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

Form 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2022

or

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

For the transition period from to

Commission File Number: 001-36510

LARIMAR THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**20-3857670**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**
Three Bala Plaza East, Suite 506 **Bala Cynwyd, PA**	**19004**
(Address of principal executive offices)	**(zip code)**

(844) 511-9056

(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	LRMR	The Nasdaq Global Market

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

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

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

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

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

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

As of August 9, 2022, there were 17,710,450 shares of the registrant's Common Stock, $0.001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

Statements made in this Quarterly Report on Form 10-Q that are not statements of historical or current facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements discuss our business, operations and financial performance and conditions, as well as our plans, objectives and expectations for our business operations and financial performance and condition. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “design,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “predict,” “positioned,” “potential,” “seek,” “should,” “target,” “will,” “would” and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. In addition, statements that “we believe” or similar statements reflect our beliefs and opinions on the relevant subject only. These forward-looking statements, which are subject to risks, uncertainties and assumptions about us, may include projections of our future financial performance, our anticipated growth strategies and anticipated trends in our business.

You should understand that the following important factors could affect our future results and could cause those results or other outcomes to differ materially from those expressed or implied in our forward-looking statements:

- our ability to successfully engage with, and satisfactorily respond to, requests for additional information from the U.S. Food and Drug Administration ("FDA") concerning the clinical hold on our investigational new drug application ("IND") for CTI-1601 and the timing and outcomes of such interactions, including our plans to engage the FDA in order to lift the clinical hold, in full or in part, and allow re-initiation of interventional clinical studies;
- uncertainties in obtaining successful non-clinical or clinical results that reliably and meaningfully demonstrate safety, tolerability and efficacy profiles that are satisfactory to the FDA, European Medicines Agency ("EMA") and other comparable regulatory authorities for marketing approval for CTI-1601 or any other product candidate that we may develop in the future and unexpected costs that may result therefrom;
- further delays or changes in our anticipated clinical timelines, due to our interactions with the FDA related to resolving the current clinical hold on CTI-1601, as well as delays in patient recruitment (including the impact of other clinical trials of competitive products), delays as a result of clinical and non-clinical results and FDA's request for additional toxicology studies, changes in clinical protocols including FDA's acceptance of our proposed four-week dose exploration study, regulatory restrictions, including additional clinical holds, and milestones for CTI-1601, including those associated with COVID-19 and the efforts to mitigate it;
- uncertainties associated with the clinical development and regulatory approval for CTI-1601 or any other product candidate that we may develop in the future, including potential delays in the commencement, enrollment and completion of clinical trials;
- the difficulties and expenses associated with obtaining and maintaining regulatory approval for CTI-1601 or any other product candidate we may develop in the future, and the indication and labeling under any such approval;
- our estimates regarding future results of operations, financial position, research and development costs, capital requirements and our needs for additional financing;
- how long we can continue to fund our operations with our existing cash, cash equivalents and marketable debt securities;
- our ability, and the ability of third-party manufacturers we engage, to optimize and scale CTI-1601 or any other product candidate’s manufacturing process and to manufacture sufficient quantities of clinical supplies, if the clinical hold on the CTI-1601 IND is lifted, and, if approved, commercial supplies of CTI-1601;

- our ability to realize any value from CTI-1601 and any other product candidate we may develop in the future in light of inherent risks and difficulties involved in successfully bringing product candidates to market and the risk that the product candidates, if approved, will not achieve broad market acceptance;
- our ability to comply with regulatory requirements applicable to our business and other regulatory developments in the United States and other countries;
- the size and growth of the potential markets for CTI-1601 or any other product candidate that we may develop in the future, the rate and degree of market acceptance of CTI-1601 or any other product candidate, if approved, that we may develop in the future and our ability to serve those markets;
- Competing therapies and products including those that are still in clinical development which become available via marketing authorizations or compassionate use and their impact on our ability to recruit and retain clinical trial patients, to obtain and maintain potential expedited regulatory pathways, and to commercialize current and future product candidates, if approved, (including the impact of potential barriers to entry if a competitor is able to establish a strong market position before we are able to commercialize our products);
- our ability to obtain and maintain patent protection and defend our intellectual property rights against third-parties;
- the performance of third parties upon which we depend, including third-party contract research organizations ("CROs") and third-party suppliers, manufacturers, distributors, and logistics providers;
- our ability to maintain our relationships, and contracts with our key vendors and to identify and contract with alternate or secondary key vendors;
- our ability to recruit and retain key scientific, technical, commercial, and management personnel and to retain our executive officers;
- our ability to maintain proper functionality and security of our internal computer and information systems and prevent or avoid cyber-attacks, malicious intrusion, breakdown, destruction, loss of data privacy or other significant disruption; and
- the extent to which health epidemics, unforeseen emergencies and other outbreaks of communicable diseases, including the ongoing COVID-19 pandemic and the efforts to mitigate it, geopolitical turmoil, including the ongoing invasion of Ukraine by Russia or increased trade restrictions between the United States, Russia, China, and other countries, social unrest, political instability, terrorism or other acts of war could disrupt our operations, the operations of third parties on which we rely or the operations of regulatory agencies we interact with in the development of CTI-1601.

These forward-looking statements are based on management’s current expectations, estimates, forecasts and projections about our business and the industry in which we operate, and management’s beliefs and assumptions are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. In light of the significant uncertainties in these forward-looking statements, you should not rely upon forward-looking statements as predictions of future events. Although we believe the expectations reflected in the forward-looking statements are reasonable, the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements may not be achieved or occur at all. The factors that could cause or contribute to such differences include, but are not limited to, those discussed in our Annual Report on Form 10-K filed on March 25, 2022. All forward-looking statements are applicable only as of the date on which they were made and, except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise after the date of this Quarterly Report on Form 10-Q or to reflect the occurrence of any unanticipated events. Comparisons of results for current and any prior periods are not intended to express any future trends or indications of future performance, unless expressed as such, and should only be viewed as historical data.

