UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

OR

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

Commission File Number: 001-38533

EIDOS THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**46-3733671**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
101 Montgomery Street, Suite 2550 San Francisco, CA	**94104**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (415) 887-1471

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

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

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

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

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

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

As of November 3, 2018, the registrant had 36,747,182 shares of common stock, $0.001 par value per share, outstanding.

Table of Contents

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

EIDOS THERAPEUTICS, INC.
Condensed Balance Sheets

(Unaudited)
(In thousands, except share and per share data)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 166,568	$ 5,497
Related party receivable	29	67
Prepaid expenses and other current assets	3,248	484
Total current assets	169,845	6,048
Property and equipment, net	218	114
Other assets	163	181
Total assets	$ 170,226	$ 6,343
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,417	$ 566
Related party payable	206	372
Accrued expenses and other current liabilities	3,427	1,300
Total current liabilities	6,050	2,238
Other liabilities	357	273
Total liabilities	6,407	2,511
Commitments and contingencies (Note 12)		
Redeemable convertible preferred stock, $0.001 par value; 0 and 14,000,000 shares authorized as of September 30, 2018 and December 31, 2017, respectively; 0 and 12,856,325 shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively; aggregate liquidation preference of $17,032 as of December 31, 2017;	—	17,028
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value, 5,000,000 and 0 shares authorized as of September 30, 2018 and December 31, 2017, respectively; and no shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively;	—	—
Common stock, $0.001 par value; 150,000,000 and 20,000,000 shares authorized as of September 30, 2018 and December 31, 2017, respectively; 36,711,661 and 5,137,771 shares issued and outstanding as of September 30, 2018 and December 31, 2017, respectively;	37	4
Additional paid-in capital	214,690	1,332
Accumulated deficit	(50,908)	(14,532)
Total stockholders' equity (deficit)	163,819	(13,196)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 170,226	$ 6,343

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Operations

(Unaudited)
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Operating expenses:				
Research and development (includes related party expense (benefit) of ($23) and $30, and $3 and $57, respectively)	$ 7,931	$ 2,283	$ 21,362	$ 5,583
General and administrative (includes related party expense of $165 and $183, and $924 and $394, respectively)	2,619	490	6,656	1,322
Total operating expenses	10,550	2,773	28,018	6,905
Loss from operations	(10,550)	(2,773)	(28,018)	(6,905)
Other income (expense), net	374	—	(1,681)	75
Loss on extinguishment of debt	—	—	(6,677)	—
Net loss	$ (10,176)	$ (2,773)	$ (36,376)	$ (6,830)
Net loss per share	$ (0.29)	$ (0.74)	$ (2.28)	$ (1.95)
Weighted-average shares used in computing net loss per share basic, and diluted	35,591,518	3,752,883	15,976,228	3,504,790

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Cash Flows

(Unaudited)
(In thousands)

	Nine Months Ended September 30,	
	2018	**2017**
Cash Flows From Operating Activities:		
Net loss	$ (36,376)	$ (6,830)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	40	2
Stock-based compensation expense	2,643	101
Accrued interest on convertible promissory notes	48	—
Change in fair value of redeemable convertible preferred stock tranche liability	(1,334)	(75)
Change in fair value of redeemable convertible preferred stock warrant liability	2,628	—
Amortization of debt discount	713	—
Loss on extinguishment of debt	6,677	—
Changes in assets and liabilities:		
Related party receivable	38	(20)
Prepaid expenses and other current assets	(2,764)	(748)
Other assets	18	6
Accounts payable	1,852	888
Accrued expenses and other liabilities	2,278	550
Related party payable	(166)	(32)
Net cash used in operating activities	(23,705)	(6,158)
Cash Flows From Investing Activities:		
Purchase of property and equipment	(144)	(6)
Net cash used in investing activities	(144)	(6)
Cash Flows From Financing Activities:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	63,875	12,993
Proceeds from issuance of convertible promissory notes	10,000	—
Proceeds from issuance of common stock upon exercise of stock options and restricted stock	75	15
Proceeds from issuance of initial public offering, net of issuance costs	110,970	—
Net cash provided by financing activities	184,920	13,008
Net increase in cash and cash equivalents	161,071	6,844
Cash and Cash Equivalents, Beginning of Period	5,497	1,956
Cash and Cash Equivalents, End of Period	$ 166,568	$ 8,800
Supplemental disclosure of non-cash financing activities:		
Settlement of fair value of redeemable convertible preferred stock put option asset	$ 1,527	$ —
Settlement of fair value of redeemable convertible preferred stock tranche liability	694	240
Vesting of restricted stock and early exercised options	135	9
Conversion of convertible note payable and accrued interest into redeemable convertible preferred stock	10,048	—
Conversion of redeemable convertible preferred stock to common stock at closing of initial public offering	97,276	—
Reclassification of redeemable convertible preferred stock warrant liability to equity	3,506	—

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

Note 1. Organization and Description of Business

Eidos Therapeutics, Inc., or the Company, was incorporated as an S corporation in the state of Delaware on August 6, 2013. The Company was converted into a C corporation on April 4, 2016 in conjunction with its Series Seed redeemable convertible preferred stock financing. The Company is advancing a drug candidate to treat multiple forms of transthyretin amyloidosis, which leads to organ damage, loss of organ function and eventual death from abnormal buildup of protein deposits predominantly in the heart and peripheral nervous system. The Company has been primarily engaged in business planning, research, recruiting personnel and raising capital. The Company is headquartered in San Francisco, California and it operates as one operating segment.

Stock Split

In June 2018, the Company's board of directors approved an amendment to the Company's amended and restated certificate of incorporation to effect a stock split of the Company's issued and outstanding common stock at a 1.196-for-1 ratio, which was effected on June 7, 2018. In connection with the stock split, the authorized shares of common stock were increased from 27,000,000 to 32,292,000. The par value of common stock and redeemable convertible preferred stock was not adjusted as a result of the stock split. All issued and outstanding common stock, options to purchase common stock and per share amounts contained in the condensed financial statements have been retroactively adjusted to reflect the stock split for all periods presented.

Initial Public Offering

On June 19, 2018, the Company's registration statement on Form S-1 (File No. 333-225235) relating to its initial public offering ("IPO") of common stock became effective. The IPO closed on June 22, 2018 at which time the Company issued 7,187,500 shares of its common stock at a price of $17.00 per share, which included shares issued upon the underwriters' exercise of their overallotment option to purchase 937,500 additional shares. In addition, upon closing the IPO, all outstanding shares of the redeemable convertible preferred stock and warrants converted into 29,564,527 shares of common stock and there are no shares of redeemable convertible preferred stock outstanding. The Company received an aggregate of $111.0 million in cash, net of underwriting discounts and commissions, and after deducting offering costs paid by the Company.

Liquidity

In accordance with Accounting Standards Update ("ASU") 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40), the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued. Since inception, the Company has funded its operations with proceeds from sales of redeemable convertible preferred stock, common stock, convertible promissory notes. The Company has incurred recurring losses since inception, including net losses of $10.2 million and $36.4 million for the three and nine months ended September 30, 2018, respectively. As of September 30, 2018, the Company had an accumulated deficit of $50.9 million. The Company expects to continue to generate operating losses for the foreseeable future. As of the issuance date of the unaudited condensed financial statements, the Company expects that funds received from the completion of its IPO in June 2018, together with its cash and cash equivalents, totaling $166.6 million as of September 30, 2018, will be sufficient to fund its anticipated operating and capital expenditure requirements through at least 12 months from the issuance date of these unaudited condensed financial statements.

Note 2. Summary of Significant Accounting Policies

Basis of preparation

These unaudited condensed financial statements have been prepared in accordance with United States generally accepted accounting principles, or GAAP. These financial statements include transactions with BridgeBio Pharma LLC and its affiliates, or BBP LLC, a controlling stockholder in the Company. For the periods presented, BBP LLC has provided consulting and management services to the Company in the ordinary course of business, including certain executive personnel, facility related costs, advisory services, insurance costs and other general corporate expenses. These allocations were made based on direct usage, when identifiable, with the remainder allocated primarily based on a proportional share of headcount. The Company's historical financial statements do not purport to reflect what the Company's results of operations, financial position, or cash flows would have been if the Company had operated as an independent entity during the periods presented. Management believes the basis on which the expenses have been

allocated to be a reasonable reflection of the utilization of services provided to or the benefit received by the Company during the periods presented. For more information on the allocated costs and related party transactions, see Note 6.

Unaudited Interim Condensed Financial Statements

The accompanying unaudited condensed financial statements have been prepared in accordance with GAAP and applicable rules and regulations of the Securities and Exchange Commission, or the SEC, regarding interim financial reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by GAAP have been condensed or omitted, and accordingly the balance sheet as of December 31, 2017 has been derived from the audited financial statements at that date but does not include all of the information required by GAAP for complete financial statements. These unaudited interim condensed financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for a fair statement of the Company's financial information. The results of operations for the three and nine months ended September 30, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018 or for any other interim period or for any other future year.

The accompanying interim unaudited condensed financial statements should be read in conjunction with the audited financial statements and the related notes thereto for the year ended December 31, 2017, which are included in the Company's prospectus related to the Company's IPO, filed with the SEC on June 21, 2018, pursuant to Rule 424(b) under the Securities Act of 1933.

Use of estimates

The preparation of the financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities as of the date of the financial statements and the reported amounts of expenses during the reporting period. On an ongoing basis, management evaluates its estimates, including those related to the fair value of the redeemable convertible preferred stock tranche liability, the preferred stock put option asset, the fair value of the redeemable convertible preferred stock warrant liability, the fair value of the Company's common stock, stock-based compensation, the useful lives of fixed assets, accruals for research and development activities and income taxes. Management bases its estimates on historical experience and on other relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents. All the Company's cash is held by one financial institution that management believes is of high credit quality. Such deposits may, at times, exceed federally insured limits.

Cash and Cash Equivalents

Cash equivalents that are readily convertible to cash are stated at cost, which approximates fair value. The Company considers all highly liquid investments purchased with an original maturity of three months or less to be cash equivalents. There were no cash equivalents at December 31, 2017. As of September 30, 2018, the Company had cash and cash equivalents of $166.6 million. The Company's cash equivalents are invested in highly-rated money market funds.

Fair Value of Financial Instruments

Fair value accounting is applied for all financial assets and liabilities that are recognized or disclosed at fair value in the condensed unaudited financial statements on a recurring basis (at least annually). The carrying amount of the Company's financial instruments, including cash equivalents, accounts payable and accrued expenses and other payables approximate fair value due to their short-term maturities. See Note 3. Fair value measurements regarding the fair value of the Company's redeemable convertible preferred stock put option asset, redeemable convertible preferred stock tranche liability and redeemable convertible preferred stock warrant liability.

Research and Development Costs and Accrued Research and Development

Research and development costs are expensed as incurred and consist of salaries and benefits, stock-based compensation expense, lab supplies and facility costs, as well as fees paid to others that conduct certain research and development activities on the Company's behalf.

The Company accrues for estimated costs of research and development activities conducted by third-party service providers, which include the conduct of preclinical studies and clinical trials, and contract manufacturing activities. The Company records the estimated costs of research and development activities based upon the estimated amount of services provided but not yet invoiced and include these costs in accrued expenses and other payables in the condensed unaudited balance sheets and within research and development expense in the condensed unaudited statements of operations. These costs are a significant component of the Company's research and development expenses. The Company accrues for these costs based on factors such as estimates of the work completed and in accordance with agreements established with its third-party service providers. The Company makes significant judgments and estimates in determining the accrued liabilities balance in each reporting period. As actual costs become known, the Company adjusts its accrued liabilities. The Company has not experienced any material differences between accrued costs and actual costs incurred. However, the status and timing of actual services performed, number of patients enrolled, and the rate of patient enrollments may vary from the Company's estimates, resulting in adjustments to expense in future periods. Changes in these estimates that result in material changes to the Company's accruals could materially affect the Company's results of operations.

Accrued repurchase liability for common stock

The Company records as a liability, within accrued expenses and other current liabilities, the purchase price of unvested common stock that the Company has a right to repurchase if and when the stockholder ceases to be a service provider to the Company before the end of the requisite service period. The proceeds are recorded as a liability and the proceeds related to the vested common stock is reclassified to additional paid-in capital as the Company's repurchase right lapses.

Redeemable convertible preferred stock put option asset

The Company has determined that its right to cause the Series B shareholders to purchase additional shares of redeemable convertible preferred stock upon the achievement of the specified milestone represented a freestanding financial instrument. The instrument was classified as an asset on the balance sheets based on its relative fair value. The put option asset balance was reclassified to redeemable convertible preferred stock upon the settlement of the additional shares in May 2018.

Redeemable convertible preferred stock tranche liability

The Company has determined that its obligation to issue additional shares of redeemable convertible preferred stock upon the achievement of certain milestones or at the option of the holder represents a freestanding financial instrument. The instrument was classified as a liability on the balance sheets and was subject to remeasurement at each balance sheet date and any change in fair value is recognized through other income (expense), net in the condensed statements of operations. The tranche liability balance was reclassified to redeemable convertible preferred stock upon the settlement of the additional shares in May 2018.

Redeemable convertible preferred stock warrant liability

The Company's redeemable convertible preferred stock warrants require liability classification and accounting as the underlying preferred stock is deemed redeemable. Upon initial recognition, the warrants are recorded at their estimated fair value. The warrants are subject to remeasurement at each balance sheet date, with changes in fair value recognized as a component of other income (expense), net.

The Company continued to adjust the liability for changes in fair value until the completion of the Company's IPO, at which time all redeemable convertible preferred stock warrants were net exercised into shares of common stock and the related redeemable convertible preferred stock warrant liability was reclassified to common stock and additional paid-in capital.

Net loss per share

Basic net loss per common share is calculated by dividing the net loss by the weighted-average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Diluted net loss per common share is the same as basic net loss per share since the effects of potentially dilutive securities are antidilutive given the Company's loss position.

Stock-Based Compensation

The Company periodically grants stock options and awards to employees and non-employees in non-capital raising transactions as compensation for services rendered. The Company accounts for stock option grants to employees whereby the fair value of the award is measured on the date of grant and recognized over the vesting period. The Company accounts for stock option grants to non-employees whereby the amount of stock compensation expense recognized is determined based upon the measurement date at either a) the date at which a performance commitment is reached, or b) at the date at which the necessary performance to earn the equity instruments is complete. Non-employee stock-based compensation expenses generally are amortized over the vesting period on a straight-line basis. In certain circumstances where there are no future performance requirements by the non-employee, option grants are immediately vested and the total stock-based compensation charge is recorded in the period of the measurement date.

The fair value of the Company's common stock option grants is estimated using a Black-Scholes option pricing model, which uses certain assumptions related to risk-free interest rates, expected volatility, expected life of the common stock options, and future dividends. Compensation expense is recorded based upon the value derived from the Black-Scholes option pricing model and based on actual experience. The assumptions used in the Black-Scholes option pricing model could materially affect compensation expense recorded in future periods.

The Company has in the past issued restricted shares of its common stock for share-based compensation programs. The Company measures the compensation cost with respect to restricted shares issued to employees based upon the estimated fair value of the equity instruments at the date of the grant and is recognized as expense over the period which an employee is required to provide services in exchange for the award.

