market funds, corporate bonds, commercial paper, asset-backed securities and government securities. The Company has not experienced any realized losses on its deposits of cash, cash equivalents, and marketable securities since inception.

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

	As of September 30, 2024				
	Fair Value Hierarchy	Amortized Cost Basis	Unrealized Gains	Unrealized Losses	Fair Market Value
Cash equivalents:					
Money market funds	Level 1	$ 44,612	$ —	$ —	$ 44,612
Marketable securities, available for sale:					
Asset-backed securities	Level 2	49,099	109	—	49,208
Corporate debt securities	Level 2	191,068	408	(5)	191,471
Commercial paper	Level 2	17,212	16	—	17,228
U.S. government treasury and agency securities	Level 2	189,300	523	—	189,823
Total		$ 491,291	$ 1,056	$ (5)	$ 492,342

	As of December 31, 2023				
	Fair Value Hierarchy	Amortized Cost Basis	Unrealized Gains	Unrealized Losses	Fair Market Value
Cash equivalents:					
Money market funds	Level 1	$ 85,897	$ —	$ —	$ 85,897
Marketable securities, available for sale:					
Asset-backed securities	Level 2	10,228	12	(2)	10,238
Corporate debt securities	Level 2	82,514	66	(113)	82,467
Commercial paper	Level 2	19,457	13	(8)	19,462
U.S. government treasury and agency securities	Level 2	116,579	151	(26)	116,704
Supranational and sovereign government securities	Level 2	3,419	6	—	3,425
Total		$ 318,094	$ 248	$ (149)	$ 318,193

The Company's money market funds are classified as Level 1 because they are valued using quoted market prices. Investments in asset-backed securities, corporate debt securities, commercial paper and U.S. government treasury and agency securities, and supranational and sovereign government securities have been classified as Level 2 as they are valued using quoted prices in less active markets or other directly or indirectly observable inputs. Fair values of asset-backed securities, corporate debt securities, commercial paper, U.S. government treasury and agency securities, and supranational and sovereign government securities were derived based on input of market prices from multiple sources at each reporting period. With regard to commercial paper, all of the securities had high credit ratings and one year or less to maturity; therefore, fair value was derived from accretion of purchase price to face value over the term of maturity or quoted market prices for similar instruments if available. There were no transfers of financial assets between Level 1, Level 2, or Level 3, during the periods presented. As of September 30, 2024, the remaining contractual maturities of $409.0 million of marketable securities were less than one year and $37.7 million of marketable securities were between 1 and 2 years.

The Company periodically reviews its portfolio of debt securities to determine if any investment is impaired due to credit loss or other potential valuation concerns. For debt securities where the fair value of the investment is less than the amortized cost basis, the Company has assessed at the individual security level for various quantitative factors including, but not limited to, the nature of the investments, changes in credit ratings, interest rate fluctuations, industry analyst reports, and the severity of impairment. Unrealized losses on marketable securities at September 30, 2024 were primarily due to changes in interest rates, including market credit spreads, and not due to increased credit risks associated with specific securities.

Comprehensive Income (Loss)

Comprehensive income (loss) is defined as the change in equity during a period from transactions and other events and/or circumstances from non-owner sources. The Company's only element of other comprehensive income was net unrealized gain on marketable securities.

Stock-Based Compensation

In accordance with ASC Topic 718, *Compensation—Stock Compensation*, the Company recognizes compensation expense for all stock-based awards issued to employees based on the estimated grant-date fair value, which is recognized as expense on a straight-line basis over the requisite service period. The Company has elected to recognize forfeitures as they occur. For restricted stock unit awards, the fair value is based on the closing price of the Company's common stock on the date of grant. The fair value of stock options is determined using the Black-Scholes option-pricing model. The determination of fair value for stock-based awards on the date of grant using an option-pricing model requires management to make certain assumptions including expected volatility, expected term, risk-free interest rate and expected dividends in addition to the Company's common stock valuation.

Research and Development Expenses and Accrued Research and Development Expenses

Expenditures made for research and development are charged to expense as incurred. External costs consist primarily of payments to contract research organizations (CROs), contract development and manufacturing organizations

(CDMOs), sample acquisition costs and laboratory supplies purchased in connection with the Company's discovery and preclinical activities, and process development and clinical development activities. Internal costs consist primarily of employee-related costs, facilities, depreciation and costs related to compliance with regulatory requirements. Non-refundable advance payments for goods and services that will be used in future research and development activities are capitalized and recorded as an expense in the period that the Company receives the goods or when services are performed.

The Company records expenses related to external research and development services based on its estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CROs and CDMOs that supply, conduct and manage preclinical studies and clinical trials on its behalf. The financial terms of these contracts vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided under such contracts. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company adjusts the accrual or the amount of prepaid expenses accordingly.

Emerging Growth Company Status

The Company is an "emerging growth company", as defined in the Jumpstart Our Business Startups Act of 2012 (JOBS Act). Under the JOBS Act, emerging growth companies 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. The Company has elected to use this extended transition period for complying with certain new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it is (1) no longer an emerging growth company or (2) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, these financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

Accounting Standards Not Yet Adopted

In November 2023, the FASB issued *ASU 2023-07, Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* to update reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses and information used to assess segment performance. This update is effective beginning with the Company's 2024 fiscal year annual reporting period, with early adoption permitted. The Company is currently evaluating the impact that the adoption of this standard will have on its financial statements.

In December 2023, the FASB issued *ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. This ASU is expected to enhance the transparency and decision usefulness of income tax disclosures by requiring public business entities on an annual basis to disclose specific categories in the rate reconciliation, additional information for reconciling items that meet a quantitative threshold, and certain information about income taxes paid. This ASU is effective beginning with the Company's 2025 fiscal year annual reporting period, with early adoption permitted. The Company is currently evaluating the impact that the adoption of this standard will have on its financial statements.

NOTE 3 PREFERRED STOCK AND COMMON STOCK

The Company is authorized to issue two classes of stock designated as common stock and preferred stock. As of September 30, 2024, the total number of shares authorized was 1,200,000,000. The total number of shares of common stock authorized was 1,000,000,000. The total number of shares of preferred stock authorized was 200,000,000. All shares of the Company's capital stock have a par value of $0.0001 per share.

Common stockholders are entitled to dividends if and when declared by the board of directors of the Company and after any convertible preferred share dividends are fully paid. The holder of each share of common stock is entitled to one vote.

NOTE 4 STOCK-BASED COMPENSATION AWARDS

Equity Incentive Plans

In March 2021, the Company's board of directors adopted, and its stockholders approved, the Company's 2021 Equity Incentive Plan (the 2021 Plan), which became effective in March 2021 in connection with the IPO. Upon adoption of the 2021 Plan, the Company restricted the grant of future equity awards under its 2017 Equity Incentive Plan, as amended and restated (the 2017 Plan).

The 2021 Plan provides for the grant of incentive stock options, within the meaning of Section 422 of the Internal Revenue Code, to the Company's employees and any of its parent and subsidiary corporations' employees, and for the grant of nonstatutory stock options, restricted stock, restricted stock units, stock appreciation rights, performance units, and performance shares to its employees, directors, and consultants and its subsidiary corporations' employees and consultants.

The vesting of stock options is stated in each individual grant agreement, which is generally four years. Options granted expire 10 years after the date of grant. A total of 5,040,000 shares of the Company's common stock were initially reserved for issuance pursuant to the 2021 Plan. The 2021 Plan share reserve increases by the number of shares under the 2017 Plan that are repurchased, forfeited, expired or cancelled after the effective date of the 2021 Plan up to the limit under the 2021 Plan. The number of shares available for issuance under the 2021 Plan increases annually on the first day of each fiscal year beginning with the Company's 2022 fiscal year, equal to the least of (1) 5,040,000 shares, (2) five percent (5%) of the outstanding shares of its common stock as of the last day of the immediately preceding fiscal year; or (3) such other amount as the Company's board of directors may determine. As of September 30, 2024, there were 1,506,606 shares available for future issuance under the 2021 Plan.

During the nine months ended September 30, 2024, the Company issued 2,751,497 stock options with a fair value of $34.5 million that vest over a weighted average period of 3.9 years and 557,027 restricted stock units (RSUs) with a fair value of $9.5 million that vest over a weighted average period of 4 years.

Inducement Equity Incentive Plan

Effective August 10, 2024, the Company's board of directors adopted the Company's 2024 Inducement Equity Incentive Plan (the Inducement Plan) and, subject to the adjustment provisions of the Inducement Plan, reserved 2,000,000 shares of the Company's common stock for issuance pursuant to equity awards granted under the Inducement Plan.

The Inducement Plan was adopted without stockholder approval pursuant to the applicable The Nasdaq Stock Market LLC's (Nasdaq) Listing Rules. The Inducement Plan provides for the grant of equity-based awards, including nonstatutory stock options, stock appreciation rights, restricted stock, restricted stock units, and performance awards, and its terms are substantially similar to the 2021 Plan, including with respect to treatment of equity awards in the event of a "merger" or "change in control" as defined under the Inducement Plan, but with such other terms and conditions intended to comply with the Nasdaq inducement award exception or to comply with the Nasdaq acquisition and merger exception.

In accordance with the Nasdaq Listing Rules, awards under the Inducement Plan may only be made to individuals not previously employees or non-employee directors of the Company (or following such individuals' bona fide period of non-employment with the Company), as an inducement material to the individuals' entry into employment with the Company, or, to the extent permitted by the Nasdaq Listing Rules, in connection with a merger or acquisition. As of September 30, 2024, there were 2,000,000 shares available for future issuance under the Inducement Plan.

Founder Stock Options

On September 19, 2017, the Company granted one of its founders the option to purchase 1,795,880 shares of the Company's common stock at an exercise price of $0.18 per share which vest monthly over a four-year period that expires 15 years after the date of grant. This grant is separate from the Company's equity incentive plans discussed above. As of September 30, 2024, 1,456,780 options were both outstanding and exercisable. 75,000 options were exercised during the three and nine months ended September 30, 2024.

Total stock-based compensation expense related to all equity plans, including Founder Stock Options was allocated as follows (in thousands):

	Three months ended September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Research and development	$ 3,884	$ 2,639	$ 9,508	$ 7,028
General and administrative	2,996	1,920	7,467	5,379
Total stock-based compensation expense	$ 6,880	$ 4,559	$ 16,975	$ 12,407

NOTE 5 COMMITMENTS AND CONTINGENCIES

Litigation

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, penalties and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated. From time to time, the Company may become involved in legal proceedings arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the three and nine months ended September 30, 2024 and no material legal proceedings are currently pending or threatened.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any claims under indemnification arrangements, and it has not accrued any liabilities related to such obligations in its financial statements as of September 30, 2024.

NOTE 6 NET LOSS PER SHARE

Basic net loss per common share is calculated by dividing the net loss by the weighted-average number of common shares outstanding during the period, without consideration of potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss by the weighted-average number of common shares and potentially dilutive securities outstanding for the period. For purposes of the diluted net loss per share calculation, common stock options and unvested restricted stock units are considered to be potentially dilutive securities. The Company’s participating securities do not have a contractual obligation to share in the Company’s losses. As such, the net loss was attributed entirely to common stockholders. As the Company has reported a net loss for all periods presented, diluted net loss per common share is the same as basic net loss per common share for those periods.

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

	Three months ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Numerator				
Net loss	$ (34,129)	$ (25,713)	$ (94,151)	$ (70,020)
Denominator				
Weighted-average shares outstanding used in computing net loss per share, basic and diluted	93,813,346	63,459,560	91,639,964	63,369,358
Net loss per share, basic and diluted	$ (0.36)	$ (0.41)	$ (1.03)	$ (1.10)

The following weighted average outstanding shares of potentially dilutive securities were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been anti-dilutive:

	Three months ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Options to purchase common stock	16,891,674	14,188,382	16,123,873	12,951,452
Unvested restricted stock units	386,465	177,871	224,615	217,657
Total	17,278,139	14,366,253	16,348,488	13,169,109

NOTE 7 PROPERTY AND EQUIPMENT

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

	As of September 30, 2024	As of December 31, 2023
Leasehold improvements	$ 9,546	$ 8,728
Laboratory equipment	3,735	3,473
Computers and software	296	281
Furniture and fixtures	497	497
Construction in process	—	80
Property and equipment, at cost	14,074	13,059
Less: accumulated depreciation	(4,108)	(2,616)
Property and equipment, net	$ 9,966	$ 10,443

Depreciation expense was $0.5 million and $1.5 million for the three and nine months ended September 30, 2024 and $0.4 million and $1.1 million for the three and nine months ended September 30, 2023, respectively.

NOTE 8 ACCRUED OTHER EXPENSES

Accrued other expenses consisted of the following amounts (in thousands):

	As of September 30, 2024	As of December 31, 2023
Accrued research and development costs	$ 5,365	$ 5,672
Accrued other	766	399
Total accrued other expenses	$ 6,131	$ 6,071

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

The following discussion of the financial condition and results of operations of Edgewise Therapeutics, Inc. should be read in conjunction with the financial statements and the related notes thereto included elsewhere in this Quarterly Report on Form 10-Q (Quarterly Report), and the audited financial statements and notes included in our Annual Report on Form 10-K (Annual Report), filed with the Securities and Exchange Commission, on February 22, 2024. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Quarterly Report, our actual results could differ materially from the results described in or implied by these forward-looking statements. 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

Since our inception in 2017, we have been drawing on our deep expertise in muscle physiology and small molecule drug discovery to drive the next generation of first-in-class therapeutics to address a variety of severe muscle diseases. We are advancing two clinical-stage programs in muscular dystrophies and severe cardiac diseases. Sevasemten (EDG-5506) is an orally administered skeletal myosin inhibitor in a pivotal stage trial for Becker muscular dystrophy (Becker) as well as ongoing Phase 2 programs in Duchenne muscular dystrophy. EDG-7500, currently in a multipart Phase 2 trial, is a novel cardiac sarcomere modulator for the treatment of hypertrophic cardiomyopathy (HCM) and other disorders of diastolic dysfunction. We also continue to advance our preclinical pipeline, including characterization of novel cardiometabolic targets. From this foundation and our dedication to muscle physiology and function, we will build a leading global biopharmaceutical company driving advances to improve the lives of people suffering from muscle diseases.

As a clinical-stage biopharmaceutical company, we are focused on the discovery, development and commercialization of innovative treatments for severe muscle diseases for which there is significant unmet medical need. Guided by our holistic drug discovery approach to targeting the muscle as an organ, we have combined our foundational expertise in muscle biology and small molecule engineering to build our proprietary, muscle focused drug discovery platform. Our platform utilizes custom-built high throughput and translatable systems that measure integrated muscle function in whole organ extracts to identify small molecule precision medicines regulating key proteins in muscle tissue, initially focused on addressing rare neuromuscular and cardiac diseases. We have developed and characterized a library of novel sarcomere modulators exhibiting a broad range of pharmacological and pharmacokinetic properties regulating disease-related muscle biology.

We have incurred significant losses since the commencement of our operations. Our net losses were $34.1 million and $94.2 million for the three and nine months ended September 30, 2024, respectively and $25.7 million and $70.0 million for the three and nine months ended September 30, 2023, respectively, and we expect to continue to incur significant losses for the foreseeable future as we advance our product candidates through preclinical development and

clinical trials and seek regulatory approval of our product candidates. Our net losses may fluctuate significantly from period to period, depending on the timing of and expenditures on our planned research and development activities.

As of September 30, 2024, we had an accumulated deficit of $338.9 million. To date, we have financed our operations primarily through private placements of convertible preferred stock and public offerings of our common stock. From inception through September 30, 2024, these private placements have provided gross proceeds of $160.7 million, and the initial public offering, follow-on public offering, issuance of our common stock under an "at the market offering" program (the ATM Program), and underwritten registered direct offering have generated net proceeds of $606.6 million. We believe that our existing cash and cash equivalents and marketable securities of $492.5 million will enable us to fund our planned operating expenses and capital expenditure requirements through at least the next 12 months.