Larimar Therapeutics, Inc.

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PART I-FINANCIAL INFORMATION

Item 1. Financial Statements

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)
(Unaudited)

	June 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 19,736	$ 70,097
Marketable debt securities	35,188	—
Prepaid expenses and other current assets	1,820	2,107
Total current assets	56,744	72,204
Property and equipment, net	986	1,049
Operating lease right-of-use assets	3,134	3,406
Restricted cash	1,339	1,339
Other assets	649	669
Total assets	$ 62,852	$ 78,667
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 425	$ 1,660
Accrued expenses	6,656	6,592
Operating lease liabilities, current	638	594
Total current liabilities	7,719	8,846
Operating lease liabilities	5,077	5,408
Total liabilities	12,796	14,254
Commitments and contingencies (See Note 8)		
Stockholders' equity:		
Preferred stock; $0.001 par value per share; 5,000,000 shares authorized as of June 30, 2022 and December 31, 2021; no shares issued and outstanding as of June 30, 2022 and December 31, 2021	—	—
Common stock, $0.001 par value per share; 115,000,000 shares authorized as of June 30, 2022 and December 31, 2021; 17,710,450 shares issued and outstanding as of June 30, 2022 and December 31, 2021	18	18
Additional paid-in capital	183,955	180,645
Accumulated deficit	(133,860)	(116,250)
Accumulated other comprehensive loss	(57)	—
Total stockholders' equity	50,056	64,413
Total liabilities and stockholders' equity	$ 62,852	$ 78,667

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 5,644	$ 9,102	$ 11,450	$ 18,076
General and administrative	3,043	3,441	6,124	6,573
Total operating expenses	8,687	12,543	17,574	24,649
Loss from operations	(8,687)	(12,543)	(17,574)	(24,649)
Other income (expense), net	20	(66)	(36)	(48)
Net loss	$ (8,667)	$ (12,609)	$ (17,610)	$ (24,697)
Net loss per share, basic and diluted	$ (0.47)	$ (0.79)	$ (0.96)	$ (1.54)
Weighted average common shares outstanding, basic and diluted	18,338,853	15,996,133	18,338,853	15,996,133
Comprehensive loss:				
Net loss	$ (8,667)	$ (12,609)	$ (17,610)	$ (24,697)
Other comprehensive loss:				
Unrealized loss on marketable debt securities	(57)	—	(57)	—
Total other comprehensive loss	(57)	—	(57)	—
Total comprehensive loss	$ (8,724)	$ (12,609)	$ (17,667)	$ (24,697)

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total Stockholders' Equity
	Shares	Par Value				
Balances as of December 31, 2021	17,710,450	$ 18	$ 180,645	$ (116,250)	$ —	$ 64,413
Stock-based compensation expense	—	—	1,635	—	—	1,635
Net loss	—	—	—	(8,943)	—	(8,943)
Balances as of March 31, 2022	17,710,450	$ 18	$ 182,280	$ (125,193)	$ —	$ 57,105
Stock-based compensation expense	—	—	1,675	—	—	1,675
Unrealized loss on marketable debt securities	—	—	—	—	(57)	(57)
Net loss	—	—	—	(8,667)	—	(8,667)
Balances as of June 30, 2022	17,710,450	$ 18	$ 183,955	$ (133,860)	$ (57)	$ 50,056

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Accumulated Other Comprehensive Gain	Total Stockholders' Equity
	Shares	Par Value				
Balances as of December 31, 2020	15,367,730	$ 15	$ 155,290	$ (65,614)	$ 1	$ 89,692
Stock-based compensation expense	—	—	1,180	—	—	1,180
Net loss	—	—	—	(12,088)	—	(12,088)
Balances as of March 31, 2021	15,367,730	$ 15	$ 156,470	$ (77,702)	$ 1	$ 78,784
Stock-based compensation expense	—	—	1,350	—	—	1,350
Net loss	—	—	—	(12,609)	—	(12,609)
Balances as of June 30, 2021	15,367,730	$ 15	$ 157,820	$ (90,311)	$ 1	$ 67,525

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

LARIMAR THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)
(Unaudited)

	Six Months Ended June 30,	
	2022	2021
Cash flows from operating activities:		
Net loss	$ (17,610)	$ (24,697)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	3,310	2,530
Loss on disposal of fixed asset	—	5
Depreciation expense	163	150
Amortization of premium on marketable securities	(3)	(11)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	287	1,908
Accounts payable	(1,235)	(1,466)
Accrued expenses	64	(168)
Right-of-use assets	272	263
Operating lease liabilities	(287)	(249)
Other assets	20	(253)
Net cash used in operating activities:	(15,019)	(21,988)
Cash flows from investing activities:		
Purchase of property and equipment	(100)	(31)
Purchase of marketable debt securities	(35,242)	(1,749)
Maturities and sales of marketable debt securities	—	26,250
Net cash provided by (used in) investing activities	(35,342)	24,470
Cash flows from financing activities:	—	—
Net cash provided by financing activities	—	—
Net increase in cash, cash equivalents and restricted cash	(50,361)	2,482
Cash, cash equivalents and restricted cash at beginning of period	71,436	69,487
Cash, cash equivalents and restricted cash at end of period	$ 21,075	$ 71,969
Supplemental disclosure of non-cash investing and financing activities:		
Property and equipment included in accounts payable and accrued expenses	$ —	$ 295

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

1. Description of Business and Basis of Presentation

Larimar Therapeutics, Inc., together with its subsidiary (the “Company” or “Larimar”), is a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using its novel cell penetrating peptide technology platform. Larimar's lead product candidate, CTI-1601, is a subcutaneously administered, recombinant fusion protein intended to deliver human frataxin ("FXN") an essential protein, to the mitochondria of patients with Friedreich’s ataxia. Friedreich’s ataxia is a rare, progressive and fatal disease in which patients are unable to produce sufficient FXN due to a genetic abnormality.