Recent accounting pronouncements

In February 2016, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, No. 2016-02, *Leases (Topic 842*), which for operating leases requires the lessee to recognize a right-of-use asset and a lease liability, initially measured at the present value of the lease payments, in its balance sheet. The guidance also requires a lessee to recognize single lease costs, calculated so that the cost of the lease is allocated over the lease term, on a generally straight-line basis. A modified retrospective transition approach is required for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, including a number of optional practical expedients that entities may elect to apply. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The Company has not determined the extent of potential effects of this ASU on its condensed financial statements.

In June 2018, the FASB issued ASU No. 2018-07, Stock-based Compensation: Improvements to Nonemployee *Share-based Payment Accounting*, which amends the existing accounting standards for share-based payments to nonemployees. This ASU aligns much of the guidance on measuring and classifying nonemployee awards with that of awards to employees. Under the new guidance, the measurement of nonemployee equity awards is fixed on the grant date. This ASU becomes effective in the first quarter of fiscal year 2019 and early adoption is permitted but no earlier than an entity’s adoption date of Topic 606. Entities will apply the ASU by recognizing a cumulative-effect adjustment to retained earnings as of the beginning of the annual period of adoption. The Company is currently evaluating the impact that ASU 2018-07 will have on its condensed financial statements.

In August 2018, the FASB issued ASU No. 2018-13, Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement, which amends ASC 820, Fair Value Measurement. This ASU modifies the disclosure requirements for fair value measurements by removing, modifying, or adding certain disclosures. The effective date is the first quarter of fiscal year 2020, with early adoption permitted for the removed disclosures and delayed adoption until fiscal year 2020 permitted for the new disclosures. The removed and modified disclosures will be adopted on a retrospective basis and the new disclosures will be adopted on a prospective basis. The Company has determined the adoption will not have a material effect on the its condensed financial statements.

In August 2018, the SEC adopted the final rule under SEC Release No. 33-10532, Disclosure Update and Simplification, amending certain disclosure requirements that were redundant, duplicative, overlapping, outdated or superseded. In addition, the amendments expanded the disclosure requirements on the analysis of stockholders' equity for interim financial statements. Under the amendments, an analysis of changes in each caption of stockholders' equity presented in the balance sheet must be provided in a note or separate statement. The analysis should present a reconciliation of the beginning balance to the ending balance of each period for which a statement of comprehensive income is required to be filed. The final rule became effective on November 5, 2018. The Company is evaluating the impact of this guidance on its condensed financial statements.

Note 3. Fair value measurement

Financial assets and liabilities are recorded at fair value. The accounting guidance for fair value provides a framework for measuring fair value, clarifies the definition of fair value and expands disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability (an exit price) in an orderly transaction between market participants at the reporting date. The accounting guidance establishes a three-tiered hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

Level 1—Quoted prices in active markets for identical assets or liabilities.
Level 2—Observable inputs other than Level 1 prices such as quoted prices for similar assets or liabilities, quoted prices in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.
Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

In determining fair value, the Company utilizes valuation techniques that maximize the use of observable inputs and minimize the use of unobservable inputs to the extent possible as well as considers counterparty credit risk in its assessment of fair value.

There were no financial assets outside of cash in an operating account as of December 31, 2017 and September 30, 2018. There were no transfers between Level 1, Level 2 and Level 3 categories during the periods presented.

There were no financial liabilities measured at fair value as of December 31, 2017 and September 30, 2018. There were no transfers between Level 1, Level 2 and Level 3 categories during the periods presented. Following is the activity related to Level 3 financial assets and liabilities of the Company during the nine months ended September 30, 2018:

Redeemable convertible preferred stock put option asset

The fair value of the redeemable convertible preferred stock put option asset was based on significant inputs not observed in the market and thus represents a Level 3 measurement. The Company estimates the fair value of the redeemable convertible preferred stock put option asset using the Black-Scholes option pricing model. The following table sets forth a summary of the changes in the fair value of the Company's redeemable convertible preferred stock put option asset (in thousands):

Redeemable convertible preferred stock put option:	
Balance at December 31, 2017	$ —
Issuance of Series B redeemable convertible preferred stock tranche put option	1,527
Settlement of redeemable convertible preferred stock tranche liability due to the issuance of Series B redeemable convertible preferred stock	(1,527)
Balance at September 30, 2018	$ —

Redeemable convertible preferred stock tranche liability

The fair value of the redeemable convertible preferred stock tranche liability was based on significant inputs not observed in the market and thus represents a Level 3 measurement. The Company estimates the fair value of the redeemable convertible preferred stock tranche liability using the Black-Scholes option pricing model. The following table sets forth a summary of the changes in the fair value of the Company's redeemable convertible preferred stock tranche liability (in thousands):

Redeemable convertible preferred stock tranche liability:	
Balance at December 31, 2017	$ —
Issuance of Series B redeemable convertible preferred stock tranche liability	2,028
Change in fair value upon revaluation	(1,334)
Settlement of redeemable convertible preferred stock tranche liability due to the issuance of Series B redeemable convertible preferred stock	(694)
Balance at September 30, 2018	$ —

Redeemable convertible preferred stock warrant liability

The fair value of the redeemable convertible preferred stock warrant liability is based on significant inputs not observed in the market and thus represents a Level 3 measurement. The Company estimates the fair value of the redeemable convertible preferred stock warrant liability using the Black-Scholes option pricing model. The following table sets forth a summary of the changes in the fair value of the Company's redeemable convertible preferred stock warrant liability (in thousands):

Redeemable convertible preferred stock warrant liability:	
Balance at December 31, 2017	$ —
Issuance of redeemable convertible preferred stock warrant liability	878
Change in fair value upon revaluation	2,628
Reclassification of redeemable convertible preferred stock warrant liability to common stock	(3,506)
Balance at September 30, 2018	$ —

Embedded derivative in convertible note

The convertible note issued in February 2018 had a redemption feature that was determined to be an embedded derivative requiring bifurcation and separate accounting. The fair value of the derivative was determined based on an income approach that identified the cash flows using a "with-and-without" valuation methodology. The inputs used to determine the estimated fair value of the derivative instrument were based largely on the probability of an underlying event triggering the embedded derivative occurring and the timing of such event. The following table sets forth a summary of the changes in the fair value of the Company's embedded derivative in convertible note (in thousands):

Derivative instrument:	
Balance at December 31, 2017	$ —
Initial fair value of the embedded derivative issued with the convertible note	4,153
Change in fair value upon revaluation	—
Extinguishment of the embedded derivative	(4,153)
Balance at September 30, 2018	$ —

Note 4. Condensed balance sheet components

Property and equipment, net

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

	September 30, 2018	December 31, 2017
Leasehold improvements	$ 86	$ 77
Computer equipment	82	29
Office furniture and equipment	94	12
Total Property and equipment, cost	262	118
Less: Accumulated depreciation and amortization	(44)	(4)
Property and equipment, net	$ 218	$ 114

The Company recorded $16,000 and $40,000 for the depreciation and amortization during the three and nine months ended September 30, 2018, respectively; and $1,000 and $2,000 for the depreciation and amortization during the three and nine months ended September 30, 2017, respectively.

Accrued expenses and other current liabilities

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

	September 30, 2018	December 31, 2017
Accrued research and development costs	$ 2,203	$ 564
Accrued employee related expenses	725	606
Liability for unvested stock, short-term	135	109
Accrued other current liabilities	364	21
	$ 3,427	$ 1,300

As of December 31, 2017, and September 30, 2018, the balances of $208,000 and $286,000, respectively, in other liabilities related to the long-term liability for unvested stock.

Note 5. Convertible promissory notes

In February 2018, the Company entered into a Note and Warrant Purchase Agreement with BBP LLC and Stanford University. The Company issued two convertible promissory notes in an aggregate principal amount of $10.0 million. The notes had a maturity date of the earliest of a qualified financing, a deemed liquidation event, a qualified initial public offering or February 2019. The convertible promissory notes had an annual interest rate of 5.0%. The convertible promissory notes were convertible into future preferred stock at a 30% discount to the price paid by investors in the Company's next preferred equity financing of at least $10.0 million or convertible into common stock at the price per share in an IPO with aggregate proceeds of at least $30.0 million. In connection with the convertible promissory notes, the Company issued warrants for the purchase of $4.0 million in shares of the Company's Series Seed redeemable convertible preferred stock or the Company's preferred stock in the next equity financing. The exercise period commences upon the earlier of the closing of the next qualified financing and the consummation of a deemed liquidation event. The exercise price of the warrant was the price per share in the next equity financing if the warrant was exercisable for the Company's redeemable convertible preferred stock in the next qualified financing, or $1.3248 per share if the warrant was exercisable for shares of Series Seed redeemable convertible preferred stock. If the warrant remained outstanding upon the consummation of the IPO, the warrant would automatically be deemed net-exercised in full immediately prior to the completion of the IPO at the initial public offering price.

Upon issuance of the convertible promissory notes, the Company recorded the fair value of the warrants of $877,000 as a debt discount and redeemable convertible preferred stock warrant liability. The convertible promissory notes also contained a redemption feature that was determined to be an embedded derivative requiring bifurcation and separate accounting. The fair value of the embedded derivative liability at issuance was determined to be $4.2 million and was recorded as an additional debt discount. The debt discount was accreted using the effective interest method as additional interest expense over the term of the convertible note. Changes in the fair value of the embedded derivative and redeemable convertible preferred stock warrant liability have also been recorded within other income (expense), net, in the condensed statement of operations for the nine months ended September 30, 2018.

During the three and nine months ended September 30, 2018, the Company recognized interest expense of $0 and $761,000 related to the accrued interest and amortization of debt discount, respectively. No expenses were recorded in 2017 related to this note.

As the convertible notes payable contained an embedded conversion feature that does not qualify for derivative treatment, the Company evaluated if there was a beneficial conversion feature (BCF). The Company determined there was a BCF of $2.4 million as the effective conversion rate of the convertible note was below market value. The Company accounted for the value of the BCF as a debt discount, which was being accreted to interest expense over the life of the related debt using the effective interest method. The value of the BCF was recorded to additional paid-in capital with the offset to discount on convertible notes payable. The debt discount was to be accreted to other income (expense), net over the one-year original term of the convertible notes payable. During the three and nine months ended September 30, 2018, the Company recorded $0 and $228,000 related to this debt discount, respectively. No expense was recorded in 2017 related to this BCF.

On March 29, 2018, the convertible notes payable were converted into Series B redeemable convertible preferred stock, and the remaining amount of unamortized debt discount was recorded as an extinguishment of debt.

In March 2018, as a result of the Series B redeemable convertible preferred stock financing event, the outstanding principal and accrued interest of $10.0 million related to the convertible promissory notes automatically converted into 1,324,823 shares of Series B redeemable convertible preferred stock using a conversion price of $7.5844.

In connection with the conversion of the convertible promissory note, for the three and nine months ended September 30, 2018, the Company recorded $0 and $6.7 million, related to the loss on the extinguishment of the convertible promissory notes, respectively.

In the addition, the warrants associated with the convertible note became warrants to purchase 369,180 shares of the Company's Series B redeemable convertible preferred stock at an exercise price of $10.8348 per share.

Note 6. Related party transactions

BridgeBio Pharma LLC

BridgeBio Pharma LLC and its affiliates, or BBP LLC, is a controlling stockholder in the Company, as it owned 75% and 53% of the Company's total outstanding shares as of December 31, 2017 and September 30, 2018. In April 2016, the Company began receiving consulting, management, facility and infrastructure services pursuant to a services agreement with BBP LLC. The initial agreement was entered into on March 1, 2016 and was superseded by the subsequent agreement effective as of May 1, 2017.

The Company incurred the following expenses under the agreement with BBP LLC (in thousands):

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30,	
	2018	2017	2018	2017
Rent	$ 14	$ 25	$ 33	$ 50
Facility	20	21	125	34
Consulting	108	167	769	367
	$ 142	$ 213	$ 927	$ 451

As of December 31, 2017, and September 30, 2018, the Company had an outstanding receivable from BBP LLC of $67,000 and $29,000, related to providing services to other subsidiaries of BBP LLC. As of December 31, 2017 and September 30, 2018, the Company had an outstanding liability due to BBP LLC of $372,000 and $206,000, respectively.

Founders

Dr. Graef Consulting Agreement

In April 2016, the Company entered into a consulting agreement with Dr. Graef, one of the Company's founders. Pursuant to the consulting agreement, Dr. Graef agreed to provide consulting services in connection with the discovery and development of novel TTR stablizers. As compensation for these services, Dr. Graef is entitled to an annual fee in the amount of up to $150,000 and reimbursement by the Company for pre-approved expenses. The consulting agreement has a term of four years but may be terminated by either party for any reason with thirty days' prior notice.

In December 2017, we issued to Dr. Graef 195,273 shares of our common stock in order to offset dilution to her ownership in connection with our issuance of additional shares of Series Seed Preferred Stock in financing transactions. In addition, we agreed to make a "gross-up" payment of $83,073 to Dr. Graef for the taxes owed by Dr. Graef as a result of such issuance of common stock, which payment was made in January 2018. As of June 20, 2018 Dr. Graef is not considered a related party.

Dr. Alhamadsheh Consulting Agreement

In August 2016, the Company entered into a consulting agreement with Dr. Alhamadsheh, one of the Company's founders. Pursuant to the consulting agreement, Dr. Alhamadsheh agreed to provide consulting services in connection with the discovery and development of novel TTR stablizers. As compensation for these services, Dr. Alhamadsheh is entitled to an annual fee in the amount of up to $115,000 and reimbursement by the Company for pre-approved expenses. The consulting agreement has a term of four years but may be terminated by either party for any reason with thirty days' prior notice.

In December 2017, we issued to Dr. Alhamadsheh 195,273 shares of our common stock in order to offset dilution to his ownership in connection with our issuance of additional shares of Series Seed Preferred Stock in financing transactions. In addition, we agreed to make a "gross-up" payment of $83,073 to Dr. Alhamadsheh for the taxes owed by Dr. Alhamadsheh as a result of such issuance of common stock, which payment was made in January 2018. As of June 20, 2018 Dr. Alhamadsheh is not considered a related party.

The Company incurred the following expenses (benefit) for services under the consulting agreements and stock-based compensation (in thousands):

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30,	
	2018	2017	2018	2017
Dr. Graef	$ (180)	$ 40	$ 602	$ 124
Dr. Alhamadsheh	(189)	31	575	98
	$ (369)	$ 71	$ 1,177	$ 222

Option Award to Dr. Huh

In May 2018, our board of directors approved a grant to Dr. Huh (a member of our board of directors) of an option to purchase 83,720 shares of our common stock pursuant to the Company's 2018 Stock Option and Incentive Plan (the "2018 Plan"). The option will vest in equal annual installments over three years from the grant date, subject to Dr. Huh's continued service as a director through the applicable vesting dates. The award is subject to full accelerated vesting upon a "sale event," as defined in the 2018 Plan. For the three and nine months ended September 30, 2018 the Company recorded $75,000 and $85,000 related to these awards.

Note 7. Redeemable convertible preferred stock

In March 2018, the Company sold an aggregate of 1,476,715 shares of Series B redeemable convertible preferred stock financing in an initial closing for total gross proceeds of $16.0 million. An additional 4,430,162 shares of Series B redeemable convertible preferred stock may be issued in an additional closing contingent upon the release of specified data study either upon the request of the Company for investors to purchase the shares (purchased put option) or the investors may call for the purchase of such shares (tranche liability or call option). The Company has determined that its right to cause the Series B shareholders to purchase additional shares of redeemable convertible preferred stock upon the achievement of the specified milestone represented a freestanding financial instrument. The Company recorded the redeemable convertible preferred stock put option asset, based on its relative fair value of $1.5 million as an asset. In addition, the Company determined it was obligated to sell additional shares of Series B redeemable convertible preferred stock contingent upon the achievement of the specified milestone. This additional closing was also deemed to be freestanding financial instrument.

