

**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 June 30, 2020

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
(State or other jurisdiction of
incorporation or organization)
101 Montgomery Street, Suite 2000
San Francisco, CA

46-3733671
(I.R.S. Employer
Identification No.)

94104

N/A
(Former address, if changed since last report)

N/A
(Former Zip Code)

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

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	EIDX	The Nasdaq Global Select Market

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

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

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

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

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

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

As of August 3, 2020, the registrant had 38,571,002 shares of common stock, \$0.001 par value per share, outstanding.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 174,821	\$ 191,157
Related party receivable	154	85
Prepaid expenses and other current assets	3,818	4,678
Total current assets	178,793	195,920
Property and equipment, net	1,317	1,259
Operating lease, right of use asset	3,781	4,010
Other assets	2,779	2,631
Total assets	<u>\$ 186,670</u>	<u>\$ 203,820</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 2,240	\$ 3,151
Related party payable	359	316
Lease liabilities	584	554
Accrued expenses and other current liabilities	12,206	6,409
Total current liabilities	15,389	10,430
Debt, non-current	16,522	16,112
Lease liabilities, non-current	4,293	4,591
Embedded derivative	1,124	1,165
Other liabilities	31	95
Total liabilities	37,359	32,393
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized; no shares issued and outstanding	-	-
Common stock, \$0.001 par value; 150,000,000 shares authorized as of June 30, 2020 and December 31, 2019, respectively; 38,561,026 and 38,040,693 shares issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	39	38
Additional paid-in-capital	304,050	274,494
Accumulated deficit	(154,778)	(103,105)
Total stockholders' equity	149,311	171,427
Total liabilities and stockholders' equity	<u>\$ 186,670</u>	<u>\$ 203,820</u>

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Operations and Comprehensive Loss

(Unaudited)

(In thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Research and development (includes related party expense (benefit) of \$(129) and \$(30) for the three months ended June 30, 2020 and 2019, respectively, and \$(371) and \$64 for the six months ended June 30, 2020 and 2019, respectively)	\$ 17,924	\$ 12,497	35,499	\$ 21,046
General and administrative (includes related party expense of \$372 and \$74 for the three months ended June 30, 2020 and 2019, respectively and \$658 and \$153 for the six months ended June 30, 2020 and 2019, respectively)	10,317	2,297	15,628	6,332
Total operating expenses	28,241	14,794	51,127	27,378
Loss from operations	(28,241)	(14,794)	(51,127)	(27,378)
Interest expense	(604)	-	(1,122)	-
Other income (expense), net	(4)	741	576	1,592
Net and comprehensive loss	\$ (28,849)	\$ (14,053)	\$ (51,673)	\$ (25,786)
Net loss per share - basic and diluted	\$ (0.75)	\$ (0.39)	\$ (1.35)	\$ (0.71)
Weighted-average shares used in computing net loss per share - basic and diluted	38,291,414	36,309,740	38,150,520	36,242,814

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Stockholders' Equity
For the three and six months ended June 30, 2020 (Unaudited)
(in thousands, except for share amounts)

	Common stock		Additional paid-in- capital	Accumulated deficit	Total stockholders' equity
	Share	Amount			
Balance—December 31, 2019	38,040,693	\$ 38	\$ 274,494	\$ (103,105)	\$ 171,427
Issuance of common stock upon exercise of stock options	39,393	—	192	—	192
Issuance of common stock in at-the-market offering, net offering cost of \$24	448,755	1	24,093	—	24,094
Vesting of restricted stock and early exercised options	—	—	36	—	36
Stock-based compensation expense	—	—	1,927	—	1,927
Net loss	—	—	—	(22,824)	(22,824)
Balance—March 31, 2020	38,528,841	\$ 39	\$ 300,742	\$ (125,929)	\$ 174,852
Issuance of common stock upon exercise of stock options	23,778	—	204	—	204
Issuance of common stock under employee stock plans	8,407	—	350	—	350
Vesting of restricted stock and early exercised options	—	—	36	—	36
Stock-based compensation expense	—	—	2,718	—	2,718
Net loss and comprehensive loss	—	—	—	(28,849)	(28,849)
Balance—June 30, 2020	38,561,026	\$ 39	\$ 304,050	\$ (154,778)	\$ 149,311

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Stockholders' Equity
For the three and six months ended June 30, 2019 (Unaudited)
(in thousands, except for share amounts)

	Common stock		Additional paid-in- capital	Accumulated deficit	Total stockholders' equity
	Share	Amount			
Balance—December 31, 2018	36,760,536	37	\$ 220,240	\$ (65,270)	\$ 155,007
Issuance of common stock upon exercise of stock options	50,533	—	25	—	25
Vesting of restricted stock and early exercised options	—	—	38	—	38
Stock-based compensation expense	—	—	964	—	964
Net loss	—	—	—	(11,733)	(11,733)
Balance—March 31, 2019	36,811,069	\$ 37	\$ 221,267	\$ (77,003)	\$ 144,301
Issuance of common stock upon exercise of stock options	34,480	—	87	—	87
Issuance of common stock under employee stock plans	25,626	—	308	—	308
Vesting of restricted stock and early exercised options	—	—	37	—	37
Stock-based compensation expense	—	—	1,166	—	1,166
Net loss and comprehensive loss	—	—	—	(14,053)	(14,053)
Balance—June 30, 2019	36,871,175	\$ 37	\$ 222,865	\$ (91,056)	\$ 131,846

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

EIDOS THERAPEUTICS, INC.
Condensed Statements of Cash Flows

(Unaudited)
(In thousands)

	Six Months Ended June 30,	
	2020	2019
Cash Flows From Operating Activities:		
Net loss	\$ (51,673)	\$ (25,786)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	183	33
Stock-based compensation expense	4,645	2,130
Amortization of debt discount and issuance costs	410	—
Change in fair value of derivative liability	(41)	—
Loss on disposal of asset	2	7
Changes in assets and liabilities:		
Related party receivable	(69)	(163)
Prepaid expenses and other current assets	860	(2,139)
Other assets	81	(2,619)
Accounts payable	(911)	(114)
Accrued expenses and other liabilities	5,537	2,431
Related party payable	43	88
Net cash used in operating activities	<u>(40,933)</u>	<u>(26,132)</u>
Cash Flows From Investing Activities:		
Purchases of property and equipment	(243)	(35)
Net cash used in investing activities	<u>(243)</u>	<u>(35)</u>
Cash Flows From Financing Activities:		
Proceeds from issuance of common stock under employee stock plan	350	308
Proceeds from issuance of common stock upon exercise of stock options	396	112
Proceeds from the issuance of common stock in at-the-market offering	24,094	—
Net cash provided by financing activities	<u>24,840</u>	<u>420</u>
Net decrease in cash and cash equivalents	(16,336)	(25,747)
Cash and cash equivalents, beginning of period	191,157	157,147
Cash and cash equivalents, end of period	<u>\$ 174,821</u>	<u>\$ 131,400</u>
Other supplemental information		
Interest paid	\$ 752	\$ —
Supplemental disclosure of non-cash activities:		
Lease liability arising from the right of use asset	\$ —	\$ 1,058
Vesting of restricted stock and early exercised options	72	75

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 is advancing a drug candidate, acoramidis (formerly AG10) to treat transthyretin, or TTR, amyloidosis, or ATTR, 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. acoramidis is an orally-administered small molecule designed to potently stabilize tetrameric TTR, thereby halting at its outset the series of molecular events that give rise to ATTR. The Company has been primarily engaged in business planning, research and development, recruiting personnel, and raising capital. The Company is headquartered in San Francisco, California and it operates as one operating segment.

The Company is currently enrolling patients in a Phase 3 clinical trial of acoramidis in patients with ATTR cardiomyopathy and planning a second Phase 3 clinical trial in patients with ATTR polyneuropathy. Due to the global outbreak of SARS-CoV-2, the novel strain of coronavirus that causes Coronavirus disease 19 (COVID-19), the Company has experienced impacts on its clinical trials, including delays in clinical site activations and enrollment of patients. The Company continues to actively monitor the situation and may take further precautionary and preemptive actions as may be required by federal, state or local authorities or that the Company determines are in the best interests of public health and safety and that of its patient community, employees, partners, suppliers and stockholders. Depending on the full impact and prevalence of COVID-19 over time, the Company currently expects enrollment of the Phase 3 clinical trial in ATTR-CM to be completed in the first half of 2021 and initiation of the Phase 3 clinical trial in ATTR-PN to occur in the second half of 2020.

Liquidity

The Company has incurred net losses from operations since inception and has an accumulated deficit of \$154.8 million as of June 30, 2020. The Company's ultimate success depends on the outcome of its research and development activities. The Company expects to incur additional losses in the future and it anticipates the need to raise additional capital to fully implement its business plan. Through June 30, 2020, the Company has financed its operations through private placements of redeemable convertible preferred stock, convertible promissory notes, an initial public offering (IPO) of common stock, at-the-market offerings of common stock and a licensing agreement with a third-party.

On August 2, 2019, the Company filed a Registration Statement on Form S-3, as amended (the "2019 Shelf") with the SEC in relation to the registration of common stock, preferred stock, debt securities, warrants and units of any combination thereof. The Company also simultaneously entered into an Open Market Sale Agreement (the "2019 Sales Agreement") with Jefferies LLC and SVB Leerink LLC (each a "Sales Agent" and together, the "Sales Agents"), to provide for the offering, issuance and sale by the Company of up to an aggregate offering price of \$100.0 million of its common stock from time to time in "at-the-market" offerings under the 2019 Shelf and subject to the limitations thereof. The Company will pay to the Sales Agent cash commissions of up to 3.0 percent of the gross proceeds of sales of common stock under the 2019 Sales Agreement. The Company issued 834,368 shares of common stock and received \$48.1 million in net proceeds under the 2019 Sales Agreement through June 30, 2020.