In May 2021, Larimar reported positive topline data from its Phase 1 Friedreich’s ataxia, ("FA") program after completing dosing of the single ascending dose, ("SAD"), trial in December 2020 and of the multiple ascending dose, ("MAD"), trial in March 2021.

Also in May 2021, the FDA placed a clinical hold on the Company's CTI-1601 clinical program after the Company notified the agency of mortalities at the highest dose levels of the 26-week non-human primate toxicology study that was designed to support extended dosing of patients with CTI-1601. At the time the hold was placed, Larimar had no interventional clinical trials with patients enrolling or enrolled.

In February 2022, in response to the Company's complete response to the clinical hold discussed above, the Agency stated that it was maintaining the clinical hold and that additional data are needed to resolve the clinical hold. Larimar subsequently submitted a request for an FDA Type C meeting.

In the third quarter of 2022, the Company and the FDA had a Type-C meeting. The purpose of the Type C Meeting was to obtain FDA feedback on the information needed to resolve CTI-1601's current clinical hold in full or in part, as well as to discuss a proposed change in CTI-1601’s clinical development plan to introduce a Phase 2 dose exploration study to precede initiation of an open label extension study. The Company plans to submit a complete response to the CTI-1601 clinical hold later in the third quarter of 2022. In conjunction with the complete response, Larimar is proposing as CTI-1601’s next clinical trial a Phase 2, four-week dose exploration study in FA patients starting at the lower dose levels tested in the Company’s Phase 1 multiple-ascending dose clinical trial. This study will provide data on extended dosing of lower doses of CTI-1601, providing insights into safety and tolerability as well as whether lower doses for longer periods of time can increase frataxin levels, while still maintaining acceptable exposures. Throughout the course of the interactions with the FDA, Larimar intends to continue to work with the Agency to enable resolution of the clinical hold and to agree on the study design and timing of the next proposed clinical trial. Larimar does not know when, or if the clinical hold will be lifted.

The Company is subject to risks and uncertainties common to pre-commercial companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with governmental regulations, failure to secure regulatory approval for its drug candidates or any other product candidates and the ability to secure additional capital to fund its operations. Drug candidates currently under development will require extensive non-clinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, and infrastructure and extensive compliance-reporting capabilities. Even if our drug development efforts are successful, it is uncertain when, if ever, we will realize significant revenue from product sales.

In late 2019, an outbreak of a novel strain of the Coronavirus 2019 Disease (COVID-19) was identified and infections have been found in a number of countries around the world, including the United States. The Company’s business, results of operations, financial condition and cash flows could be materially and adversely affected. Specifically, the Company could experience additional delays in future clinical trial timelines as a result of additional travel and hospital restrictions related to the COVID-19 pandemic and the efforts to mitigate it which may be imposed or could experience supply shortages or manufacturer shutdowns impacting the manufacture of drug substance or drug product, which could also impact clinical trial timelines. The condensed consolidated financial statements do not reflect any adjustments as a result of the pandemic.

Basis of Presentation

The condensed consolidated financial statements include the accounts of Larimar and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated. The accompanying condensed consolidated financial statements have been prepared in conformity with GAAP.

Going Concern Assessment

The Company's condensed consolidated financial statements have been presented on the basis that it will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business.

Since its inception, the Company has incurred significant recurring operating losses and negative cash flows from operations. The Company has incurred net losses of $17.6 million and $24.7 million for the six months ended June 30, 2022 and 2021, respectively. In addition, as of June 30, 2022, the Company had an accumulated deficit of $133.9 million. The Company expects to continue to generate operating losses for the foreseeable future. As of June 30, 2022, the Company had approximately $54.9 million of cash, cash equivalents and marketable debt securities available for use to fund its operations and capital requirements.

In August 2020, the Company entered into an Equity Distribution Agreement (the "ATM Agreement") with an investment bank in connection with the establishment of an "at-the-market" offering program under which the Company could sell up to an aggregate of $50,000,000 of shares of its common stock from time to time through this investment bank as sales agent. In 2021, the Company sold 2,342,720 shares pursuant to the ATM Agreement for gross proceeds of $20.5 million. As of June 30, 2022, $29.2 million of additional shares of common stock remained available for sale by the Company under the ATM Agreement. See Note 7 for a further discussion of the ATM Agreement.

In accordance with Accounting Standards Update ("ASU") No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the condensed consolidated financial statements are issued. As of the issuance date of these condensed consolidated financial statements, the Company expects its cash, cash equivalents and marketable debt securities will be sufficient to fund its forecasted operating expenses and capital expenditure requirements, for at least twelve months from the issuance of these financial statements. If the timing of our clinical assumptions were delayed or if there were other forecasted assumption changes that negatively impact our operating plan, the Company could reduce expenditures in order to further extend cash resources.