Upon issuance of the Series B redeemable convertible preferred stock, the Company recorded the redeemable convertible preferred stock tranche liability incurred in connection with its Series B redeemable convertible preferred stock as a derivative financial instrument liability at the fair value of $2.0 million on the date of issuance and remeasured the liability on each subsequent balance sheet dates through the issuance of the additional shares. The changes in fair value are recognized as a gain or loss within other income (expense), net in the statements of operations and the liability is remeasured at each reporting period and settlement of the related tranche closing.

In May 2018, the Company issued 4,430,162 shares of Series B redeemable convertible preferred stock at a purchase price of $10.8348 per share, for total proceeds of $48.0 million. The issuance of the shares is in connection with the additional shares related to the put option asset pertaining to the Series B redeemable convertible preferred stock financing in March 2018. The tranche liability and put option asset balances were reclassified to redeemable convertible preferred stock upon the closing of the sale of additional shares.

Following the closing of the IPO, all outstanding shares of the redeemable convertible preferred stock converted into 24,025,270 shares of common stock and the related carrying value was reclassified to common stock and additional paid-in capital. There were no shares of redeemable convertible preferred stock outstanding as of September 30, 2018.

Note 8. Redeemable convertible preferred stock tranche liability and put option asset

In March 2018, the Company entered into a Series B Preferred Stock Purchase Agreement, or the Agreement, for the issuance of up to 7,231,700 shares of Series B redeemable convertible preferred stock at a price of $10.8348 per

share in two closings. Upon the initial closing on March 29, 2018, 1,476,715 shares of Series B redeemable convertible preferred stock were issued for gross proceeds of $16.0 million and 1,324,823 shares were issued upon conversion of the outstanding convertible promissory note principal balance and accrued interest of $10.0 million.

The Agreement, provided that the Company could issue an additional 4,430,162 shares under the same terms as the initial closing, in an additional closing contingent upon the achievement of certain milestone. Either the investors or the Company could provide written notice for the additional closing to occur.

The Company determined that its obligation to issue additional shares of its redeemable convertible preferred stock and the Company's right to request investors to purchase additional shares of its redeemable convertible preferred stock represent freestanding financial instruments. The freestanding redeemable convertible preferred stock tranche liability was initially recorded at fair value, with fair value changes recorded within other income (expense), net in the condensed statement of operations. The purchased put option was recorded at fair value without subsequent remeasurement.

The Company continued to adjust the tranche liability for changes in the fair value until the settlement of the redeemable convertible preferred stock additional closing in May 2018. At such time, the remaining value of the redeemable convertible preferred stock tranche liability and the put option asset were reclassified to redeemable convertible preferred stock with no further remeasurement required. The Company has recorded a redeemable convertible preferred stock tranche liability and a put option asset in March 2018 of $2.0 million and $1.5 million, respectively, related to the Series B redeemable convertible preferred stock financing.

The Company estimated the fair value of the preferred stock liability and the put option asset using a Black-Scholes option pricing model using the following assumptions:

Expected term—The expected term represents the period for which the redeemable convertible preferred stock tranche liability and put option asset are expected to be outstanding, which is estimated to be the remaining contractual term.

Expected volatility—The volatility data was estimated based on a study of publicly traded industry peer companies, as there is no trading history for our redeemable convertible preferred stock. For purposes of identifying these comparable peer companies, the Company considered the industry, stage of development, size and financial leverage. The Company has measured historical volatility over a period equivalent to the expected term and believes that historical volatility provides a reasonable estimate of future expected volatility.

Expected dividends—The Black-Scholes valuation model calls for a single expected dividend yield as an input. The Company currently has no history or expectation of paying cash dividends on its redeemable convertible preferred stock.

Risk-free interest rate—The risk-free interest rate is based on the yield available on U.S. Treasury zero-coupon issues similar in duration to the expected term of the redeemable convertible preferred stock tranche liability and put option asset.

The Company used the following assumptions: a term of 0.08 years, a risk-free rate of 1.63%, a volatility of 36.4%, and a dividend yield of 0.0%.

In May 2018, the Company completed the closing of the Series B Second Tranche and issued 4,430,162 shares of Series B redeemable convertible preferred stock for net cash proceeds of $48.0 million. At this time the Series B redeemable convertible preferred stock liability was remeasured at $0.7 million, determined using a probability-weighted expected return method ("PWERM"). The PWERM included probabilities of three IPO scenarios occurring in June 2018. The scenarios were weighted based on the Company's estimate of each event occurring in deriving the estimated fair value. Upon the closing of the Series B Second Tranche, the Series B redeemable convertible preferred stock liability was terminated and the balance of the liability of $0.7 million was reclassified to redeemable convertible preferred stock.

For the three and nine months ended September 30, 2018, the Company recorded a charge of $0 and $1.3 million, respectively, for the change in the fair value of the Series B redeemable convertible preferred stock liability in the condensed statements of operations.

Note 9. Stockholders' Equity and Stock-Based Compensation

Stock Plans

Equity Incentive Plan

2016 Equity Incentive Plan

In April 2016, the Company established its 2016 Equity Incentive Plan, or the 2016 Plan, which provides for the granting of equity awards to employees and consultants of the Company. Awards granted under the 2016 Plan may be either incentive stock options, or ISOs, nonqualified stock options, or NSOs or restricted stock awards. ISOs may be granted only to Company employees (including officers and directors who are also employees). NSOs may be granted to Company employees and consultants. The exercise price of an ISO and NSO shall not be less than 100% of the estimated fair value of the shares on the date of grant, as determined by the board of directors. The exercise price of an ISO granted to an employee who at the time of grant is a 10% stockholder shall not be less than 110% of the estimated fair value of the shares on the date of grant, as determined by the board of directors. To date, options have a term of ten years and generally vest over a four-year period with annual cliff vesting and the balance monthly over 36 months. Upon completion of the Company's IPO, the remaining shares available for issuance under the 2016 Plan were retired.

2018 Stock Option and Incentive Plan

In May 2018, the Company's board of directors and stockholders approved the 2018 Stock Option and Incentive Plan, or the 2018 Plan, to replace the 2016 Plan. The 2018 Plan became effective upon the IPO and is administered by the board of directors or a committee appointed by the board of directors, which determines the types of awards to be granted, including the number of shares subject to the awards, the exercise price and the vesting schedule. Under the 2018 Plan, 598,000 shares of the Company's common stock have been initially reserved for the issuance of stock options, restricted stock units and other awards to employees, directors and consultants. Options granted under the 2018 Plan expire no later than 10 years from the date of grant. The exercise price of each option may not be less than 100% of the fair market value of the common stock at the date of grant. Options may be granted to stockholders possessing more than 10% of the total combined voting power of all classes of stocks of the Company at an exercise price at least 110% of the fair value of the common stock at the date of grant and the options are not exercisable after the expiration of 10 years from the date of grant. Employee stock options generally vest 25% upon one year of continued service to the Company, with the remainder in monthly increments over three additional years. Upon adoption of the 2018 Plan, no additional stock awards will be issued under the 2016 Plan. Options granted under the 2016 Plan that were outstanding on the date the 2018 plan became effective remain subject to the terms of the 2016 Plan. As of September 30, 2018, the Company has reserved 598,000 shares of common stock for issuance under the 2018 Plan.

Employee Stock Purchase Plan

In May 2018, the Company's board of directors and stockholders approved the 2018 Employee Stock Purchase Plan, or the 2018 ESPP, which became effective upon the IPO. The 2018 ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Internal Revenue Code of 1986, as amended, and is administered by the Company's board of directors and the Compensation Committee of the board of directors. Under the 2018 ESPP, 143,520 shares of the Company's common stock have been initially reserved for employee purchases of the Company's common stock. The 2018 ESPP allows eligible employees to purchase shares of the Company's common stock at a discount through payroll deductions of up to 20% of their eligible compensation. At the end of each offering period, employees are able to purchase shares at 85% of the lower of the fair market value of the Company's common stock at the beginning of the offering period or at the end of each applicable purchase period. The first purchase period commenced upon the completion of the Company's IPO, and ends on November 30, 2018.

Stock Options

The following table summarizes the Company's stock option activity for the nine months ended September 30, 2018:

	Options Available for Grant	Options Outstanding	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding—December 31, 2017	670,996	846,164	$ 0.59		$ 4,383
Options granted	(399,074)	399,074	$ 5.03		
Options cancelled	321,916	(321,916)	$ 0.59		
Options retired	(593,838)	—	$ -		
Additional authorized	598,000	—	$ -		
Options granted	(283,808)	283,808	$ 18.64		
Options exercised	—	(149,350)	$ 1.70		
Exercised options repurchased	40,366	—	$ 0.33		
Options canceled	16,446	(16,446)	$ 0.59		
Outstanding—September 30, 2018	371,004	1,041,334	$ 7.05	9.46	$ 3,051
Options exercisable – September 30, 2018		153,813	$ 1.02	9.19	$ 1,378
Options vested and expected to vest – September 30, 2018		1,041,334	$ 7.05	9.46	$ 3,051

During the three months ended September 30, 2018, the estimated weighted-average grant-date fair value of common stock underlying options granted to employees was $13.18 per share.

Accrued repurchase liability for common stock early exercises

Stock awards granted pursuant to the 2016 Plan permitted option holders to elect to exercise unvested options in exchange for unvested common stock. Awards granted under the 2016 Plan that are exercised prior to vesting will continue to vest according to the respective award agreement, and such unvested shares are subject to repurchase by the Company at the optionee's original exercise price or fair market value in the event the optionee's service with the Company voluntarily or involuntarily terminates.

As of December 31, 2017 and September 30, 2018, 1,219,389 and 988,238 shares, respectively, remained subject to a repurchase right by the Company, with a related liability included in accrued expenses and other liabilities in the condensed balance sheet of $317,000 and $421,000, respectively.

Restricted stock

In December 2017, the Company issued 390,546 shares of common stock for no consideration to the founders pursuant to the Series Seed Preferred Stock Purchase Agreement and license agreement in connection with certain anti-dilution rights held by these parties. If the shares issued under the license agreement represent less than 1% of the shares issued and outstanding common stock on an as-converted basis, the Company will issue additional common stock to the founders and Stanford University. The Company has the right to repurchase the common stock at the fair value per share on the date of repurchase, which right lapses as the shares vest, which is 25% cliff after one year and monthly thereafter over 36 months. In order to vest, the holders are required to provide continued service to the Company. As of December 31, 2017, and September 30, 2018, 390,546 and 292,910 shares remained subject to repurchase.

The Company recognizes stock-based compensation expense over the period in which the related services from the founders are received. Stock-based compensation expense related to the restricted stock is recognized based on the vesting date fair value of stock using Black-Scholes pricing model and recorded as a research and development expense. During the three and nine months ended September 30, 2018 the Company recognized a benefit of $0.4 million and an expense of $1.0 million in connection with the stock-based compensation expense related to the restricted stock. During the three and nine months ended September 30, 2017 the Company did not recognize any stock-based compensation expense related to these awards.

Stock-based compensation expense

Total stock-based compensation expense related to all our stock-based awards was recorded on the statements of operations as follows (in thousands):

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30,	
	2018	2017	2018	2017
Research and development	$ (187)	$ 26	$ 2,016	$ 100
General and administrative	443	1	627	2
Total stock-based compensation expense	$ 256	$ 27	$ 2,643	$ 102

As of September 30, 2018, there was $11.6 million of total unrecognized compensation cost related to unvested stock-based compensation arrangements under the 2016 and 2018 Plans. The unrecognized stock-based compensation cost is expected to be recognized over a weighted-average period of 3.07 years.

10. Net Loss per share

As the Company had net losses for the three and nine months ended September 30, 2018 and 2017, all potentially dilutive shares were determined to be anti-dilutive. The following table sets forth the computation of basic and diluted net loss per share (in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Numerator:				
Net loss	$ (10,176)	$ (2,773)	$ (36,376)	$ (6,830)
Denominator:				
Weighted-average shares used to compute net loss per common share, basic and diluted	35,591,518	3,752,883	15,976,228	3,504,790
Net loss per shares, basic and diluted	$ (0.29)	$ (0.74)	$ (2.28)	$ (1.95)

The following shares of potentially dilutive securities have been excluded from the diluted net loss per share computations for the three and nine months ended September 30, 2018 and 2017 because their inclusion would be anti-dilutive:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Redeemable convertible preferred stock on an as-converted basis	—	15,376,164	—	15,376,164
Options to purchase common stock	1,041,334	100,719	1,041,334	100,719
Common stock subject to vesting or repurchase	988,238	424,475	988,238	424,475
	2,029,572	15,901,358	2,029,572	15,901,358

11. License agreement

In April 2016, the Company entered into a license agreement with the Board of Trustees of the Leland Stanford Junior University, or Stanford University relating to the Company's drug discovery and development initiatives. Under this agreement, the Company has been granted certain worldwide exclusive licenses to use the licensed compounds. The Company paid an upfront license payment of $25,000 in April 2016, which was recorded as research and development expense and issued 56,809 shares of common stock. The value of this equity was recorded, at fair value of $0.18 per share, as research and development expense of $8,000 during the year ended December 31, 2016. In March 2017, the Company paid a license fee of $10,000, which was recorded as research and development expense during the year ended December 31, 2017. The Company may also be required to make future payments of up to approximately $1.0 million to Stanford University upon achievement of specific intellectual property, clinical and regulatory milestone events, as well as pay royalties in the low single digits on future net sales, if any. In addition, the Company is obligated to pay Stanford University a percentage of non-royalty revenue received by the Company from its sublicensees, with the amount owed decreasing annually for three years based on when the applicable sublicense agreement is executed. In March 2018, the Company recorded $50,000 under the Stanford agreement in connection with the achievement of a development milestone. During the three and nine months ended September 30, 2017, the Company recognized $0 and $10,000, respectively, and during the three and nine months ended September 30, 2018 the Company recognized $0 and $63,000, respectively, in connection with this agreement.

12. Commitments and contingencies

Lease arrangements

In September 2017, the Company entered into a one-year operating lease for laboratory facilities in San Francisco, California. In November 2017, the Company entered into an operating lease for an administrative facility in San Francisco, California, which expires in November 2022. The Company has provided a security deposit of $158,000 as collateral for the lease, which is included in other assets on the condensed balance sheet at September 30, 2018.

Future minimum lease payments as of September 30, 2018 are as follows (in thousands):

Year	Operating Lease Commitments
2018 (remaining three months)	$ 80
2019	327
2020	337
2021	347
2022	327
	$ 1,418

The Company's rent expense was $29,000 and $54,000 for the three and nine months ended September 30, 2017 and $107,000 and $311,000 for the three and nine months ended September 30, 2018, respectively. The amounts include the amounts incurred pursuant to the service agreement with BridgeBio Services, Inc., an affiliate of BridgeBio Pharma LLC (see Note 6).