Based on current business plans and assuming no additional financing, the Company believes that its existing cash and cash equivalents will be sufficient to fund its cash requirements through at least the next twelve months from the date of these financial statements. The Company will need to obtain additional financing in the future and may seek financing through the issuance of its common stock, through other equity or debt financings or through collaborations or partnerships with other companies. The amount and timing of the Company's future funding requirements will depend on many factors, including the pace and results of the Company's clinical development efforts for acoramidis and other research and development activities. In addition, the Company is closely monitoring ongoing developments in connection with the COVID-19 pandemic, which may negatively impact its financial and operating results. The Company will continue to assess its operating expenses and cash and cash equivalents and, if circumstances warrant, the Company will make appropriate adjustments to its operating plan. The Company may not be able to raise additional capital on terms acceptable to the Company, or at all, and any failure to raise capital as and when needed would compromise the Company's ability to execute on our business plan and the Company may have to significantly delay, scale back, or discontinue the development of acoramidis or curtail any efforts to expand the Company's product pipeline.

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 unaudited condensed financial statements include transactions with BridgeBio Pharma LLC and its affiliates ("BBP LLC"), a controlling stockholder in the Company. Upon the closing of the BBP LLC IPO on July 1, 2019, all unitholders of BridgeBio Pharma LLC exchanged their units for shares of common stock of BridgeBio Pharma, Inc. ("BridgeBio" or "BBP, Inc."), and BridgeBio Pharma LLC became a wholly-owned subsidiary of BBP, Inc. (the "Reorganization"). As the sole managing member, BBP, Inc. will operate and control all of BridgeBio Pharma LLC's businesses and affairs after the Reorganization.

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 and the Company has provided consulting and management services to BBP LLC and affiliates. 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 interim 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, 2019 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 six months ended June 30, 2020 are not necessarily indicative of the results to be expected for the year ending December 31, 2020 or for any future year or interim period.

The accompanying unaudited interim condensed financial statements and related financial information should be read in conjunction with the audited financial statements and the related notes thereto for the year ended December 31, 2019 included in the Company's Annual Report on Form 10-K, filed with the SEC on February 26, 2020.

Use of estimates

The preparation of 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, but not limited to, those related to revenue recognition, including deductions from revenues (cost of license revenues), the period of performance, identification of deliverables and evaluation of regulatory and royalty milestones with respect to our license agreement, the fair value of the embedded derivative liability, the assumptions used to estimate the fair value of stock-based compensation, 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 funds are 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

All highly-liquid investments with an original maturity date of three months or less when purchased that are readily convertible into cash and have an insignificant interest rate risk are considered to be cash equivalents. As of June 30, 2020 and December 31, 2019, the Company had cash and cash equivalents of \$174.8 million and \$191.2 million, respectively. The Company's cash equivalents are invested in highly-rated money market funds.

Fair value of financial instruments

The carrying amount of the Company's short-term financial instruments, including 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 embedded derivative liability related to its convertible promissory notes.

Impairment of long-lived assets

The Company reviews long-lived assets, primarily comprised of property and equipment, for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability is measured by comparison of the carrying amount to the estimated undiscounted future cash flows which the assets or asset groups are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is the amount by which the carrying amount of the assets or asset groups exceeds the estimated discounted future cash flows arising from the assets or asset groups. There have been no such impairments of long-lived assets for any of the periods presented.

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 third parties 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 includes these costs in accrued expenses and other payables in the balance sheets and within research and development expense in the 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 at each reporting date. 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.

Embedded derivative liability on Loan Agreement

For the SVB and Hercules Loan Agreement entered into in November 2019 (see Note 5), the Company elected to pay a fee (“Success Fee”) upon certain events which is recorded as an embedded derivative liability to be measured at fair value. The Success Fee amount is \$1.0 million if conditions are met prior to November 13, 2021 and \$2.0 million if conditions are met after November 13, 2021. The Company also determined that certain events of default provisions resulting in the prepayment of the loan or a change in the default rate of interest should also be recorded as an embedded derivative liability but were deemed immaterial for all periods presented due to the triggers being deemed unlikely. The compound embedded derivative related to the SVB and Hercules Loan Agreement is subject to remeasurement with changes in fair value recognized in other income (expense), net in the statements of operations.

Net income (loss) attributable to common stockholders and net income (loss) per share

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

Recently issued accounting standards adopted

In August 2018, the FASB issued ASU No. 2018-13, Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement (“ASU 2018-13”), 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 Company has adopted this disclosure requirement in the quarter ended March 31, 2020.

Recently issued accounting standards not yet adopted

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments (“ASU 2016-13”). ASU 2016-13 requires an entity to utilize a new impairment model that requires measurement and recognition of expected credit losses for most financial assets and certain other instruments, including but not limited to available-for-sale debt securities. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. The standard is effective for fiscal years, and interim periods within those years, beginning after December 15, 2019. In November 2019, the FASB issued ASU No. 2019-10, Financial Instruments—Credit Losses (Topic 326), Derivatives and Hedging (Topic 815) and Leases (Topic 842): Effective Dates, which defers the effective date of this ASU to fiscal years beginning after December 15, 2022 for all entities except SEC reporting companies that are not smaller reporting companies. As a smaller reporting company, this ASU will now be effective for the Company beginning January 1, 2023; however, early adoption is permitted. The Company is currently evaluating the impact of adopting the updated provisions and does not anticipate that the adoption of this standard will have a material impact on its condensed financial statements.

Note 3. Fair value measurements

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; and

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.

Financial assets and liabilities measured and recognized at fair value are as follows (in thousands):

	June 30, 2020			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds	\$ 174,821	\$ 174,821	\$ —	\$ —
Total financial assets	\$ 174,821	\$ 174,821	\$ —	\$ —
Liabilities:				
Embedded derivative	\$ 1,124	\$ —	\$ —	\$ 1,124
Total financial liabilities	\$ 1,124	\$ —	\$ —	\$ 1,124
	December 31, 2019			
	Total	Level 1	Level 2	Level 3
Assets:				
Money market funds	\$ 191,157	\$ 191,157	\$ —	\$ —
Total financial assets	\$ 191,157	\$ 191,157	\$ —	\$ —
Liabilities:				
Embedded derivative	\$ 1,165	\$ —	\$ —	\$ 1,165
Total financial liabilities	\$ 1,165	\$ —	\$ —	\$ 1,165

Since the embedded derivative liability is the Company's only Level 3 financial instrument, the following disclosure regarding the embedded derivative liability outlines the activity related to Level 3 financial liabilities of the Company.

Embedded derivative liability in the loan payable

For the SVB and Hercules Loan Agreement entered into in November 2019 (see Note 5), the Company determined that the requirement to pay a Success Fee upon certain events is an embedded derivative liability to be measured at fair value. 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 primarily on the probability of an underlying event triggering the embedded derivative occurring and the timing of such event. The embedded derivative liability was \$1.1 million at inception in November 2019 and \$1.2 million for the year ended December 31, 2019. The following table sets forth a summary of the changes in the fair value of the Company’s embedded derivative liability in the loan payable for the three and six months ended June 30, 2020 (in thousands):

Derivative instrument:	
Beginning balance - December 31, 2019	\$ 1,165
Change in fair value upon revaluation recognized in other income (expense), net	(62)
Balance March 31, 2020	1,103
Change in fair value upon revaluation recognized in other income (expense), net	21
Ending balance - June 30, 2020	\$ 1,124

Quantitative Information about Level 3 Fair Value Measurements

	Fair Value Estimate	Valuation Technique	Unobservable Input	Range of probability
Dollars in thousands				
June 30, 2020	\$ 1,124	Appraisal	Appraisal adjustments	0%-75%
December 31, 2019	\$ 1,165	Appraisal	Appraisal adjustments	0%-75%

Note 4. Condensed balance sheet components

Property and equipment, net

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

	June 30, 2020	December 31, 2019
Leasehold improvements	\$ 1,115	\$ 1,112
Computer equipment	198	139
Office furniture and equipment	288	109
Total property and equipment, cost	1,601	1,360
Less: accumulated depreciation and amortization	(284)	(101)
Total property and equipment, net	\$ 1,317	\$ 1,259

The Company recognized \$156,000 and \$183,000 of depreciation and amortization expense during the three and six months ended June 30, 2020, respectively, and \$18,000 and \$33,000 of depreciation and amortization expense during the three and six months ended June 30, 2019, respectively.

Accrued expenses and other current liabilities

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

	June 30, 2020	December 31, 2019
Accrued research and development costs	\$ 5,498	\$ 3,968
Accrued other current liabilities	5,365	433
Accrued employee related expenses	\$ 1,210	\$ 1,865
Liability for unvested stock, short-term	133	143
Total accrued expenses and other current liabilities	<u>\$ 12,206</u>	<u>\$ 6,409</u>

As of June 30, 2020 and December 31, 2019, \$31,000 and \$95,000, respectively, related to the long-term liability for unvested stock that was recorded in other liabilities.

Lease liabilities

Lease liabilities consist of the following (in thousands):

	June 30, 2020	December 31, 2019
Lease liabilities, current	\$ 584	\$ 554
Lease liabilities, non-current	4,293	4,591
Total lease liabilities	<u>\$ 4,877</u>	<u>\$ 5,145</u>

Note 5. Debt obligation

Silicon Valley Bank and Hercules loan agreement

On November 13, 2019, the Company entered into a Loan and Security Agreement with Silicon Valley Bank and Hercules Capital, Inc. ("SVB and Hercules Loan Agreement"). The SVB and Hercules Loan Agreement provides for up to \$55.0 million in term loans to be drawn in three tranches as follows: (i) Tranche A loan of \$17.5 million, (ii) Tranche B loan of up to \$22.5 million which is available to be drawn until October 31, 2020, and (iii) Tranche C loan of up to \$15.0 million available to be drawn upon a clinical trial milestone. The Tranche C loan is available to be drawn until September 30, 2021. The Tranche A loan of \$17.5 million was drawn on November 13, 2019. There have not been any additional draws on the other tranches as of June 30, 2020.

The Tranche A loan bears interest at a fixed rate equal to the greater of either (i) 8.50% or (ii) 3.25% plus the prime rate as reported in The Wall Street Journal (8.50% as of June 30, 2020). The Tranche A loan repayment schedule provides for interest only payments until November 1, 2021, followed by consecutive equal monthly payments of principal and interest commencing on this date continuing through the maturity date of October 2, 2023. The Tranche A loan also provides for a \$0.3 million commitment fee that was paid at closing and a final payment charge equal to 5.95% multiplied by the amount funded to be paid when the loan becomes due or upon prepayment of the facility. If the Company elects to prepay the Tranche A loan, there is also a prepayment fee of between 0.75% and 2.50% of the principal amount being prepaid depending on the timing and circumstances of prepayment. The Tranche A loan is secured by substantially all of the Company's assets, except the Company's intellectual property, which is the subject of a negative pledge.