The Company has not yet commercialized any products and does not expect to generate revenue from the commercial sale of any products for several years, if at all. The Company expects that its research and development and general and administrative expenses will continue to increase and, as a result, it will need additional capital to fund its future operating and capital requirements. Until the Company can generate substantial revenue, if ever, management continuously evaluates different strategies to obtain the required funding for future operations. These strategies include seeking additional funding through a combination of public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. The incurrence of indebtedness would result in increased fixed payment obligations and the Company may be required to agree to certain restrictive covenants, such as limitations on its ability to incur additional debt, limitations on its ability to acquire, sell or license intellectual property rights, minimum required cash balances and other operating restrictions that could adversely impact the Company's ability to conduct its business. Any additional fundraising efforts may divert the Company's management from their day-to-day activities, which may adversely affect its ability to develop and commercialize our product candidates.

There can be no assurance that the Company will be able to raise sufficient additional capital on acceptable terms, if at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or if the Company does not have sufficient authorized shares, the Company may be required to delay, limit, or eliminate the development of business opportunities and its ability to achieve its business objectives, its competitiveness, and its business, financial condition, and results of operations will be materially adversely affected. The Company could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and it may be required to relinquish rights to some of its technologies or product candidates or otherwise agree to terms unfavorable to it, any of which may have a material adverse effect on the Company's business, operating results and prospects. In addition, geopolitical unrest including the potential impact of the Russian invasion of Ukraine, the possibility that the conflict could expand beyond eastern Europe, the impact of the COVID-19 pandemic and/or other health crises on the global financial markets may reduce the Company's ability to access capital, which could negatively affect its liquidity and ability to continue as a going concern.

If the Company is unable to obtain funding when needed and/or on acceptable terms, the Company may be required to significantly curtail, delay or discontinue one or more of its research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion or pre commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations.

2. Summary of Significant Accounting Policies

Unaudited Interim Financial Information

The condensed consolidated balance sheet as of December 31, 2021 was derived from the Company's audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited condensed consolidated financial statements as of June 30, 2022 and for the three and six months ended June 30, 2022 and 2021, have been prepared by the Company, pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC"), for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2021 included in the Company's Annual Report on Form 10-K filed with the SEC on March 25, 2022.

In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair statement of the Company's condensed consolidated financial position as of June 30, 2022, condensed consolidated results of operations for the three and six months ended June 30, 2022 and condensed consolidated statement of cash flows for the six months ended June 30, 2022 have been made. The results of operations for the three and six months ended June 30, 2022 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2022.

Use of Estimates

The preparation of condensed consolidated 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 at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual of research and development expense, valuation of stock-based awards and valuation of leases. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions.

Research and Development Costs

Costs associated with internal research and development and external research and development services, including drug development, clinical studies and non-clinical studies, are expensed as incurred. Research and development expenses include costs for salaries, employee benefits, subcontractors, facility-related expenses, depreciation, stock-based compensation, third-party license fees, laboratory supplies, and external costs of outside vendors engaged to conduct discovery, non-clinical and clinical development activities, and clinical trials as well as to manufacture clinical trial materials, and other costs. The Company recognizes external research and development costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its service providers.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such prepaid expenses are recognized as an expense when the goods have been delivered or the related services have been performed, or when it is no longer expected that the goods will be delivered, or the services rendered.

Upfront payments, milestone payments and annual maintenance fees under license agreements are currently expensed in the period in which they are incurred.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

The Company measures all stock-based awards granted to employees, directors and non-employee consultants based on the fair value on the date of grant using the Black-Scholes option-pricing model. Compensation expense of those awards is recognized over the requisite service period, which is the vesting period of the respective award. Typically, the Company issues awards with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company accounts for forfeitures as they occur.

The Company classifies stock-based compensation expense in its condensed consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Prior to May 28, 2020, the Company had been a private company and lacked company-specific historical and implied volatility information for its common stock. Therefore, the Company estimates its expected common stock price volatility based on the historical volatility of publicly traded peer companies and expects to continue to do so until it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield considers the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

Net Loss Per Share

Basic net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period. Basic shares outstanding includes the weighted average effect of the Company's prefunded warrants issued in June 2020, the exercise of which requires little or no consideration for the delivery of shares of common stock. Basic and diluted weighted average shares of common stock outstanding for the three and six months ended June 30, 2022 and 2021 includes the weighted average effect of 628,403 prefunded warrants for the purchase of shares of common stock, for which the remaining unfunded exercise price is $0.01 per share.

Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of common shares, including potentially dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted common shares, as determined using the treasury stock method. For periods in which the Company has reported net losses, diluted net loss per common share attributable to common stockholders is the same as basic net loss per common share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is antidilutive.

The Company excluded 3,130,370 and 2,546,962 common stock equivalents outstanding as of June 30, 2022 and 2021, respectively, from the computation of diluted net loss per share for the three and six months ended June 30, 2022 and 2021 because they had an anti-dilutive impact due to the net loss incurred for the periods presented.

Recently Issued and Adopted Accounting Pronouncements

From time to time, new accounting guidance is issued by the FASB or other standard setting bodies that is adopted by us as of the effective date or, in some cases where early adoption is permitted, in advance of the effective date. We have assessed the recently issued guidance that is not yet effective and believe the new guidance will not have a material impact on the condensed consolidated results of operations, cash flows or financial position.