Rent expense is recognized on a straight-line basis over the terms of the Company's leases and accordingly, the Company recorded the difference between rent expense and amount paid under the leases as deferred rent liability within other liabilities in the balance sheets. Incentives granted under the Company's facility lease, including allowances to fund leasehold improvements, are deferred and recognized as adjustments to rent expense on a straight-line basis over the term of the lease.

Indemnification

In the ordinary course of business, the Company may provide indemnifications of varying scope and terms to vendors, lessors, business partners, board members, officers, and other parties with respect to certain matters, including, but not limited to, losses arising out of breach of such agreements, services to be provided by the Company, negligence or willful misconduct of the Company, violations of law by the Company, or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with directors and certain officers and employees 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, officers or employees. No demands have been made upon the Company to provide indemnification under such agreements, and thus, there are no claims that the Company is aware of that could have a material effect on the Company's balance sheets, statements of operations, or statements of cash flows.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our unaudited condensed financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and related notes thereto for the year ended December 31, 2017, included in our final prospectus filed with the Securities and Exchange Commission on June 21, 2018 pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended (the "Prospectus")

Forward-Looking Statements

This discussion contains certain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements are identified by words such as "believe," "will," "may," "estimate," "continue," "anticipate," "intend," "should," "plan," "expect," "predict," "could," "potentially" or the negative of these terms or similar expressions. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in this report in Part II, Item 1A -"Risk Factors," and elsewhere in this Quarterly Report on Form 10-Q. Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to our management. These statements, like all statements in this report, speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. We caution investors that our business and financial performance are subject to substantial risks and uncertainties.

Overview

We are a clinical stage biopharmaceutical company focused on addressing the large and growing unmet need in transthyretin, or TTR, amyloidosis, or ATTR. We are advancing our product candidate, AG10, to treat ATTR, a progressive and fatal family of diseases.

Our financial information includes allocations of expenses attributable to certain corporate functions that were provided to us by BridgeBio and its affiliates, including expenses attributable to certain executive personnel, facility-related costs, advisory services, insurance costs and other general corporate expenses. These allocations were made based on direct usage or estimates which are considered to be reasonable by our management and in accordance with our services agreement with BridgeBio. We have moved into our own leased facility and expect to reduce the services provided by BridgeBio as we hire additional personnel.

Since the commencement of our operations, we have devoted substantially all of our resources to research and development activities in support of our product development efforts, hiring personnel, raising capital to support and expand such activities and general and administrative support for these operations. We have funded our operations to date primarily from the issuance and sale of shares of redeemable convertible preferred stock and notes convertible into shares of redeemable convertible preferred stock.

In April 2016, we entered into a license agreement with the Board of Trustees of the Leland Stanford Junior University, or Stanford, for rights relating to novel transthyretin aggregation inhibitors. Under the license agreement, Stanford has granted us an exclusive worldwide license to make, use and sell products that are covered by the licensed patent rights.

In connection with the execution of the license agreement, we paid an upfront license fee in April 2016 and issued Stanford shares of common stock, which were recorded as research and development expense during the year ended December 31, 2016. In March 2017, we paid an annual maintenance fee under the license agreement, which was recorded as research and development expense during the year ended December 31, 2017. In the quarter ended March 31, 2018 we recorded expense of $60,000 under the license agreement related to the annual minimum royalty and a license milestone which was achieved in connection with the license agreement. We are obligated to make future payments to Stanford upon the achievement of specific intellectual property, clinical and regulatory milestone events, as well as pay royalties in the low single digits on future net sales, if any.

In October 2018, the FDA granted orphan drug designation in the United States to AG10 for the treatment of ATTR, and the Committee for Orphan Medicinal Products of the European Medicines Agency, or EMA, adopted a positive opinion for

the designation of AG10 as an orphan medicinal product in the European Union, or EU, for the treatment of ATTR. The EMA also granted a product-specific pediatric investigational plan waiver to the Company for AG10.

We have not generated any revenue to date. Since inception, we have incurred significant operating losses. We have incurred net losses of $2.5 million and $11.9 million during the years ended December 31, 2016 and 2017, and $6.8 million and $36.4 million during the nine months ended September 30, 2017 and 2018, respectively, and we expect to continue to incur significant losses for the foreseeable future. As of September 30, 2018, we had an accumulated deficit of $50.9 million. We expect these losses to increase as we continue our development of, and seek regulatory approvals for our product candidate, AG10, begin to commercialize AG10, if approved, and engage in any other research and development activities. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

In June 2018, we completed our initial public offering ("IPO") of our common stock pursuant to which we issued 7,187,500 shares of our common stock at a price of $17.00 per share and received $111.0 million in cash, net of underwriting discounts and commissions and offering costs.

As of September 30, 2018, we had $166.6 million in cash and cash equivalents.

We will need to obtain additional financing in the future and may seek financing through the issuance of our common stock, through other equity or debt financings or through collaborations or partnerships with other companies. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our clinical development efforts for AG10 and other research and development activities. We may not be able to raise additional capital on terms acceptable to us, or at all, and any failure to raise capital as and when needed would compromise our ability to execute on our business plan and we may have to significantly delay, scale back, or discontinue the development of AG10 or curtail any efforts to expand our product pipeline. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

Financial operations overview

Research and development expense

Research and development expense consists primarily of costs incurred for the development of AG10, which include:

- employee-related expenses, including salaries, benefits and stock-based compensation;
- laboratory, manufacturing and other vendor expenses related to the execution of preclinical studies and clinical trials;
- the costs related to the production of clinical supplies and the engagement of consultants that conduct research and development activities on our behalf;
- fees paid under our license agreement with Stanford; and
- facilities and other allocated expenses, expenses for rent, depreciation and amortization, maintenance of facilities and other supplies.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors, collaborators and third-party service providers. Nonrefundable payments made prior to the receipt of goods or services that will be used or rendered for future research and development activities are deferred and capitalized. The capitalized amounts are recognized as expense as the goods are delivered or the related services are performed.

The following table summarizes our research and development expenses incurred during the respective periods (in thousands):

	Three-Month Periods Ended September 30,		Nine-Month Periods Ended September 30,	
	2018	2017	2018	2017
Clinical development	$ 3,808	$ 342	$ 8,277	$ 342
Contract manufacturing	2,106	1,220	4,534	1,267
Preclinical, discovery and other research and development costs	1,305	244	2,823	3,070
Compensation and related personnel costs	627	436	5,410	814
Facility and other costs	85	41	318	90
	$ 7,931	$ 2,283	$ 21,362	$ 5,583

We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to AG10 as we advance AG10 into later stages of clinical development, including our ongoing Phase 2 clinical trial of AG10 in ATTR-CM and our planned Phase 3 clinical trial of AG10 in ATTR-PN and any subsequent clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of AG10 is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization of our product candidate, if at all.

General and administrative expense

Our general and administrative expenses consist primarily of personnel costs, allocated facility costs and other expenses for outside professional services, including legal, human resource, audit and accounting services. Personnel costs consist of salaries, benefits and stock-based compensation. We expect to incur additional expenses as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and listing standards applicable to companies listed on a national securities exchange, additional insurance expenses, investor relations activities and other administrative and professional services. We also expect to increase the size of our administrative function to support the growth of our business.

Other income (expense), net

Other income (expense), net primarily includes gains and losses from the remeasurement of our liabilities related to our redeemable convertible preferred stock tranche liability and our redeemable convertible preferred stock warrant liability. We continued to adjust the liabilities for changes in estimated fair value until the settlement of the redeemable convertible preferred stock tranche liability and redeemable convertible preferred stock warrant liability. In May 2018, the redeemable convertible preferred stock tranche liability was reclassified to redeemable convertible preferred stock and we will no longer recorded any related periodic fair value adjustments. We continued to record adjustments to the estimated fair value of the redeemable convertible preferred stock warrants until such time as these instruments were net exercised upon the completion of our IPO in June 2018.

Loss on extinguishment of debt

Loss on extinguishment of debt resulted from the conversion of our convertible promissory notes into Series B redeemable convertible preferred stock prior to its maturity date, resulting in the immediate recognition of unamortized debt discount amounts and related settlement of the embedded derivative liability.

Comparison of the three and nine months ended September 30, 2018 and 2017

Research and development expense

	Three Months Ended September 30,		Increase (Decrease)		Nine Months Ended September 30,		Increase (Decrease)	
	2018	2017	$	%	2018	2017	$	%
Research and development	$ 7,931	$ 2,283	5,648	247%	$ 21,362	$ 5,583	15,779	283%

Research and development expense increased by $5.6 million, or 247%, during the three months ended September 30, 2018, compared to the three months ended September 30, 2017. The increase was primarily attributable to increased personnel costs of $0.4 million due to a higher headcount, an increase of $5.2 million in clinical trial related activities and contract manufacturing activities for our clinical trials and their product supply, partially offset by a decrease in stock-based compensation of $0.2 million.

Research and development expense increased by $15.8 million, or 283%, during the nine months ended September 30, 2018, compared to the nine months ended September 30, 2017. The increase was primarily attributable to increased personnel costs of $2.7 million due to a higher headcount, an increase of $10.4 million in clinical trial related activities and contract manufacturing activities for our clinical trials and their product supply and an increase in stock-based compensation of $1.9 million.

General and administrative expense

	Three Months Ended September 30,		Increase (Decrease)		Nine Months Ended September 30,		Increase (Decrease)	
	2018	2017	$	%	2018	2017	$	%
General and administrative	$ 2,619	$ 490	2,129	434%	$ 6,656	$ 1,322	5,334	403%

General and administrative expense increased by $2.1 million, or 434%, during the three months ended September 30, 2018, compared to the three months ended September 30, 2017. The increase was primarily attributable to an increase of $1.1 million in professional service fees and consulting services, primarily for financial, legal and accounting fees and an increase of $0.5 million in personnel-related expenses due to an increase in headcount to support the growth of our operations and an increase in stock-based compensation of $0.4 million.

General and administrative expense increased by $5.3 million, or 403%, during the nine months ended September 30, 2018, compared to the nine months ended September 30, 2017. The increase was primarily attributable to an increase of $3.7 million in professional service fees and consulting services, primarily for financial, legal and accounting fees and an increase of $1.1 million in personnel-related expenses due to an increase in headcount to support the growth of our operations and an increase in stock-based compensation of $0.6 million.

Other income (expense), net

	Three Months Ended September 30,		Increase (Decrease)		Nine Months Ended September 30,		Increase (Decrease)	
	2018	2017	$	%	2018	2017	$	%
Other income (expense), net	$ 374	$ —	374	*	$ (1,681)	$ 75	(1,756)	-2341%

Other income (expense), net was an income of $0.3 million during the three months ended September 30, 2018, compared to income of $0 during the three months ended September 30, 2017. The income during the three months ended September 30, 2018 is primarily from the Company investing the funds from the IPO.

Other income (expense), net was an expense of $2.1 million during the nine months ended September 30, 2018, compared to income of $75,000 during the nine months ended September 30, 2017. The expense during the nine months ended September 30, 2018 is primarily from the amortization of the debt discount of $0.7 million related to the convertible promissory note payable which was converted into Series B redeemable convertible preferred stock in March 2018 and the net revaluation of the redeemable convertible preferred stock warrant liability and tranche liability of $1.3 million, partially offset by the interest income of $0.4 million. The other income during the nine months ended September 30, 2017 was due to the settlement of the redeemable convertible preferred stock tranche liability in March 2017 related to the Series Seed redeemable convertible preferred stock financing.

Loss on extinguishment of debt

	Three Months Ended September 30,		Increase (Decrease)		Nine Months Ended September 30,		Increase (Decrease)	
	2018	2017	$	%	2018	2017	$	%
Loss on extinguishment of debt	$ —	$ —	$ —	0%	$ (6,677)	$ —	(6,677)	0%

Loss on extinguishment of debt was due to our convertible promissory notes converting into Series B redeemable convertible preferred stock. The convertible promissory notes had a contractual term of one year, however, they were converted in March 2018, as such the remaining debt discounts were recognized immediately upon the conversion of the notes. There was no similar activity during the three and nine months ended September 30, 2017.

Critical accounting policies and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

There have been no material changes in our critical accounting policies during the nine months ended September 30, 2018, as compared to those disclosed in the *"Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates'* in our prospectus filed with the SEC on June 21, 2018 pursuant to Rule 424(b) under the Securities Act.

Liquidity and Capital Resources

Liquidity and Capital Expenditures

Liquidity

As of September 30, 2018, we had $166.6 million of cash and cash equivalents and an accumulated deficit of $50.9 million. In June 2018, we completed our IPO of our common stock pursuant to which we issued 7,187,500 shares of our common stock at a price of $17.00 per share and received $111.0 million in cash, net of underwriting discounts and commissions and offering costs paid by us.

Our primary uses of cash are to fund operating expenses, primarily research and development expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

We believe, based on our current operating plan and expected expenditures, that our existing cash and cash equivalents will be sufficient to meet our anticipated operating and capital expenditure requirements for at least the next 12 months from the filing of this Quarterly Report on Form 10-Q. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Our ultimate success depends on the outcome of our research and development activities. We expect to incur additional losses in the future and we anticipate the need to raise additional capital to fully implement our business plan.

We expect to further increase our research and development activities, which will increase the amount of cash used during the remainder of 2018 and beyond. Specifically, we expect continued spending on clinical trials, continued manufacturing activities and higher payroll expenses as we increase our professional and scientific staff and research and development activities. Based on the funds we have available as of the date of the filing of this Quarterly Report on Form 10-Q, we believe that we have sufficient capital to fund our anticipated operating expenses for at least 12 months. We will require additional financing to fund working capital and pay our obligations. We may pursue financing opportunities through the issuance of debt or equity to private investors. There can be no assurance that we will be successful in acquiring additional funding at levels sufficient to fund our operations or on terms favorable to us. Our future funding requirements will depend on many factors, including the following:

- the progress, timing, scope, results and costs of our ongoing and planned clinical trials and other research and development activities related to AG10 and any other product candidates we may identify and pursue, including the ability to enroll patients in a timely manner in our clinical trials;
- the costs of obtaining AG10 in amounts sufficient for our ongoing and planned clinical trials and, if approved, for commercialization;
- the cost, timing and outcomes of any regulatory approvals for AG10;
- our ability to successfully commercialize AG10, if approved;
- the extent to which we may acquire or in-license other product candidates and technologies;
- our ability to attract, hire and retain qualified personnel; and
- the cost of obtaining, maintaining, preparing, filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights related to AG10 and any other product candidates we may identify and pursue.

If we need to raise additional capital to fund our operations, funding may not be available to us on acceptable terms, or at all. If we are unable to obtain adequate financing when needed, we may have to delay, reduce the scope of or suspend one or more of our preclinical studies, research and development programs or commercialization efforts. We may seek to raise any necessary additional capital through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing and distribution arrangements.

To the extent that we raise additional capital through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

Cash flows

The following table summarizes our cash flows for the periods indicated (in thousands):

	Nine Months Ended	
	2018	2017
Net cash used in operating activities	$ (23,705)	$ (6,158)
Net cash used in investing activities	(144)	(6)
Net cash provided by financing activities	184,920	13,008
	$ 161,071	$ 6,844

Cash flows from operating activities

During the nine months ended September 30, 2018, cash used in operating activities was $23.7 million and consisted primarily of a net loss of $36.4 million. Our non-cash charges primary consisted of $6.7 million on extinguishment of debt, a benefit of $1.3 million in regards to the redeemable convertible preferred stock tranche liability revaluation, a cost of $2.6 million in regards to the revaluation of the convertible redeemable preferred stock warrant liability and $2.6 million for stock-based compensation expense. The change in our net operating assets of $1.3 million was primarily due to an increase in accounts payable and accrued expenses of $4.1 million, as a result of an increase in operating expenses and

timing of payments, partially offset by the change in prepaid expenses and other current assets of $2.8 million, due to timing of payments and the timing of activities in regards to the payments.