Embedded derivatives and debt discounts

On issuance, the net carrying value of the Tranche A loan was \$16.1 million after deducting for various discounts on issuance of \$1.4 million. The debt included discounts and other issuance type costs related to the recognition of a bifurcated compound embedded derivative liability of \$1.1 million treated as a debt discount, the final payment charge of \$1.0 million due on maturity, the \$0.3 million commitment fee paid at closing treated as a debt discount and \$0.1 million in other debt issuance costs. The debt discounts are being amortized to interest expense over the life of the Tranche A loan using the effective interest rate method.

The Company determined that the requirement in its SVB and Hercules Loan Agreement to pay a Success Fee in certain events is an embedded derivative liability requiring bifurcation from the Tranche A loan proceeds and separate accounting. The Success Fee amount is \$1.0 million if conditions are met prior to November 13, 2021 and \$2.0 million if conditions are met after November 13, 2021. The Company also determined that certain events of default provisions resulting in the prepayment of the loan or a change in the default rate of interest should also be recorded as an embedded derivative liability but were deemed immaterial for all periods presented due to the triggers being deemed unlikely. The Company recorded a compound embedded derivative liability of \$1.1 million on issuance, which was recorded as a derivative liability in other long-term liabilities on the balance sheet and as a corresponding debt discount.

The Company calculated the fair values of the derivative liability on issuance as of December 31, 2019 and for each subsequent balance sheet date based on a probability weighted valuation of certain event outcomes and discounted to the present value. The key valuation assumptions used consist of the discount rate of 13.6% as of June 30, 2020 and the probability of an underlying event triggering the Success Fee payment and the timing of such events, which changes each reporting period. The derivative liability is being remeasured at each financial reporting period with any changes in fair value being recognized as a component of other income (expense), net. The fair value of the derivative liability was approximately \$1.1 million and \$1.2 million as of June 30, 2020 and December 31, 2019 and was classified as other long-term liabilities on the balance sheet. There was a change in the fair value of the derivative liability of \$41,000 for the six months ended June 30, 2020.

The facility fee, fair value of the bifurcated embedded derivative liability on issuance, and other debt issuance costs have been treated as debt discounts on the Company's balance sheet and together with the final payment charge are being amortized to interest expense throughout the life of the Tranche A loan using the effective interest rate method. As of June 30, 2020 and December 31, 2019, the net carrying value of the Tranche A loan was \$16.5 million and \$16.1 million, respectively.

As of June 30, 2020 and December 31, 2019, there were unamortized debt discounts of \$2.0 million and \$2.4 million. The Company recorded interest expense and amortization of the debt discount in the amount of \$0.6 million and \$1.2 million on the Tranche A loan for the three months and six months ended June 30, 2020, respectively.

Future minimum payments

The following table presents future payments of principal, interest and final payment charge on the Tranche A loan as of June 30, 2020:

Year Ending December 31:	
2020 (remainder of the year)	\$ 756
2021	2,961
2022	9,786
2023	8,621
Total	<u>22,124</u>
Less: amount representing interest	(3,583)
Less: unamortized debt discount associated with the issuance of a compound embedded derivative liability, final payment charge and other debt issuance costs	(2,019)
Total carrying value	<u>\$ 16,522</u>

Note 6. Related party transactions

BridgeBio Pharma LLC

BridgeBio through its ownership of BBP LLC, is a controlling stockholder in the Company, as it owned 63.7% and 64.6% of the Company's total outstanding shares as of June 30, 2020 and December 31, 2019, respectively. In April 2016, the Company began receiving consulting, management, facility and infrastructure services pursuant to a services agreement with BBP LLC and the Company also provides similar services to BBP LLC and affiliates. The initial agreement was entered into on March 1, 2016 and was superseded by the subsequent agreement that was effective as of May 1, 2017.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Rent	\$ (19)	\$ (8)	\$ (44)	\$ (11)
Facility	(13)	(4)	(44)	75
Consulting	275	56	375	153
	<u>\$ 243</u>	<u>\$ 44</u>	<u>\$ 287</u>	<u>\$ 217</u>

As of June 30, 2020 and December 31, 2019, the Company had outstanding receivables from BBP LLC of \$0.2 million and \$0.1 million, respectively, related to providing services to other related companies of BBP LLC. As of June 30, 2020 and December 31, 2019, the Company had outstanding liabilities due to BBP LLC of \$0.4 million and \$0.3 million, respectively.

Note 7. Stockholders' equity and stock-based compensation

Common stock

The Company has reserved shares of common stock for issuance as follows:

	As of June 30,	
	2020	2019
Options issued and outstanding	1,832,415	1,454,461
Options available for future grants	1,375,223	537,345
Employee Stock Purchase Plan shares available for future issuance	89,398	104,540
Total	<u>3,297,036</u>	<u>2,096,346</u>

Stock options

The following table summarizes the Company's stock option activity for the six months ended June 30, 2020:

	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, 2019	1,935,054	1,335,755	\$ 16.91	8.77	\$ 54,071
Options granted	(567,123)	567,123	\$ 46.77		
Options exercised		(63,171)	\$ 6.28		
Options cancelled	7,292	(7,292)	\$ 16.88		
Outstanding — June 30, 2020	<u>1,375,223</u>	<u>1,832,415</u>	\$ 26.52	8.76	\$ 38,756
Options exercisable – June 30, 2020		<u>483,345</u>	\$ 16.70	8.25	\$ 14,969
Options vested and expected to vest – June 30, 2020		<u>1,832,415</u>	\$ 26.52	8.76	\$ 38,756

Employee stock options valuation

The fair value of employee and non-employee director stock option awards was estimated at the date of grant using a Black-Scholes option-pricing model with the following assumptions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Expected term in years	6.06	6.07	6.06	6.07
Expected volatility	72.48%	72.27%	72.29%	72.31%
Risk-free interest rate	0.45%	2.07%	0.57%	2.10%
Dividend yield	—	—	—	—
Weighted average fair value of share-based awards granted	\$ 29.03	\$ 18.62	\$ 29.65	\$ 18.22

Stock options granted to non-employees

Stock-based compensation related to stock options granted to non-employees is recognized as the stock options are earned. The fair value of the stock options granted to non-employees was calculated at each reporting date using the Black-Scholes option-pricing model with the following assumptions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Expected term in years	6.06	N/A	6.06	6.08
Expected volatility	72.48%	N/A	72.45%	73.54%
Risk-free interest rate	0.41%	N/A	0.44%	2.74%
Dividend yield	—	N/A	—	—

During the three and six months ended June 30, 2020 and 2019, the Company granted 17,130, 17,595, 0, and 18,500 shares, respectively, to non-employee consultants. The Company recognized stock-based compensation expense for non-employee awards during the three and six months ended June 30, 2020 and 2019 of \$0.2 million, \$0.4 million, \$0.1 million, and \$0.1 million, respectively.

Accrued repurchase liability for common stock early exercises

Stock awards granted pursuant to the 2016 Equity Incentive Plan, or 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 June 30, 2020, and December 31, 2019, 339,102 and 524,513 shares, respectively, remained subject to a repurchase right by the Company, with a related liability included in accrued expenses and other liabilities in the balance sheet of \$164,000 and \$238,000, respectively.

Stock-based compensation expense

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Research and development	\$ 1,448	\$ 552	\$ 2,363	\$ 1,004
General and administrative	1,270	614	2,282	1,126
Total stock-based compensation expense	\$ 2,718	\$ 1,166	\$ 4,645	\$ 2,130

As of June 30, 2020, there was \$25.7 million of total unrecognized compensation cost related to unvested stock-based compensation arrangements under the 2016 Plan and the Company's Amended and Restated 2018 Stock Option and Incentive Plan. The unrecognized stock-based compensation cost is expected to be recognized over a weighted-average period of 2.98 years.

Note 8. Net income (loss) per share

The basic and diluted net income per share were computed as follows (in thousands, except per share amounts):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Numerator:				
Net loss attributable to common shareholders	\$ (28,849)	\$ (14,053)	\$ (51,673)	\$ (25,786)
Denominator:				
Weighted average common shares outstanding	38,542,591	36,834,135	38,435,846	36,801,357
Weighted average unvested common shares subject to repurchase	(251,177)	(524,395)	(285,326)	(558,543)
Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted	<u>38,291,414</u>	<u>36,309,740</u>	<u>38,150,520</u>	<u>36,242,814</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.75)</u>	<u>\$ (0.39)</u>	<u>\$ (1.35)</u>	<u>\$ (0.71)</u>

The following shares of potentially dilutive securities have been excluded from the diluted net loss per share computations for the three and six months ended June 30, 2020 and 2019 because their inclusion would be anti-dilutive:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Employee Stock Purchase Plan shares	2,075	1,475	2,075	1,475
Options to purchase common stock	1,832,415	1,454,461	1,832,415	1,454,461
Common stock subject to vesting or repurchase	217,054	490,241	217,054	490,241
Total	<u>2,051,544</u>	<u>1,946,177</u>	<u>2,051,544</u>	<u>1,946,177</u>

Note 9. License agreements

Alexion license agreement

In September 2019, the Company entered into an exclusive license agreement with Alexion Pharma International Operations Unlimited Company, a subsidiary of Alexion Pharmaceuticals, Inc. (together, "Alexion") to develop, manufacture and commercialize the compound known as acoramidis and any of its various chemical forms and any pharmaceutical products containing acoramidis in Japan. Under the agreement, the Company received an upfront nonrefundable payment of \$25.0 million.

The Company also entered into a stock purchase agreement with Alexion, under which the Company sold to Alexion 556,173 shares of the Company's common stock at a price per share of \$44.95, for an aggregate purchase price of approximately \$25.0 million. The excess of the purchase price over the value of the Company's shares, determined based on the closing price of a share of the Company's common stock of \$41.91 as reported on The Nasdaq Global Select Market as of the date of execution, was \$1.7 million and recognized in revenue as part of the upfront payment as discussed below.

The Company is also eligible to receive \$30.0 million in regulatory milestone payments subject to the achievement of regulatory milestones. The Company will also receive royalty payments in the low-teens based on net sales of acoramidis in Japan. The royalty rate is subject to reduction if Alexion is required to obtain intellectual property rights from third parties to develop, manufacture or commercialize acoramidis in Japan, or upon the introduction of generic competition into market.