Macroeconomic and Geopolitical Developments

We are monitoring macroeconomic and geopolitical developments, such as inflation, instability in the banking and financial services sector, tightening of the credit markets, the upcoming change in the U.S. presidential administration, international conflicts, public health pandemics, cybersecurity and sanctions so that the Company can be prepared to react to new developments as they arise.

Components of Our Results of Operations

Operating expenses

Research and development expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We record research and development expenses when these are incurred. Such expenses include:

- employee and external consultant-related expenses including salaries, bonuses, benefits and stock-based compensation expense for employees engaged in research and development functions;
- external expenses incurred in connection with the clinical development of our product candidates including under agreements with third parties, such as consultants and contract research organizations (CROs);
- the cost of external manufacturing drug products for use in our preclinical studies and ongoing and planned clinical trials including under agreements with third parties such as consultants and contract development and manufacturing organizations (CDMOs);
- expenses incurred in connection with the preclinical development of our product candidates including external, or outsourced professional scientific development services, consulting research fees and payments made under sponsored research arrangements with third parties;
- laboratory supplies;
- facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities;
- expenses related to compliance with regulatory requirements; and
- payments made under third-party licensing agreements.

The majority of these expenses have been incurred to advance our lead product candidate, sevasemten and to a lesser degree EDG-7500. We expect that significant additional spending will be required to progress these and other potential discoveries through the remainder of the clinical development phases. These expenses will primarily consist of expenses for the administration of clinical trials as well as manufacturing costs for clinical material supply.

We track our direct research and development expenses on a program-by-program basis once a lead compound has been selected and clinical trials have been initiated. These direct costs consist primarily of external costs such as fees paid to outside consultants, CROs, CDMOs, clinical trial sites and central laboratories in connection with our discovery and preclinical activities, process development, manufacturing and clinical development activities. These expenses are recognized based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers or our estimate of the level of service that has been performed at each reporting date. Our direct research and development expenses by program also include costs of laboratory supplies that can be directly attributed to a specific program as well as any fees incurred under license agreements. We do not allocate employee-related costs, including stock-based compensation, or facility expenses, including rent, depreciation or other indirect costs, to specific programs because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to conduct our research and discovery activities and to manage our preclinical development, manufacturing and clinical development activities.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages, primarily due to the increased size and duration of later-stage clinical trials. We are currently conducting four Phase 2 clinical trials with sevasemten for people with muscular dystrophy (CANYON, LYNX, FOX and DUNE), a potentially registrational, or pivotal cohort, in individuals with Becker (GRAND CANYON), and a multipart Phase 2 study with EDG-7500 for people with HCM (CIRRUS-HCM). As a result, we expect that our research and development expenses will increase substantially over the next several years as we advance sevasemten, EDG-7500, a product from our EDG-003 cardiometabolic discovery program through clinical trials and additional product candidates; continue to develop our proprietary drug discovery platform; continue to discover and develop additional product candidates; and hire additional personnel.

The successful development of our product candidates is highly uncertain, and we do not believe it is possible at this time to accurately project the nature, timing and extent of expenses necessary to complete the development of our product candidates. We are also unable to predict when, if ever, we will generate revenue from our product candidates to offset these expenses. Our expenditures on current and future preclinical and clinical development programs are subject to numerous uncertainties in timing and cost to completion. The duration, costs and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with IND-enabling studies;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt of regulatory approvals from applicable regulatory authorities;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- our ability to establish new licensing or collaboration arrangements;
- the performance of our future collaborators, if any;

- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- development and timely delivery of sufficient supplies of our drug product that can be used in our planned clinical trials and for commercial launch upon approval;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- launching commercial sales of our product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

Any changes in the outcome of any of these factors could significantly impact the costs and timing associated with the development of our product candidates.

General and administrative expenses

General and administrative expenses consist primarily of salaries, related benefits and stock-based compensation expense for personnel in executive, finance, accounting, legal and administrative functions. General and administrative expenses also include facilities and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance, not otherwise included in research and development expenses, as well as professional fees for legal, patent, consulting, investor and public relations, accounting and audit services.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support the continued research and development of our programs.

Interest income

Interest income primarily consists of interest income generated from our cash, cash equivalents and marketable securities.

Results of Operations

Comparison of the three months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the three months ended September 30, 2024 and 2023:

	Three months ended September 30,		
	2024	2023	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 32,222	$ 23,786	$ 8,436
General and administrative	8,210	5,666	2,544
Total operating expenses	40,432	29,452	10,980
Loss from operations			
Interest income	6,303	3,739	2,564
Net loss	$ 34,129	$ 25,713	$ 8,416

Research and development expenses

The following table summarizes our research and development expenses:

	Three months ended September 30,		
	2024	2023	Change
	(in thousands)		
External research and development expenses:			
Sevasemten clinical program	$ 13,119	$ 8,538	$ 4,581
EDG-7500 clinical program	4,118	3,979	139
Discovery and preclinical	3,040	2,450	590
Internal costs, including personnel related	11,945	8,819	3,126
Total research and development expenses	$ 32,222	$ 23,786	$ 8,436

Research and development expenses were $32.2 million and $23.8 million for the three months ended September 30, 2024 and 2023, respectively. The increase of $8.4 million was driven by an increase of $4.6 million in sevasemten clinical program expenses, which was primarily due to increased clinical trial activity from site activations, patient enrollment, and manufacturing costs in our GRAND CANYON and FOX trials that were in start-up phases during the comparable period in 2023 and general clinical and outreach costs incurred in connection with all sevasemten trials. Internal costs increased $3.1 million, primarily related to personnel costs and stock-based compensation resulting from increased employee headcount to support the growth of our research and development programs. Discovery and preclinical expenses increased $0.6 million as a result of additional manufacturing and nonclinical costs related to our EDG-003 program and other cardio-metabolic research. EDG-7500 clinical program expenses increased $0.1 million primarily related to the start-up, site activation, and patient enrollment for our multipart Phase 2 CIRRUS-HCM trial; our Phase 1 trial was in a similar start-up phase in the comparable period in 2023.

General and administrative expenses

General and administrative expenses were $8.2 million and $5.7 million for the three months ended September 30, 2024 and 2023, respectively. The increase of $2.5 million was primarily due to $1.6 million in increased personnel-related costs from increased headcount and stock-based compensation and $0.9 million in increased professional and consulting costs and other administrative costs.

Interest income

Interest income was $6.3 million and $3.7 million for the three months ended September 30, 2024 and 2023, respectively. The increase of $2.6 million was primarily due to higher balances of marketable securities during the three months ended September 30, 2024 as compared to the three months ended September 30, 2023.

Comparison of the nine months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the nine months ended September 30, 2024 and 2023:

	Nine months ended September 30,		
	2024	2023	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 90,596	$ 63,221	$ 27,375
General and administrative	22,696	17,274	5,422
Total operating expenses	113,292	80,495	32,797
Loss from operations			
Interest income	19,141	10,475	8,666
Net loss	$ 94,151	$ 70,020	$ 24,131

Research and development expenses

The following table summarizes our research and development expenses:

	Nine months ended September 30,		
	2024	2023	Change
	(in thousands)		
External research and development expenses:			
Sevasemten clinical program	$ 37,886	$ 23,231	$ 14,655
EDG-7500 clinical program	13,208	3,979	9,229
Discovery and preclinical	7,120	11,885	(4,765)
Internal costs, including personnel related	32,382	24,126	8,256
Total research and development expenses	$ 90,596	$ 63,221	$ 27,375

Research and development expenses were $90.6 million and $63.2 million for the nine months ended September 30, 2024 and 2023, respectively. The increase of $27.4 million was primarily driven by an increase of $14.7 million in sevasemten clinical program expenses which was primarily due an $8.6 million increase in our GRAND CANYON trial, $3.5 million increase in our FOX trial, $2.4 million increase in our LYNX trial, and $2.0 million increase in our MESA trial related to site activation and patient enrollment as the trials were in startup phases in the third quarter of 2023; offset by a $1.3 million decrease in our CANYON trial, which is nearing completion. EDG-7500 clinical program expenses increased $9.2 million related to the completion of our Phase 1 trial that was activated in late 2023 and manufacturing, site activation, and patient enrollment costs related to our CIRRUS-HCM trial that began in 2024. Internal costs increased $8.3 million, primarily related to a $5.0 million increase in personnel costs related to salaries, benefits, and employee expenses resulting from increased employee headcount to support the growth of our research and development programs, a $2.5 million increase in stock-based compensation related to increased headcount and overall higher stock prices driving increased option fair values, and $0.8 million related to depreciation and other facilities costs. Discovery and preclinical expenses decreased $4.8 million as a result of EDG-7500 costs, now shown separately, which were a significant portion of the first nine months of 2023's discovery and preclinical expenses along with other costs from our EDG-003 program.

General and administrative expenses

General and administrative expenses were $22.7 million and $17.3 million for the nine months ended September 30, 2024 and 2023, respectively. The increase of $5.4 million was primarily due to $3.8 million in increased personnel-related costs from increased headcount and stock-based compensation and $1.6 million in increased professional and consulting costs and other administrative costs.

Interest income

Interest income was $19.1 million and $10.5 million for the nine months ended September 30, 2024 and 2023, respectively. The increase of $8.7 million was primarily due to higher balances of marketable securities during the nine months ended September 30, 2024, as compared to the nine months ended September 30, 2023.

Liquidity and Capital Resources

Sources of liquidity

Since our inception, we have not generated any revenue and have incurred significant operating losses and negative cash flows from our operations. To date, we have financed our operations primarily through private placements of convertible preferred stock and public offerings of our common stock. From inception through September 30, 2024, these private placements have provided gross proceeds of $160.7 million, and the initial public offering, follow-on public offering, issuance of our common stock under the ATM Program, and underwritten registered direct offering have generated net proceeds of $606.6 million. As of September 30, 2024, we had cash, cash equivalents and marketable securities in the amount of $492.5 million.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Nine months ended September 30,	
	2024	2023
	(in thousands)	
Net cash used in operating activities	$ (82,071)	$ (65,118)
Net cash (used in) provided by investing activities	(205,578)	75,903
Net cash provided by financing activities	246,358	179
Net (decrease) increase in cash and cash equivalents	$ (41,291)	$ 10,964

Operating activities

Cash used in operating activities during the nine months ended September 30, 2024 was primarily driven by our net loss for the period of $94.2 million, and was also impacted by changes in operating assets and liabilities which decreased net working capital by $3.4 million. Cash used in operating activities was reduced by non-cash charges of $8.7 million relating to stock-based compensation expense of $17.0 million, amortization of premium and accretion of discounts, net on marketable securities of $9.9 million, depreciation of $1.5 million and amortization of right-of-use asset of $0.2 million.

Cash used in operating activities during the nine months ended September 30, 2023 was primarily driven by our net loss for the period of $70.0 million, and was also impacted by changes in operating assets and liabilities which increased net working capital by $1.4 million. Cash used in operating activities was reduced by non-cash charges of $6.3 million relating to stock-based compensation expense of $12.4 million, amortization of premium and accretion of discounts, net on marketable securities of $7.3 million, depreciation of $1.1 million and amortization of right-of-use asset of $0.1 million.

Investing activities

Cash used in investing activities during the nine months ended September 30, 2024 amounted to $205.6 million, which was due to $413.9 million in purchases of marketable securities and $1.0 million for purchases of equipment, which was partially offset by $196.6 million in maturities of marketable securities and $12.7 million in sales of marketable securities.

Cash provided by investing activities during the nine months ended September 30, 2023 amounted to $75.9 million, which was due to $278.4 million in maturities of marketable securities and $19.2 million in sales of marketable securities, which was partially offset by $216.1 million in purchases of marketable securities and $5.6 million for purchases of equipment.

Financing activities

Cash provided by financing activities during the nine months ended September 30, 2024 was $246.4 million, which was due to cash proceeds of $7.0 million from the ATM Program, $240.0 million from the underwritten registered direct offering, $7.1 million in proceeds from the issuance of common stock upon exercise of stock options, and $0.4 million in proceeds from the employee stock purchase plan, which was partially offset by $7.9 million for the payment of underwriting commissions and offering costs and $0.3 million for the payment of deferred offering costs.

Cash provided by financing activities during the nine months ended September 30, 2023 was $0.2 million, which was due to cash proceeds of $0.3 million in proceeds from the employee stock purchase plan and cash proceeds of 0.1 million from the issuance of common stock upon the exercise of stock options, which was partially offset by $0.2 million for the payment of deferred offering costs.

Funding requirements

We will continue to require substantial additional capital to develop our product candidates and fund operations for the foreseeable future. On May 10, 2024, we filed an automatic shelf registration statement on Form S-3ASR that allows us to undertake various equity and debt offerings and entered into the Leerink Sales Agreement under which we may offer and sell shares of common stock, having aggregate sales proceeds of up to $175,000,000 from time to time, through the Leerink ATM. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the development of and seek regulatory approvals for our product candidates and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may harm our business. In addition, we expect to continue to incur additional costs associated with operating as a public company. Our expenses will also increase if, and as, we:

- advance our product candidates through preclinical and clinical development;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- continue to invest in our proprietary drug discovery platform;
- seek to discover and develop additional product candidates;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval and intend to commercialize on our own or jointly;
- hire additional clinical, quality control, scientific and other personnel;
- expand our operational, financial and management systems and increase personnel including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand, protect and enforce our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

We do not currently have any long-term material capital requirements other than what will be required to fund operations for the foreseeable future and the amounts disclosed on the contractual obligations and commitments section below. In order to complete the process of obtaining regulatory approval for our product candidates and to build the sales, marketing and distribution infrastructure that we believe will be necessary to commercialize our product candidates, if approved, we will require substantial additional funding.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of product candidates, we are unable to estimate the exact amount and timing of our working capital requirements. Our future funding requirements will depend on many factors, including:

- the scope, progress, results and costs of researching and developing our product candidates including:
    - conducting preclinical studies and clinical trials;
    - the costs, timing and outcome of regulatory review of our product candidates;
    - the number and characteristics of other product candidates that we pursue;

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

A change in the outcome of any of these or other factors with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate. Furthermore, our operating plans may change in the future, and we may need additional funds to meet operational needs and capital requirements associated with such operating plans.

If we are unable to raise additional funds when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts. We may also be required to grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

The issuance of additional equity securities may cause our stockholders to experience dilution. Future equity or debt financings may contain terms that are not favorable to us or our stockholders including debt instruments imposing covenants that restrict our operations and limit 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.

Operating and Capital Expenditure Requirements and Contractual Obligations

We expect that our existing cash and cash equivalents and marketable securities, will be sufficient to enable us to fund our planned operating expenses and capital expenditure requirements through at least the next 12 months.

Our short-term material cash requirements as of September 30, 2024 are to fund our operations, which consist primarily of research and development expenses related to our programs, and to a lesser extent, general and

administrative expenses. We have entered into contracts in the normal course of business with CROs, CDMOs and other third parties for preclinical research studies and testing, clinical trials and manufacturing services. These contracts do not contain any minimum purchase commitments and are cancelable by us upon prior notice. Payments due upon cancellation consist only of payments for services provided and expenses incurred, including non-cancelable obligations of our service providers, up to the date of cancellation.

Our long-term cash requirements as of September 30, 2024 includes our lease obligations. In January 2022, we entered into a lease agreement for approximately 18,614 square feet of office and laboratory space in Boulder, Colorado which includes escalating rent payments and an 8.2 year term, plus our share of operating expenses. In February 2023, the lease was modified to occupy an additional 9,624 square feet of office space, with aggregate payments of approximately $1.5 million over the initial 7.3 year term, plus our share of operating expenses. As of September 30, 2024 our total operating lease liability balance is $4.4 million, of which $1.0 million is a current liability.