During the nine months ended September 30, 2017, cash used in operating activities was $6.2 million and consisted primarily of a net loss of $6.8 million, partially offset by a decrease in net operating assets of $0.7 million. The change in our net operating assets of $0.7 million was primarily due to an increase in accounts payable and accrued expenses of $1.4 million as a result of an increase in operating expenses and timing of payments, partially offset by the change in prepaid expenses and other current assets of $0.8 million, due to timing of payments and the timing of activities in regards to the payments.

Cash flows from investing activities

During the nine months ended September 30, 2018 and 2017, cash used in investing activities was $0.1 million and $6,000, respectively, which consisted of our purchase of property and equipment for our office and lab facilities.

Cash flows from financing activities

During the nine months ended September 30, 2018, cash provided by financing activities was $184.9 million, which consisted of net proceeds from the issuance of Series B redeemable convertible preferred stock of $63.9 million and proceeds from the issuance of convertible promissory notes of $10.0 million, and the receipt of funds in connection with our IPO of $111.0 million.

During the nine months ended September 30, 2017, cash provided by financing activities was $13.0 million, which consisted of net proceeds from the issuance of Series Seed redeemable convertible preferred stock.

Contractual Obligations and Other Commitments

During the nine months ended September 30, 2018, there were no material changes to our contractual obligations and commitments described under *Management's Discussion and Analysis of Financial Condition and Results of Operations* in our prospectus filed on June 21, 2018 with the SEC pursuant to Rule 424(b) under the Securities Act.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not have any off-balance sheet arrangements, as defined under SEC rules, including the use of structured finance, special purpose entities or variable interest entities.

Recent Accounting Pronouncements

For information on Recent Accounting Pronouncements refer to Note 2 of Notes to Unaudited Condensed Financial Statements

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities.

Our exposure to market risk is limited primarily to interest income sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because a significant portion of our investments are in interest bearing cash accounts and a mutual fund consisting of short-term debt securities issued by the U.S. government. The primary objective of our investment activities is to preserve principal. At September 30, 2018, we do not have any marketable securities, and therefore we believe that we are not exposed to any material market risk. We do not have any derivative financial instruments or foreign currency instruments. If interest rates had varied by 10% in the nine months ended September 30, 2018, it would not have had a material effect on our results of operations or cash flows for that period.

Item 4. Controls and Procedures.

The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required

to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2018, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q 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.

We are not currently a party to any material litigation or other material legal proceedings.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, together with the other information included in this Quarterly Report on Form 10-Q, including our financial statements and the related notes as well as our other public filings. We cannot assure you that any of the events discussed in the risk factors below will not occur. The occurrence of any of the events or developments described below could have a material and adverse impact on our business, results of operations, financial condition, and cash flows and future prospects and, if so, our future prospects would likely be materially and adversely affected. If any of such events were to happen, the trading price of our common stock could decline, and you could lose all or part of your investment. The risks described below are not the only ones that we may face, and additional risks or uncertainties not known to us or that we currently deem immaterial may also impair our business and future prospects.

Risk related to our financial position and need for additional capital

Drug development is a highly uncertain undertaking and involves a substantial degree of risk. We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have only one product candidate in development and have not generated any revenue since our inception, which, together with our limited operating history, may make it difficult for you to assess our future viability.

We are a clinical development-stage biopharmaceutical company with a limited operating history upon which you can evaluate our business and prospects. We have no products approved for commercial sale and have not generated any revenue from product sales. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. To date, we have focused principally on developing our only product candidate, AG10, which is in clinical development and will require substantial additional development time and resources before we would be able to apply for or receive regulatory approvals and begin generating revenue from product sales.

We are not profitable and have incurred losses in each year since our inception in August 2013. Our net losses for the years ended December 31, 2016 and 2017 and nine months ended September 30, 2018 were $2.5 million, $11.9 million and $36.4 million, respectively. As of September 30, 2018, we had an accumulated deficit of $50.9 million. We have not generated any revenue since our inception and have financed our operations solely through the sale of equity securities and convertible debt. We continue to incur significant research and development and other expenses related to our ongoing operations and expect to incur losses for the foreseeable future. We anticipate these losses will increase significantly and we will not generate any revenue from product sales until after we have successfully completed clinical development and received regulatory approval for the commercial sale of AG10 or any other product candidate that we may identify and pursue.

Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of our expenses, or when we will be able to generate any meaningful revenue or achieve or maintain profitability, if ever. In addition, our expenses could increase beyond our current expectations if we are required by the U.S. Food and Drug Administration, or FDA, or comparable foreign regulatory authorities, to perform studies in addition to those that we currently anticipate, or if there are any delays in any of our or our future collaborators' clinical trials or the development of AG10 or other product candidates that we may identify. Even if AG10 or any future product candidate that we may identify is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate and ongoing compliance efforts.

We may never be able to develop or commercialize a marketable drug or achieve profitability. Revenue from the sale of any product candidate for which regulatory approval is obtained will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain reimbursement at any price and whether we own the commercial rights for that territory. If the number of addressable patients is not as significant as we anticipate, the indication approved by regulatory authorities is narrower than we expect, or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. Even if we are able to generate revenue from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations. Even if we achieve profitability in the future, we may not be able to sustain profitability in

subsequent periods. Our failure to achieve sustained profitability would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our research and development pipeline, market AG10 or any other product candidates we may identify and pursue, if approved, or continue our operations. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. In any particular quarter, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

We will require substantial additional funding to achieve our business goals. If we are unable to obtain this funding when needed and on acceptable terms, we could be forced to delay, limit or terminate our product development efforts.

We are currently advancing AG10, our only clinical development candidate, in a Phase 2 clinical trial. Developing biopharmaceutical products is expensive and time-consuming, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance AG10 in planned and future clinical trials. We are also responsible for license maintenance fees, milestone payments and royalties to Stanford University, or Stanford. Because the outcome of any clinical development and regulatory approval process is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development, regulatory approval process and commercialization of AG10 and any future product candidates we may identify.

Based on current business plans and assuming no financing, we believe that our existing cash and cash equivalents will be sufficient to fund our cash requirements through at least the next twelve months. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other sources, such as strategic collaborations or license and development agreements. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize AG10 and other product candidates that we may identify and pursue. Moreover, such financing may result in dilution to stockholders, imposition of debt covenants and repayment obligations, or other restrictions that may affect our business. In addition, 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.

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

- the time and cost necessary to complete our ongoing Phase 2 clinical trial of AG10 in ATTR-CM, to initiate and complete any pivotal clinical trials of AG10 and to pursue regulatory approvals for AG10, and the costs of post-marketing studies that could be required by regulatory authorities;
- the progress and results of our ongoing Phase 2 and planned Phase 3 clinical trials of AG10;
- the progress, timing, scope and costs of our nonclinical studies, clinical trials and other related activities, including the ability to enroll patients in a timely manner for our Phase 2 clinical trial of AG10 and potential future clinical trials;
- the costs of obtaining clinical and commercial supplies of AG10 and any other product candidates we may identify and develop;
- our ability to successfully commercialize AG10 and any other product candidates we may identify and develop;
- the manufacturing, selling and marketing costs associated with AG10 and any other product candidates we may identify and develop, including the cost and timing of expanding our sales and marketing capabilities;
- the amount and timing of sales and other revenues from AG10 and any other product candidates we may identify and develop, including the sales price and the availability of adequate third-party reimbursement;
- the cash requirements of any future acquisitions or discovery of product candidates;
- the time and cost necessary to respond to technological and market developments;
- the costs of acquiring, licensing or investing in intellectual property rights, products, product candidates and businesses;
- our ability to attract, hire and retain qualified personnel; and
- the costs of maintaining, expanding and protecting our intellectual property portfolio.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit or terminate one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, prospects, financial condition and results of operations.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to AG10 or any future product candidates which we develop on unfavorable terms to us.

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

Risk related to our business and the clinical development, regulatory review and approval of our product candidates

We are heavily dependent on the success of our only product candidate, AG10, and we have not identified any other clinical development candidates through our research activities. If we are unable to successfully complete clinical development, obtain regulatory approval for, or commercialize AG10, or experience delays in doing so, our business will be materially harmed.

To date, we have invested all of our efforts and financial resources to the development of AG10, including conducting preclinical studies and clinical trials and providing general and administrative support for these operations. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and commercialize AG10. Before we can generate any revenues from sales of AG10, we will be required to conduct additional clinical development, including, among other things, additional toxicology studies that may be required before we can conduct longer-term clinical trials and a larger pivotal clinical trial if our ongoing clinical trial of AG10 is successful, seek and obtain regulatory approval, secure adequate manufacturing supply to support larger clinical trials and commercial sales and build a commercial organization. Further, the success of AG10 will depend on patent and trade secret protection, obtaining and maintaining regulatory exclusivity, acceptance of AG10 by patients, the medical community and third-party payors, its ability to compete with other therapies, healthcare coverage and reimbursement, and maintenance of an acceptable safety profile following approval, among other factors. If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize AG10, which would materially harm our business.

Currently, AG10 is our only product candidate, and it may be years before we can advance AG10 into a pivotal trial, if at all. We have not yet identified any other product candidates for studies that would enable the filing of an investigational new drug application, or IND, or for clinical evaluation. We cannot be certain that AG10 will be successful in clinical trials or receive regulatory approval. If we do not receive regulatory approval for, or otherwise fail to successfully commercialize, AG10, we may need to discontinue our operations as currently contemplated unless we identify other product candidates, advance them through preclinical and clinical development and apply for regulatory approvals, which could be time-consuming and costly, and may adversely affect our business, prospects, financial condition and results of operations.

If we are unable to obtain regulatory approval in one or more jurisdictions for AG10 or any other product candidates that we may identify and develop, our business will be substantially harmed.

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Approval by the FDA and comparable foreign regulatory authorities is lengthy and unpredictable and depends upon numerous factors. 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. We have not obtained regulatory approval for AG10, and it is possible that neither AG10 nor any other product candidates which we may seek to develop in the future will ever obtain regulatory approval.

Applications for AG10 or any other product candidates we may develop could fail to receive regulatory approval for many reasons, including but not limited to:

- our inability to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that AG10 or any other product candidate we may develop is safe and effective;
- the FDA or comparable foreign regulatory authorities may disagree with the design, endpoints or implementation of our clinical trials, including those of our planned Phase 3 clinical trial;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA's or comparable foreign regulatory authorities' requirement for additional preclinical studies or clinical trials beyond those that we currently anticipate;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- the data collected from clinical trials of AG10 and other product candidates that we may identify and pursue may not be sufficient to support the submission of a new drug application, or NDA, or other submission for regulatory approval in the United States or elsewhere;
- we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may change in a manner that renders our clinical trial design or data insufficient for approval.

The lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failure to obtain regulatory approval to market AG10 or any other product candidates that we may pursue in the United States or elsewhere, which would significantly harm our business, prospects, financial condition and results of operations.

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

Clinical testing is expensive, time consuming, and subject to uncertainty. We cannot guarantee that any of our ongoing and planned clinical trials will be conducted as planned or completed on schedule, if at all. Moreover, even if these trials are initiated or conducted on a timely basis, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical trials can occur at any stage of testing, and our ongoing and future clinical trials may not be successful. Events that may prevent successful or timely initiation or completion of clinical trials include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of clinical trials;
- delays in confirming target engagement, patient selection or other relevant biomarkers to be utilized in preclinical and clinical product candidate development;
- delays in reaching a consensus with regulatory agencies on study design;

- delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in identifying, recruiting and training suitable clinical investigators;
- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical trial site;
- imposition of a temporary or permanent clinical hold by regulatory agencies for a number of reasons, including after review of an IND or amendment, clinical trial application, or CTA, or amendment, or equivalent application or amendment; as a result of a new safety finding that presents unreasonable risk to clinical trial participants; a negative finding from an inspection of our clinical trial operations or study sites;
- developments in trials for other drug candidates targeting ATTR conducted by competitors that raise regulatory or safety concerns about risk to patients of the treatment, including the approach of TTR stabilization; or if the FDA finds that the investigational protocol or plan is clearly deficient to meet its stated objectives;
- delays in identifying, recruiting and enrolling suitable patients to participate in our clinical trials, and delays caused by patients withdrawing from clinical trials or failing to return for post-treatment follow-up;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's or any other regulatory authority's current good clinical practices, or cGCP, requirements, or regulatory guidelines in other countries;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- the cost of clinical trials of AG10 or any our product candidates that we may identify and pursue being greater than we anticipate;
- clinical trials of AG10 or any other product candidates that we may identify and pursue producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon product development programs;
- transfer of manufacturing processes to larger-scale facilities operated by a contract manufacturing organization, or CMO, or by us, and delays or failure by our CMOs or us to make any necessary changes to such manufacturing process; and
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of AG10 or other product candidates that we may identify for use in clinical trials or the inability to do any of the foregoing.

Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to AG10 or other product candidates that we may identify, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize AG10 or other product candidates that we may identify and may harm our business and results of operations.

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

Delays in the initiation, conduct or completion of any clinical trial of AG10 or other product candidates that we may develop will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of AG10 or any future product candidates which we may develop. In the event we identify any additional product candidates to pursue, we cannot be sure that submission of an IND or a CTA will result in the FDA or comparable foreign regulatory authority allowing clinical trials to begin in a timely manner, if at all. Any of these events could have a material adverse effect on our business, prospects, financial condition and results of operations.

Our clinical trials may fail to demonstrate substantial evidence of the safety and effectiveness of AG10 or any other product candidates that we may identify and pursue, which would prevent, delay or limit the scope of regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of AG10 or any other product candidate that we may identify and pursue, we must demonstrate through lengthy, complex and expensive preclinical studies and clinical trials that the applicable product candidate is both safe and effective for use in each target indication. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical development process. Most product candidates that begin clinical trials are never approved by regulatory authorities for commercialization. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. We cannot be certain that our current clinical trials or any other future clinical trials will be successful. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operations. In addition, even if such clinical trials are successfully completed, we cannot guarantee that the FDA or comparable foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. To the extent that the results of the trials are not satisfactory to the FDA or comparable foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates. Even if regulatory approval is secured for AG10 or any other product candidate we may identify and pursue, the terms of such approval may limit the scope and use of our product candidate, which may also limit its commercial potential.

Results of earlier studies or clinical trials may not be predictive of future clinical trial results, and initial studies or clinical trials may not establish an adequate safety or efficacy profile for AG10 and other product candidates that we may pursue to justify proceeding to advanced clinical trials or an application for regulatory approval.

The results of nonclinical and preclinical studies and Phase 1 or Phase 2 clinical trials of AG10 or any other product candidates that we may pursue may not be predictive of the results of later-stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. For example, our preclinical and preliminary clinical observations that AG10 potently stabilizes TTR in human serum may not be replicated in later stage clinical trials.