The Company accounted for the license agreement under ASC 606 and identified the exclusive license as a distinct performance obligation since Alexion can benefit from the license on its own by developing and commercializing the underlying product using its own resources. In addition, the Company entered into a clinical supply agreement with Alexion for the licensed territory and will enter into a commercial supply agreement for the licensed territory. The Company determined that the optional right to future products under these supply agreements is not considered to represent a material right. The Company recognized the \$25.0 million upfront fee and \$1.7 million premium paid for the Company's stock of for a total upfront payment of \$26.7 million in license revenue upon the effective date of the license agreement in September 2019. The Company determined that the license was a right to use the Company's intellectual property and as of the effective date, the Company had provided all necessary information to Alexion to benefit from the license and the license term had begun.

The Company considers the future potential regulatory milestones of up to approximately \$30.0 million and the sales-based royalties to be variable consideration. The Company excluded the regulatory milestones from the transaction price because the Company determined such payments to be fully constrained under ASC 606 due to the inherent uncertainty in the achievement of such milestone payments and are highly susceptible to factors outside of the Company's control. As the sales-based royalties are all related to the license of the IP, the Company will recognize revenue in the period when subsequent sales are made pursuant to the sales-based royalty exception under ASC 606-10-55-65. The Company will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

The Company finalized the clinical supply agreement with Alexion on July 10, 2020. There are no additional performance obligations to be accounted for as there is no minimum purchase requirement in the clinical supply agreement.

During the three months and six months ended June 30, 2020, the Company did not recognize any revenue related to the license agreement.

Acquired license

Stanford license agreement

In April 2016, the Company entered into a license agreement with the Board of Trustees of the Leland Stanford Junior University ("Stanford"), 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. In consideration for the license the Company paid an upfront license payment of \$25,000 in April 2016 and also issued 56,809 shares of common stock.

In March 2017, the Company paid Stanford an annual 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 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 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 recognized \$50,000 of research and development expense in connection with the achievement of a development milestone under the Stanford agreement. In February 2019, the Company recognized \$200,000 of research and development expense in connection with the achievement of a development milestone under the Stanford agreement.

During the three and six months ended June 30, 2020 and 2019, the Company recognized expense of \$0.0, \$0.0, \$0.0 million and \$0.2 million, respectively, in connection with this agreement.

Under the license agreement with Stanford, the Company will pay Stanford a portion of all nonroyalty sublicensing consideration attributable to the sublicense of the licensed compounds. The license agreement states that if this event occurred in the third year, 10% is payable to Stanford. During the year ended December 31, 2019, the Company recognized expense of \$2.5 million related to the Alexion license agreement recorded as cost of license revenue.

Note 10. Commitments and contingencies

Lease arrangements

The Company entered into a one-year operating lease in September 2017 for laboratory facilities in San Francisco, California, which continued thereafter on a month-to-month basis. This was terminated in May 2020. In May 2020, the Company entered into a one-year operating lease for laboratory facilities in San Carlos, CA. The Company has provided \$30,000 as a security deposit for the lease, which is included in the current assets on the condensed balance sheet at June 30, 2020.

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 \$0.2 million as collateral for the lease, which is included in non-current assets on the condensed balance sheet at June 30, 2020.

On March 27, 2019 the Company entered into an amendment to the lease dated November 14, 2017 and the new lease commenced in August 2019. In connection with the amendment, the Company leases 10,552 rentable square feet. The amended lease is for a term of 87 months and provide for \$6.4 million in payments over the lease term. The Company has provided an additional security deposit of \$0.2 million which is included in non-current assets on the condensed balance sheet at June 30, 2020.

Upon the adoption of ASU 2016-02 on January 1, 2019, the Company recognized a lease liability and related ROU asset of \$1.2 million and \$1.1 million, respectively, based on the present value of lease payments for the remaining term of the Company's prior lease. Upon the commencement of the amended lease, the Company recognized \$56,000 in tenant improvements from the prior lease as expense, a gain on extinguishment of the previous lease liability of \$69,000, and wrote-off the total ROU assets and lease liabilities of \$1.0 million and \$1.0 million, respectively. As of June 30, 2020 total ROU assets and lease liabilities per the amended lease were approximately \$3.8 million and \$4.9 million, respectively. All operating lease expense is recognized on a straight-line basis over the lease term.

Other information related to the operating lease:

	Six months ended June 30, 2020
Cash payments over lease term (in thousands)	\$ 5,880
Weighted average remaining lease term (months)	76
Weighted average discount rate (1)	6%

- (1) Because the rate implicit in our lease is not readily determinable, the Company used the Company's incremental borrowing rate. In determining our incremental borrowing rate for each lease, we considered recent rates on secured borrowings, observable risk-free interest rates and credit spreads correlating to our creditworthiness, the impact of collateralization and the term of each of our lease agreements.

Future minimum lease payments as of June 30, 2020 are as follows (in thousands):

Year	Operating Lease Commitments
2020 (remaining six months)	\$ 427
2021	869
2022	895
2023	922
2024	950
Thereafter	1,817
Total	5,880

The Company's rent expense was \$0.2 million and \$0.4 million for the three and six months ended June 30, 2020 and \$0.1 million and \$0.2 million for the three and six months ended June 30, 2019.

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.

Note 11. Income taxes

Income tax expense during interim periods is based on applying an estimated annual effective income tax rate to year-to-date income, plus any significant unusual or infrequently occurring items which are recorded in the interim period. The provision for income taxes for the six months ended June 30, 2020 and 2019 differs from the amount that would be provided by applying the statutory U.S. federal income tax rate of 21%, to pre-tax income primarily due to (i) an increase in uncertain tax positions related to tax credits generated during the quarter and (ii) for the three and six months ended June 30, 2020 and 2019, a full valuation allowance was in effect, which reduced the Company's net tax expense to zero.

In assessing the realizability of deferred tax assets, management considers whether it is more likely than not that some portion, or all, of the Company's deferred tax assets will not be realized. In making such determination, the Company considers all available positive and negative evidence, including future reversals of temporary differences, tax-planning strategies and projected future taxable income and results of operations. If the Company concludes that it is more likely than not that some portion, or all, of its deferred tax assets will not be realized, the tax asset is reduced by a valuation allowance. At December 31, 2019, the Company maintained a full valuation allowance on its net deferred tax assets. The Company assesses the appropriateness of its valuation allowance on a quarterly basis. As of June 30, 2020, there was no change in the Company's assessment of the realizability of its deferred tax assets, and the full valuation allowance remains in effect.

In June 2019, the Company entered into a tax sharing agreement with BridgeBio Pharma, Inc. Based on the agreement, in the case that the Company and BridgeBio Pharma, Inc. file a consolidated or combined tax return and BridgeBio Pharma, Inc. utilizes the Company's tax attributes, BridgeBio Pharma, Inc. will pay the Company the tax benefit it takes on a "with and without" basis. Based on the current ownership structure at June 30, 2020, the Company and BridgeBio Pharma, Inc. may qualify for filing a consolidated or combined tax returns in certain tax jurisdictions.

Note 12. Subsequent Events

Subsequent to period end the Company amended the payment terms related to fees owed to a financial advisory consulting services. As such, \$2.5 million was payable immediately, and \$2.5 million is payable upon the earlier of certain clinical milestones or June 30, 2022.

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

You should read the following management's discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed financial statements and notes thereto included in Part I, Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and notes thereto for the year ended December 31, 2019, included in our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the U.S. Securities and Exchange Commission (SEC) on February 26, 2020 (the "Annual Report").

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 caused by transthyretin, or TTR, amyloidosis, or ATTR. We are advancing our product candidate, acoramidis (formerly 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 and services we provide to 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.

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 common stock, redeemable convertible preferred stock, notes convertible into shares of redeemable convertible preferred stock and licensing arrangements.

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 TTR 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 October 2018, the U.S. Food and Drug Administration, or FDA, granted orphan drug designation in the United States to acoramidis 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 acoramidis 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 us for acoramidis.

We have incurred net losses of \$37.8 million during the year ended December 31, 2019 and \$28.8 and \$51.7 million during the three and six months ended June 30, 2020, respectively, and we expect to continue to incur significant losses for the foreseeable future. As of June 30, 2020, we had an accumulated deficit of \$154.8 million. We expect these losses to increase as we continue our development of, and seek regulatory approvals for our product candidate, acoramidis, begin to commercialize acoramidis, 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.

On August 2, 2019, we filed a Registration Statement on Form S-3, as amended (the “2019 Shelf”) with the SEC in relation to the registration of common stock, preferred stock, debt securities, warrants and units of any combination thereof. We also simultaneously entered into an Open Market Sale Agreement (“2019 Sales Agreement”) with Jefferies LLC and SVB Leerink LLC (together, the “Sales Agents”), to provide for our offering, issuance and sale of up to an aggregate offering price of \$100.0 million of our common stock from time to time in “at-the-market” offerings under the 2019 Shelf and subject to the limitations thereof. We will pay to the Sales Agents cash commissions of up to 3.0 percent of the gross proceeds of sales of common stock under the 2019 Sales Agreement. We issued 834,368 shares of common stock and received \$48.1 million in net proceeds under the 2019 Sales Agreement through June 30, 2020..

In September 2019, we entered into a license agreement (the “License Agreement”) with Alexion Pharma International Operations Unlimited Company, a subsidiary of Alexion Pharmaceuticals, Inc. (together, “Alexion”) to develop and commercialize the Company’s product candidate, acoramidis, in Japan. Additionally, in September 2019, we entered into a Stock Purchase Agreement (the “Stock Purchase Agreement”) with Alexion, pursuant to which we sold to Alexion 556,173 shares of our common stock, for aggregate cash proceeds of \$25.0 million. Under the terms of the License Agreement, we granted Alexion an exclusive license to certain of our intellectual property rights to develop, manufacture and commercialize acoramidis in Japan. In consideration for the license grant, we were entitled to receive an upfront payment of \$25 million, with the potential for an additional one-time payment of \$30.0 million subject to the achievement of a regulatory milestone. In addition, we are entitled to receive royalties in the low-teens on net sales by Alexion of acoramidis in Japan. The royalty rate is subject to reduction if Alexion is required to obtain intellectual property rights from third parties to develop, manufacture or commercialize acoramidis in Japan, or upon the introduction of generic competition into the market.