Critical Accounting Estimates

Our financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on a periodic basis. Our actual results may differ from these estimates.

Our critical accounting policies are described in the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Estimates” in our Annual Report on Form 10-K filed with the SEC on February 22, 2024 and the notes to the financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. During the three and nine months ended September 30, 2024, there were no material changes to our critical accounting estimates from those discussed in our Annual Report on Form 10-K filed on February 22, 2024.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our financial statements appearing in our Quarterly Report.

Emerging Growth Company and Smaller Reporting Company Status

Section 107 of The Jumpstart Our Business Startups Act of 2012 (JOBS Act) permits an “emerging growth company” such as us to take advantage of an extended transition time to comply with new or revised accounting standards as applicable to public companies. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for private companies.

We will remain an emerging growth company until December 31, 2024.

We are also currently a “smaller reporting company,” and based on the market value of our common stock held by non-affiliates exceeding $700.0 million as of June 30, 2024, we will transition to a “large accelerated filer” status with effect as of January 1, 2025.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are a smaller reporting company as defined by Rule 12b-2 of the Exchange Act and are not required to provide the information required under this item.

Item 4. Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rule 13a-15(e) of the Securities Exchange Act of 1934, as amended, or the Exchange Act) as of September 30, 2024. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures as of September 30, 2024 were effective at a reasonable assurance level in ensuring that information required to be disclosed by us in reports that we file or submit under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the Securities and Exchange Commission's rules and forms; and (ii) accumulated and communicated to management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely discussions 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.

There was no change in our internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) that occurred during the three and nine months ended September 30, 2024 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings. The outcome of litigation cannot be predicted with certainty and some lawsuits, claims or proceedings may be disposed of unfavorably to us, which could materially affect our financial condition or results of operations.

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

Investing in shares of our common stock involves a high degree of risk because our business is subject to numerous risks and uncertainties, as fully described below. The principal factors and uncertainties that make investing in shares of our common stock risky include, among others:

- We have a limited operating history, some of our product candidates are early in development and we have no products approved for commercial sale.
- We have not generated any revenue to date, have incurred significant net losses since our inception, and expect to continue to incur significant net losses for the foreseeable future.

- Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve several objectives relating to the discovery, development and commercialization of our product candidates, if approved.
- We will require substantial additional capital to finance our operations. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs or future commercialization efforts.
- Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.
- We are substantially dependent on the success of our lead product candidate, sevasemten (EDG-5506).
- In addition to sevasemten, our prospects depend in part upon developing and commercializing EDG-7500 and product candidates from our EDG-003 cardiometabolic discovery program and discovering, developing and commercializing product candidates in future programs, which may fail or suffer delays that adversely affect their commercial viability.
- Clinical drug development involves a lengthy and expensive process with an uncertain outcome. The clinical trials of our product candidates may not demonstrate safety and efficacy to the satisfaction of the FDA, 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.
- 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.
- The outcome of preclinical testing 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.
- 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 have limited resources and are currently focusing the majority of our efforts on developing sevasemten and EDG-7500 for particular indications. As a result, we may fail to capitalize on other indications or product candidates that may ultimately have proven to be more profitable.
- 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.
- Interim, topline and preliminary data from our clinical trials that we announce or publish may change as more patient data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.
- We may not be successful in our efforts to develop a proprietary drug discovery platform to build a pipeline of product candidates.
- We may develop sevasemten and potentially other programs in combination with other therapies, which would expose us to additional risks.
- The manufacture of drugs is complex, and our third-party manufacturers may encounter difficulties in production.
- Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.
- Our product candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

- The patient population suffering from Duchenne muscular dystrophy (Duchenne), Becker muscular dystrophy (Becker) and Limb-girdle muscular dystrophy is small and has not been established with precision.
- The regulatory approval processes of the FDA, EMA and other comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable.
- Our success is highly dependent on our ability to attract and retain highly skilled executive officers and employees.
- Our success depends on our ability to protect our intellectual property and our proprietary technologies.
- We rely, and expect to continue to rely, on third parties to conduct our clinical trials and those third parties may not perform satisfactorily.
- We contract with third parties for the production of our product candidates.
- Our reliance on third parties may require us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.
- 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.
- Our operations and financial results could be adversely impacted by public health pandemics, such as COVID-19 and other related outbreaks in the United States and the rest of the world.

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

We have a limited operating history, some of our product candidates are early in development and we have no products approved for commercial sale, which may make it difficult for you to evaluate our current business and likelihood of success and future viability.

We are a clinical stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. We are developing precision medicines for rare neuromuscular diseases which is an unproven and highly uncertain undertaking and involves a substantial degree of risk. We commenced operations in 2017, have no products approved for commercial sale and have not generated any revenue. In July 2022, we initiated the first of four Phase 2 clinical trials for our lead product candidate, sevasemten, and we are enrolling Part B and Part C of a multipart Phase 2 clinical trial with our product candidate EDG-7500 for people with HCM. We have not yet initiated clinical trials for any other product candidate, including product candidates from our EDG-003 cardiometabolic discovery program. Since our inception in 2017, we have devoted substantially all of our focus and financial resources to discovering, identifying and developing potential product candidates, including advancing our development programs, conducting preclinical studies of our product candidates and initiating clinical trials, organizing and staffing our company, business planning, raising capital and securing related intellectual property rights.

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

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

We have not generated any 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 revenue to date and have financed our operations principally through private placements of our convertible preferred stock and public offerings of our common stock. Our net loss was $34.1 million and $94.2 million for the three and nine months ended September 30, 2024, respectively. As of September 30, 2024, we had an accumulated deficit of $338.9 million. We are advancing sevasemten and EDG-7500 in clinical development. Our other programs, including EDG-003, are in preclinical discovery and research stages. 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 approved product candidates, we expect that we will continue to incur substantial research and development and other expenses in order to discover, develop and market additional potential products.

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

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

Our business depends entirely on the successful discovery, development, regulatory approval and commercialization of product candidates. We have no products approved for commercial sale and do not anticipate generating any revenue from product sales for the next several years, if ever. 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 sevasemten, EDG-7500, and product candidates from our EDG-003 cardiometabolic discovery program and our other future product candidates and programs;
- establishing and maintaining relationships with CROs and clinical sites for the clinical development of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any other future product candidates and programs;
- the initiation and successful patient enrollment and completion of additional clinical trials on a timely basis;
- acceptable frequency and severity of adverse events in the clinical trials;
- the efficacy and safety profiles that are satisfactory to the 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;
- 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;
- a continued acceptable safety profile following any marketing approval of our product candidates;
- commercial acceptance of our product candidates by patients, the medical community and third-party payors;
- timely receipt of reimbursement from applicable authorities for any product candidates for which we successfully receive regulatory approval;
- satisfying any required post-marketing approval commitments to applicable regulatory authorities;
- identifying, assessing and developing new product candidates;
- obtaining, maintaining and expanding patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting our rights in our intellectual property portfolio;
- defending against third-party infringement claims, if any;
- entering into, on favorable terms, any collaboration, licensing or other arrangements that may be necessary or desirable to develop, manufacture or commercialize our product candidates;
- obtaining coverage and adequate reimbursement by third-party payors for our products and patients' willingness to pay in the absence of such coverage and adequate reimbursement;
- obtaining additional funding to develop and potentially manufacture and commercialize our product candidates;
- addressing any competing therapies and technological and market developments;
- managing costs, including any unforeseen costs, that we may incur as a result of nonclinical study or clinical trial delays due to COVID-19 or other public health outbreaks or emergencies, inflation or other causes; and
- attracting, hiring and retaining qualified personnel, including clinical, scientific, management and administrative personnel.

We may never be successful in achieving our objectives and, even if we do, may never generate revenue that is significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease

the value of our company and could impair our ability to maintain or further our research and development efforts, raise additional necessary capital, grow our business and continue our operations.

We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our product candidates on a timely or profitable basis, if at all. Changes in the manufacturing process or facilities will require further comparability analysis and approval by the FDA before implementation, which could delay our clinical trials and product candidate development, and could require additional clinical trials, including bridging studies, to demonstrate consistent and continued safety and efficacy.

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

As of September 30, 2024, we had $492.5 million in cash, cash equivalents and marketable securities. We expect our current cash, cash equivalents and marketable securities will be sufficient to fund our current operating plan for at least the next 12 months. On May 10, 2024, we filed an automatic shelf registration statement on Form S-3ASR that allows us to undertake various equity and debt offerings. We additionally filed a prospectus supplement to the shelf registration statement and entered into a sales agreement with Leerink Partners LLC (Leerink Sales Agreement) on May 10, 2024, under which we may offer and sell shares of common stock, having aggregate sales proceeds of up to $175,000,000 from time to time, through an "at the market offering" program (Leerink ATM). We have not yet offered or sold any shares of common stock related to the Leerink ATM. Our estimate as to how long we expect our existing cash, cash equivalents and marketable securities to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. Our operations have consumed substantial amounts of cash since inception, and we expect our expenses to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek marketing approval for, sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program, as well as develop our proprietary drug discovery platform. 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, the EMA or other regulatory agencies to perform clinical trials or preclinical studies in addition to those that we currently anticipate. Other unanticipated costs may also arise. Because the design and outcome of our planned and anticipated preclinical studies and clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of resources and funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop. We are not permitted to market or promote sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program or any other product candidate before we receive marketing approval from the FDA. We also expect to incur costs associated with operating as a public company. Our cash, cash equivalents and marketable securities will not be sufficient for us to fund any of our product candidates through regulatory approval, and we will need to raise additional capital to complete the development and commercialization of our products. Accordingly, we will need to obtain substantial additional funding in order to continue our operations.

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

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

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

A change in the outcome of any of these or other factors with respect to the development of any of our product candidates could significantly change the costs and timing associated with the development of that product candidate.

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. In the event that we would need to obtain additional funding, our ability to raise or access capital may be affected by macroeconomic events and disruptions to the U.S. banking and financial sectors. Failures of banks and other financial institutions, such as Silicon Valley Bank in March 2023, or issues in the broader U.S. financial system may impact the broader capital markets, and in turn, may impact our ability to access those markets. Further, a tightening of credit markets and lending standards could it make more difficult for us to raise capital through either debt or equity offerings on commercially reasonable terms or at all.

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 research-stage programs, clinical trials or future commercialization efforts.

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

Until such time, if ever, as we can generate substantial revenues, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources, which may dilute our stockholders or restrict our operating activities. As summarized in the risk factor entitled, "*We will require substantial additional capital to finance our operations. If we are unable to raise such capital when needed, or on acceptable terms, we may be forced to delay, reduce and/or eliminate one or more of our research and drug development programs or future commercialization efforts*.", we have previously raised capital under our shelf registration statement that was filed on April 1, 2022 with the SEC that became effective on May 5, 2022 and was amended on January 19, 2024. On May 10, 2024, we filed an automatic shelf registration statement on Form S-3ASR that allows us to undertake various equity and debt offerings and entered into the Leerink Sales Agreement under which we may offer and sell shares of common stock, having aggregate sales proceeds of up to $175,000,000 from time to time, through the Leerink ATM.

Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, 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, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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

Our net operating loss (NOL) carryforwards may be unavailable to offset future taxable income because of restrictions under U.S. tax law. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 taxable years under applicable U.S. federal tax law, and therefore could expire unused. Under tax legislation commonly referred to as the Tax Cuts and Jobs Act of 2017 (Tax Act) as amended by the Coronavirus Aid, Relief, and Economic Security Act, our federal NOLs generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but for taxable years beginning after December 31, 2020, the deductibility of federal NOLs generated in tax years beginning after December 31, 2017 is limited to 80% of our current year taxable income. Our state NOLs may be subject to similar or different limitations. As of December 31, 2023, we had available federal NOL carryforwards of approximately $109.4 million, of which $108.2 million do not expire, and state NOL carryforwards of approximately $114.3 million, of which $32.8 million do not expire.

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 may 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. We have not conducted any studies to determine annual limitations, if any, that could result from such changes in the ownership of our stock. Our ability to utilize our NOLs and certain other tax attributes could be limited by an "ownership change" as described above and consequently, we may not be able to utilize a material portion of our NOLs and certain other tax attributes, which could have a material adverse effect on our cash flows and results of operations.

Changes in tax laws could have a material adverse effect on our business, cash flow, results of operations or financial conditions.

We are subject to tax laws, regulations, and policies of several taxing jurisdictions. Changes in tax laws, as well as other factors, could cause us to experience fluctuations in our tax obligations and effective tax rates and otherwise adversely affect our tax positions and/or our tax liabilities. For example, in August 2022, the United States enacted the Inflation Reduction Act of 2022, which imposes a 1% non-deductible excise tax on certain stock buybacks and a 15% alternative minimum tax on global adjusted financial statement income. In addition, beginning in 2022, the Tax Act eliminated the option to deduct research and development expenditures currently and requires taxpayers to capitalize and amortize them over five or fifteen years, and this requirement may impact our effective tax rate and our cash tax liability in future years. When and if we achieve profitability, these changes may cause us to pay federal income taxes earlier than under prior law and may increase our total federal tax liability attributable to orphan drug programs and other research and development activities. Further, many countries, and organizations such as the Organization for Economic Cooperation and Development have proposed implementing changes to existing tax laws, including a proposed 15% global minimum tax that has been and is being adopted by several countries, with implementation beginning in 2024. Any of these developments or changes in U.S. federal, state, or international tax laws or tax rulings could adversely affect our effective tax rate and our operating results. There can be no assurance that our effective tax rates, tax payments, or tax credits and incentives will not be adversely affected by these or other developments or changes in law.

Market conditions and changing circumstances, some of which may be beyond our control, could impair our ability to access our existing cash, cash equivalents and investments and to timely pay key vendors and others.

Market conditions and changing circumstances, some of which may be beyond our control, could impair our ability to access our existing cash, cash equivalents and investments and to timely pay key vendors and others. For example, on March 10, 2023, Silicon Valley Bank (SVB), where we maintain certain operating accounts, was placed into receivership with the Federal Deposit Insurance Corporation (FDIC), which resulted in all funds held at SVB being temporarily inaccessible by SVB's customers. If other banks and financial institutions with whom we have banking relationships enter receivership or become insolvent in the future, we may be unable to access, and we may lose, some or all of our existing cash, cash equivalents and investments to the extent those funds are not insured or otherwise protected by the FDIC. In addition, in such circumstances we might not be able to timely pay key vendors and others. We regularly maintain cash balances that are not insured or are in excess of the FDIC's insurance limit. Any delay in our ability to access our cash, cash equivalents and investments (or the loss of some or all of such funds) or to timely pay key vendors and others could have a material adverse effect on our operations and cause us to need to seek additional capital sooner than planned.

Our operations and financial results could be adversely impacted by public health pandemics, such as COVID-19 and other related outbreaks in the United States and the rest of the world.

Disruptions caused by the COVID-19 pandemic impacted our productivity, resulted in increased operational expenses, certain adjustments to the operations of our clinical trial, delays in the enrollment of new patients at our clinical trial site, and delays in certain supply chain activities and collecting and analyzing data from patients in our clinical trial.