3. Fair Value Measurements and Marketable Debt Securities

Fair Value Measurements

The Company's assets and liabilities that are measured at fair value on a recurring basis as of June 30, 2022 and December 31, 2021 are measured in accordance with the standards of ASC 820, *Fair Value Measurements and Disclosures*, which establishes a three-level valuation hierarchy for measuring fair value and expands financial statement disclosures about fair value measurements. The valuation hierarchy is based on the transparency of inputs to the valuation of an asset or liability as of the measurement date. The three levels are defined as follows:

Level – 1	Inputs to the valuation methodology are quoted prices (unadjusted) for identical assets or liabilities in active markets.
Level – 2	Inputs to the valuation methodology include quoted prices for similar assets and liabilities in active markets, and inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the financial instrument.
Level – 3	Inputs to the valuation methodology are unobservable and significant to the fair value measurement.

The Company's financial instruments consist primarily of cash, cash equivalents, marketable debt securities, accounts payable and accrued liabilities. For accounts payable and accrued liabilities, the carrying amounts of these financial instruments as of June 30, 2022 and December 31, 2021 were considered representative of their fair values due to their short term to maturity.

The following tables summarize the Company's cash equivalents and marketable debt securities as of June 30, 2022 and cash equivalents as of December 31, 2021:

	Total	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
	(in thousands)			
June 30, 2022				
Cash equivalents:				
Money market funds	$ 17,678	17,678		
Total cash equivalents	17,678	17,678	—	—
Marketable debt securities:				
U.S. Government securities	35,188	—	35,188	
Total marketable debt securities	35,188	—	35,188	—
Total cash equivalents and marketable debt securities	$ 52,866	$ 17,678	$ 35,188	$ —
December 31, 2021				
Cash equivalents:				
Money market funds	$ 6,137	$ 6,137	$ —	$ —
Commercial paper	7,549	7,549	—	—
Corporate bonds	1,219	1,219	—	—
Total cash equivalents	14,905	14,905	—	—

The accrued interest receivable related to the Company's investments was $0.1 million as of June 30, 2022 and is included in prepaid expenses and other current assets on the condensed consolidated balance sheet. There was no

accrued interest receivable and December 31, 2021.

The Company classifies its money market funds which are valued based on quoted market prices in active markets with no valuation adjustment, as Level 1 assets within the fair value hierarchy.

The Company classifies its investments in U.S. treasury securities corporate commercial paper, and corporate debt securities as Level 2 assets within the fair value hierarchy. The fair values of these investments are estimated by taking into consideration valuations obtained from third-party pricing services. The pricing services utilize industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs

As of June 30, 2022 and December 31, 2021, the unrealized losses for available-for-sale investments were non-credit related, and the Company does not intend to sell the investments that were in an unrealized loss position, nor will it be required to sell those investments before recovery of their amortized cost basis, which may be maturity. As of June 30, 2022 and December 31, 2021, no allowances for credit losses for the Company's investments were recorded. During the three and six months ended June 30, 2022 and 2021, the Company did not recognize any impairment losses related to investments.

Marketable Debt Securities

The following table summarizes the Company's marketable debt securities as of June 30, 2022. The Company held no marketable debt securities as of December 31, 2021.

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
June 30, 2022				
Assets:				
U.S. Government securities	$ 35,245	—	(57)	$ 35,188
	$ 35,245	$ —	$ (57)	$ 35,188

4. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	June 30, 2022	December 31, 2021
	(in thousands)	
Prepaid research and development expenses	$ 1,031	$ 676
Prepaid insurance	186	944
Payroll tax receivable	237	208
Other prepaid expenses and other assets	366	279
	$ 1,820	$ 2,107

5. Fixed Assets

Fixed assets, net consisted of the following:

	Useful Life	June 30, 2022	December 31, 2021
		(in thousands)	
Computer equipment	5 years	$ 66	$ 66
Lab equipment	5 years	1,192	1,092
Furniture and fixtures	7 years	456	456
Leasehold improvements	lease term	31	31
		1,745	1,645
Less: Accumulated depreciation		(759)	(596)
		$ 986	$ 1,049

Depreciation expense during the three and six months ended June 30, 2022 was $0.1 million, respectively. Depreciation expense during the three and six months ended June 30, 2021 was $0.1 million and $0.2 million, respectively.

6. Accrued Expenses

	June 30, 2022	December 31, 2021
	(in thousands)	
Accrued research and development expenses	$ 5,135	$ 5,042
Accrued payroll and related expenses	1,002	1,098
Accrued other	519	452
	$ 6,656	$ 6,592

7. Stockholders' Equity and Stock Options

Common Stock and Prefunded Warrants

As of June 30, 2022, the Company's Certificate of Incorporation, as amended and restated, authorized the Company to issue up to 115,000,000 shares of $0.001 par value common stock, of which 17,710,450 shares were issued and outstanding, and up to 5,000,000 shares of $0.001 par value undesignated preferred stock, of which no shares were issued or outstanding. The voting, dividend, and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers, and preferences of the holders of the preferred stock. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors of the Company (the "Board"), if any. No cash dividends have been declared or paid to date.

On May 28, 2020, the Company entered into a securities purchase agreement with certain accredited investors (the "Purchasers") for the sale by the Company in a private placement of 6,105,359 shares of the Company's common stock and prefunded warrants to purchase an aggregate of 628,403 shares of the Company's common stock, for a price of $11.88 per share of the common stock and $11.87 per prefunded warrant. The prefunded warrants are exercisable at an exercise price of $0.01 and are exercisable indefinitely. The Purchasers may exercise the prefunded warrants on a cashless basis in the event that there is no effective registration statement covering the resale of the shares of common stock underlying the prefunded warrants on the date in which the Company is required to deliver the shares. The private placement closed on June 1, 2020. The aggregate gross proceeds for the issuance and sale of the common stock and prefunded warrants were $80.0 million; transaction costs totaled $4.6 million and resulted in net proceeds of $75.4 million. The Company's Registration Statement on Form S-3, filed with the SEC on June 26, 2020, registered the resale of 6,105,359 shares of common stock sold and the 628,403 shares of common stock underlying the prefunded warrants. MTS Health Partners served as placement agent to the Company in connection with the private placement. As partial compensation for these services, the Company issued MTS Health Partners 35,260 shares of common stock.