In November 2019, we entered into a Loan and Security Agreement with Silicon Valley Bank and Hercules Capital, Inc. (“SVB and Hercules Loan Agreement”). The SVB and Hercules Loan Agreement provides for up to \$55.0 million in term loans to be drawn in three tranches as follows: (i) Tranche A loan of \$17.5 million, (ii) Tranche B loan of up to \$22.5 million which is available to be drawn until October 31, 2020, and (iii) Tranche C loan of up to \$15.0 million available to be drawn upon a clinical trial milestone. The Tranche C loan is available to be drawn until September 30, 2021. The Tranche A loan of \$17.5 million was drawn on November 13, 2019 and there have not been any additional draws on the other tranches as of June 30, 2020. The Tranche A loan bears interest at a fixed rate equal to 8.50% per annum that is due and payable monthly.

As of June 30, 2020, we had \$174.8 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 acoramidis 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 acoramidis 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.

We experienced impacts on certain aspects of our business, including delays in activation of clinical sites and enrollment of patients in our clinical trials, during the quarter ended June 30, 2020 due to the global outbreak of SARS-CoV-2, the novel strain of coronavirus that causes Coronavirus disease 19 (COVID-19). We are currently enrolling patients in a Phase 3 clinical trial of acoramidis in patients with ATTR cardiomyopathy and planning a second Phase 3 clinical trial in patients with ATTR polyneuropathy. Depending on the full impact and prevalence of COVID-19 over time, we currently expect enrollment of the Phase 3 clinical trial in ATTR-CM to be completed in the first half of 2021 and initiation of the Phase 3 clinical trial in ATTR-PN to occur in the second half of 2020. The ultimate impacts of the COVID-19 pandemic on our business are currently unknown. We will continue to actively monitor the situation and may take further precautionary and preemptive actions as may be required by federal, state or local authorities or that we determine are in the best interests of public health and safety and that of our patient community, employees, partners, suppliers and stockholders. We cannot predict the effects that such actions, or the impact of the COVID-19 pandemic on global business operations and economic conditions may have on our business or strategy, including the effects on our ongoing and planned clinical development activities and prospects, or on our financial and operating results.

Financial operations overview

Research and development expense

Research and development expense consists primarily of costs incurred for the development of acoramidis, 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 and other third-party service providers 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 Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Clinical development	\$ 6,665	\$ 4,437	\$ 16,148	\$ 8,201
Contract manufacturing	5,217	4,908	9,152	7,245
Preclinical, discovery and other research and development costs	660	504	1,237	1,196
Compensation and related personnel costs	5,052	2,529	8,385	4,186
Facility and other costs	330	119	577	218
	<u>\$ 17,924</u>	<u>\$ 12,497</u>	<u>\$ 35,499</u>	<u>\$ 21,046</u>

We expect our research and development expenses to increase substantially for the foreseeable future as we continue to conduct research and development activities related to acoramidis and advance acoramidis into later stages of clinical development, including our ongoing and planned Phase 2 and Phase 3 clinical trials of acoramidis in ATTR-CM and ATTR-PN and any subsequent preclinical or clinical development activities. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of acoramidis is highly uncertain. In addition, we believe that delays in our ongoing and planned clinical trials and adjustments to certain of our study procedures, such as increased frequency of home visits, as a result of the COVID-19 pandemic, could increase our expenditures, although it is difficult to predict the full effects of the COVID-19 pandemic on our research and development activities at this time. 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.

Interest expense

Interest expense consists of cash and non-cash components. The cash component of interest expense is attributable to borrowings under our loan agreements. Refer to Note 5 Debt obligation, for further information on our loan agreements. The non-cash component consists of interest expense recognized from the amortization of debt discounts derived from the debt issuance costs capitalized on our balance sheet.

Other income (expense), net

Other income (expense), net primarily includes interest income during the three and six months ended June 30, 2020 and 2019.

Comparison of the three months and six months ended June 30, 2020 and 2019

Research and development expense

<i>in thousands</i>	Three Months Ended June 30,		Increase (Decrease)		Six Months Ended June 30,		Increase (Decrease)	
	2020	2019	\$	%	2020	2019	\$	%
Research and development	\$ 17,924	\$ 12,497	5,427	43%	\$ 35,499	\$ 21,046	14,453	69%

Research and development expense increased by \$5.4 million, or 43%, during the three months ended June 30, 2020, compared to the three months ended June 30, 2019. The increase was primarily attributable to an increase of \$2.7 million in clinical trial related activities and contract manufacturing activities for our clinical trials and drug supply, and an increase in personnel costs of \$1.8 million, and an increase in stock-based compensation costs of \$0.9 million.

Research and development expense increased by \$14.5 million, or 69%, during the six months ended June 30, 2020, compared to the six months ended June 30, 2019. The increase was primarily attributable to an increase of \$10.3 million in clinical trial related activities and contract manufacturing activities for our clinical trials and drug supply, an increase in personnel costs of \$2.8 million, and an increase in stock-based compensation of \$1.4 million.

General and administrative expense

<i>in thousands</i>	Three Months Ended June 30,		Increase (Decrease)		Six Months Ended June 30,		Increase (Decrease)	
	2020	2019	\$	%	2020	2019	\$	%
General and administrative	\$ 10,317	\$ 2,297	8,020	349%	\$ 15,628	\$ 6,332	9,296	147%

General and administrative expense increased by \$8.0 million, or 349%, during the three months ended June 30, 2020, compared to the three months ended June 30, 2019. The increase was primarily attributable to an increase in financial advisory consulting costs of \$5.0 million, an increase in marketing costs of \$1.0 million, an increase in stock-based compensation of \$0.7 million, an increase of consulting costs of \$0.8 million, an increase of \$0.3 million other expenses due to an increase in costs for director and officers insurance, and an increase of \$0.4 million in personnel-related expenses due to an increase in headcount to support the growth of our operations, offset by a decrease of \$0.2 million for related party expenses.

General and administrative expense increased by \$9.3 million, or 147%, during the six months ended June 30, 2020, compared to the six months ended June 30, 2019. The increase was primarily attributable to an increase in financial advisory consulting costs of \$5.0 million, an increase in marketing costs of \$2.3 million, an increase in stock-based compensation of \$1.2 million, an increase of \$0.6 million in personnel-related expenses due to an increase in headcount to support the growth of our operations, an increase of \$0.5 million other expenses due to an increase in costs for director and officers insurance, an increase of \$0.3 for related party expenses, offset by a decrease of \$0.5 million due decrease in consulting costs compared to the prior year primarily for financial, legal and accounting fees.

Interest expense

<i>in thousands</i>	Three Months Ended June 30,		Increase (Decrease)		Six Months Ended June 30,		Increase (Decrease)	
	2020	2019	\$	%	2020	2019	\$	%
Interest expense	\$ (604)	\$ —	(604)	100%	\$ (1,122)	\$ —	—	100%

Interest expense of \$0.6 million and \$1.1 million during the three months and six months ended June 30, 2020, respectively, was related to the obligations under the SVB and Hercules Loan Agreement, whereby we are required to pay interest for money received. This also reflects amortization of issuance costs and debt discount, accretion of the end of term payment and change in the derivative liability, which were not present during the three months and six months ended June 30, 2019.

Other income (expense), net

<i>in thousands</i>	Three Months Ended June 30,		Increase (Decrease)		Six Months Ended June 30,		Increase (Decrease)	
	2020	2019	\$	%	2020	2019	\$	%
Other income (expense), net	\$ (4)	\$ 741	(745)	-101%	\$ 576	\$ 1,592	(1,016)	-64%

Other income (expense), net was an expense of \$4,000 during the three months ended June 30, 2020, compared to an income of \$0.7 million during the three months ended June 30, 2019. The decrease in other income during the three months ended June 30, 2020 reflected a decrease of interest income related to our money market funds due to a decrease in interest rates.

Other income (expense), net was an income of \$0.6 million during the six months ended June 30, 2020, compared to an income of \$1.6 million during the six months ended June 30, 2019. The decrease in other income during the six months ended June 30, 2019 was due to a decrease in interest income related to our money market funds due to a decrease in interest rates.

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.

Our significant accounting policies are fully described in Note 2 of our Annual Report. There were no significant changes to our critical accounting policies disclosed in our audited financial statements as of December 31, 2019. We believe that the accounting policies discussed are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Liquidity and Capital Resources

Liquidity and Capital Expenditures

Liquidity

As of June 30, 2020, we had \$174.8 million of cash and cash equivalents and an accumulated deficit of \$154.8 million. In September 2019, we received \$50.0 million in aggregate cash proceeds from Alexion upon the execution of the License Agreement and Stock Purchase Agreement. In November 2019, we entered into the SVB and Hercules Loan Agreement and drew proceeds of \$17.5 million in debt financing.

On August 2, 2019, we filed a Registration Statement on Form S-3, as amended (the "2019 Shelf") with the SEC in relation to the registration of common stock, preferred stock, debt securities, warrants and units of any combination thereof. We also simultaneously entered into an Open Market Sale Agreement ("2019 Sales Agreement") with Jefferies LLC and SVB Leerink LLC (each a "Sales Agent" and together, the "Sales Agents"), to provide for the offering, issuance and sale by the Company of up to an aggregate offering price of \$100.0 million of its common stock from time to time in "at-the-market" offerings under the 2019 Shelf and subject to the limitations thereof. We will pay to the Sales Agent cash commissions of up to 3.0 percent of the gross proceeds of sales of common stock under the 2019 Sales Agreement. We issued 834,368 shares of common stock and received \$48.1 million in net proceeds under the 2019 Sales Agreement through June 30, 2020.

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 2020 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 and advance acoramidis into later-stage clinical development, including our ongoing and planned Phase 3 clinical trials. 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 acoramidis 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 acoramidis 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 acoramidis;
- our ability to successfully commercialize acoramidis, 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 acoramidis 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):

	Six Months Ended June 30,	
	2020	2019
Net cash used in operating activities	\$ (40,933)	\$ (26,132)
Net cash used in investing activities	\$ (243)	\$ (35)
Net cash provided by financing activities	\$ 24,840	\$ 420

Cash flows from operating activities

During the six months ended June 30, 2020, cash used in operating activities was \$40.9 million and consisted primarily of a net loss of \$51.7 million. Our non-cash charges of \$5.2 million primarily consisted of stock-based compensation expense. The change in our net operating assets of \$5.5 million was primarily due to an increase in accounts payable and accrued expenses of \$4.6 million due to the timing of payments and the timing of activities for which payments were made, as well as prepaid expenses and other current and non-current assets of \$0.9 million.