Additionally, some of our preclinical studies in which AG10 demonstrated greater TTR stabilization and inhibition of amyloid fibril formation than tafamidis were conducted using synthesized, research-grade tafamidis and therefore may not be indicative of the comparative efficacy of AG10 to commercially available tafamidis. The results of clinical trials in one set of patients or disease indications may not be predictive of those obtained in another. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. In addition, preclinical and clinical data are often susceptible to various 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. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies, and we cannot be certain that we will not face similar setbacks. Even if early stage clinical trials are successful, we may need to conduct additional clinical trials of AG10 or other product candidates that we may pursue in additional patient populations or under different treatment conditions before we are able to seek approvals from the FDA and regulatory authorities outside the United States to market and sell these product candidates. Our failure obtain marketing approval for AG10 or any other product candidate we may choose to develop in our ongoing and any future clinical trials would substantially harm our business, prospects, financial condition and results of operations.

We may encounter difficulties enrolling patients in our clinical trials, and our clinical development activities could thereby be delayed or otherwise adversely affected.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the applicable clinical trial protocols, which may limit the patient populations eligible for our clinical trials to a greater extent than competing clinical trials for the same indication;
- the size of the study population required for analysis of the trial's primary endpoints;
- the proximity of patients to a trial site;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- the approval of competing product candidates currently under development for ATTR, or competing clinical trials for similar therapies or targeting patient populations meeting our patient eligibility criteria;
- clinicians' and patients' perceptions as to the potential advantages and side effects of the product candidate being studied in relation to other available therapies and product candidates;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will not complete such trials, for any reason.

If we have difficulty enrolling sufficient numbers of patients to conduct our clinical trials as planned, we may need to delay or terminate ongoing or planned clinical trials, either of which would have an adverse effect on our business.

If serious adverse events or unacceptable side effects are identified during the development of AG10 or other product candidates that we may develop, we may need to delay, limit or terminate our clinical development activities.

Clinical trials by their nature utilize a sample of the potential patient population. Our Phase 2 clinical trial of AG10 for ATTR-CM is designed to enroll approximately 45 subjects. Subject to the successful completion of our Phase 2 clinical trial of AG10 in ATTR-CM and authorization from applicable regulatory authorities, we also plan to initiate a Phase 3 clinical trial of AG10 in up to 130 symptomatic ATTR-PN subjects in early 2019. To date, we have only begun to evaluate AG10 in a limited number of subjects at a limited duration of exposure in our Phase 1 clinical trial and the duration of exposure in our Phase 2 and 3 clinical trials is expected to be significantly longer. Accordingly, any rare and severe side effects of AG10 may be uncovered in later stages of our Phase 2 clinical trial or in any larger, subsequent trials that we may conduct, such as our planned Phase 3 clinical trial of AG10 for ATTR-PN. Additionally, although our animal safety pharmacology studies of AG10 demonstrated a wide safety margin between anticipated therapeutic exposures and doses associated with toxicity and no dose limiting toxicities were established in the 90 day GLP toxicology dog study, in prior toxicology studies of shorter duration, at doses above the no adverse effect level, dogs experienced dose limiting toxicities of gastrointestinal effects including vomiting, dehydration and weight loss. Many product candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented their further development. If AG10 or any product candidates that we may develop are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit their 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, which could adversely affect our business, prospects, financial condition and results of operations.

We may in the future conduct clinical trials for AG10 or other product candidates that we may identify outside the United States, and the FDA and comparable foreign regulatory authorities may not accept data from such trials.

We may in the future choose to conduct one or more of our clinical trials outside the United States, including in Europe. For instance, subject to the successful completion of our Phase 2 clinical trial of AG10 in ATTR-CM and authorization from applicable regulatory authorities, we plan to initiate a Phase 3 clinical trial of AG10 in up to 130 symptomatic ATTR-PN subjects in early 2019. We do not intend to file an IND with the FDA in connection with this clinical trial as it will be conducted outside of the United States. The acceptance of study data from clinical trials conducted outside the United

States or another jurisdiction by the FDA or comparable foreign regulatory authority may be subject to certain conditions. In cases where data from foreign clinical trials are intended to serve as the basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; and (ii) the trials were performed by clinical investigators of recognized competence and pursuant to cGCP regulations. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction, including our planned Phase 3 clinical trial of AG10 in ATTR-PN. If the FDA or any comparable 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 AG10 or other product candidates that we may develop not receiving approval or clearance for commercialization in the applicable jurisdiction.

Even if we obtain FDA approval for AG10 or any other product candidates that we may identify and pursue in the United States, we may never obtain approval to commercialize AG10 or other product candidates that we may develop outside of the United States, which would limit our ability to realize their full market potential.

In order to market any products outside of the United States, we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and effectiveness. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional non-clinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of AG10 or any other product candidates that we may identify and pursue in those countries. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approval in international markets is delayed, our target market will be reduced and our ability to realize the full market potential of our products will be harmed.

Although the FDA has granted orphan drug designation for AG10 and the EMA adopted a positive opinion for the designation of AG10 as an orphan medicinal product in the EU for the treatment of transthyretin amyloidosis, we may not receive orphan drug designation for AG10 in the European Union or for any other product candidates for which we may submit orphan drug designation requests, and any orphan drug designations that we have received or may receive in the future may not confer marketing exclusivity or other expected commercial benefits for AG10 or any of our other product candidates.

Our business strategy focuses on the development of product candidates for the treatment of transthyretin amyloidosis that may be eligible for FDA or EU orphan drug designation. 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 drug as an orphan drug if it is 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. In the EU, the Committee for Orphan Medicinal Products of the EMA grants orphan drug designation to promote the development of medical products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the EU (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention, or treatment is authorized or, if a method exists, the product would be of significant benefit to those affected by the condition. In October 2018, the FDA granted orphan drug designation to AG10 in the United States for the treatment of ATTR, and the EMA adopted a positive opinion for the designation of AG10 as an orphan medicinal product in the EU for the treatment of ATTR. Although the diagnosed ATTR patient population in the United States is currently below 200,000, if the size of the population is shown to be greater as a result of increased rates of diagnosis or otherwise, ATTR may not in the future qualify as an orphan indication for any other product candidate we pursue.

Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA, the EMA or comparable foreign regulatory authority from approving another marketing application for the same drug for the

same indication for that time period. The applicable period is seven years in the United States and 10 years in the EU. The EU 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. Orphan drug exclusivity may be lost if the FDA, the EMA or comparable foreign regulatory authority determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Although the FDA has granted orphan drug designation to AG10 and the EMA adopted a positive opinion for the designation of AG10 as an orphan medicinal product in the EU for the treatment of transthyretin amyloidosis, we may apply for orphan drug designation for AG10 in other jurisdictions, or for other product candidates we may develop and pursue in the future. Applicable regulatory authorities may not grant us these additional designations. In addition, the exclusivity granted under any orphan drug designation that we have received from the FDA or may receive from any other regulatory authorities, including the EMA, may not effectively protect AG10 or any other product candidate that we may develop and pursue from competition because different drugs can be approved for the same condition and the same drug can be approved for different conditions but used off-label. Even after an orphan drug is approved, the FDA can subsequently approve the same drug 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. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Further, orphan drug designation neither shortens the development or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Any inability to secure or maintain orphan drug designation or the exclusivity benefits of this designation would have an adverse impact on our ability to develop and commercialize our product candidates. In addition, even if any orphan drug designations we receive are maintained, we may be unable to realize significant commercial benefits from these orphan drug designations or exclusivities for AG10 (if approved) or any other product candidate we pursue.

We may not elect or be able to take advantage of any expedited development or regulatory review and approval processes available to product candidates granted breakthrough therapy or fast track designation by the FDA.

We intend to evaluate and continue ongoing discussions with the FDA on regulatory strategies to rapidly advance the development of AG10. For example, potential expedited development pathways include breakthrough therapy or fast track designation. The breakthrough therapy program is designed for product candidates intended to treat a serious or life-threatening condition, and preliminary clinical evidence indicates that the product candidate may demonstrate substantial improvement on a clinically significant endpoint(s) over available therapies. The fast track program is designed for product candidates that treat a serious or life-threatening condition, and nonclinical or clinical data demonstrate the potential to address an unmet medical need. Although we believe AG10 could potentially qualify under either or both of the breakthrough therapy and fast track programs, we may elect not to pursue either of these programs, and the FDA has broad discretion whether or not to grant these designations. Accordingly, even if we believe a particular product candidate is eligible for breakthrough therapy or fast track designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive breakthrough therapy or fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw breakthrough therapy or fast track designation if it believes that the product no longer meets the qualifying criteria. Our business may be harmed if we are unable to avail ourselves of these or any other expedited development and regulatory pathways.

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

If AG10 or other product candidates that we may develop are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

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

Any regulatory approvals that we receive for AG10 or other product candidates that we may develop will be subject to limitations on the approved indicated uses for which the product may be marketed and promoted or to the conditions of approval, or contain requirements for potentially costly post-marketing testing. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have approval. The holder of an approved NDA or MAA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical trials to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval was obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical trial to confirm clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

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

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

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

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.

If AG10 or other product candidates that we may identify are approved and we are found to have improperly promoted off-label uses of those products, we may become subject to significant liability. The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as AG10 if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

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

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

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

In addition, if we undertake such a transaction, we may 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.

Risks related to our reliance on third parties

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

We currently rely and expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions, and clinical investigators, to conduct some aspects of our research and preclinical testing and our clinical trials. Any of these third parties may terminate their engagements with us or be unable to fulfill their contractual obligations. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, or at all. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA and comparable foreign regulatory authorities require us to comply with cGCPs for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible, reproducible and accurate and that the rights, integrity, and confidentiality of trial participants are protected. For any violations of laws and regulations during the conduct of our clinical trials, we could be subject to untitled and warning letters or enforcement action that may include civil penalties up to and including criminal prosecution. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database within certain timeframes. Failure to do so can result in fines, adverse publicity, and civil and criminal sanctions.

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

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

We rely entirely on third parties for the manufacturing of AG10 or other product candidates that we may develop for preclinical studies and clinical trials and expect to continue to do so for commercialization. Our business could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture drug supplies for our ongoing Phase 2 clinical trial of AG10 or any future clinical trials that we may conduct, and we lack the resources to manufacture any product candidates on a commercial scale. We rely, and expect to continue to rely, on third-party manufacturers to produce AG10 or other product candidates that we may identify for our clinical trials, as well as for commercial manufacture if any of our product candidates receives marketing approval. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the trial, any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay the clinical development and potential regulatory approval of our product candidates, which could harm our business and results of operations. We also expect to rely on third parties for the manufacturing of commercial supply of AG10 or any other product candidates, if approved.

We may be unable to establish any 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:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or non-renewal of the agreement by the third party at a time that is costly or inconvenient for us.

Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks for the production of such materials and products. As a result, failure to meet the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally. If the FDA or a comparable foreign regulatory agency does not approve these facilities for the manufacture of our product candidates or if any agency withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would negatively impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

AG10 and any future product candidates that we may develop may compete with other product candidates and marketed drugs for access to manufacturing facilities. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We are currently manufacturing AG10 through a third party and have adequate supplies to conduct our ongoing Phase 2 clinical trial. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. If we are unable to enter into relationships with additional contract manufacturers, or our current or future contract manufacturers cannot perform as agreed, we may experience delays and incur additional costs in our clinical development and commercialization activities. Our current and anticipated future dependence upon others for the manufacturing of AG10 or other product candidates that we may identify, or marketed drugs may adversely affect our future profit margins and our ability to commercialize any product candidates that receive marketing approval on a timely and competitive basis.

If the contract manufacturing facilities on which we rely do not continue to meet regulatory requirements or are unable to meet our supply demands, our business will be harmed.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for AG10, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP, or similar regulatory requirements outside the United States. These regulations govern manufacturing processes and procedures, including recordkeeping, and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of AG10. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, suspension of production, seizures or recalls of product candidates or marketed drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect clinical or commercial supplies of AG10.

We or our contract manufacturers must supply all necessary documentation in support of an NDA or MAA on a timely basis and must adhere to regulations enforced by the FDA and other regulatory agencies through their facilities inspection program. Some of our contract manufacturers have never produced a commercially approved pharmaceutical product and therefore have not obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of AG10 or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of AG10 or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we oversee the contract manufacturers, we cannot control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the products may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

Additionally, if supply from one approved manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical trials, regulatory submissions, required approvals, or commercialization of AG10 or other product candidates that we may identify. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Risks related to our intellectual property

If we are unable to obtain and maintain sufficient intellectual property protection for AG10 or other product candidates that we may identify, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize product candidates similar or identical to ours, and our ability to successfully commercialize AG10 and other product candidates that we may pursue may be impaired.

As is the case with other biopharmaceutical companies, our success depends in large part on our ability to obtain and maintain protection of the intellectual property we may own solely and jointly with others, particularly patents, in the United States and other countries with respect to our product candidates and technology. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to AG10 or other product candidates that we may identify.

Obtaining and enforcing biopharmaceutical patents is costly, time consuming and complex, and we may not be able to file and prosecute all necessary or desirable patent applications, or maintain, enforce and license any patents that may issue from such patent applications, at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal, technological and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. Further, we may not be aware of all third-party intellectual property rights potentially relating to our product candidates. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive product candidates. Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative product candidates in a non-infringing manner.

Moreover, we may be subject to a third-party preissuance submission of prior art to the United States Patent and Trademark Office, or the USPTO, or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, 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 drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical product candidates, or limit the duration of the patent protection of our product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

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

We are a party to an exclusive license agreement with Stanford and may need to obtain additional licenses from others to advance our research and development activities or allow the commercialization of AG10 or any other product candidates we may identify and pursue. Our license agreement with Stanford imposes, and we expect that future license agreements will impose, various development, diligence, commercialization, and other obligations on us. For example, under our license agreement with Stanford we are required to use commercially reasonable efforts to engage in various development and commercialization activities with respect to licensed products, and must satisfy specified milestone and royalty payment obligations. In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If our license agreement with Stanford is terminated, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to AG10 and we may be required to cease our development and commercialization of AG10. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Moreover, disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our product candidates, technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, certain provisions in our license agreement with Stanford 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 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.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our 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. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and inter partes reexamination proceedings before the USPTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that AG10 or other product candidates that we may identify may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. 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 AG10 or other product candidates that we may identify. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that AG10 or other product candidates that we may identify may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of AG10 or other product candidates that we may identify, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all, or it may be non-exclusive, which could result in our competitors gaining access to the same intellectual property.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize AG10 or other product candidates that we may identify. 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. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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 material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

Patent 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, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we are not able to obtain patent term extension or non-patent exclusivity in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for AG10 or other product candidates that we may identify, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of AG10 or other product candidates that we may identify, one of the U.S. patents covering each of such product candidates or the use thereof may be eligible for up to five years of patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority 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, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration sooner, and our revenue could be reduced, possibly materially.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a U.S. patent covering AG10 or other product candidates that we may identify even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for our licensed patents, we do not have the right to control prosecution, including filing with the USPTO, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO.