To the extent we may experience any disruptions directly or indirectly through our contractors or partners as a result of any ongoing pandemic, outbreaks or other public health emergencies or disruptions, including any resurgence in COVID-19 cases in the future, that could severely impact our business and clinical trials, including:

- further delays or difficulties in enrolling and retaining patients in our clinical trials or those conducted by third parties and further incurrence of additional costs as a result of preclinical study and clinical trial delays and adjustments;
- challenges related to ongoing and increased operational expenses related to pandemics or public health emergencies or disruptions;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays, difficulties or increased costs to comply with COVID-19 or other public health related protocols at our leased facilities and clinical sites;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others;
- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;
- delays in preclinical and clinical sites receiving the supplies and materials needed to conduct our clinical trials;
- interruption in global shipping that may affect the transport of clinical trial materials, such as investigational drug product used in our clinical trials;
- changes in regulations as part of a response to the COVID-19 pandemic or other public health emergencies or disruptions which may require us to change the ways in which our clinical trials are conducted, or to discontinue the clinical trials altogether, or which may result in unexpected costs;
- delays in necessary interactions with regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government or contractor personnel;
- refusal of the FDA to accept data from clinical trials in affected geographies outside the United States; and
- increased competition for contract research organizations (CROs), suppliers and vendors.

Additionally, certain third parties with whom we engage, including our collaborators, contract organizations, third-party manufacturers, suppliers, clinical trial sites, regulators and other third parties with whom we conduct business may adjust their operations in light of the COVID-19 pandemic or other public health emergencies. If these third parties experience shutdowns or continued business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. For example, we experienced delays in trial initiation for our Phase 1 clinical trial of sevasemten and switched from an international third-party manufacturer to a third-party manufacturer based in the United States to minimize manufacturing supply chain disruptions as a result of COVID-19. Changing our third-party manufacturer could result in delays in our manufacturing supply chain which could delay or otherwise impact our development of sevasemten and result in increased costs related to sevasemten. Additionally, certain preclinical studies for our discovery research programs are conducted by CROs, which could be discontinued or delayed as a result of the pandemic. We could also experience delays if our suppliers are delayed in delivering raw materials to our third-party manufacturers. For example, we experienced delays in enrolling patients for our Phase 1 clinical trial for sevasemten. In addition, our clinical trial sites could experience delays in collecting, receiving, and analyzing data from patients enrolled in our

clinical trial for sevasemten due to limited staff at such sites, limitation or suspension of on-site visits by patients, or patients' reluctance to visit the clinical trial sites during the pandemic. As a result, research and development expenses and general and administrative expenses may vary significantly if there is an increased impact from COVID-19 or other public health emergencies on the costs and timing associated with the conduct of our clinical trial and other related business activities.

In the event of a resurgence of COVID-19 or other public health emergencies, we could be required to develop and implement additional clinical trial policies and procedures designed to help protect subjects from such diseases. During the COVID-19 pandemic, the FDA has issued various COVID-19 related guidance documents for sponsors and manufacturers, many of which have expired or were withdrawn with the expiration of the COVID-19 public health emergency declaration on May 11, 2023, although some COVID-19 related guidance documents continue in effect.

Any continued and prolonged public health crisis, such as the COVID-19 pandemic, could have a material negative impact on our business, financial condition and operating results.

To the extent the COVID-19 pandemic or other public health emergencies or outbreaks adversely affect our business, financial condition and operating results, it may also have the effect of heightening many of the risks described in this "Risk Factors" section.

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

We are substantially dependent on the success of our lead product candidate, sevasemten. If we are unable to complete further development of, obtain approval for and commercialize sevasemten for one or more indications in a timely manner, our business will be harmed.

Our future success is dependent on our ability to timely and successfully complete clinical trials, obtain marketing approval for and successfully commercialize sevasemten, our lead product candidate. We are investing the majority of our efforts and financial resources in the research and development of sevasemten. Sevasemten is in advanced clinical trials in patients with Becker, Duchenne, and Limb-Girdle muscular dystrophies as well as McArdle Disease.

Sevasemten will require additional clinical development, expansion of manufacturing capabilities, marketing approval from government regulators, substantial investment and significant marketing efforts before we can generate any revenues from product sales. We are not permitted to market or promote sevasemten, or any other product candidate before we receive marketing approval from the FDA and comparable foreign regulatory authorities, and we may never receive such marketing approvals.

The success of sevasemten will depend on several factors, including the following:

- the successful and timely completion of our ongoing nonclinical studies and clinical trial of sevasemten;
- the initiation and successful patient enrollment and completion of additional clinical trials of sevasemten on a timely basis;
- maintaining and establishing relationships with CROs and clinical sites for the clinical development of sevasemten;
- the frequency and severity of adverse events in clinical trials;
- demonstrating efficacy, safety and tolerability profiles that are satisfactory to the FDA, EMA or any comparable foreign regulatory authority for marketing approval;

- the timely receipt of marketing approvals for sevasemten from applicable regulatory authorities;
- maintaining the ODD and RPDD for sevasemten;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- the maintenance of existing or the establishment of new supply arrangements with third-party drug product suppliers and manufacturers for clinical development and, if approved, commercialization of sevasemten;
- obtaining and maintaining patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting our rights in our intellectual property portfolio;
- our ability to expand sevasemten into multiple indications;
- the successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- the actual market-size, ability to identify patients and the demographics of patients eligible for our product candidates, which may be different than expected;
- commercial acceptance by patients, the medical community and third-party payors, particularly since the product candidates we develop may be novel;
- our ability to compete or combine with other therapies; and
- addressing any delays, necessary adjustments and additional costs in nonclinical study and clinical trials resulting from factors related to public health pandemics, including the COVID-19 pandemic.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize sevasemten, which would materially harm our business. If we do not receive marketing approvals for sevasemten, we may not be able to continue our operations.

In addition to sevasemten, our prospects depend in part upon developing and commercializing EDG -7500 and product candidates from our EDG-003 cardiometabolic discovery program and discovering, developing and commercializing product candidates in future programs, which may fail or suffer delays that adversely affect their commercial viability.

Our future operating results are dependent on our ability to successfully develop, obtain regulatory approval for and commercialize EDG-7500, product candidates from our research program currently focused on cardiometabolic indications, or EDG-003, and our lead product candidate, sevasemten. EDG-7500 is currently in a multipart Phase 2 trial. However, research and development related to novel therapeutics is inherently risky. A product candidate can unexpectedly fail at any stage of preclinical and/or clinical development. The historical failure rate for product candidates is high due to risks relating to safety, efficacy, clinical execution, changing standards of medical care and other unpredictable variables. The results from preclinical testing or early clinical trials of a product candidate may not be predictive of the results that will be obtained in later stage clinical trials of the product candidate.

The success of other product candidates we may develop will depend on many factors, including the following:

- generating sufficient data to support the initiation or continuation of clinical trials;
- obtaining regulatory permission to initiate clinical trials;
- contracting with the necessary parties to conduct clinical trials;
- successful enrollment of patients in, and the completion of, clinical trials on a timely basis;
- the timely manufacture of sufficient quantities of a product candidate for use in clinical trials;
- adverse events in clinical trials; and
- addressing any delays in our research programs resulting from factors related to public health pandemics, including the COVID-19 pandemic.

Even if we successfully discover and advance any other 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, commercialize, or generate significant revenue from any product candidates.

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

Although we have announced positive results from our preclinical studies and clinical trials, our product candidates' risk of failure is high and it is impossible to predict when or if sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program or any other product candidate that we develop will prove effective or safe in humans or will receive marketing approval. Before obtaining marketing approval from the FDA, EMA or other comparable foreign regulatory authorities for the sale of our product candidates, we must complete preclinical development and extensive clinical trials to demonstrate with substantial evidence the safety and efficacy of such product candidates.

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

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. 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 other regulatory authorities to require additional testing before approving any of our product candidates.

We have experienced delays in completing our ongoing clinical trial and may experience additional delays in initiating or completing additional clinical trials including delays as a result of COVID-19. We may also experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent receipt of marketing approval or our ability to commercialize our product candidates, including:

- receipt of feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trial observations or results that require us to modify the design of our clinical trials;
- negative or inconclusive clinical trial results that may require us to conduct additional clinical trials or abandon certain drug development programs;
- obtaining approval from one or more institutional review boards (IRB);
- the number of patients required for clinical trials being larger than anticipated, enrollment in these clinical trials being slower than anticipated or participants dropping out of these clinical trials at a higher rate than anticipated;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the suspension or termination of our clinical trials for various reasons, including non-compliance with regulatory requirements or a finding that our product candidates have undesirable side effects or other unexpected characteristics or risks;
- changes to clinical trial protocol;
- clinical sites deviating from trial protocol or dropping out of a trial;
- the cost of clinical trials of our product candidates being greater than anticipated;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates being insufficient or inadequate;
- subjects experiencing severe or unexpected drug-related adverse effects;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of cGMPs, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process that may be necessary or desired;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practices (GCP) or other regulatory requirements;

- third-party contractors not performing data collection or analysis in a timely or accurate manner;
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications; and
- regulators revising the requirements for approving our product candidates.

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

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

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

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

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

We are developing novel biologically active small molecules for muscle related diseases. As a result, there is uncertainty as to the safety profile of product candidates we may develop. In addition, our product candidates may be used in combination with certain other therapies, including corticosteroids, which may have undesirable side effects. If our product candidates are associated with undesirable side effects or have unexpected characteristics in preclinical studies or clinical trials when used alone or in combination with other approved products or

investigational new drugs we may need to interrupt, delay or abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Treatment-related side effects could also affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Any of these occurrences may prevent us from achieving or maintaining market acceptance of the affected product candidate and may harm our business, financial condition and prospects significantly.

Patients in our ongoing and planned clinical trials may in the future suffer other adverse events or other side effects not observed in our preclinical studies or previous clinical trials. For example, in the single ascending dose (SAD) trial for sevasemten, dose limiting somnolence was observed at the 135 mg level. In addition, in the multiple ascending dose (MAD) trial for sevasemten, the most common adverse events were dizziness and somnolence, all of which were mild and transient. In the ARCH trial of sevasemten in adults wit Becker, the most common adverse events were dizziness, fall, and arthralgia, which were mild and transient. Sevasemten or other product candidates may be used in pediatric populations for which safety concerns may be particularly scrutinized by regulatory agencies. In addition, if sevasemten is studied in combination with other therapies, it may exacerbate adverse events associated with the therapy. Patients treated with sevasemten or our other product candidates may also be undergoing other therapies which can cause side effects or adverse events that are unrelated to our product candidate but may still impact the success of our clinical trials. The inclusion of critically ill patients in our clinical trials may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using or due to the gravity of such patients' illnesses, which could occur either during the course of our clinical trials or after participating in such clinical trials.

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

The outcome of preclinical testing 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. Although we have announced positive results from our preclinical studies and clinical trials, we do not know whether sevasemten or EDG-7500 will perform in current or future clinical trials as sevasemten has performed in preclinical studies or earlier clinical trials, nor do we know whether any product candidate in our EDG-003 cardiometabolic discovery program will perform in current or future preclinical studies or future clinical trials as it has in prior preclinical studies. Product candidates in clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of the FDA, EMA and other comparable foreign regulatory authorities despite having progressed through preclinical studies. Regulatory authorities may also limit the scope of later-stage trials until we have demonstrated satisfactory safety, which could delay regulatory

approval, limit the size of the patient population to which we may market our product candidates, or prevent regulatory approval.

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

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

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

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

For sevasemten trials, we completed our open-label ARCH trial and completed enrollment of the DUNE Phase 2 exercise challenge study at a single clinical trial site and our CANYON Phase 2 clinical trial at multiple sites. All other Phase 2 clinical trials are also being conducted at multiple sites. We have initiated an industry-sponsored, global, prospective registry investigating the natural history of adults with Becker aged 18 years and older. However, we may not be successful in achieving our goal of establishing natural history reference data points and identifying future eligibility for recruitment into our planned registrational trial in Becker. In addition, we completed the Phase 1 trial with EDG-7500 in healthy subjects and the Part A single-dose arm of the multipart Phase 2 Cirrus-CM trial in patient with oHCM. However, we may experience difficulty with enrollment and/or maintenance of patients in the ongoing enrollment of Part B and Part C of the Phase 2 trial.

We are developing product candidates for severe muscle diseases with limited patient pools from which to draw for clinical trials. Such trials may be difficult to enroll and the lack of data on these patients may negatively impact the approvability of sevasemten. We also may encounter difficulties in identifying and enrolling subjects with a stage of disease appropriate for our planned clinical trials and monitoring such subjects adequately during and after treatment. We may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible subjects to participate in the clinical trials required by the FDA or comparable foreign regulatory authorities. In addition, the process of finding and diagnosing subjects may prove costly. Further, the treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies.

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

- size and nature of the patient population;
- perceived risks and benefits of novel, unproven approaches;
- severity of the disease under investigation;

- availability and efficacy of approved drugs for the disease under investigation;
- patient eligibility criteria for the trial in question as defined in the protocol;
- perceived risks and benefits of the product candidate under study;
- clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new products that may be approved or other product candidates being investigated for the indications we are investigating;
- patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- the activities of key opinion leaders (KOLs) and patient advocacy groups;
- proximity and availability of clinical trial sites for prospective patients; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion or, because they may have an advanced disease, will not survive the full terms of the clinical trials.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates and jeopardize our ability to obtain marketing approval for the sale of our product candidates. Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining participation in our clinical trials through the treatment and any follow-up periods.

We have limited resources and are currently focusing the majority of our efforts on developing sevasemten and EDG-7500 for particular indications. As a result, we may fail to capitalize on other indications or product candidates that may ultimately have proven to be more profitable.

We are currently focusing the majority of our resources and efforts on developing sevasemten and EDG-7500. As a result, because we have limited resources, we may forgo or delay the pursuit of opportunities for other indications or with other product candidates that may have greater commercial potential, including product candidates from our EDG-003 cardiometabolic discovery program. In addition, while we currently have multiple compounds in our programs, we are focusing our efforts on select product candidates from each of these programs to develop as lead product candidates in each program. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development activities for sevasemten, EDG-7500 and our EDG-003 cardiometabolic discovery program may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target markets for sevasemten, EDG-7500 or the product candidates we are currently researching, such as those from our EDG-003 cardiometabolic discovery program, we may relinquish valuable rights to our product candidates or programs through collaboration, licensing or other strategic arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate or program.

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

The biotechnology and biopharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary and novel products and product candidates. Our competitors have developed, are developing or may develop products, product candidates and processes

competitive with our product candidates. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may attempt to develop product candidates.

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

With sevasemten, we expect to face competition from existing products and products in development. Approximately 70% of patients with Duchenne are treated with corticosteroids to manage the inflammatory component of the disease. Deflazacort and prednisone are FDA-approved corticosteroids and are marketed by multiple companies. In October 2023, the FDA granted Agamree (vamorolone) approval in Duchenne patients aged 2 years and older and Catalyst Pharmaceuticals, Inc. announced commercialization of this product in the United States in March 2024 following its North America exclusive license deal with Santhera. In addition, there are four exon skipping drugs which are marketed under an accelerated approval pathway from the FDA: EXONDYS 51 (eteplirsen), AMONDYS 45 (casimersen) and VYONDYS 53 (golodirsen), which are naked phosphorodiamidate morpholino oligomers (PMOs) approved for the treatment of Duchenne patients amenable to Exon 51, Exon 45 and Exon 53 skipping, respectively, and are marketed by Sarepta Therapeutics, Inc., and VILTEPSO (vitolarsen), a naked PMO approved for the treatment of Duchenne patients amenable to Exon 53 skipping, which is marketed by Nippon Shinyaku Co. Ltd. In May 2024, Nippon Shinyaku Co. Ltd. announced that no statistical significance was observed between the treatment group and the placebo group in VILTEPSO's confirmatory study. This result may affect VILTEPSO's accelerated FDA approval. In June 2022, PTC Therapeutics presented new topline results with Translarna (ataluren), for patients with nonsense mutation Duchenne, a subset of the disease that impacts between 10% and 15% of patients. It remains unclear if the data will lead to FDA approval of Translarna, for which the company resubmitted the NDA in July 2024. Translarna has been conditionally approved in the European Union and Brazil for ambulatory patients aged 2 years and older with Duchenne resulting from a nonsense mutation in the dystrophin gene. However, in January and June 2024, the Committee for Medicinal Products for Human Use of the EMA delivered negative opinions on the re-examination procedure for the conditional marketing authorization of Translarna. This is expected to result in the withdrawal of Translarna from the EMA markets. In June 2023, the FDA approved Sarepta's Biologics License Application seeking accelerated approval of their microdystrophin gene therapy, Elevidys (delandistrogene moxeparvovec), for the treatment of ambulant individuals with Duchenne between the ages of four to five years. In June 2024, the FDA granted Elevidys full approval for the treatment of ambulatory individuals aged 4 years and older, and accelerated approval for the treatment of non-ambulatory individuals aged 4 years and older. Other companies focused on developing genetic based therapies for Duchenne that target dystrophin mechanisms include Solid Biosciences Inc., Genethon, PepGen, Dyne Therapeutics, Avidity Biosciences, REGENXBIO, Wave Life Sciences, and Entrada Therapeutics. In June 2024, Pfizer announced its gene therapy Phase 3 study failed to meet the primary and key secondary endpoints and is no longer under development. Gene editing treatments that are in preclinical development are also being pursued by Vertex and Sarepta Therapeutics.