During the six months ended June 30, 2019, cash used in operating activities was \$26.1 million and consisted primarily of a net loss of \$25.8 million. Our non-cash charges of \$2.1 million primarily consisted of stock-based compensation expense. The change in our net operating assets of \$2.5 million was primarily due to an increase in accounts payable and accrued expenses of \$2.4 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 and non-current assets of \$4.8 million, due to the timing of payments and the timing of activities for which payments were made.

Cash flows from investing activities

During the six months ended June 30, 2020 and June 30, 2019, cash used in investing activities was \$0.2 million and \$35,000; respectively, which consisted of our purchase of property and equipment for our office and employees.

Cash flows from financing activities

During the six months ended June 30, 2020, cash provided by financing activities was \$24.8 million; which consisted of \$24.1 million from proceeds from the issuance of common stock under our at-the-market offering facility; \$0.7 million due to the receipt of funds from stock purchases under our employee stock purchase plan and the exercise of common stock options.

During the six months ended June 30, 2019, cash provided by financing activities was \$0.4 million related to the receipt of funds from our employee stock purchase plan and the exercise of common stock options.

Contractual Obligations and Other Commitments

There have been no material changes outside the ordinary course of our business to our contractual obligations during the six months ended June 30, 2020, as compared to those disclosed in our Annual Report.

Off-Balance Sheet Arrangements

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.

Under SEC rules and regulations, as a smaller reporting company, we are not required to provide the information required by this item.

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, who currently serves as our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), as of the end of the period covered by this Form 10-Q. Based on such evaluation, our Chief Executive Officer has concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of June 30, 2020.

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.

Item 1. Legal Proceedings.

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

Item 1A. Risk Factors.

You should consider carefully the following risk factors, together with all the other information in this report, including our consolidated financial statements and notes thereto, and in our other public filings with the SEC. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

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 from product sales 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, acoramidis (formerly 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 year ended December 31, 2019, and six months ended June 30, 2020 were \$37.8 million and \$51.7 million, respectively. As of June 30, 2020, we had an accumulated deficit of \$154.8 million. We have not generated any revenue from product sales since our inception and have financed our operations solely through the sale of equity securities, debt financings and our license agreement with Alexion. 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 acoramidis 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 existing or future collaborators' clinical trials or the development of acoramidis or other product candidates that we may identify. Even if acoramidis 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 acoramidis 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 enrolling patients in a Phase 3 clinical trial of acoramidis, our only clinical development candidate, in ATTR cardiomyopathy (ATTR-CM) and are preparing to advance acoramidis into a Phase 3 clinical trial in ATTR polyneuropathy (ATTR-PN). 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 acoramidis in planned and future clinical trials. We are also responsible for license maintenance fees, milestone payments and royalties to the Board of Trustees of the Leland Stanford Junior University ("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 acoramidis 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 from the date of this Quarterly Report on Form 10-Q. 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 acoramidis 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, our disposition of intellectual property or other rights to acoramidis or other product candidates we may identify and pursue, 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 initiate, conduct and complete our Phase 3 clinical trials of acoramidis in ATTR-CM and ATTR-PN, and to pursue regulatory approvals for acoramidis, and the costs of post-marketing studies that could be required by regulatory authorities;
- the progress and results of our ongoing and planned Phase 3 clinical trials of acoramidis;
- 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 planned Phase 3 clinical trials of acoramidis and potential future clinical trials;
- the costs of obtaining clinical and commercial supplies of acoramidis and any other product candidates we may identify and develop;
- our ability to successfully commercialize acoramidis and any other product candidates we may identify and develop;
- the manufacturing, selling and marketing costs associated with acoramidis 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 acoramidis 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 competing products and product candidates, technological changes 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.

We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities.

In November 2019, we entered into a Loan and Security Agreement with Silicon Valley Bank (“SVB”) and Hercules Capital, Inc. (the “SVB and Hercules Loan Agreement”), pursuant to which we were extended term loans in the aggregate principal amount of \$55 million. Borrowings under the SVB and Hercules Loan Agreement are secured by substantially all of our assets, excluding intellectual property. The SVB and Hercules Loan Agreement restricts our ability, among other things, to:

- sell, transfer or otherwise dispose of any of our business or property, subject to limited exceptions;
- make material changes to our business or management;
- enter into transactions resulting in significant changes to the voting control of our stock;
- make certain changes to our organizational structure;
- consolidate or merge with other entities or acquire other entities;
- incur additional indebtedness or create encumbrances on our assets;
- pay dividends, other than dividends paid solely in shares of our common stock, or make distributions on and, in certain cases, repurchase our stock;
- enter into transactions with our affiliates, subject to limited exceptions;
- repay subordinated indebtedness; or
- make certain investments.

In addition, we are required under our SVB and Hercules Loan Agreement to maintain our deposit and securities accounts with SVB and to comply with various operating covenants and default clauses that may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. A breach of any of these covenants or clauses could result in a default under the loan and security agreement, which could cause all of the outstanding indebtedness under the facility to become immediately due and payable.

If we are unable to generate sufficient cash to repay our debt obligations when they become due and payable, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively affect our business operations and financial condition.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to acoramidis 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 and of negotiating, entering into and maintaining such strategic partnership or other arrangements. 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 financing transactions or other equity or debt issuances. 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

The outbreak of the novel strain of coronavirus, SARS-CoV-2, which causes COVID-19, could adversely impact our business, including our preclinical studies and clinical trials.

Public health crises such as pandemics or similar outbreaks could adversely impact our business. In December 2019, a novel strain of coronavirus, SARS-CoV-2, which causes coronavirus disease 2019 (COVID-19), surfaced in Wuhan, China. Since then, COVID-19 has spread globally. In response to the spread of COVID-19 and governmental “shelter-in-place” orders, we have closed our executive offices with our administrative employees continuing their work outside of our offices, restricted on-site staff to only those required to execute their job responsibilities.

As a result of the COVID-19 outbreak or any future pandemics, we have experienced, and may in the future experience disruptions that severely impact our business, clinical trials and preclinical studies, including:

- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays or disruptions in non-clinical experiments due to unforeseen circumstances at contract research organizations and vendors along their supply chain;
- increased rates of patients withdrawing from our clinical trials following enrollment as a result of contracting COVID-19, being forced to quarantine, or not accepting home health visits;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures (particularly any procedures that may be deemed non-essential), which may impact the integrity of subject data and clinical study endpoints;
- interruption or delays in the operations of the U.S. Food and Drug Administration and comparable foreign regulatory agencies, which may impact review and approval timelines;
- interruption of, or delays in receiving, supplies of our product candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems; and
- limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, including because of sickness of employees or their families, the desire of employees to avoid contact with large groups of people, an increased reliance on working from home or mass transit disruptions.

These and other factors arising from the COVID-19 pandemic could worsen in countries that are already afflicted with COVID-19, could continue to spread to additional countries, or could return to countries where the pandemic has been partially contained, each of which could further adversely impact our ability to conduct clinical trials and our business generally, and could have a material adverse impact on our operations and financial condition and results.

In addition, the trading prices for our common stock and other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms. The COVID-19 outbreak continues to rapidly evolve. The extent to which the outbreak may impact our business, preclinical studies and clinical trials will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the duration of the outbreak, travel restrictions and actions to contain the outbreak or treat its impact, such as social distancing and quarantines or lock-downs in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease.

To the extent the COVID-19 pandemic adversely affects our business and financial results, it may also have the effect of heightening many of the other risks described in this "Risk Factors" section, such as those relating to our clinical development operations, the supply chain for our ongoing and planned clinical trials, our indebtedness under the SVB and Hercules Loan Agreement, our need to raise additional capital to support our operations and to service our indebtedness, and our ability to comply with the covenants contained in the agreements that govern our indebtedness.

We are heavily dependent on the success of our only product candidate, acoramidis, 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 acoramidis, 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 acoramidis, 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 acoramidis. Before we can generate any revenues from sales of acoramidis, we will be required to complete additional clinical development, including, among other things, longer-term and larger registrational clinical trials of acoramidis, 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 acoramidis will depend on patent and trade secret protection, obtaining and maintaining regulatory exclusivity, acceptance of acoramidis by patients, the medical community and third-party payors, its ability to compete with other therapies, including therapies that are currently on the market, 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 acoramidis, which would materially harm our business.

Currently, acoramidis is our only product candidate, and it may be years before we can complete the clinical development activities necessary to apply for regulatory approval of acoramidis, 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 acoramidis will be successful in clinical trials or receive regulatory approval. If we do not receive regulatory approval for, or otherwise fail to successfully commercialize, acoramidis, 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 acoramidis 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 acoramidis, and it is possible that neither acoramidis nor any other product candidates which we may seek to develop in the future will ever obtain regulatory approval.

Applications for acoramidis 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 acoramidis 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 ongoing and planned Phase 3 clinical trials;
- 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 acoramidis 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 acoramidis 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 marketed drugs for ATTR or in clinical trials conducted by competitors for other drug candidates targeting ATTR that raise regulatory or safety concerns about risk to patients of the treatment, including the approach of TTR stabilization;
- a finding by the FDA or foreign regulatory agency 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 acoramidis or any our product candidates that we may identify and pursue being greater than we anticipate;
- clinical trials of acoramidis 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 acoramidis 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 on a timely basis, or at all, could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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.

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 availability of competing products approved for the treatment of ATTR or product candidates currently under development for ATTR, including Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine), for which Pfizer Inc. has been approved for the treatment of ATTR-CM in the United States and Japan (Vyndaqel only) and is approved in certain countries outside the United States for the treatment of ATTR-PN (Vyndaqel only), 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.