Also, there are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Approved Drug Products with Therapeutic Equivalence Evaluations, or the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of our product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, a manufacturer of generic drugs would not have to provide advance notice to us of any abbreviated new drug application filed with the FDA to obtain permission to sell a generic version of such product candidate.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

We seek to protect our confidential proprietary information, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and collaborators. These agreements are designed to protect our proprietary information. However, we cannot be certain that such agreements have been entered into with all relevant parties, and we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. For example, 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. We also seek to preserve the integrity and confidentiality of our confidential proprietary information by maintaining physical security of our premises and physical and electronic security of our information technology systems, but it is possible that these security measures could be breached. If any of our confidential proprietary information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Although we are not currently involved in any litigation, we may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents or other intellectual property. Although we are not currently involved in any litigation, if we were to initiate legal proceedings against a third party to enforce a patent covering AG10 or other product candidates that we may identify, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. Interference or 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. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference or 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 litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring AG10 or other product candidates that we may identify to market. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

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

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets, or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of AG10 or other product candidates that we may identify, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover AG10 or other product candidates that we may identify. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industry, we employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. 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 impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. governmental patent agencies 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 the applicable rules. However, there are situations in which non-compliance 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. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

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

Filing, prosecuting and defending patents on our product candidates 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 may also export 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 products and our patents 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 certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith America Invents Act, or the America Invents Act, enacted in September 2011, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Risks related to commercialization

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

The commercial success of AG10 or other product candidates that we may identify will depend upon its degree of market acceptance by physicians, patients, third-party payors, and others in the medical community. Even if any product candidates we may develop receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors, and others in the medical community. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in pivotal clinical trials and published in peer-reviewed journals;
- the potential and perceived advantages compared to alternative treatments;
- the ability to offer our products for sale at competitive prices;
- the ability to offer appropriate patient access programs, such as co-pay assistance;
- the extent to which physicians recommend our products to their patients;

- convenience and ease of dosing and administration compared to alternative treatments;
- the clinical indications for which the product candidate is approved by FDA or comparable regulatory agencies;
- product labeling or product insert requirements of the FDA or other comparable foreign regulatory authorities, including any limitations, contraindications or warnings contained in a product's approved labeling;
- restrictions on how the product is distributed;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- the strength of marketing and distribution support;
- favorable third-party coverage and sufficient reimbursement; and
- the prevalence and severity of any side effects.

If any product candidates we develop do not achieve an adequate level of acceptance, we may not generate significant product revenue, and we may not become profitable.

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

We do not have a sales or marketing infrastructure and have little experience in the sale, marketing, or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales, marketing, and commercial support infrastructure to market and sell AG10 and any other product candidates we may identify, if and when they are approved. We may also elect to enter into collaborations or strategic partnerships with third parties to engage in commercialization activities, although there is no guarantee we will be able to enter into these arrangements even if we intend to do so.

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

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

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

If we enter into arrangements with third parties to perform sales, marketing, commercial support, and distribution services, our product revenue or the profitability of product revenue may be lower than if we were to market and sell any products we may develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to commercialize AG10 or other product candidates that we may identify or may be unable to do so on terms that are

favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish commercialization capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates if approved.

The insurance coverage and reimbursement status of newly-approved products is uncertain. AG10 and any other product candidates that we may develop may become subject to unfavorable pricing regulations, third-party reimbursement practices, or healthcare reform initiatives, which would harm our business. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

Our ability to successfully commercialize AG10 or any other products that we may develop also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford treatments such as AG10. Sales of AG10 or other product candidates that we may identify will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize AG10 or any other product candidates we may identify. 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 a sufficient return on our investment.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, 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 AG10 or other product candidates that we may identify. 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 revenues and profits.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. No uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize product candidates, and our overall financial condition.

Government authorities currently impose mandatory discounts for certain patient groups, such as Medicare, Medicaid and Veterans Affairs, or VA, hospitals, and may seek to increase such discounts at any time. Future regulation both domestically and abroad may negatively impact the price of our products, if approved. 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. We cannot be sure that reimbursement will be available for any product candidate that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. In order to obtain reimbursement, physicians may need to show that patients have superior treatment outcomes with our products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

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

If we fail to comply with healthcare laws, we could face substantial penalties and our business, operations and financial conditions could be adversely affected.

Our ongoing and planned operations, including clinical research, sales, marketing and promotion of AG10 or other product candidates that we may identify and begin commercializing in the United States, may subject us to various federal and state fraud and abuse laws and other healthcare laws. The laws that may impact our operations include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the False Claims Act, which impose criminal and civil penalties, including through civil “qui tam” or “whistleblower” actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false or fraudulent; knowingly making or causing a false statement material to a false or fraudulent claim or an obligation to pay money to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing such an obligation. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of these statutes or specific intent to violate them in order to have committed a violation;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;

- the federal Physician Payments Sunshine Act, created under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program to report annually to the U.S. Department of Health and Human Services under the Open Payments Program, information related to payments or other transfers of value made to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payer, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could, despite our efforts to comply, be subject to challenge under one or more of such laws. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and 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 civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, integrity and oversight agreements to resolve allegations of non-compliance, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Healthcare legislative measures aimed at reducing healthcare costs may have a material adverse effect on our business and results of operations.

Third-party payors, whether domestic or foreign, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010 the ACA was enacted, which, among other things increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research.

There have been a number of significant changes to the ACA and its implementation. The Tax Cuts and Jobs Act of 2017, or Tax Act, includes a provision repealing effective January 1, 2019 the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from

terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Moreover, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, also amends the ACA, effective January 1, 2019, by increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". Similarly, on April 9, 2018, CMS issued a final rule that will give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces by relaxing certain requirements for essential health benefits required under the ACA for plans sold through such marketplaces. Congress will likely consider additional legislation to repeal, replace, or modify other elements of the ACA. The implications of the ACA, its possible repeal, replacement, or modification, and the political uncertainty surrounding these matters for our business and financial condition, if any, are not yet clear.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in 2013, and, due to subsequent legislative amendments, will remain in effect through 2027 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012 further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for AG10 or other product candidates that we may identify, if we obtain regulatory approval;
- our ability to receive or set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, lower reimbursement, and new payment methodologies. This could lower the price that we receive for any approved product. Any denial in coverage or reduction in reimbursement from Medicare or other government-funded programs may result in a similar denial or reduction in payments from private payors, which may prevent us from being able to generate sufficient revenue, attain profitability or commercialize AG10 or other product candidates that we may identify, if approved.

We face significant competition in an environment of rapid technological and scientific change, and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer, more advanced or more effective than ours, which may negatively impact our ability to successfully market or commercialize any product candidates we may develop and ultimately harm our financial condition.

The development and commercialization of new drug products is highly competitive. We may face competition with respect to any product candidates that we seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies, and other public and private research organizations that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing, and commercialization.

There are a number of large pharmaceutical and biotechnology companies that are currently pursuing the development of products for the treatment of ATTR. Companies that we are aware are developing therapeutics for ATTR include large companies with significant financial resources, such as Pfizer Inc., Alnylam Pharmaceuticals Inc., Ionis Pharmaceuticals Inc./Akcea Therapeutics, Inc., Corino Therapeutics Inc./SOM Innovation Biotech, S.L., GlaxoSmithKline plc, Intellia Therapeutics Inc., Arcturus Therapeutics Inc., Neurimmune Holding AG and Prothena Therapeutics plc. In particular, in March 2018, Pfizer announced that its Phase 3 clinical trial of tafamidis in ATTRwt-CM and ATTRm-CM patients (ATTR-ACT) reportedly met its primary endpoint of a reduction in the combination of all-cause mortality and cumulative incidence of cardiovascular-related hospitalizations. If tafamidis receives FDA approval for one or both forms of ATTR-CM, AG10 would not be the first treatment on the market for ATTR, and its market share may be limited. In addition to competition from other companies targeting ATTR, any products we may develop may also face competition from other types of therapies.

Many of our current or potential competitors, either alone or with their strategic partners, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than any products that we may develop. Furthermore, currently approved products could be discovered to have application for treatment of TTR, which could give such products significant regulatory and market timing advantages over AG10 or other product candidates that we may identify. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours and may obtain orphan product exclusivity from the FDA for indications, other than treatment of transthyretin amyloidosis, that AG10 or other product candidates that we may identify are targeting, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, products or technologies developed by our competitors may render our potential product candidates uneconomical or obsolete and we may not be successful in marketing any product candidates we may develop against competitors.

In addition, we could face litigation or other proceedings with respect to the scope, ownership, validity and/or enforceability of our patents relating to our competitors' products and our competitors may allege that our products infringe, misappropriate or otherwise violate their intellectual property. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize. See "Risks related to our intellectual property."

If the market opportunities for AG10 are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Our ability to successfully identify patients and acquire a significant market share will be necessary for us to achieve profitability and growth.

We focus our research and product development on treatments for ATTR. Our projections of both the number of individuals who have a form of ATTR, as well as the subset of individuals with a form of ATTR who have the potential to benefit from treatment with AG10 or other product candidates that we may identify, are based on our beliefs and estimates, including our belief that the availability of minimally invasive diagnostics will result in increased rates of diagnosis for ATTR. These estimates have been derived from a variety of sources, including the scientific literature, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. 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. Additionally, the potentially addressable patient population for AG10 or other product candidates that we may identify may be limited or may not be amenable to treatment with AG10 or other product candidates that we may identify, 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 AG10 or other product candidates that we may identify, because the potential target populations are small, we may never achieve profitability despite obtaining such significant market share. In addition, our market share could be limited by the availability of other treatments for ATTR, such as tafamidis, that could receive regulatory approval or otherwise be commercially launched before AG10.

Risks related to our business and industry

Our future success depends on our ability to retain key management, employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, research and development, clinical, financial and business development expertise of our executive officers, as well as the other members of our scientific and clinical teams. However, some of these executive officers and other personnel are not our full-time employees. For example, Neil Kumar, our Chief Executive Officer, who does not directly receive any cash or equity compensation from us, is employed by BridgeBio and spends a portion of his time on other BridgeBio matters, including involvement with other BridgeBio subsidiaries. Christine Siu, our Chief Financial Officer, also serves as the Chief Operating Officer for other BridgeBio subsidiaries. Uma Sinha, our Chief Scientific Officer, also serves as the Chief Scientific Officer of BridgeBio and other BridgeBio subsidiaries. Jonathan Fox, our Chief Medical Officer, also serves as the Therapeutic Area Lead of Cardiovascular and Renal Diseases for BridgeBio. As a result, these executive officers may not be able to devote their full attention to our Company, which could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Since joining us, all of our executives, including Dr. Kumar, have each spent the majority of their time devoted to us. While none of the executives has a minimum time commitment to us, each retains flexibility to ensure that he or she can re-allocate his or her time based on the needs of each business. The particulars of these executives' time-allocation strategy may change over time based on these needs or the executives' individual incentives to provide services to us relative to other businesses. The risks related to our dependence upon Dr. Kumar are compounded by BridgeBio's significant ownership percentage and Dr. Kumar's role in our company, as well as the absence of any contract between us and Dr. Kumar for his services. If we were to lose Dr. Kumar or any of our other executives or key personnel, we may not be able to find appropriate replacements on a timely basis and our financial condition and results of operations could be materially adversely affected. Furthermore, although we have employment offer letters with each of our executive officers other than Dr. Kumar, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or employees. Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our drug pipeline toward scaling up for commercialization, sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval for and commercialize AG10 or other product candidates that we may identify. Competition to hire qualified personnel in our industry is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. Furthermore, to the extent we hire personnel from competitors, we may be subject to allegations that they have been improperly solicited or that they have divulged proprietary or other confidential information, or that their former employers own their research output. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Certain of our directors and officers may have actual or potential conflicts of interest because of their positions with BridgeBio.

Neil Kumar, founder and Chief Executive Officer of BridgeBio, Ali Satvat, a member of the Board of Managers of BridgeBio and Hoyoung Huh, a member of BridgeBio, serve on our board of directors and retain their positions and affiliations with BridgeBio. Similarly, Christine Siu, our Chief Financial Officer, also serves as the Chief Operating Officer for other BridgeBio subsidiaries. In addition, certain of these individuals own equity interests in BridgeBio, which represent a significant portion of these individuals' net worth, while Dr. Kumar, in particular, does not currently hold any direct equity interest in us. These individuals' respective positions at BridgeBio and the ownership of any BridgeBio equity or equity awards creates, or may create the appearance of, conflicts of interest when we ask these individuals to make decisions that could have different implications for BridgeBio than the decisions have for us.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2018, we had 21 full-time employees. As we mature, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time toward managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

We may expend our limited resources to pursue a particular product candidate and fail to capitalize on development opportunities or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and personnel resources, we are placing significant focus on the development of our product candidate, AG10. As a result, we may forgo or delay pursuit of opportunities with other future product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and other future product candidates for specific indications may not yield any commercially viable future product candidates. If we do not accurately evaluate the commercial potential or target market for a particular future product candidate, we may relinquish valuable rights to that future product candidates through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such future product candidates.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of AG10 or other product candidates that we may identify in human clinical trials and will face an even greater risk if we commercially sell any medicines that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or medicines caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or medicines that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize AG10 or any other product candidates that we may develop.

Although we maintain product liability insurance, including coverage for clinical trials that we sponsor, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage as we commence additional clinical trials and if we successfully commercialize any product candidates. The market for insurance coverage is increasingly expensive, and the costs of insurance coverage will increase as our clinical programs increase in size. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations

involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We 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 for failure to comply with such laws and regulations.

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 biological, hazardous or radioactive 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 production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our ability to invest in and expand our business and meet our financial obligations, to attract and retain third-party contractors and collaboration partners and to raise additional capital depends on our operating and financial performance, which, in turn, is subject to numerous factors, including the prevailing economic and political conditions and financial, business and other factors beyond our control, such as the rate of unemployment, the number of uninsured persons in the United States, political influences and inflationary pressures. For example, an overall decrease in or loss of insurance coverage among individuals in the United States as a result of unemployment, underemployment or the repeal of certain provisions of the ACA, may decrease the demand for healthcare services and pharmaceuticals. If fewer patients are seeking medical care because they do not have insurance coverage, we may experience difficulties in any eventual commercialization of our product candidates and our business, results of operations, financial condition and cash flows could be adversely affected.

In addition, our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets upon which biopharmaceutical companies such as us are dependent for sources of capital. In the past, global financial crises have caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including a reduced ability to raise additional capital when needed on acceptable terms, if at all, and weakened demand for AG10 or other product candidates that we may identify. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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

Despite the implementation of security measures, our internal computer systems and those of our future CROs and other contractors and consultants may be vulnerable to damage from computer viruses and unauthorized access. Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on our third-party research institution collaborators for research and development of AG10 and other third parties for the manufacture of AG10 and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of AG10 could be delayed.

Certain data breaches must also be reported to affected individuals and the government, and in some cases to the media, under provisions of HIPAA, as amended by HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive, and financial penalties may also apply.

Our insurance policies may not be adequate to compensate us for the potential losses arising from breaches, failures or disruptions of our infrastructure, catastrophic events and disasters or otherwise. 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 defending a suit, regardless of its merit, could be costly and divert management's attention.

Furthermore, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on other third parties for the manufacture of AG10 and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business.

We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

Our anticipated international operations may expose us to business, regulatory, political, operational, financial, pricing and reimbursement and economic risks associated with doing business outside of the United States.

We currently have no international operations, but our business strategy incorporates potential international expansion to target ATTR patient populations outside the United States. If we receive regulatory approval for and commercialize AG10 in patient populations outside the United States, we may hire sales representatives and conduct physician and patient association outreach activities outside of the United States. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations;
- natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our potential international expansion and operations and, consequently, our results of operations.

If we fail to maintain an effective system of disclosure controls and internal control over financial reporting, our ability to produce timely and accurate financial statements or comply with applicable regulations could be impaired.