We are also aware of several companies targeting non-dystrophin mechanisms for the treatment of Duchenne. In June 2022, Italfarmaco announced positive topline data from its completed Phase 3 trial with givinostat, a histone deacetylase (HDAC) inhibitor, in boys with Duchenne. In March 2024, the FDA approved Duvyzat (givinostat) for the treatment of Duchenne muscular dystrophy in patients aged six years and older. Moreover, in June 2021, Italfarmaco released top line Phase 2 data for givinostat in Becker. Givinostat did not show a significant difference in the primary endpoint compared to placebo. The future of this program in Becker is uncertain.

With EDG-7500, we expect to face competition from existing products and products in development. Current pharmaceutical treatment is intended to improve diastolic filling in both obstructive hypertrophic cardiomyopathy

(oHCM) and nonobstructive hypertrophic cardiomyopathy (nHCM) and reduce left ventricular outflow tract gradient in oHCM patients only. The goal of current therapies is to achieve meaningful symptom relief. Non-vasodilating beta blockers and non-dihydropyridine calcium channel blockers are the first-line therapies for symptomatic oHCM and nHCM patients. Commonly prescribed beta-blockers are atenolol, propranolol, and metoprolol. Verapamil and diltiazem are calcium channel blockers used in the treatment of symptomatic oHCM and nHCM. For oHCM patients who remain symptomatic, disopyramide (either Norpace, marketed by Pfizer, or a generic form marketed by several companies) or Camzyos (mayacamten) may also be added.

In the field of emerging treatments intended to treat HCM, competitors include Bristol-Myers Squibb (BMS), Cytokinetics, Imbria Pharmaceuticals, and Celltrion. Cytokinetics is developing a CMI, aficamten (CK-274), for which positive topline Phase 3 oHCM trial results were reported in December 2023. In June 2023, Cytokinetics initiated another Phase 3 active-comparator clinical trial of aficamten compared to metoprolol in symptomatic oHCM patients (MAPLE trial). In the second quarter of 2024, BMS and Cytokinetics initiated a study of mavacamten and aficamten, respectively, in pediatric population with symptomatic oHCM. BMS and Cytokinetics are also exploring their respective CMIs in ongoing Phase 3 nHCM clinical trials, and both are also developing next generation CMIs for the treatment of symptomatic HCM, CK-271 and MYK-224, respectively. A Phase 2 oHCM clinical trial of MYK-224 is currently ongoing.

Non-CMI targeting drugs in development include IMB-101(Imbria Pharmaceuticals), a prodrug of a 3-ketoacyl CoA thiolase enzyme inhibitor, CT-G20 (Celltrion), an anti-arrhythmic cibenzoline succinate, and trientine dihydrochloride (Univar Solutions), a selective copper II chelator. Lexicon Pharmaceuticals announced plans to investigate sotagliflozin, an SGLT1 and SGLT2 inhibitor, in a Phase 3 study of obstructive and non-obstructive HCM patients. In March 2024, the results of a Phase 2 trial in nHCM of IMB-101 were published. We have limited knowledge of CT-G20's Phase 1 oHCM trial status, while the trientine Phase 2 oHCM clinical trial is ongoing. A gene therapy approach, TN-201, a myosin binding protein C3-targeting gene therapy candidate being developed by Tenaya Therapeutics for genetic HCM, is currently in Phase 1b. We are aware of several preclinical HCM programs including: JN-210, a microRNA activating gene therapy approach being developed by Jaan Biotherapeutics; HTX-001, an antisense oligonucleotide approach being developed by Haya Therapeutics; CDR348T and CDR641L, both are non-coding RNA-based therapies being developed by Cardior Pharmaceuticals (acquired by Novo Nordisk in May 2024). We are also aware of several early-stage preclinical HCM assets being developed by DiNAQOR in collaboration with BioMarin Pharmaceuticals (BMN-293/DINA-001) and Lexeo Therapeutics (LX2022), both are gene therapy approaches for genetic HCM. In April 2024, BioMarin announced the discontinuation of the development of BMN-293.

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

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient, have a broader label, are marketed more effectively, are more widely reimbursed or are less expensive than any products that we may develop. Our competitors also may obtain marketing approval from the FDA, EMA or other comparable foreign regulatory authorities for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Even if the product candidates we develop achieve marketing approval, they may be priced at a significant premium over competitive

products if any have been approved by then, resulting in reduced competitiveness. Technological advances or products developed by our competitors may render our technologies or product candidates obsolete, less competitive or not economical. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Interim, topline and preliminary data from our clinical trials that we announce or publish may change as more patient data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary, interim or topline data from our clinical trials. For example, on April 15, 2024, we announced positive two-year topline results from the ARCH open label trial of sevasemten in adults with Becker and on September 19, 2024, we announced positive topline data from the Phase 1 trial of EDG-7500 in healthy subjects and the Part A single-dose arm of the Phase 2 multipart CIRRUS-HCM trial in patients with oHCM. These interim updates are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data are available. In addition, we may report interim analyses of only certain endpoints rather than all endpoints. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data becomes available. Adverse changes between interim data and final data could significantly harm our business and prospects. Further, additional disclosure of interim data by us or by our competitors in the future could result in volatility in the price of our common stock.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the preliminary or topline data that we report differ from late, final or actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program or any other product candidates may be harmed, which could harm our business, financial condition, results of operations and prospects.

We may not be successful in our efforts to develop a proprietary drug discovery platform to build a pipeline of product candidates.

A key element of our strategy is to leverage our proprietary drug discovery platform and our ability to design small molecule inhibitors of fast skeletal myosin to expand our pipeline of product candidates. We are leveraging our proprietary drug discovery platform and capabilities to create precision medicines for muscle diseases with high levels of unmet need. In order to do so, we must continue to invest in our proprietary drug discovery platform and development capabilities. Although our research and development efforts to date have resulted in a pipeline of product candidates, these product candidates may not be safe and effective. In addition, although we expect that our proprietary drug discovery platform will allow us to develop a diverse pipeline of product candidates across multiple therapeutic areas, we may not prove to be successful at doing so. Furthermore, we may also find that the uses of our proprietary drug discovery platform are limited because alternative uses of our therapeutics prove not to be safe or effective. Even if we are successful in building our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side

effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval or achieve market acceptance. Further, because our product candidates and development programs are based on our proprietary drug discovery platform, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs.

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

We may develop sevasemten and potentially other programs in combination with other therapies, which would expose us to additional risks.

We may develop sevasemten and potentially other programs, in combination with one or more currently approved therapies or therapies in development. Patients may not be able to tolerate sevasemten or any other product candidates in combination with other therapies or dosing of sevasemten in combination with other therapies may have unexpected consequences. Even if any of our product candidates were to receive marketing approval or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA, EMA or other comparable foreign regulatory authorities could revoke approval of the therapy used in combination with any of our product candidates, or safety, efficacy, manufacturing or supply issues could arise with these existing therapies. In addition, it is possible that existing therapies with which our product candidates are approved for use could themselves fall out of favor. This could result in the need to identify other combination therapies for our product candidates, or our own products being removed from the market or being less successful commercially.

We may also evaluate our product candidates in combination with one or more other therapies that have not yet been approved for marketing by the FDA, EMA or comparable foreign regulatory authorities. We will not be able to market and sell any product candidate in combination with any such unapproved therapies that do not ultimately obtain marketing approval.

If the FDA, EMA or other comparable foreign regulatory authorities do not approve or revoke their approval of these other therapies, or if safety, efficacy, commercial adoption, manufacturing or supply issues arise with the therapies we may choose to evaluate in combination with sevasemten or any other product candidate, we may be unable to obtain approval of or successfully market any one or all of the product candidates we develop.

Additionally, if the third-party providers of therapies or therapies in development used in combination with our product candidates are unable to produce sufficient quantities for clinical trials or for commercialization of our product candidates, or if the cost of combination therapies are prohibitive, our development and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and growth prospects.

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

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

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

As product candidates progress through preclinical 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. For example, we may explore alternate sevasemten formulations for use with pediatric patients, particularly Duchenne patients, who may have difficulty taking adult formulations. 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.

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. The degree of market acceptance of any of our approved product candidates will depend on a number of factors, including:

- the efficacy and safety profile as demonstrated in clinical trials compared to alternative treatments;
- the 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;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- the availability of an approved product candidate for use as a combination therapy;

- relative convenience and ease of administration;
- the willingness of the target patient population or their caregivers to try new therapies and of physicians to prescribe these therapies;
- the availability of coverage and adequate reimbursement by third-party payors, including government authorities;
- patients' willingness to pay for these therapies in the absence of such coverage and adequate reimbursement;
- the effectiveness of sales and marketing efforts;
- support from KOLs and patient advocacy groups;
- unfavorable publicity relating to our product candidates; and
- the approval of other new therapies for the same indications.

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

The patient population suffering from Duchenne, Becker and Limb-girdle muscular dystrophy (LGMD) is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected. Because the target patient populations of our programs 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.

Duchenne and Becker are rare, genetic neuromuscular disorders. We estimate that Duchenne occurs in approximately 35,000 patients in the US, EU (5) and Japan. Becker has a much lower incidence of approximately 1 in every 18,450 live male births. We estimate that Becker occurs in approximately 12,000 patients in the US, EU (5) and Japan. The approximate global prevalence of LGMDs as a group is estimated to be from 0.56 to 5.75 per 100,000. Our estimates of the size of these patient populations are based on published studies. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. 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. Various factors may decrease the market size of our product and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our product candidates. If the results of these studies or our analysis of them do not accurately reflect the relevant patient population, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability.

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. A newborn screening initiative was put into place with the goal of identifying and providing care for every child born with Duchenne muscular dystrophy and achieving Recommended Uniform Screening Panel (RUSP) status. An Ohio newborn screening (NBS) program was announced in April 2024 in which all newborns in the state of Ohio are screened for Duchenne. A newborn screening pilot program in New York State tested this and other aspects of a comprehensive newborn screening program at a large scale. The pilot was completed in October 2021 and screened more than 36,000 babies born in New York State over two years. Four babies were confirmed to have Duchenne/Becker muscular dystrophy, and one baby was identified as a carrier female. Two other pilot programs have been successfully conducted. In June 2022, Parent Project Muscular Dystrophy, a nonprofit organization leading the fight to end Duchenne, announced that the organization submitted a nomination package to add Duchenne to the RUSP to the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), initiating the review process. The review process

typically takes more than a year and requires two key votes of experts in NBS to move forward. In February 2023, the first key vote took place and the ACHDNC decided that more information was needed before proceeding to the second vote. Work is ongoing to provide the required additional information. However, the ACHDNC may decide that the algorithm developed for accurately detecting muscular dystrophy is not scalable or cost-effective, thus not appropriate for national and state level implementation. In addition, the ACHDNC may decide not to add Duchenne to the RUSP for other reasons. Furthermore, even if Duchenne is added to the RUSP, states may not be able to effectively implement a NBS program. This could reduce the identifiable patient population for the diseases we seek to treat and result in our therapies not being able to be initiated early in the course of the disease.

Additionally, the potentially addressable patient population for each of 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.

We may not be successful in augmenting our product pipeline through acquisitions and in-licenses.

We intend to evaluate select external opportunities to strategically expand our pipeline. While we plan to leverage our leadership team's prior business development experience as we evaluate potential in-licensing and acquisition opportunities to expand our portfolio, we may not be able to identify suitable licensing or acquisition opportunities, and even if we do, we may not be able to successfully secure such licensing and acquisition opportunities. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These 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 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 license or acquire additional product candidates to expand our portfolio, our pipeline, competitive position, business, financial condition, results of operations, and prospects may be materially harmed.

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 initial targets in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size.

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

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved products. In the United States, for example, principal decisions about reimbursement for new products are typically

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

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

Outside the United States, the commercialization of therapeutics is generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the European Union (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 United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

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

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

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing and marketing of therapeutic treatments. We currently have product liability insurance that we believe is appropriate for our stage of development and may need to obtain higher levels prior to marketing any of our product candidates, if approved. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have an adverse effect on our business and financial condition. Also, our insurance policies may have various exclusions, and we may be subject to a product liability claim for

which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

We may be sued if any of our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing, or sale post-approval. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, or a breach of warranties. Claims could also be asserted under state consumer protection laws. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit testing and commercialization of our products. Even successful defense would require significant financial and management resources.

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

- delays in the development of our product candidates;
- FDA, EMA or other regulatory authority investigation of the safety and effectiveness of our products, our manufacturing processes and facilities or our marketing programs;
- decreased or interrupted demand for our products;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing, or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources; and
- the inability to commercialize any products.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

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

Our product candidates are and will continue to be subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, safety, efficacy, approval, recordkeeping, reporting, labeling, storage, packaging, advertising and promotion, pricing, marketing and distribution of drugs. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process must be successfully

completed in the United States and in many foreign jurisdictions before a new drug can be approved for marketing. Obtaining approval by the FDA, EMA and other comparable foreign regulatory authorities is costly, unpredictable, typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved. In addition, approval policies, regulations or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other data. Even if we eventually complete clinical testing and receive approval for our product candidates, the FDA, EMA and other comparable foreign regulatory authorities may approve our product candidates for a more limited indication or a narrower patient population than we originally requested or may impose other prescribing limitations or warnings that limit the product's commercial potential. We have not submitted for, or obtained, regulatory approval for any product candidate, and it is possible that none of our product candidates will ever obtain regulatory approval. Further, development of our product candidates and/or regulatory approval may be delayed for reasons beyond our control. We cannot provide any assurance that any product candidate we may develop will progress through required clinical testing and obtain the regulatory approvals necessary for us to begin selling them.

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

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

This lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, results of operations and prospects. Any delay or failure in seeking or obtaining required approvals would have a material and adverse effect on our ability to generate revenue from any particular product candidates we are developing and for which we are seeking approval. Furthermore, any regulatory approval to market a drug may be subject to significant limitations on the approved uses or indications for which we may market, promote and advertise the drug or the labeling or other restrictions. In addition, the FDA has the authority to require a Risk Evaluation and Mitigation Strategy (REMS) plan as part of approving an NDA, or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug. These requirements or restrictions might include limiting prescribing to certain physicians or medical centers that have undergone

specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. These limitations and restrictions may significantly limit the size of the market for the drug and affect reimbursement by third-party payors.