Our clinical trials may fail to demonstrate substantial evidence of the safety and effectiveness of acoramidis 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 acoramidis 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 acoramidis 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. For example, in the event another therapy in the same class as acoramidis is approved with one or more claims with respect to efficacy endpoints that are demonstrated with greater statistical significance than the same or similar claim(s) in our clinical trials for acoramidis, the scope of the approval for acoramidis could be limited to a second-line claim only for those patients who cannot tolerate the first-line product. Any of these events could limit the commercial potential of acoramidis and have a material adverse effect on our business, prospects, financial condition and results of operations.

Results of earlier studies or clinical trials, including cross-trial comparisons of results that are not derived from head-to-head 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 acoramidis 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 acoramidis 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 acoramidis potentially stabilizes TTR in human serum may not be replicated in later stage clinical trials.

Additionally, some of our preclinical studies in which acoramidis 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 acoramidis 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, certain of our hypotheses regarding the potential clinical and therapeutic benefit of acoramidis compared to other TTR stabilizers are based on cross-trial comparisons of results that were not derived from head-to-head preclinical studies or clinical trials. These observations, which do not reflect robust comparative analyses, may suggest misleading similarities or differences due to differences in study protocols, conditions and patient populations, and may not be reliable predictors of the relative efficacy or other benefits of acoramidis compared to other product candidates that may be approved or are in development for the treatment of ATTR.

Further, 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 acoramidis 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 acoramidis 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.

If serious adverse events or unacceptable side effects are identified during the development of acoramidis 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. To date, we have only evaluated acoramidis in a limited number of subjects at a limited duration of exposure in our Phase 1 and Phase 2 clinical trials and the duration of exposure in our Phase 3 clinical trials is expected to be significantly longer. Accordingly, any rare and severe side effects of acoramidis may be uncovered in our ongoing studies or in larger, subsequent trials that we may conduct, such as our ongoing Phase 2 OLE and Phase 3 clinical trials of acoramidis in ATTR-CM and our planned Phase 3 clinical trial of acoramidis in ATTR-PN. Additionally, although our animal safety pharmacology studies of acoramidis demonstrated a wide safety margin between anticipated therapeutic exposures and doses associated with toxicity and no dose limiting toxicities were established in the 9 month 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 acoramidis 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 intend to conduct clinical trials for acoramidis 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 intend to conduct one or more of our clinical trials outside the United States, including in Europe. For instance, subject to authorization from applicable regulatory authorities, we plan to initiate a Phase 3 clinical trial of acoramidis in ATTR-PN in the second half of 2020, subject to potential delays related to the COVID-19 global pandemic. 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 acoramidis 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 acoramidis 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 acoramidis or any other product candidates that we may identify and pursue in the United States, we may never obtain approval to commercialize acoramidis 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 acoramidis 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 and EMA have granted orphan drug designation for acoramidis for the treatment of transthyretin amyloidosis, we may not receive orphan drug designation 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 acoramidis 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 acoramidis in the United States for the treatment of transthyretin amyloidosis, or ATTR, and in November 2018, the EMA granted the designation of acoramidis as an orphan medicinal product in the EU for the treatment of transthyretin amyloidosis, or 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 and EMA have granted orphan drug designation for acoramidis for the treatment of ATTR, we may apply for orphan drug designation for acoramidis 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 and EMA or may receive from any other regulatory authorities, may not effectively protect acoramidis 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 acoramidis (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 acoramidis. 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 for additional assets or programs, 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 for additional assets or programs in the future, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any such 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.

The United Kingdom's withdrawal from the EU may have a negative effect on our business, global economic conditions, and financial markets.

As a result of the United Kingdom's vote to leave the EU in March 2019, often referred to as Brexit, the EMA relocated its headquarters from London to Amsterdam. Following such referendum vote, effective as of January 31, 2020, the United Kingdom formally left the EU, subject to a transition period that is set to end on December 31, 2020. Since a significant proportion of the regulatory framework in the United Kingdom is derived from EU directives and regulations, Brexit could materially increase the time and costs associated with our ongoing clinical trials of acoramidis at one or more investigative sites in the UK, impact the regulatory regime with respect to the approval of product candidates, disrupt the manufacture of our products and product candidates in the United Kingdom or the EU, disrupt the import and export of active substances and other components of drug formulations, and disrupt the supply chain for clinical trial product and final authorized formulations. The specific impact to the supervision, regulation and supply of medicines in the United Kingdom and Europe still remains unclear. The cumulative effect of disruptions to the regulatory framework or supply chains may add considerably to the development lead time to, and expense of, marketing authorization and commercialization of products in the EU and/or the United Kingdom. In view of the uncertainty surrounding the United Kingdom's decision to leave the EU, we are unable to predict the effects of such disruption to the regulatory framework and supply chain in Europe and the impact of such effects on our business, financial condition and operations. In addition, Brexit may lead other EU member countries to consider referendums regarding their EU membership. Any of these events, along with any political, economic and regulatory changes that may occur, could cause political and economic uncertainty in Europe and internationally and harm our business, financial condition and results of operations.

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 acoramidis 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 and planned Phase 3 clinical trials of acoramidis or any other 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 acoramidis 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 acoramidis 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.

Acoramidis 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 acoramidis through a third party and have adequate supplies to conduct our ongoing and planned Phase 3 clinical trials of acoramidis in ATTR-CM and ATTR-PN. 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 acoramidis 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 acoramidis, 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 acoramidis. 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 acoramidis.

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 acoramidis 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 acoramidis 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 acoramidis 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.

We are dependent upon our license agreement with Alexion for the development and eventual commercialization of acoramidis in Japan. If this collaboration is unsuccessful or is terminated, we may be unable to commercialize acoramidis in Japan and we will not receive additional funding from this relationship.

We depend upon our license agreement (the "Alexion License Agreement") with Alexion Pharma International Operations Unlimited Company, a subsidiary of Alexion Pharmaceuticals, Inc. (together, "Alexion") for the clinical development and commercialization of acoramidis in Japan. While Alexion is responsible for various research and development activities under the Alexion License Agreement and with respect to the commercialization of acoramidis in Japan, our ability to benefit from Alexion's development and commercialization activities and to receive future payments under the Alexion License Agreement will depend upon the ability and willingness of Alexion to successfully meet its responsibilities under the Alexion License Agreement and continue the collaboration. We may not receive some or all of the future payments and other benefits that we currently expect to receive under our Alexion License Agreement.

Our ability to generate additional funding from the Alexion License Agreement may be impaired by several factors including:

- Alexion may shift its priorities and resources away from acoramidis or the market in Japan due to a change in business strategies, or a merger, acquisition, sale or downsizing of its company or business unit;
- Alexion may change the success criteria for acoramidis, thereby delaying or ceasing its development;
- Alexion may exercise its rights to terminate the Alexion License Agreement; or
- a dispute may arise between us and Alexion concerning financial obligations or the research, development or commercialization of acoramidis in Japan, resulting in a delay in payments or termination of the collaboration and possibly resulting in costly litigation or arbitration which may divert management attention and resources.

Specifically, with respect to termination, unless earlier terminated, the Alexion License Agreement will expire upon the later of the expiration of the last-to-expire valid claim under any licensed patents covering acoramidis in Japan or the tenth anniversary of the first commercial sale of acoramidis in Japan. Either party may terminate the Alexion License Agreement in the event of a material breach or insolvency of the other party. Additionally, Alexion may terminate the Alexion License Agreement for convenience upon at least 180 days prior written notice, and we may terminate the Alexion License Agreement in the event Alexion ceases development or commercialization of acoramidis under certain circumstances or challenges the validity or enforceability of our patent rights.

If the Alexion License Agreement is terminated, then in order to fund further development and commercialization of acoramidis in Japan, we may need to seek out and establish alternative strategic collaborations with third-party partners, which may not be possible; or we may not be able to do so on terms which are acceptable to us, in which case it may be necessary for us to limit the size or scope of one or more of our programs or increase our expenditures and seek additional funding by other means.

Any of these events could have a material adverse effect on our results of operations and financial condition.

Risks related to our intellectual property

If we are unable to obtain and maintain sufficient intellectual property protection for acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis and we may be required to cease our development and commercialization of acoramidis. 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 acoramidis 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 the revocation of or amendment to our patents in such a way that they no longer cover acoramidis 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 acoramidis or any other product candidates we may 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 acoramidis or any other product candidate 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 acoramidis or any other product candidates we may 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 acoramidis 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 acoramidis 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. Acoramidis 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 acoramidis 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 acoramidis. Sales of acoramidis 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 acoramidis 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 acoramidis 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 and data privacy 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 acoramidis 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 and regulations. In the United States, these laws include, without limitation, state and federal anti-kickback, false claims, physician transparency and patient data privacy and security laws and regulations.

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. Regulators globally are also imposing greater monetary fines for privacy violations. The GDPR, which went into effect in May 2018, applies to any company established in the European Union (EU) as well as to those outside the EU if they collect and use personal data in connection with the offering of goods or services to individuals in the EU or the monitoring of their behavior. Noncompliance with the GDPR may result in monetary penalties of up to €20 million or 4% of worldwide revenue, whichever is higher. The GDPR may increase our responsibility and liability in relation to personal data that we process where such processing is subject to the GDPR, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, including as implemented by individual countries. Compliance with the GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of personal data, such as healthcare data or other sensitive information, could greatly increase our cost of developing our products and services or even prevent us from offering any of our products in certain jurisdictions. Given the limited enforcement of the GDPR to date, particularly in the pharmaceutical space, we face uncertainty as to the exact interpretation of the new requirements on our clinical trials and we may be unsuccessful in implementing all measures that may be required by data protection authorities or courts in interpretation of the new law.

If we or any of the physicians or other providers or entities with whom we expect to do business with are found not to be in compliance with applicable laws, we or they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs, which may also adversely affect our business.

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.

Since its enactment, some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial, congressional, or executive challenges. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects 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 2029 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 acoramidis 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 acoramidis 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., Intellia Therapeutics Inc., Arcturus Therapeutics Inc., Neurimmune Holding AG and Prothena Therapeutics plc. In particular, Vyndamax (tafamidis) and Vyndaqel (tafamidis meglimume) are approved in the United States and Japan for the treatment of ATTR-CM and in certain countries outside the United States for the treatment of ATTR-PN. Accordingly, acoramidis will not be the first treatment on the market for ATTR-CM, and its market share may be limited. acoramidis also will not be the first treatment on the market for ATTR-PN as tafamidis, patisiran, and inotersen are approved for the treatment of ATTR-PN in a variety of countries globally. 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 acoramidis 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 that acoramidis 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 acoramidis 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 acoramidis 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 acoramidis or other product candidates that we may identify may be limited or may not be amenable to treatment with acoramidis 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 acoramidis 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 and pricing of other treatments for ATTR, including Vyndamax (tafamidis) and Vyndaqel (tafamidis meglumine), for which Pfizer Inc. has been approved for the treatment of ATTR-CM in the United States and Japan (Vyndaqel only). As a result, even if acoramidis is approved, it will not be the first treatment on the market for ATTR and will face competition from existing marketed drugs.