Equity Distribution Agreement

On August 14, 2020, the Company entered into the ATM Agreement with an investment bank in connection with the establishment of an "at-the-market" offering program under which the Company may sell up to an aggregate of $50,000,000 of shares of common stock (the "ATM Shares") from time to time (the "Offering").

Under the ATM Agreement, the Company sets the parameters for the sale of ATM Shares, including the number of ATM Shares to be issued, the time period during which sales are requested to be made, limitations on the number of ATM Shares that may be sold in any one trading day and any minimum price below which sales may not be made. Sales of the ATM Shares, if any, under the ATM Agreement may be made in transactions that are deemed to be “at-the-market offerings” as defined in Rule 415 under the Securities Act. The Company pays its investment bank a commission equal to 3.0% of the gross proceeds of any ATM Shares sold through its investment bank under the ATM Agreement and reimburses the investment bank for certain specified expenses. The ATM Agreement contains customary representations, warranties and agreements by the Company, indemnification obligations of the Company and its investment bank, other customary obligations of the parties and termination provisions. The Company has no obligation to sell any of the ATM Shares and may at any time suspend offers under the ATM Agreement.

In 2021, the Company sold 2,342,720 shares under the ATM Agreement for net proceeds of $19.9 million.

As of June 30, 2022, 2,354,244 shares of Common Stock have been sold under the ATM Agreement for net proceeds of $20.1 million. As of June 30, 2022, approximately $29.2 million shares of common stock still remained available for sale by the Company under the ATM Agreement.

2020 Equity Incentive Plan

The Board adopted the 2020 Equity Incentive Plan (the "2020 Plan") on July 16, 2020 and the stockholders of the Company approved the 2020 Plan on September 29, 2020. The 2020 Plan replaces the predecessor plans (the "Prior Plans") that the Company assumed following its merger with Zafgen, Inc. ("Zafgen") in May 2020. Options outstanding under the Prior Plans will remain outstanding, unchanged, and subject to the terms of the Prior Plans and the respective award agreements, and no further awards will be made under the Prior Plans. However, if any award previously granted under the Prior Plans, expires, terminates, is canceled, or is forfeited for any reason after the approval of the 2020 Plan, the shares subject to that award will be added to the 2020 Plan share pool so that they can be utilized for new grants under the 2020 Plan.

The 2020 Plan provides for the grant of incentive stock options (“ISOs”), nonstatutory stock options (“NSOs”), stock appreciation rights, restricted stock awards, restricted stock unit awards, and cash or other stock-based awards. ISOs may be granted only to the Company’s employees, including the Company’s officers, and the employees of the Company’s affiliates. All other awards may be granted to the Company’s employees, including the Company’s officers, the Company’s non-employee directors and consultants, and the employees and consultants of the Company’s affiliates.

The maximum number of shares that may be issued in respect of any awards under the 2020 Plan is the sum of: (i) 1,700,000 shares plus (ii) an annual increase on January 1, 2021 and each anniversary of such date thereafter through January 1, 2030, equal to the lesser of (A) 4% of the shares issued and outstanding on the last day of the immediately preceding fiscal year, or (B) such smaller number of shares as determined by the Board (collectively, the “Plan Limit”). The maximum aggregate number of shares that may be issued under the 2020 Plan is 8,000,000 over the ten-year term of the 2020 Plan. During the six months ended June 30, 2022 and twelve months ended December 31, 2021, respectively, options to purchase 130,308 and 56,966 shares issued under the Prior Plans were cancelled and became available for grant under the 2020 Plan.

As permitted by the 2020 Plan, the Company added 614,709 and 708,418 shares available for grant to the 2020 Plan on January 1, 2021 and January 1, 2022, respectively. As of June 30, 2022, 998,301 shares of common stock were available for grant under the 2020 Plan.

Stock Valuation

The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees:

	June 30, 2022	December 31, 2021
Risk-free interest rate	2.11%	0.89%
Expected term (in years)	6.21	6.19
Expected volatility	89%	91%
Dividend yield	0.00%	0.00%

Stock Options

The following table summarizes the Company's stock option activity for the six months ended June 30, 2022 (amounts in millions, except for share and per share data):

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (a) (in millions)
Outstanding as of December 31, 2021	2,523,305	$ 18.88	7.6	
Granted	852,450	7.08		
Forfeited/Expired	(245,385)	61.54		
Outstanding as of June 30, 2022	3,130,370	$ 12.32	8.3	$ —
Exercisable as of June 30, 2022	1,201,628	$ 14.98	7.4	$ —
Vested and expected to vest as of June 30, 2022	3,130,370	$ 12.32	8.3	$ —

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were "in the money" at June 30, 2022. At June 30, 2022, none of the Company's stock options were "in the money."

2022 Option Grants

During the six months ended June 30, 2022, the Company granted options to purchase 852,450 shares of common stock to employees and directors of the Company under the 2020 Plan. The options have an exercise price equal to the closing stock price as of the grant date. Of the 852,450 options granted in 2022, 810,950 were granted to employees and vest over four years with 25% vesting on the first anniversary of the grant and the remainder vesting in equal monthly installments thereafter. The remaining 41,500 options were annual grants to the Company's directors and vest one year from the grant date. The weighted-average grant date fair value of options granted during the six months ended June 30, 2022 was $5.31.