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

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

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

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

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction. For example, even if the FDA or EMA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion and reimbursement of the product candidate in those countries. Complying with new requirements and changes in other foreign regulations that apply to clinical trials and drug development activities can delay our clinical trials and regulatory approval timelines in the EU and other foreign jurisdictions. For example, the Clinical Trials Regulation EU No. 536/2014 entered into application on January 31, 2022 and is intended to simplify the current rules for clinical trial authorization and standards of performance in EU. Complying with such new legislation or changes in healthcare regulation could be time-intensive and expensive, resulting in a material adverse effect on our business.

However, a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

Obtaining foreign regulatory approvals and establishing and maintaining compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we or any future collaborator fail to comply with the regulatory requirements in international markets or fail to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our potential product candidates will be harmed.

The regulatory approval processes for product candidates that target rare diseases, including Duchenne, Becker and LGMD are uncertain.

Due to the lack of precedent, broad discretion of regulatory authorities, and a multitude of unique factors that impact the regulatory approval process, the likelihood of the approval of any of our product candidates that target rare diseases, such as Duchenne, Becker and LGMD is uncertain, and we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned Investigational New Drug (IND) and NDA for our product candidates, in a timely manner, or at all. For example, Duchenne is a rare disease for which there are only two FDA approved therapeutics. In addition, no therapies are currently approved for Becker in the United States or the EU. Further, the FDA may determine, after evaluation of our data and analyses, that such data and analyses do not support an NDA submission, filing or approval. Due to this lack of predictability, we may not have the resources necessary to meet regulatory requirements and successfully complete a potentially protracted, expensive and wide-ranging approval process for commercialization of product candidates for rare diseases.

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

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

- delays in or the rejection of product approvals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on the products, manufacturers or manufacturing process;
- warning or untitled letters;
- civil and criminal penalties;
- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions or import bans;
- voluntary or mandatory product recalls and publicity requirements;

- total or partial suspension of production; and
- imposition of restrictions on operations, including costly new manufacturing requirements.

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

Further, the FDA's or other ex-U.S. regulators' policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. Recently, 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. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action. 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, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

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

Regulatory authorities in some jurisdictions, including the United States and the 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 United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. However, there can be no assurances that we will be able to obtain orphan designations for our product candidates.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the United States provides that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances. The applicable exclusivity period is 10 years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

The FDA granted sevasemten Fast Track designation for the treatment of Duchenne in February 2024, and ODD for the treatment of Duchenne and Becker and RPDD for the treatment of Duchenne in November 2023. The FDA previously granted Fast Track designation for the investigation and development of sevasemten for the treatment of Becker. EMA granted ODD for sevasemten for the treatment of Becker and Duchenne in April 2024. We may seek orphan drug designation for other product candidates. Even after obtaining orphan drug designation, we may not be able to obtain or maintain orphan drug exclusivity for that product candidate. We may not be the first to obtain marketing approval of any product candidate for which we have obtained orphan drug designation for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for

designation was materially defective or if we are unable to ensure that we will be able to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even after obtaining 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. In view of the court decision in *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021), in January 2023, the FDA published a notice in the Federal Register to clarify that while the agency complies with the court's order in *Catalyst*, FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the *Catalyst* order – that is, the agency will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, which permits other sponsors to obtain approval of a drug for new uses or indications within the same orphan designated disease or condition that have not yet been approved. It is unclear how future litigation, legislation, agency decisions, and administrative actions will impact the scope of the orphan drug exclusivity.

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

Where possible, we plan to pursue accelerated development strategies in areas of high unmet need. We may seek an accelerated approval pathway for our one or more of our product candidates. Under the accelerated approval provisions in the Federal Food, Drug, and Cosmetic Act, and the FDA's implementing regulations, the FDA may grant accelerated approval to a product candidate designed to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. If such post-approval studies fail to confirm the drug's clinical benefit, the FDA may withdraw its approval of the drug. The Food and Drug Omnibus Reform Act reformed the accelerated approval pathway, such as requiring the FDA to specify conditions for post-approval study requirements and setting forth procedures for the FDA to withdraw a product on an expedited basis for non-compliance with post-approval requirements.

Prior to seeking such accelerated approval, we will seek feedback from the FDA and will otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that after subsequent FDA feedback we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for

accelerated approval or under another expedited regulatory designation (e.g., breakthrough therapy designation), there can be no assurance that such submission or application will be accepted or that any expedited development, review or approval will be granted on a timely basis, or at all. The FDA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our product candidate would result in a longer time period to commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

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

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

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the current U.S. administration may impact our business and industry, which could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how current and future legislation, executive actions, and litigation, including the executive orders referenced below, will be implemented, and the extent to which they will impact our business, our clinical development, and the FDA's and other agencies' ability to exercise their regulatory authority, including FDA's pre-approval inspection and timely review of any regulatory filings or applications we submit to the FDA. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course or constraints on our business operations, including operations of our contractors, our business may be negatively impacted.

For example, in March 2010, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the ACA), was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and continues to significantly impact the U.S. pharmaceutical industry. Since its enactment, there have been judicial and congressional challenges to certain aspects of the ACA. In June 2021, the United States Supreme Court held that Texas and other challengers had no legal standing to challenge the ACA, dismissing the case without specifically ruling on the constitutionality of the ACA. Accordingly, the ACA remains in effect in its current form. It is unclear how this Supreme Court decision, future litigation, or healthcare measures promulgated by the Biden administration will impact our business, financial condition and results of operations. On January 28, 2021, President Biden issued an executive order to initiate a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. Complying with any new legislation or changes in healthcare regulation could be time-intensive and expensive, resulting in a material adverse effect on our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, effective April 1, 2013, which will remain in effect through 2032, unless additional congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in

additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and accordingly, our financial operations.

Moreover, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which has resulted in several congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, the American Rescue Plan Act of 2021 eliminated the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs. Elimination of this cap may require pharmaceutical manufacturers to pay more in rebates than it receives on the sale of products, which could have a material impact on our business. In July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at increasing competition for prescription drugs. In August 2022, Congress passed the Inflation Reduction Act of 2022, 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 industry stakeholders, including pharmaceutical companies, the U.S. Chamber of Commerce, and the Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of the Inflation Reduction Act are unconstitutional. The impact of these judicial challenges as well as future judicial challenges in view of the U.S. Supreme Court's overturn of the Chevron doctrine, and other legislative, executive, and administrative actions and any future healthcare measures and agency rules implemented by the government on us and the pharmaceutical industry as a whole is unclear. The implementation of cost containment measures, including the prescription drug provisions under the Inflation Reduction Act, 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. For example, FDA recently authorized the state of Florida to import certain prescription drugs from Canada for a period of two years to help reduce drug costs, provided that Florida's Agency for Health Care Administration meets the requirements set forth by the FDA. Other states may follow Florida. We are unable to predict the future course of federal or state healthcare measures in the United States directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. These and any further changes in the law or regulatory framework that reduce our revenue or increase our costs could also have a material and adverse effect on our business, financial condition and results of operations.

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

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.

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 and regulations limiting, or laws and regulations regarding, the collection, distribution, use, disclosure, storage, security and other processing of personal information.

Additionally, the collection and use of health data and other personal data is governed in the EU by the General Data Protection Regulation (GDPR), which extends the geographical scope of EU data protection law to entities and operations outside of the EU under certain conditions and imposes substantial obligations upon companies and new rights for individuals, and by certain EU member state-level legislation. Failure to comply with the GDPR may result in fines up to €20,000,000 or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, and other administrative penalties. The GDPR has increased our responsibility and liability in relation to applicable personal data that we or our CROs and other contractors and service providers may process, and we may be required to put in place additional measures in an effort to comply with the GDPR and with other laws and regulations in the EU, including those of EU member states, relating to privacy and data protection. These efforts may require substantial efforts and incurring significant costs. If our efforts to comply with the GDPR or other applicable EU laws and regulations are not successful, or are perceived to be unsuccessful, it could adversely affect our business in the EU. Further, the Court of Justice of the European Union (CJEU) invalidated the EU-U.S. Privacy Shield, which had enabled the transfer of personal data from the EU to the U.S. for companies that had self-certified to the Privacy Shield in July 2020. The CJEU decision also raised questions about the continued validity of one of the primary alternatives to the EU-U.S. Privacy Shield, namely the European Commission's standard contractual clauses (SCCs), and EU regulators have issued additional guidance regarding considerations and requirements that we and other companies must consider and undertake when using the SCCs. EU regulators have released updated standard contractual clauses that are required to be implemented. The CJEU's decision and other regulatory guidance or developments otherwise may impose additional obligations with respect to the transfer of personal data from the EU, United Kingdom (UK) and Switzerland to the U.S., and we may be required to engage in additional contractual negotiations relating to the new SCCs or otherwise, each of which could restrict our activities in those jurisdictions, limit our ability to provide our products and services in those jurisdictions, or increase our costs and obligations and impose limitations upon our ability to efficiently transfer personal data from the EU, UK and Switzerland to the U.S.

Further, the exit of the UK from the EU has created uncertainty with regard to data protection regulation in the UK. The UK has implemented legislation similar to the GDPR, referred to as the UK GDPR, which provides for fines of up to the greater of £17.5 million or 4% of global turnover. On June 28, 2021, the European Commission issued an adequacy decision in respect of the UK's data protection framework, allowing personal data transfers from EU member states to the UK to continue without requiring additional contractual or other measures in order to lawfully transfer personal data between the territories. This decision is subject to renewal after four years, however, and may be revisited by the European Commission at any time. In the medium and longer terms, however, the relationship between the UK and EU in relation to aspects of data protection law remains unclear, which exposes us to further compliance risk. The UK also has issued its own standard contractual clauses that are required to be implemented. We may incur liabilities, expenses, costs, and other operational losses relating to the GDPR, the UK GDPR, and other laws and regulations in the EU and UK relating to privacy and data protection, including those of applicable EU member states in connection with any measures we take to comply with them.

In the United States, a broad variety of data protection laws and regulations may apply to our activities such as state data breach notification laws, state personal data privacy laws (for example, the California Consumer Privacy Act of 2018 (CCPA)), state health information privacy laws, and federal and state consumer protection laws. A range of enforcement agencies exist at both the state and federal levels that can enforce these laws and regulations. For example, the CCPA requires covered businesses that process personal information of California residents to disclose their data collection, use and sharing practices. Further, the CCPA provides California residents with certain data privacy rights (including the ability to opt out of certain disclosures of personal data),

imposes operational requirements for covered businesses, provides for civil penalties for violations as well as a private right of action for data breaches and statutory damages (that is expected to increase data breach class action litigation and result in significant exposure to costly legal judgements and settlements). Aspects of the CCPA and its interpretation and enforcement remain uncertain. In addition, the CCPA was expanded on January 1, 2023, when the California Privacy Rights Act of 2020 (CPRA) became operative. The CPRA, among other things, gives California residents the ability to limit use of certain sensitive personal information, establishes restrictions on the retention of personal information, expand the types of data breaches subject to the CCPA's private right of action, provides for increased penalties for CPRA violations concerning California residents under the age of 16, and establishes a new California Privacy Protection Agency to implement and enforce the new legislation. Although there are limited exemptions for clinical trial data under the CCPA, the CCPA and other similar laws could impact our business activities, depending on their interpretation. Additionally, other state legislatures have enacted or are currently contemplating, and may pass, their own data privacy and security laws, with potentially greater penalties and more rigorous compliance requirements relevant to our business. Many of these laws are comprehensive privacy statutes that impose obligations similar to the CCPA. For example, Colorado has enacted a Colorado Privacy Act (CPA) in June 2021 that went into effect on July 1, 2023, with enforcement commencing on the same date. The Colorado Attorney General released its rules implementing the CPA on March 15, 2023. Connecticut, Utah and Virginia have also enacted legislation similar to the CCPA and the CPA that have taken effect in 2023; Florida, Montana, Oregon and Texas have enacted similar legislation that has taken, or will take effect in 2024; Delaware, Iowa, Minnesota, New Hampshire, New Jersey, Nebraska and Tennessee have enacted similar legislation that will take effect in 2025; and Indiana, Kentucky and Rhode Island have enacted similar legislation that will take effect in 2026.

Additionally, state and foreign laws may apply generally to the privacy and security of information we maintain, and may differ from each other in significant ways, thus complicating compliance efforts and potentially requiring us to undertake additional measures to comply with them. With the GDPR, CCPA, CPRA, CPA and other laws, regulations and other obligations relating to privacy and data protection imposing new and relatively burdensome obligations, and with substantial uncertainty over the interpretation and application of these and other obligations, we may face challenges in addressing their requirements and in making necessary changes to our policies and practices, and may incur significant costs and expenses in an effort to do so. Additionally, if third parties we work with, such as vendors or service providers, violate applicable laws or regulations or our policies, such violations may also put our or our customers' data at risk and could in turn have an adverse effect on our business. Any failure or perceived failure by us or our 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 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. Further, other states have enacted laws that cover certain aspects of the collection, use, disclosure, and/or other processing of health information, such as Washington's My Health, My Data Act, which, among other things, provides for a private right of action.

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

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the U.S. Securities and Exchange Commission (SEC) and other government agencies on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies, including delays or disruptions due to the COVID-19 pandemic or other public health emergencies, travel restrictions, staffing shortages, may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in 2018 and 2019, the U.S. government shut down several times and certain regulatory

agencies, such as the FDA and the SEC, had to furlough critical employees and stop critical activities. If any prolonged government shutdown or disruption occurs, including due to any public health emergencies, resurgence in COVID-19 cases, travel restrictions, or COVID-19-related policies, staffing shortages, it could significantly impact the ability of the FDA and other regulatory authorities to timely review and process our regulatory submissions and provide feedback on our clinical development plans, which could have a material adverse effect on our business and our anticipated timelines. Further, future government shutdowns or disruptions to normal operations could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

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

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

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

- the federal false claims laws, including the civil False Claims Act, which can be enforced by private citizens through civil whistleblower or qui tam actions, and civil monetary penalties laws, prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), prohibits, among other things, executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH) and their implementing regulations, also imposes obligations, including mandatory contractual terms, on covered entities, which are health plans, healthcare clearinghouses, and certain health care providers, as those terms are defined by HIPAA, and their respective business associates and their subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- 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 made to covered recipients, including 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

- 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 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, suppliers and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

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

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

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

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

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

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

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

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

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 and prohibitions on the conduct of our business including our ability to offer our products in one or more countries. Responding to any investigation or action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees. As a general matter, investigations, enforcement actions and sanctions could damage our reputation, our brand, our international activities, our ability to attract and retain employees and our business, prospects, operating results and financial condition.

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

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

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

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

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

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

maintain consulting relationships with our scientific founders or if they provide services to our competitors, our development and commercialization efforts will be impaired and our business will be significantly harmed.