Risks related to our business and industry

Certain of our directors and officers may have actual or potential conflicts of interest because of their positions with BridgeBio and may not be able to or may choose not to devote sufficient time and attention to our company.

Neil Kumar, founder, Chief Executive Officer and a member of the Board of Directors of BridgeBio, Eric Aguiar, a member of the Board of Directors of BridgeBio, Ali Satvat, a member of the Board of Directors of BridgeBio, and Uma Sinha, Chief Scientific Officer of BridgeBio, serve on our board of directors and retain their positions and affiliations with BridgeBio. Drs. Kumar and Sinha spend a significant portion of their time on other BridgeBio matters, including involvement with other BridgeBio subsidiaries. Additionally, Jonathan Fox, our Chief Medical Officer, serves as the Therapeutic Area Lead of Cardiovascular and Renal Diseases for BridgeBio and Cameron Turtle, our Chief Business Officer, serves as Senior Vice President, Portfolio Management and Corporate Development of BridgeBio. As a result, these executive officers may not be able to devote their full time and 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 a significant portion 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. 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 receive any cash or equity compensation from us and does not 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.

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. 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 acoramidis 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.

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

As of June 30, 2020 we had 64 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, acoramidis. 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 acoramidis 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 acoramidis 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 due to high levels of unemployment (particularly as a result of the COVID-19 pandemic), underemployment or the repeal of certain provisions of the ACA, may decrease the demand for healthcare services and pharmaceuticals. Additionally, the availability of healthcare services and resources has been and continues to be constrained due to the COVID-19 pandemic. If fewer patients are seeking medical care because they do not have insurance coverage or are unable to obtain medical care for their conditions due to resource constraints on the healthcare system, 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, including as a result of the COVID-19 pandemic, 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 acoramidis 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 COVID-19 pandemic, 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 service providers or consultants, may fail or suffer security breaches, which could result in a material disruption of our product candidates' development programs and have a material adverse effect on our reputation, business, financial condition or results of operations.

Despite the implementation of security measures, our internal computer systems and those of our current and future CROs and other service providers 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 or 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 acoramidis and other third parties for the manufacture of acoramidis 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 acoramidis could be delayed. We also rely on third-party service providers for aspects of our internal control over financial reporting and such service providers may experience a material system failure or fail to carry out their obligations in other respects, which may impact our ability to produce accurate and timely financial statements, thus harming our operating results, our ability to operate our business, and our investors' view of us.

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 acoramidis 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, outbreak of disease or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Earthquakes, outbreak of disease, 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. For example, we may experience delays in the supply of drug product for our clinical trials as a result of disruptions to the operations of manufacturing facilities of some of our third-party contract manufacturers in China due to the COVID-19 pandemic. Any continued or subsequent measures taken by governmental authorities or businesses to contain the spread of COVID-19, or the perception that such measures may be required in the future should another outbreak occur, could adversely affect our business, financial condition or results of operations by limiting our contract manufacturers' ability to manufacture product and forcing temporary closure of facilities that we rely upon. The extent to which COVID-19 impacts our results will depend on future developments, which are highly uncertain and cannot be accurately predicted, including new information which may emerge concerning the severity of COVID-19 and the actions to contain COVID-19 or treat its impact, among others. 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 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 employees outside the United States, but we are conducting clinical trials internationally through a global CRO, and our business strategy incorporates potential international expansion to target ATTR patient populations outside the United States. If we receive regulatory approval for and commercialize acoramidis 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.

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 (a) following the fifth anniversary of the completion of our initial public offering, or IPO, (b) in which we have total annual gross revenue of at least \$1.07 billion or (c) 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 (2) 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.

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 are 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 continue to design and implement 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 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 has been and 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;
- reports of adverse events or other negative results in clinical trials of third parties' product candidates for ATTR or similar indications;
- inability to obtain additional funding;
- any delay in filing an NDA for acoramidis or an IND or NDA for other product candidates that we may identify and pursue, 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 acoramidis or other product candidates that we may identify;
- failure to maintain our existing license and collaboration 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, including the effects of the COVID-19 pandemic on the global economy, 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 Amended and Restated 2018 Stock Option and Incentive Plan, or the 2018 Plan, we are authorized to grant stock options and other equity-based awards to our employees, directors and consultants. Most recently, in June 2020, our stockholders approved an increase in the number of shares reserved for future grant under the 2018 Plan. If our board of directors elects to further increase the number of shares available for future grant, and our stockholders approve any such further increase in the number of shares reserved under our equity incentive plans, 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, 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. In the past, the representatives of the underwriters in our IPO, in their discretion, released a portion of the shares subject to lock-up agreements, which resulted in sales by certain of our stockholders prior to the expiration of the lock-up period. Shares issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available for sale in the public market to the extent permitted by the provisions of applicable vesting schedules and Rule 144 and Rule 701 under the Securities Act of 1933, as amended, or the Securities Act. Additionally, in September 2019, we issued 556,173 shares of common stock to Alexion in connection with the Alexion License Agreement, which will also become available for public resale as and when permitted under applicable securities laws. 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 71.6% of our voting stock as of June 30, 2020. 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, or BridgeBio alone, 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. For example, in August 2019, we announced the receipt from BridgeBio of a non-binding proposal for BridgeBio to purchase all of our outstanding common stock not already held by BridgeBio. Although discussions between a special committee comprised of our disinterested and independent directors and BridgeBio with respect to the proposed transaction have terminated, BridgeBio continues to exercise significant control over our decisions and may, in the future, engage in similar discussions with us. Additionally, BridgeBio's ownership of a majority of the voting power of our common stock may enable it to block third-party acquisition proposals or offers for our common stock that our other stockholders may believe are in their best interests.

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 63.7% of the voting power of our outstanding common stock as of June 30, 2020. 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, Uma Sinha, Chief Scientific Officer of BridgeBio, Eric Aguiar, a member of the Board of Directors of BridgeBio, and Ali Satvat, a member of the Board of Directors of BridgeBio, serve on our board of directors and retain their positions and affiliations with BridgeBio. Additionally, Jonathan Fox, our Chief Medical Officer, and Cameron Turtle, our Chief Business Officer, also have roles within BridgeBio and/or its other subsidiaries. Our other stockholders may not have visibility into the BridgeBio ownership positions or other affiliations of any of our directors or officers with BridgeBio or its other subsidiaries, which may change at any time through acquisition, disposition, dilution, or otherwise. Any change in our directors' or officers' ownership in BridgeBio or its other subsidiaries 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 majority of our outstanding voting securities, BridgeBio will continue to control all matters that are subject to approval by our stockholders and will be able to strongly influence and significantly control our other decisions.

Although we do not currently 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 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 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; and
- expressly authorize our board of directors to modify, alter or repeal our amended and restated bylaws.

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

Furthermore, under our amended and restated bylaws, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for any state law claim for (i) any derivative action or proceeding brought on behalf of the Company, (ii) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of the Company to the Company or the Company's stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law or the certificate of incorporation or bylaws, or (iv) any action asserting a claim against the Company governed by the internal affairs doctrine (the “Delaware Forum Provision”); provided, however, that this Delaware Forum Provision does not apply to any actions arising under the Securities Act or the Exchange Act. The Delaware Forum Provision may impose additional litigation costs on stockholders in pursuing such claims, particularly if the stockholders do not reside in or near the State of Delaware. Additionally, the Delaware Forum Provision may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage the filing of such lawsuits. The Court of Chancery of the State of Delaware may also reach different judgment or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

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 acoramidis and any other product candidates we may identify and pursue, which may change from time to time;
- the cost of manufacturing acoramidis 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 acoramidis in accordance with our plans and to obtain regulatory approval for acoramidis 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 acoramidis or other product candidates that we may identify, should they receive approval, which may vary significantly;
- our ability to successfully commercialize acoramidis, if approved, in light of competition from other available products and product candidates under development;
- future accounting pronouncements or changes in our accounting policies;
- the risk/benefit profile, cost and reimbursement policies with respect to acoramidis 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, including as a result of the global COVID-19 pandemic.

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, the amount of post 2017 NOLs that we are permitted to deduct in any taxable year beginning after December 31, 2020 is limited to 80% of our taxable income in such year, where taxable income is determined without regard to the NOL deduction itself. Any NOL arising in a taxable year beginning after December 31, 2020 may not be carried back to prior taxable years, while post 2017 unused NOLs may be carried forward indefinitely. There is a risk that due to legislative or 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.

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 acoramidis 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 public company, and our management will devote substantial time to compliance initiatives.

As a public company, we 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, under the Dodd-Frank Wall Street Reform and Consumer Protection Act, there are significant corporate governance and executive compensation-related provisions 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*

None.

b) *Use of Proceeds*

Not applicable.

c) *Issuer Purchases of Company Equity Securities*

None.

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	10-Q	10/31/2019	3.1
3.2	Amended and Restated Bylaws	10-Q	10/31/2019	3.2
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
10.1#	Amended and Restated 2018 Stock Option and Incentive Plan and forms of award agreements thereunder	S-8	6/9/2020	4.4
31+	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			
32*	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			
101.SCH+	Inline XBRL Taxonomy Extension Schema Document			
101.CAL+	Inline XBRL Taxonomy Extension Calculation Linkbase Document			
101.DEF+	Inline XBRL Taxonomy Extension Definition Linkbase Document			
101.LAB+	Inline XBRL Taxonomy Extension Labels Linkbase Document			
101.PRE+	Inline XBRL Taxonomy Extension Presentation Linkbase Document			
104+	Cover Page Interactive Data File (formatted as inline XBRL with applicable taxonomy extension information contained in Exhibits 101.)			

Represents management compensation plan, contract or arrangement.

+ Filed herewith.

* The certification attached as Exhibit 32 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