Stock-Based Compensation

Stock-based compensation expense was classified in the condensed consolidated statements of operations as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Research and development	$ 694	$ 515	$ 1,349	$ 953
General and administrative	981	835	1,961	1,577
	$ 1,675	$ 1,350	$ 3,310	$ 2,530

As of June 30, 2022, total unrecognized compensation expense related to unvested stock options was $15.7 million, which is expected to be recognized over a weighted average period of 2.47 years.

8. Commitments and Contingencies

Intellectual Property Licenses

The Company is party to an exclusive License Agreement (the "WFUHS License"), dated November 30, 2016 with Wake Forest University Health Sciences ("WFUHS") and an exclusive License Agreement (the "IU License"), dated November 30, 2016, as amended, with Indiana University ("IU"). Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology used by the Company with respect to the development of CTI-1601.

In partial consideration for the right and license granted under these agreements, the Company will pay each of WFUHS and IU a royalty of a low single digit percentage of net sales of licensed products depending on whether there is a valid patent covering such products. As additional consideration for these agreements, the Company is obligated to pay each of WFUHS and IU certain milestone payments of up to $2.6 million in the aggregate upon the achievement of certain developmental milestones, commencing on the enrollment of the first patient in a Phase 1 clinical trial. The Company enrolled the first patient in its SAD trial on December 11, 2019 and paid WFUHS and IU less than $0.1 million. The Company will also pay each of WFUHS and IU sublicensing fees ranging from a high-single digit to a low double-digit percentage of sublicense consideration depending on the Company's achievement of certain regulatory milestones as of the time of receipt of the sublicense consideration. The Company is also obligated to reimburse WFUHS and IU for patent-related expenses. In the event that the Company disputes the validity of any of the licensed patents, the royalty rate would be tripled during such dispute. The Company is also obligated to pay to IU a minimum annual royalty of less than $0.1 million per annum starting in the 2020 calendar year for the term of the agreement.

In the event that the Company is required to pay IU consideration, then the Company may deduct 20% of such IU consideration on a dollar-for-dollar basis from the consideration due to WFUHS. In the event that the Company is required to pay WFUHS consideration, then the Company may deduct 60% of such WFUHS consideration on a dollar-for-dollar basis from the consideration due to IU.

During the three and six months ended June 30, 2022, no milestones were achieved and no milestone expense was recognized. Both agreements continue from their effective date through the last to expire of the licensed patents unless earlier terminated by either party in accordance with their terms.

Leases

On August 8, 2019, the Company entered into an operating lease for office space in Bala Cynwyd, Pennsylvania, effective as of December 15, 2019, for a period of three years and six months with an option to extend the lease for three additional years. Due to required tenant improvements to be completed by the landlord, the Company did not take immediate possession of the leased property and the lease term commenced on February 15, 2020.

On May 28, 2020, the Company acquired a non-cancellable operating lease for approximately 17,705 square feet of office space (the "Premises"). The lease expires on October 30, 2029. As part of the agreement, the Company is required to maintain a letter of credit, which upon signing was $1.3 million and is classified as restricted cash within the condensed consolidated financial statements. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The right-of-use asset is being amortized to other income/(expense) over the remaining lease term as a result of the sublease described below.

On October 27, 2020, the Company entered into a sublease agreement (the "Sublease") with Massachusetts Municipal Association, Inc. (the "Subtenant"), whereby the Company sublet the entire Premises to the Subtenant. The initial term of the Sublease commenced on December 4, 2020 and continues until October 30, 2029. In connection with the Sublease, the Company evaluated the need for impairment under ASC 360 and determined there was no impairment.

The Sublease provided for an initial annual base rent of $0.8 million, which increases annually up to a maximum annual base rent of $1.0 million. The Subtenant also is responsible for paying to the Company future increases in operating costs (commencing on January 1, 2022), future increases in annual tax costs (commencing July 1, 2021) and all utility costs (commencing March 1, 2021) attributable to the Premises during the term of the Sublease. As part of the Sublease, the subtenant deposited a letter of credit in the amount of $0.8 million to assure their performance under the sublease. If there are no uncured events of default under the sublease, the amount of this security deposit decreases over time to $0.4 million on the sixth anniversary of the Sublease. The Company records sublease income on this sublease on a straight-line basis as a component of other income/(expense).

On November 5, 2018, the Company entered into an operating lease for office and lab space in Philadelphia, Pennsylvania, effective as of January 1, 2019, and expiring on December 31, 2020 with an option to extend the lease for two additional years. On August 4, 2020, the Company executed the first option to extend the lease for an additional year, expiring on December 31, 2021. On August 9, 2021, the Company executed the remaining option to extend the lease for an additional year, expiring on December 31, 2022. The Company has determined this lease extension qualifies as a short-term lease and have applied the accounting policy election to not record the related right-of-use asset and lease liabilities.

Expense arising from operating leases was $0.1 million and $0.2 million during the three and six months ended June 30, 2022 and $0.1 million during the three and six months ended June 30, 2021, respectively. For operating leases, the weighted-average remaining lease term for leases at June 30, 2022 and December 31, 2021 was 7.2 and 7.6 years, respectively. For operating leases, the weighted average discount rate for leases at June 30, 2022 and December 31, 2021 was 11.0%. The Company has not entered into any financing leases.