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

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

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

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

As of September 30, 2024, we had 108 full-time employees. Of these employees, 84 are engaged in research or product development and clinical activities. 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. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA, EMA and other comparable foreign regulatory agencies' review process for sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any other product candidates, while complying with any contractual obligations to contractors and other third parties we may have; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to successfully develop and, if approved, commercialize sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other 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 and manufacturing. 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 clinical trials may be extended, delayed or terminated, and we may not be able to obtain marketing approval of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any other product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing third-party service providers or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and/or engaging additional third-party service providers, we may not be able to successfully implement the tasks necessary to further develop and commercialize sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and other product candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

Despite the implementation of security measures in an effort to protect systems that store our information, given their size and complexity and the increasing amounts of information maintained on our internal information technology systems, and those of our third-party CROs, other contractors (including sites performing our clinical trials) and consultants, these systems are potentially vulnerable to breakdown or other damage or interruption from service interruptions, system malfunction, technical errors, natural disasters, terrorism, war and telecommunication and electrical failures, as well as security breaches and incidents from inadvertent or intentional actions by our employees, contractors, consultants, business partners, and/or other third parties, or from cyber-attacks by malicious third parties (including supply chain cyber-attacks or the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information), which may compromise our system infrastructure or availability or lead to the loss, destruction, alteration, prevention of access to, disclosure, or dissemination of, or damage or unauthorized access to, our data (including trade secrets or other confidential information, intellectual property, proprietary business information, and personal information) or data that is processed or maintained on our behalf, or other assets, which could result in financial, legal, business and reputational harm to us. For example, in 2019, one of our CROs experienced a cybersecurity breach which resulted in unauthorized access to certain of our preclinical data. Additionally, in 2023, one of our CROs experienced a cyber-attack for which an investigation found that no unauthorized access to Edgewise data occurred in connection with this event. We have received phishing attacks, and companies have, in general, experienced an increase in phishing and social engineering attacks from third parties in connection with remote working, which has increased these and other cybersecurity risks. Additionally, cybersecurity researchers have warned of heightened risks of cyberattacks in connection with Russia's war with Ukraine, and war and instability in Israel and the surrounding region. Any disruption or security incident resulting in any loss, destruction, unavailability, alteration, disclosure, disruption or dissemination of, or damage or unauthorized access to, our applications, any other data processed or maintained on our behalf or other assets, or for it to be believed or reported that any of these occurred, could cause us to incur costs, liability, and other financial harm and reputational damage and could contribute to delays in the development and commercialization of our product candidates. We cannot assure you that our data protection efforts and our investment in information technology, or the efforts or investments of CROs, consultants or other third parties, will prevent significant breakdowns in systems or will prevent, or have prevented, other cyber incidents that could disrupt our programs and operations and the development of our product candidates or result in loss, destruction, unavailability, alteration or dissemination of, or damage or unauthorized access to, our systems and data and other data processed or maintained on our behalf or other assets, any of which could have a material adverse effect upon

our reputation, business, operations or financial condition. Any such event that leads to loss, damage, or unauthorized access to, or use, alteration, or disclosure, dissemination, or other processing of, personal information, including personal information regarding our clinical trial subjects or employees, or the perception that any such event has occurred, could harm our reputation directly, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, cause us to incur costs, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages that could potentially have an adverse effect on our business.

Notifications and follow-up actions related to a security breach or incident could impact our reputation and cause us to incur significant costs, including legal expenses and remediation costs. For example, the loss, corruption or (temporary or permanent) unavailability of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the impacted data. We expect to incur significant costs in an effort to detect and prevent security breaches and incidents, and we may face increased costs and requirements to expend substantial resources in the event of an actual or perceived security breach or incident. We also rely on third parties to manufacture our product candidates, and for other purposes, and similar events relating to their infrastructure and systems could also have a material adverse effect on our business.

Our insurance policies may not be adequate to compensate us for the potential losses arising from any such disruption in or, failure or security breach or incident of or impacting our systems or third-party systems where information important to our business operations or commercial development is stored. In addition, such insurance may not be available to us in the future on economically reasonable terms, or at all. Further, our insurance may not cover all claims made against us and could have high deductibles in any event, and defending a suit, regardless of its merit, could be costly and divert management attention.

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 Boulder, Colorado. We have not undertaken a systematic analysis of the potential consequences to our business and financial results from a major flood, blizzard, fire, earthquake, power loss, terrorist activity, pandemics or other disasters and do not have a recovery plan for such disasters. In addition, we do not carry sufficient insurance to compensate us for actual losses from interruption of our business that may occur, and any losses or damages incurred by us could harm our business. Also, our contract development and manufacturing organizations' (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.

Our business may become subject to economic, political, regulatory and other risks associated with international operations directly or indirectly. A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.

Our business is subject to risks associated with business operations we conduct internationally, as well as indirect impacts from our relationships with collaborators, partners, or contractors who conduct business internationally. We may seek regulatory approval of our product candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements and reimbursement regimes in foreign countries, including changes in existing regulatory requirements and implementation of new regulatory requirements or policies that impact our clinical development and business operations in foreign countries;

- foreign regulatory authorities may disagree with the design, implementation or results of our clinical trials or our interpretation of data from preclinical studies or clinical trials;
- approval policies or regulations of foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, change in political condition, including as a result of the upcoming change in the U.S. presidential administration, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the FCPA or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- impact of the COVID-19 pandemic or other public health concerns on our ability to produce our product candidates and conduct clinical trials in foreign countries;
- production or supply shortages or other disruptions resulting from any events affecting raw material supply or manufacturing capabilities abroad, including, but not limited to, impacts due to the ongoing Ukraine-Russia war, addition of certain suppliers or companies to the Unverified List or other export restrictions under the Export Administration Regulations, implementation of other export controls, restrictions or sanctions, including impact of the upcoming change in the U.S. presidential administration, that can impact the supply chain, our business, or business operations of our suppliers, contractors or partners; and
- business interruptions resulting from geo-political actions, including war, such as the ongoing war in Ukraine and war and instability in Israel and the surrounding region, other regional or geo-political conflicts, and terrorism.

In particular, there is currently significant uncertainty about the future relationship between the United States and various other countries, most significantly China, with respect to trade policies, treaties, tariffs, taxes, and other limitations on cross-border operations. The U.S. government has and continues to make significant additional changes in U.S. trade policy and may continue to take future actions, including the impact of the upcoming change in the U.S. presidential administration, that could negatively impact U.S. trade. For example, legislation has been introduced in Congress to limit 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. 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.

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

Inflation in the global economy could negatively impact our business and results of operations.

General inflation in the United States, Europe and other geographies has risen to levels not experienced in recent decades. General inflation, including rising prices for our trial drug supply, CROs, CDMOs and rising salaries negatively impact our business by increasing our operating expenses. To the extent general inflation results in rising interest rates and has other adverse effects on the market, it may continue to adversely affect our business, financial condition and results of operations.

Risks Related to Our Intellectual Property

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

Our commercial success depends in part on our ability to obtain and maintain patent protection and trade secret protection for our product candidates, proprietary technologies and their uses as well as our ability to operate without infringing upon the proprietary rights of others. We generally seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates, proprietary technologies and their uses that are important to our business. We also seek to protect our proprietary position by acquiring or in-licensing relevant issued patents or pending applications from third parties.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issue from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our patent applications or the patent applications of any licensor will result in additional patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents issued will not be infringed, designed around or invalidated by third parties.

Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. The degree of future protection for our and any licensor's 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/or limitations in our ability to properly protect the intellectual property rights relating to our product candidates could have a material adverse effect on our financial condition and results of operations.

We cannot be certain that the claims in our U.S. pending patent applications, corresponding international patent applications and patent applications in certain foreign territories, or that of any licensor, will be considered patentable by the United States Patent and Trademark Office (USPTO), courts in the United States or by the patent offices and courts in foreign countries, nor can we be certain that issued claims will not be found invalid or unenforceable if challenged.

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 collaborators will be successful in protecting our product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance

with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;

- patent applications may not result in any patents being issued;
- patents may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable 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.

The patent prosecution process is also expensive and time-consuming, and we and any licensor may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner or in all jurisdictions where protection may be commercially advantageous. It is also possible that we or any licensor will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

In addition, although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

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 biopharmaceutical 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 any licensor may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive product candidates.

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. Any patents that we own or in-license may be challenged or circumvented by third parties or may be narrowed or invalidated as a result of challenges by third parties. Consequently, we do not know whether our

product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents or the patents of any licensors by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

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

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

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

- others may be able to develop products that are similar to our product candidates but that are not covered by the claims of the patents that we own or license;
- we or any licensor or collaborators might not have been the first to make the inventions covered by the patent applications that we own or license;
- we or any licensor or collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that the pending patent applications we own or license will not lead to issued patents;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and

- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third-party may subsequently file a patent covering such intellectual property.

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

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and products that may be approved in the future, or impair our competitive position. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biopharmaceutical industry, including patent infringement lawsuits, oppositions, reexaminations, IPR proceedings and PGR proceedings before the USPTO and/or corresponding foreign patent offices. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates.

There are numerous U.S. and foreign issued patents and pending patent applications owned by third-parties in the fields in which we are developing our product candidates. Because patent applications are maintained as confidential for a certain period of time, until the relevant application is published, we may be unaware of third-party patents that may be infringed by commercialization of any of our product candidates, and we cannot be certain that we were the first to file a patent application related to a product candidate or technology. Moreover, because patent applications can take many years to issue, there may be currently-pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. There is also no assurance that there is not prior art of which we are aware, but which we do not believe is relevant to our business, which may, nonetheless, ultimately be found to limit our ability to make, use, sell, offer for sale or import our products that may be approved in the future, or impair our competitive position. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation that may cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing any of our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- subject us to significant liability to third parties; or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

Although no third-party has asserted a claim of patent infringement against us as of the date of this periodic report, others may hold proprietary rights that could prevent our product candidates from being marketed. For example, we are aware of an issued patent that claims a method of treatment based upon a general mode of action. These claims could be alleged to cover sevasemten in certain treatment indications. While we believe that these patents are difficult to enforce and that we would have valid defenses to these claims of patent infringement, we cannot be certain that we would prevail in any dispute and we cannot be certain how an adverse determination would affect our business.

It is possible that a third party may assert a claim of patent infringement directed at any of our product candidates. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our products, treatment indications, or processes could subject us to significant liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Moreover, even if we or our future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. In addition, we cannot be certain that we could redesign our product candidates, treatment indications, or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates, which could harm our business, financial condition and operating results. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

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.

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

Many pharmaceutical companies, biotechnology companies, and academic institutions may have patents and patent applications potentially relevant to our business. We may find it necessary or prudent to obtain licenses to such patents from such third-party intellectual property holders, for example, in order to avoid infringing these third-party patents. We may also require licenses from third parties for certain technologies for use with future product candidates. We may be unable to acquire or in-license any compositions, methods of use, processes or

other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We may be involved in lawsuits to protect or enforce our patents or any licensor's patents, which could be expensive, time consuming and unsuccessful. Further, our issued patents or any licensor's patents could be found invalid or unenforceable if challenged in court.

Competitors may infringe our intellectual property rights. To prevent infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in a patent infringement proceeding, a court may decide that a patent we own or in-license is not valid, is unenforceable and/or is not infringed. If we or any of our potential future collaborators were to initiate legal proceedings against a third-party to enforce a patent directed at one of our product candidates, the defendant could counterclaim that our patent or the patent of any licensor is invalid and/or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of sufficient written description, non-enablement, or obviousness-type double patenting. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution.

Third parties may also raise similar invalidity claims before the USPTO or patent offices abroad, even outside the context of litigation. Such mechanisms include re-examination, PGR, IPR, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). The outcome following legal assertions of invalidity and/or unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, any licensor, and the patent examiners are unaware during prosecution. There is also no assurance that there is not prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim in our patents and patent applications or the patents and patent applications of any licensor, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. If a third-party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or proprietary drug discovery platform, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

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

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

compromise our ability to compete in the marketplace. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings.

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

In Europe, as of June 1, 2023, European applications and patents may be subjected to the jurisdiction of the Unified Patent Court (UPC). Also, European applications now have the option, upon grant of a patent, of becoming a Unitary Patent which is subject to the jurisdiction of the UPC. This may be a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. As a single court system can invalidate a European patent, we, where applicable may opt out of the UPC, and as such, each European patent would need to be challenged in each individual country.

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

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

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

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

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

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

For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this

combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the U.S. federal courts, the USPTO, or similar authorities in foreign jurisdictions, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patent and the patents we might obtain or license in the future.

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

We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Such an outcome could 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.

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

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

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

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

Filing, prosecuting and defending patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may

use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our product candidates, and our patents, the patents of any licensors, or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or any future licensors' patents or marketing of competing products in violation of our proprietary rights. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents or the patents of any licensors at risk of being invalidated or interpreted narrowly and our patent applications or the patent applications of any licensor at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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.

Geo-political actions in the United States 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. For example, the United States and foreign government actions related to Russia's invasion of Ukraine, which could be subject to change as a result of the upcoming change in the U.S. presidential administration, may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

In Europe, as of June 1, 2023, European applications and patents may be subjected to the jurisdiction of the Unified Patent Court (UPC). Also, European applications now have the option, upon grant of a patent, of becoming a Unitary Patent which is subject to the jurisdiction of the UPC. This may be a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. As a single court system can invalidate a European patent, we, where applicable may opt out of the UPC, and as such, each European patent would need to be challenged in each individual country.

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 applications will be due to the USPTO and various foreign patent offices at various points over the lifetime of our

patents and/or applications and those of any licensors. We have systems in place to remind us to pay these fees, and we rely on our outside patent annuity service to pay these fees when due. Additionally, the USPTO and various foreign patent offices require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can 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.

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. Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights 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, we 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, and confidential information and inventions agreements with employees, consultants and advisors, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced, and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

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

We have entered into and may enter in the future into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, lessees of shared multi-company property and other third parties. We may become subject to litigation where a third-party asserts that we or our employees inadvertently or otherwise

breached the agreements and used or disclosed trade secrets or other information proprietary to the third parties. Defense of such matters, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions. Moreover, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology. Failure to defend against any such claim could subject us to significant liability for monetary damages or prevent or delay our developmental and commercialization efforts, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Parties making claims against us may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, operating results, financial condition and prospects.

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

As is common in the 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 pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

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

We may enter into license agreements in the future to advance our research or allow commercialization of product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

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

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

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

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

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

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patents and other rights to third parties;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- our right to transfer or assign the license;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by any licensors and us and our partners; and
- the priority of invention of patented technology.

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

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

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

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

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

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

Risks Related to Our Dependence on Third Parties

We rely, and expect to continue to rely, on third parties to conduct our clinical trials and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research and studies, which may harm our business.

We do not have the ability to independently conduct our clinical trials. We currently rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our current and planned clinical trials of sevasemten and EDG-7500 and we expect to continue to rely upon third parties to conduct additional clinical trials for sevasemten, EDG-7500 and other product candidates. Third parties have a significant role in the conduct of our clinical trials and the subsequent collection and analysis of data. These third parties are not our employees, and except for remedies available to us under our agreements with such third

parties, we have limited ability to control the amount or timing of resources that any such third-party will devote to our 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. Some of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements with a third-party, it would delay our drug development activities.

Our reliance on these third parties for such drug development activities will reduce our control over these activities but will not relieve us of our regulatory responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCP standards, regulations for conducting, recording and reporting the results of clinical trials to assure that data and reported results are reliable and accurate and that the rights, integrity and confidentiality of trial participants are protected. The EMA also requires us to comply with similar standards. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials substantially comply with GCP regulations. In addition, our clinical trials must be conducted with product produced under current cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the marketing approval process.

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

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

We contract with third parties for the production of sevasemten and EDG-7500 for our ongoing clinical trials and the production of product candidates from our EDG-003 cardiometabolic discovery program for our ongoing preclinical studies, and expect to continue to do so for additional clinical trials, preclinical studies and ultimately for commercialization. This reliance on third parties increases the risk that we will not have sufficient quality and quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not currently have the infrastructure or internal capability to manufacture supplies of our product candidates for use in development and commercialization. We rely, and expect to continue to rely, on third-party manufacturers for the production of our product candidates for preclinical studies and clinical trials under the guidance of members of our organization. We will be relying on a single third-party manufacturer and we currently have no alternative manufacturer in place. Changing our third-party manufacturer could result in delays in our manufacturing supply chain which could delay or otherwise impact our development of sevasemten, EDG-7500, and product candidates from our EDG-003 cardiometabolic discovery program and result in increased costs related to sevasemten, EDG-7500, and product candidates from our EDG-003 cardiometabolic discovery program. We do not have long-term supply agreements, and we purchase our required drug product on a purchase order basis, which means that aside from any binding purchase orders we have from time to time, our supplier could cease supplying to us or change the terms on which it is willing to continue supplying to us at any time. If we were to experience an unexpected loss of supply of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program or any other 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 clinical trials and preclinical studies.