As a public company, we will be required to maintain internal control over financial reporting and to report any material weaknesses in such internal controls. Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, requires that we evaluate and determine the effectiveness of our internal control over financial reporting and, provide a management report on internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm.

Any failure to develop or maintain effective controls, or any difficulties encountered in their implementation or improvement, could harm our results of operations, cause us to fail to meet our reporting obligations, result in a restatement of our financial statements for prior periods, or adversely affect the results of management evaluations and independent registered public accounting firm audits of our internal control over financial reporting that we will eventually be required to include in our periodic reports that will be filed with the SEC. Ineffective disclosure controls and procedures and internal controls over financial reporting could also cause investors to lose confidence in our reported financial and other information, which would likely have a negative effect on the trading price of our common stock.

We are in the process of designing and implementing the internal control over financial reporting required to comply with Section 404 of the Sarbanes-Oxley Act. This process will be time consuming, costly, and complicated. If we are unable to assert that our internal control over financial reporting is effective or when required in the future, if our independent registered public accounting firm issues an adverse opinion on the effectiveness of our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock could be adversely affected and we could become subject to investigations by the stock exchange on which our securities are listed, the SEC, or other regulatory authorities, which could require additional financial and management resources.

The report of our independent registered public accounting firm on our 2017 financial statements contains an explanatory paragraph regarding our ability to continue as a going concern, and we will need additional financing to execute our business plan, to fund our operations and to continue as a going concern.

Since inception, we have experienced recurring operating losses and negative cash flows and we expect to continue to generate operating losses and consume significant cash resources for the foreseeable future. These conditions raise substantial doubt about our ability to continue as a going concern without additional financing. As a result, our independent registered public accounting firm included an explanatory paragraph in its report on our 2017 financial statements with respect to this uncertainty. Substantial doubt about our ability to continue as a going concern may materially and adversely affect the price per share of our common stock and we may have a more difficult time obtaining financing.

Risks related to our equity securities

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

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act, and we intend to take advantage of some of the exemptions from 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 prior to our first filing of our Annual Report on Form 10-K, 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;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- 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; and
- not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year following the fifth anniversary of the completion of our IPO, (2) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (3) the last day of the fiscal year in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior September 30th and (4) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

The market price of our common stock may be highly volatile.

The market price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- adverse results or delays in our preclinical studies or clinical trials, including our ongoing Phase 2 and planned Phase 3 clinical trials of AG10;
- reports of adverse events or other negative results in clinical trials of third parties' product candidates for ATTR or similar indications, including the Phase 3 ATTR-ACT clinical trial of tafamidis;
- inability to obtain additional funding;
- any delay in filing an IND or NDA for AG10 or other product candidates that we may identify and any adverse development or perceived adverse development with respect to the FDA's review of that IND or NDA;
- failure to develop successfully and commercialize AG10 or other product candidates that we may identify;
- failure to maintain our existing license arrangements or enter into new licensing and collaboration agreements;
- failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate clinical or commercial supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions, including failure to reach agreement with applicable regulatory authorities on the design or scope of our planned clinical trials;
- failure to obtain and maintain regulatory exclusivity for our product candidates;
- regulatory approval or commercialization of new products or other methods of treating our target disease indications by our competitors;
- failure to meet or exceed financial projections we may provide to the public or to the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic collaboration partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and

- trading volume of our common stock.

In addition, companies trading in the stock market in general, and Nasdaq, 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.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, would result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We will need additional capital in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2018 Stock Option and Incentive Plan, or the 2018 Plan, which became effective upon the completion of our IPO, we are authorized to grant stock options and other equity-based awards to our employees, directors and consultants. If our board of directors elects to increase the number of shares available for future grant and our stockholders approve of such an increase at our annual meeting, our stockholders may experience additional dilution, and our stock price may fall.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. In particular, sales upon the expiration of market standoff and lock-up agreements entered into by us and certain of our stockholders in connection with our IPO, the early release of the sale restrictions imposed by these agreements, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. The representatives of the underwriters in our IPO may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. Shares issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market standoff and lock-up agreements, and Rule 144 and Rule 701 under the Securities Act of 1933, as amended, or the Securities Act. If any of these additional 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 securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. If no or few analysts commence or continue coverage of us, the trading price of our stock could decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

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

Our executive officers, directors, five percent stockholders and their affiliates beneficially own approximately 78.0% of our voting stock as of September 30, 2018. Therefore, these stockholders, and in particular, our controlling stockholder, BridgeBio, will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, 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 transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

BridgeBio owns a significant percentage of our common stock, will be able to exert significant control over matters subject to stockholder approval and may have interests that conflict with those of our other stockholders.

BridgeBio is currently our majority stockholder and we will continue to be controlled by BridgeBio. BridgeBio beneficially owns approximately 53.4% of the voting power of our outstanding common stock as of September 30, 2018. As such, BridgeBio has the ability to substantially influence us and exert significant control through this ownership position. For example, BridgeBio will be able to control elections of directors, amendments of our organizational documents, or approval of any merger, amalgamation, sale of assets or other major corporate transaction. Any transferees or successors of all or a significant portion of BridgeBio's ownership in us will be able to exert a similar amount of control over us through their ownership position.

Furthermore, certain of our directors and officers may have actual or potential conflicts of interest with us because of their positions or affiliations with BridgeBio or their equity ownership in BridgeBio. For example, Neil Kumar, founder and Chief Executive Officer of BridgeBio, Ali Satvat, a member of the Board of Managers of BridgeBio and Hoyoung Huh, a member of BridgeBio, serve on our board of directors and retain their positions and affiliations with BridgeBio. Christine Siu, our Chief Financial Officer, also serves as the Chief Operating Officer for other BridgeBio subsidiaries. Uma Sinha, our Chief Scientific Officer, also serves as the Chief Scientific Officer of BridgeBio and other BridgeBio subsidiaries. Jonathan Fox, our Chief Medical Officer, also serves as the Therapeutic Area Lead of Cardiovascular and Renal Diseases for BridgeBio. Further, our other shareholders may not have visibility into the BridgeBio ownership positions or other affiliations of any of our directors or officers with BridgeBio, which may change at any time through acquisition, disposition, dilution, or otherwise. Any change in our directors' or officers' ownership in BridgeBio could impact the interests of those holders. BridgeBio's interests may not always coincide with our corporate interests or the interests of other stockholders, and it may exercise its voting and other rights in a manner with which you may not agree or that may not be in the best interests of our other stockholders. So long as it continues to own a significant amount of our equity, BridgeBio will continue to be able to strongly influence and significantly control our decisions.

Although we do not expect to rely on the "controlled company" exemption under the rules and regulations of Nasdaq, we expect to have the right to use such exemption and therefore we could in the future avail ourselves of certain reduced corporate governance requirements.

BridgeBio holds a majority of the voting power of our outstanding capital stock, and therefore we are considered a "controlled company" as that term is set forth in the rules and regulations of Nasdaq. Under these rules, a company of which more than 50% of the voting power is held by a person or group of persons acting together is a "controlled company" and may elect not to comply with certain rules and regulations of Nasdaq regarding corporate governance, including:

- the requirement that a majority of its board of directors consist of independent directors;
- the requirement that its director nominees be selected or recommended for the board's selection by a majority of the board's independent directors in a vote in which only independent directors participate or by a nominating committee comprised solely of independent directors, in either case, with board resolutions or a written charter, as applicable, addressing the nominations process and related matters as required under the federal securities laws; and
- the requirement that its compensation committee be composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities.

These requirements would not apply to us if, in the future, we choose to avail ourselves of the "controlled company" exemption. Although we qualify as a "controlled company," we do not currently expect to rely on these exemptions and intend to fully comply with all corporate governance requirements under the rules and regulations of Nasdaq. However, if we were to utilize some or all of these exemptions, we would not comply with certain of the corporate governance standards of Nasdaq, which could adversely affect the protections for our other stockholders.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Our restated certificate of incorporation, amended and restated bylaws and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our restated certificate of incorporation and amended and restated bylaws, which became effective upon the completion of our IPO, include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;

- specify that special meetings of our stockholders can be called only by our board of directors or stockholders holding at least 25% of our outstanding voting stock;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum, or by the holders of a majority of the outstanding shares of capital stock then entitled to vote at an election of directors;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated bylaws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated bylaws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us. Any provision of our restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for 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. Our operating results may fluctuate due to a variety of factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing, results and cost of, and level of investment in, our clinical development activities for AG10 and any other product candidates we may identify and pursue, which may change from time to time;
- the cost of manufacturing AG10 or other product candidates that we may identify, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- our ability to conduct clinical trials of AG10 in accordance with our plans and to obtain regulatory approval for AG10 or other product candidates that we may identify, and the timing and scope of any such approvals we may receive;
- the timing and success or failure of clinical trials for competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies;
- our ability to attract, hire and retain qualified personnel;
- the level of demand for AG10 or other product candidates that we may identify, should they receive approval, which may vary significantly;
- future accounting pronouncements or changes in our accounting policies;
- the risk/benefit profile, cost and reimbursement policies with respect to AG10 or other product candidates that we may identify, if approved, and existing and potential future drugs that compete with our product candidates; and
- the changing and volatile U.S., European and global economic environments.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. 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 operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially.

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

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset a portion of future taxable income, if any, subject to expiration of such carryforwards in the case of carryforwards generated prior to 2018. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. Our prior equity offerings and other changes in our stock ownership may have resulted in ownership changes. In addition, we may experience ownership changes in the future as a result of future offerings or subsequent shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. At the state level, there may also be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. In addition, under the Tax Act, the amount of post 2017 NOLs that we are permitted to deduct in any taxable year is limited to 80% of our taxable income in such year, where taxable income is determined without regard to the NOL deduction itself. The Tax Act generally eliminates the ability to carry back any NOL to prior taxable years, while allowing post 2017 unused NOLs to be carried forward indefinitely. There is a risk that due to changes under the Tax Act, regulatory changes, or other unforeseen reasons, our existing NOLs could expire or otherwise be unavailable to offset future income tax liabilities. For these reasons, we may not be able to realize a tax benefit from the use of our NOLs, whether or not we attain profitability.

Comprehensive tax reform legislation could adversely affect our business and financial condition.

On December 22, 2017, the Tax Act, was signed into law. The Tax Act, among other things, contains significant changes to corporate taxation, including (i) reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, (ii) limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), (iii) limitation of the deduction for net operating losses to 80% of current year taxable income in respect of net operating losses generated during or after 2018 and elimination of net operating loss carrybacks, (iv) one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, (v) immediate deductions for certain new investments instead of deductions for depreciation expense over time, and (vi) modifying or repealing many business deductions and credits. Any federal net operating loss incurred in 2018 and in future years may now be carried forward indefinitely pursuant to the Tax Act. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. We will continue to examine the impact the Tax Act may have on our business.

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

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

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

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to: comply with the laws of the FDA and comparable foreign regulatory authorities; provide true, complete and accurate information to the FDA and comparable foreign regulatory authorities; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of AG10 or other product candidates that we may identify and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. In particular, research, sales, marketing, education and other business arrangements in the healthcare industry are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, educating, marketing and promotion, sales and commission, certain customer incentive

programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter misconduct by employees and third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. 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 fines or other sanctions.

We will incur significant costs as a result of operating as a new public company, and our management will devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which require, among other things, that we file with the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation-related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of an initial public offering. We intend to take advantage of this new legislation, but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot 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.

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

a) Sale of Unregistered Securities

During the three months ended June 30, 2018, we issued the following unregistered securities:

- In May 2018, we sold an aggregate of 4,430,162 shares of Series B redeemable convertible preferred stock to 10 accredited investors for an aggregate purchase price of approximately $48.0 million.
- During the three months ended June 30, 2018, we granted stock options to purchase an aggregate of 213,844 shares of our common stock, with an exercise price of $7.24 per share, to employees, directors and consultants pursuant to the 2016 Plan.
- During the three months ended June 30, 2018, we granted stock options to purchase an aggregate of 107,640 shares of our common stock, with an exercise price of $17 per share, to employees, directors and consultants pursuant to the 2018 Plan.

The issuances of these securities were exempt either pursuant to Rule 701, as a transaction pursuant to a compensatory benefit plan, or pursuant to Section 4(a)(2), as a transaction by an issuer not involving a public offering.

b) ***Use of Proceeds***

Not applicable.

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

Period	(a) Total Number of Shares Purchased (1)	(b) Average Price Paid per Share	(c) Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	(d) Maximum Number (or Approximate Dollar Value) of Shares that May Yet Be Purchased Under the Plans or Programs
		(in thousands, except share and per share amounts)		
July 1, 2018 through July 31, 2018	—	$ —	—	—
August 1, 2018 through August 31, 2018	40,366	0.33	—	—
September 1, 2018 through September 30, 2018	—	—	—	—
Total	40,366	$ 0.33	—	—

(1) Under certain stock purchase agreements with employees, we have the right to repurchase common stock at the lower of fair value and the stockholders' original purchase price, which right lapses according to individual vesting schedules. Reflects shares of common stock repurchased in connection with the termination of services by certain employees.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

The documents listed in the Exhibit Index of this Quarterly Report on Form 10-Q are incorporated by reference or are filed with this Quarterly Report on Form 10-Q, in each case as indicated therein (numbered in accordance with Item 601 of Regulation S-K).

Exhibit Number	Exhibit Description	Incorporated by Reference		
		Form	Date	Number
3.1	Amended and Restated Certificate of Incorporation	S-1/A	6/15/2018	3.2
3.2	Amended and Restated Bylaws	S-1/A	6/15/2018	3.4
4.1	Specimen Common Stock Certificate	S-1/A	6/8/2018	4.1
4.2	Amended and Restated Investors' Rights Agreement by and among the Registrant and certain of its stockholders dated March 29, 2018	S-1	5/25/2018	4.2
31.1+	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.			

31.2+	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1+*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
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.
101.INS+	XBRL Instance Document
101.SCH+	XBRL Taxonomy Extension Schema Document
101.CAL+	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB+	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document

* The certifications attached as Exhibit 32.1 and Exhibit 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filing of Eidos Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

\+ Filed herewith.

SIGNATURES

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

EIDOS THERAPEUTICS, INC.

Date: November 6, 2018

By: /s/ Neil Kumar

Neil Kumar
Chief Executive Officer and Director (Principal Executive Officer)

Date: November 6, 2018

By: /s/ Christine Siu

Christine Siu
Chief Financial Officer (Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION 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, Neil Kumar, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Eidos Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

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

   (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 6, 2018

By: /s/ Neil Kumar

Neil Kumar
Chief Executive Officer and Director (Principal Executive Officer)

Exhibit 31.2

CERTIFICATION 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, Christine Siu, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Eidos Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

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

   (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 6, 2018

By: /s/ Christine Siu

Christine Siu
Chief Financial Officer (Principal Financial and Accounting Officer)

Exhibit 32.1

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

In connection with the Quarterly Report of Eidos Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended September 30, 2018, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: November 6, 2018

By: /s/ Neil Kumar

Neil Kumar
Chief Executive Officer and Director (Principal Executive Officer)

Exhibit 32.2

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

In connection with the Quarterly Report of Eidos Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended September 30, 2018, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to the best of my knowledge:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: November 6, 2018

By: /s/ Christine Siu

Christine Siu
Chief Financial Officer (Principal Financial and Accounting Officer)