We expect to continue to rely on third-party manufacturers for the commercial supply of any of our product candidates for which we obtain marketing approval. We may be unable to maintain or establish required agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

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

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

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

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

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

Because we currently rely on third parties in the course of our business, we may share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements, and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are intentionally or inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets and despite our efforts to protect our trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

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

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

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and marketing approvals; and
- 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.

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

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

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We may seek to selectively form collaborations to expand our capabilities, potentially accelerate research and development activities and provide for commercialization activities by third parties. In addition, we intend to explore strategic partnering and collaboration opportunities to out-license rights to our research programs and drug candidates for indications in which we are unlikely to pursue development and commercialization. In parallel, we will also evaluate select external opportunities to strategically expand our portfolio. Any of these relationships may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business.

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

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

If and when we seek to enter into collaborations, we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to, and the manner in which they perform their obligations under, these collaborations and may not perform their obligations as expected;

- collaborators may deemphasize or not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus, including as a result of a business combination or sale or disposition of a business unit or development function, or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- a collaborator with marketing and distribution rights to multiple products may not commit sufficient resources to the marketing and distribution of our product relative to other products;

- we may grant exclusive rights to our collaborators that would prevent us from collaborating with others;

- collaborators may not properly obtain, maintain, defend 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.

We also collaborate with a network of experts who advise and support our development efforts. In the future, such experts may not collaborate with us which could affect our ability to develop our product candidates and proprietary drug discovery platform as such experts potentially provide us with access to ideas to address the needs of muscle diseases.

Risks Related to the Securities Markets and Ownership of Our Common Stock

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

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

The price of our stock has been and may continue to be volatile, and you could lose all or part of your investment.

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

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

- the timing and results of preclinical studies and clinical trials of our product candidates, those conducted by third parties or those of our competitors;
- the success of competitive products or announcements by potential competitors of their product development efforts;
- regulatory actions with respect to our products or our competitors' products;
- actual or anticipated changes in our growth rate relative to our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, collaborations or capital commitments;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- market conditions in the pharmaceutical and biotechnology sector;
- changes in the structure of healthcare payment systems;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;

- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- the impact of any natural disasters or public health emergencies, such as the COVID-19 pandemic; and
- general economic, political, industry and market conditions including the impact of the change in the U.S. presidential administration and the impact of increasing inflation.

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

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

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

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

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

- the timing and cost of, and level of investment in, research and development activities relating to our current product candidates and any future product candidates and research-stage programs, which will change from time to time;
- our ability to enroll patients in clinical trials and the timing of enrollment;
- the cost of manufacturing our current product candidates and any future product candidates, which may vary depending on FDA, EMA or other comparable foreign regulatory authority guidelines and requirements, the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies or other assets;
- the timing and outcomes of clinical trials or preclinical studies (as applicable) for sevasemten, EDG-7500, and our EDG-003 cardiometabolic discovery program and any of our other product candidates, or competing product candidates;
- the need to conduct unanticipated clinical trials or trials that are larger or more complex than anticipated;
- competition from existing and potential future products that compete with sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any of our other product candidates

or programs, and changes in the competitive landscape of our industry, including consolidation among our competitors or partners;

- any delays in regulatory review or approval of sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program or any of our other product candidates;
- the level of demand for sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any of our other product candidates, if approved, which may fluctuate significantly and be difficult to predict;
- the risk/benefit profile, cost and reimbursement policies with respect to our product candidates, if approved, and existing and potential future products that compete with sevasemten and any of our other product candidates;
- our ability to commercialize sevasemten, EDG-7500, product candidates from our EDG-003 cardiometabolic discovery program and any of our other product candidates, if approved, inside and outside of the United States, either independently or working with third parties;
- our ability to establish and maintain collaborations, licensing or other arrangements;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies;
- the changing and volatile global economic and political environment, including as a result of the upcoming change in the U.S. presidential administration; and
- increased impact from COVID-19 on the costs and timing associated with the conduct of our clinical trial and other related business activities.

The cumulative effect of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated guidance we may provide.

Our affiliated 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, 2024, our executive officers, directors, affiliated holders of 5% or more of our capital stock and their respective affiliates beneficially owned approximately 22.7% of our outstanding common stock. These stockholders, acting together, may be able to control matters requiring stockholder approval. For example, they may be able to control elections of directors, amendments of our organizational documents or approval of any merger, sale of assets or other major corporate transactions. This concentration of ownership control may delay, discourage or prevent a change of control, including unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders, entrench our management and board of directors or delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire. The interests of this group of stockholders may not always coincide with your

interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. On May 10, 2024, we filed an automatic shelf registration statement on Form S-3ASR that allows us to undertake various equity and debt offerings and entered into the Leerink Sales Agreement under which we may offer and sell shares of common stock, having aggregate sales proceeds of up to $175,000,000 from time to time, through the Leerink ATM.

Moreover, certain holders of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Registration of these shares, under the Securities Act, would result in the shares becoming freely tradeable in the public market, subject to the restrictions of Rule 144 in the case of our affiliates. In addition, shares registered under Form S-8 to register shares of our common stock reserved for issuance under our equity compensation plans become available for sale in the public market subject to the satisfaction of applicable vesting arrangements and the exercise of such options and, in the case of our affiliates, the restrictions of Rule 144. If any of these shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amount of our common stock in the public market, the market price of our common stock could decline significantly.

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

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

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

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to avail ourselves of this exemption from new or revised accounting standards and, therefore, will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, our financial

statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We will remain an emerging growth company and a smaller reporting company until December 31, 2024. We will transition to a "large accelerated filer" status with effect as of January 1, 2025, because the aggregate market value of our common stock held by non-affiliates exceeded $700 million as of June 30, 2024. As a large accelerated filer, we will have to provide more expansive disclosure regarding executive compensation in our periodic reports and be subject to shorter filing deadlines, which will require additional time and expense. We will also be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act.

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

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

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

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an "emerging growth company." We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and the Nasdaq Stock Market LLC (Nasdaq). Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have substantially increased our legal and financial compliance costs and makes some activities more time-consuming and costly, which has increased our operating expenses. For example, these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance and we have been required to incur substantial costs to maintain sufficient coverage, particularly in light of recent cost increases related to coverage. We cannot accurately predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

In addition, we are required to incur additional costs and obligations in order to comply with SEC rules that implement Section 404 of the Sarbanes-Oxley Act. We are required to make a formal assessment of the effectiveness of our internal control over financial reporting. Additionally, as a result of our ceasing to be an emerging growth company and being deemed a large accelerated filer as of January 1, 2025, commencing with our Annual Report on Form 10-K for the year ending December 31, 2024, we will be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we have engaged 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 engage outside consultants and adopt a detailed work plan to assess and document the adequacy of our internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are designed and operating

effectively, and implement a continuous reporting and improvement process for internal control over financial reporting.

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

Our internal control over financial reporting will not prevent or detect all errors and all fraud. 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, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

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

We are subject to the periodic reporting requirements of the Exchange Act. We designed our disclosure controls and procedures to reasonably assure that information we must disclose in reports we file or submit under the Exchange Act is accumulated and communicated to management, and recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well-conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

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

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

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

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

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

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

Our amended and restated certificate of incorporation and amended and restated bylaws contains provisions that could depress the market price of our common stock by acting to discourage, delay or prevent a change in control of our company or changes in our management that the stockholders of our company may deem advantageous. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. These provisions, among other things:

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

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

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

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America are the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

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

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of fiduciary duty;
- any action asserting a claim against us arising under the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

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

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

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

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

Unregistered Sales of Equity Securities

None.

Use of Proceeds from Public Offering of Common Stock

On March 25, 2021, our registration statement on Form S-1 was declared effective by the SEC for our initial public offering of common stock. We began trading on the Nasdaq Global Select Market on March 26, 2021 and the IPO formally closed on March 30, 2021. In connection with our IPO, we issued and sold an aggregate of 12,650,000 shares of our common stock at a price of $16.00 per share, including 1,650,000 shares of our common stock issued and sold in connection with the full exercise by the underwriters of their option to purchase additional shares of common stock. The gross proceeds from the offering for shares sold in our IPO was $202.4 million. The joint book-running managers for the

initial public offering were J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and SVB Leerink LLC. After deducting underwriting discounts and commissions and offering costs paid or payable by us of approximately $16.3 million, the net proceeds from the offering were approximately $186.1 million. No payments were made by us to directors, officers or persons owning ten percent or more of our common stock or to their associates, or to our affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors pursuant to our director compensation policy.

There has been no material change in the planned use of proceeds from our IPO as described in our final prospectus filed with the SEC on March 26, 2021 pursuant to Rule 424(b)(4). We invested the funds received in marketable securities as described in more detail in Note 2 to our financial statements appearing in this Quarterly Report until such time the funds are required for use in operations.

Use of Proceeds from Follow-On Offering

On September 16, 2022, we completed a follow-on offering and issued 13,372,093 shares of our common stock at a price to the public of $10.32 per share, including 1,744,186 shares of common stock issued in connection with the full exercise by the underwriters of their options to purchase additional shares of common stock. The aggregate gross proceeds from the follow-on offering were $138.0 million. After deducting underwriting discounts and commissions of $8.3 million and offering costs of $0.5 million, the net proceeds from the follow-on offering were approximately $129.2 million.

There has been no material change in the planned use of proceeds in our follow-on offering as described in our final prospectus filed with the SEC on September 14, 2022 pursuant to Rule 424(b)(5). We invested the funds received in interest-bearing investment-grade securities.

Use of Proceeds from the ATM Program

On June 16, 2023, we entered into a Sales Agreement with BofA Securities under which we may offer and sell shares of common stock, having aggregate sales proceeds of up to $125,000,000 from time to time, through an ATM Program. On January 19, 2024, we filed a prospectus supplement to suspend the ATM Program. Through the suspension of the ATM program, we sold 7,560,068 shares of common stock at a weighted average price of $7.93 per share. The gross proceeds were $59.9 million, and the net proceeds were $59.4 million after deducting underwriting discounts and commissions of $0.2 million and offering expenses of $0.3 million.

There has been no material change in the planned use of proceeds from the ATM Program as described in our final prospectus filed with the SEC on June 16, 2023 pursuant to Rule 424(b)(5). We invested the funds received in interest-bearing investment-grade securities.

Use of Proceeds from Underwritten Registered Direct Offering

On January 23, 2024, we closed an underwritten registered direct offering of 21,818,182 shares of common stock at a public offering price of $11.00 per share. The aggregate gross proceeds from the underwritten registered direct offering were $240.0 million, and the net proceeds were $231.9 million after deducting underwriting discounts and commissions of $7.5 million and offering expenses of $0.6 million.

There has been no material change in the planned use of proceeds in our January 2024 Offering as described in our final prospectus filed with the SEC on January 19, 2024 pursuant to Rule 424(b)(5). We invested the funds received in interest-bearing investment-grade securities.

Repurchase of our Common Stock

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Director and Officer Trading Arrangements

A portion of the compensation of our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended, or the Exchange Act), is in the form of equity awards and, from time to time, directors and officers engage in open-market transactions with respect to the securities acquired pursuant to such equity awards or other securities of ours, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in our securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in our securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

The following table describes, for the third quarter of 2024, each trading arrangement for the sale or purchase of our securities adopted by our directors and officers that is either (1) a contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c), which we refer to as a "Rule 10b5-1 trading arrangement" or (2) a "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K):

Name (Title)	Action Taken (Date of Action)	Type of trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
Kevin Koch (Chief Executive Officer & Director)	Adoption (September 30, 2024)	Rule 10b5-1 trading arrangement	Sale	(1)	(1)
Alan Russell (Chief Scientific Officer & Director)	Adoption (September 30, 2024)	Rule 10b5-1 trading arrangement	Sale	(2)	(2)
R. Michael Carruthers (Chief Financial Officer)	Adoption (September 27, 2024)	Rule 10b5-1 trading arrangement	Sale	(3)	(3)
Behrad Derakhshan (Chief Business Officer	Adoption (September 30, 2024)	Rule 10b5-1 trading arrangement	Sale	(4)	(4)
Joanne Donovan (Chief Medical Officer)	Adoption (September 30, 2024)	Rule 10b5-1 trading arrangement	Sale	(5)	(5)
John Moore (General Counsel)	Adoption (September 30, 2024)	Rule 10b5-1 trading arrangement	Sale	(6)	(6)
Marc Semigran (Chief Development Officer)	Adoption (September 24, 2024)	Rule 10b5-1 trading arrangement	Sale	(7)	(7)

(1) This trading plan has a scheduled expiration date of September 30, 2025. The number of shares of our common stock to be sold under the plan is up to 169,241 shares.
(2) This trading plan has a scheduled expiration date of September 30, 2025. The number of shares of our common stock to be sold under the plan is up to 300,000 shares.
(3) This trading plan has a scheduled expiration date of January 31, 2026. The number of shares of our common stock to be sold under the plan is up to 75,000 shares.
(4) This trading plan has a scheduled expiration date of January 5, 2026. The number of shares of our common stock to be sold under the plan is up to 40,000 shares.
(5) This trading plan has a scheduled expiration date of December 31, 2025. The number of shares of our common stock to be sold under the plan is up to 81,735 shares.

(6) This trading plan has a scheduled expiration date of December 31, 2025. The number of shares of our common stock to be sold under the plan is up to 50,000 shares.
(7) This trading plan has a scheduled expiration date of December 31, 2025. The number of shares of our common stock to be sold under the plan is up to 75,000 shares.

Item 6. Exhibits

See Exhibit Index.

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference			
		Form	File No.	Exhibit	Filing Date
10.1	Edgewise Therapeutics, Inc. 2024 Inducement Equity Incentive Plan and related forms of stock option and restricted stock unit agreements.	8-K	001-40236	10.1	August 12, 2024
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.	Filed herewith			
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 14d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	Filed herewith			
32.1†	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	Furnished herewith			
32.2†	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	Furnished herewith			
101.INS	Inline XBRL Instance Document	Filed herewith			
101.SCH	Inline XBRL Taxonomy Extension Schema Document	Filed herewith			
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document	Filed herewith			
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	Filed herewith			
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	Filed herewith			
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	Filed herewith			
104	The cover page for the Company's Quarterly Report on Form 10-Q has been formatted in Inline XBRL and contained in Exhibit 101	Filed herewith			

^ Indicates management contract or compensation plan

† The certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of 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, duly authorized.

Date: November 7, 2024

EDGEWISE THERAPEUTICS, INC.

By: /s/ Kevin Koch
Name: Kevin Koch
Title: President, Chief Executive Officer and Director
(Principal Executive Officer)

By: /s/ R. Michael Carruthers
Name: R. Michael Carruthers
Title: 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, Kevin Koch, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Edgewise 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 7, 2024

EDGEWISE THERAPEUTICS, INC.

By: /s/ Kevin Koch
Name: Kevin Koch
Title: President, 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, R. Michael Carruthers, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Edgewise 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 7, 2024

EDGEWISE THERAPEUTICS, INC.

By: /s/ R. Michael Carruthers
Name: R. Michael Carruthers
Title: Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.2

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

I, R. Michael Carruthers, 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 Edgewise Therapeutics, Inc. for the quarterly period ended September 30, 2024, 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 Edgewise Therapeutics, Inc.

/s/ R. Michael Carruthers

R. Michael Carruthers

Chief Financial Officer
(Principal Financial and Accounting Officer)

Date: November 7, 2024

Exhibit 32.1

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

I, Kevin Koch, 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 Edgewise Therapeutics, Inc. for the quarterly period ended September 30, 2024, 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 Edgewise Therapeutics, Inc.

/s/ Kevin Koch

Kevin Koch

Chief Executive Officer and Director
(Principal Executive Officer)

Date: November 7, 2024