Maturities of lease liabilities due under these lease agreements as of June 30, 2022 are as follows:

(in thousands)	Operating Leases
Six months ending December 31, 2022	$ 600
Year ended December 31, 2023	1,146
Year ended December 31, 2024	1,065
Year ended December 31, 2025	1,083
Year ended December 31, 2026	1,101
Thereafter	3,213
Total lease payments	8,208
Less: imputed interest	(2,493)
Present value of lease liabilities	$ 5,715

Legal Proceedings

The Company is not currently a party to any litigation, nor is management aware of any pending or threatened litigation against the Company, that it believes would materially affect the Company's business, operating results, financial condition or cash flows.

9. Related Party Transactions

In November 2016, the Company entered into a consulting agreement with Mark Payne, M.D (the “Consulting Engagement”). Dr. Payne was a director of Chondrial at that time, a full-time employee of IU and one of the inventors of the licensed IU intellectual property, and as such is entitled to a certain share of the revenues received by IU under the IU License. Pursuant to the terms of his consulting agreement the Company agreed to pay Dr. Payne $0.1 million per year over the term of the agreement and granted Dr. Payne 123,853 restricted Common Units in Chondrial Holdings, LLC, the sole investor in Chondrial Therapeutics which merged into the Company in May of 2020 ("Holdings"). On November 30, 2016, 30% immediately vested and was associated with Chondrial Therapeutics IP, LLC (“IP LLC”) becoming a subsidiary of Holdings, which was subsequently contributed to the Company on December 31, 2018. The remaining 70% vested ratably over 48 months beginning on December 1, 2016 and were fully vested as of December 31, 2020. The consulting agreement has a four-year term, subject to earlier termination. On November 30, 2020, The Company entered into a 1-month extension of the Consulting Engagement, expiring on December 31, 2020 and on January 1, 2021, the Company entered into a new consulting agreement with Dr. Payne which extended the term of the Consulting Engagement for a four-year term beginning on January 1, 2021. During the three and six months ended June 30, 2022 and 2021 the Company recognized less than $0.1 million and $0.1 million, respectively, related to this consulting agreement, recorded as research and development expense in the Condensed Consolidated Statements of Operations.

During 2021, the Company purchased a piece of laboratory equipment and lab supplies for a cumulative $0.1 million from a supplier of which one of the Company's directors is also a current director.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q ("Quarterly Report"), and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2021 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 25, 2022 ("2021 Annual Report"). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks, uncertainties, and assumptions. These statements are based on our beliefs and expectations about future outcomes and are subject to risks and uncertainties that could cause our actual results to differ materially from anticipated results. We undertake no obligation to publicly update these forward-looking statements, whether as a result of new information, future events or otherwise. You should read the "Risk Factors" section included in our 2021 Annual Report and our subsequently filed Quarterly Report on Form 10-Q for the period ended March 31, 2022, in addition to the "Risk Factors" and "Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biotechnology company focused on developing treatments for patients suffering from complex rare diseases using our novel cell penetrating peptide technology platform. Our lead product candidate, CTI-1601, is a subcutaneously administered, recombinant fusion protein intended to deliver human frataxin ("FXN"), an essential protein, to the mitochondria of patients with Friedreich's ataxia ("FA"). FA is a rare, progressive, and fatal disease in which patients are unable to produce enough FXN due to a genetic abnormality. There is currently no effective therapy for FA.

We have completed two Phase 1 clinical trials in patients with FA. We have received an orphan drug designation, fast track designation and rare pediatric disease designation, from the U.S. Food and Drug Administration (the "FDA") for CTI-1601. In addition, we received orphan drug designation for CTI-1601 from the European Commission and a Priority Medicines ("PRIME") designation from the European Medicines Agency ("EMA"). The receipt of such designations or positive opinions may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA or EMA procedures and does not assure ultimate approval by the FDA or EMA.

Our cell penetrating peptide technology platform, which enables a therapeutic molecule to cross a cell membrane in order to reach intracellular targets, has the potential to enable the treatment of other rare and orphan diseases. We intend to use our proprietary platform to target additional orphan indications characterized by deficiencies in or alterations of intracellular content or activity.

Since our inception, we have devoted substantially all of our resources to developing CTI-1601, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, and providing general and administrative support for such operations.

CTI-1601 Program Update

On May 20, 2021 we announced that we had received an EMA PRIME designation for CTI-1601 in FA. Through PRIME, the EMA offers early and proactive support to medicine developers to optimize the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications so that these medicines can reach patients earlier. The PRIME designation was based on both pre-clinical data as well as tolerability data from the CTI-1601 Phase 1 program in patients with FA.

In May 2021, we reported positive topline data from our Phase 1 FA program after completing dosing of the single ascending dose ("SAD") trial in December 2020 and of the multiple ascending dose ("MAD") trial in March 2021. Data from these trials demonstrate proof-of-concept by showing that daily subcutaneous injections of CTI-1601 for up to 13 days resulted in dose-dependent increases in FXN levels from baseline compared to placebo in all evaluated tissues (buccal cells, skin, and platelets). FXN levels achieved in peripheral tissues (buccal cells) following daily 50 mg and 100 mg subcutaneous injections of CTI-1601 were at or in excess of FXN levels that would be expected in phenotypically normal heterozygous carriers. There were no serious adverse events ("SAEs") associated with either the MAD or SAD trials.

Also in May 2021, the FDA placed a clinical hold on our CTI-1601 clinical program after we notified the agency of mortalities at the highest dose levels of the 26-week non-human primate toxicology study that was designed to support extended dosing of patients with CTI-1601. At the time the hold was placed, we had no interventional clinical trials with patients enrolling or enrolled.

In February 2022, in response to the complete response we submitted to the FDA, the Agency stated that it was maintaining the clinical hold and that additional data are needed to resolve the clinical hold. We subsequently submitted a request to the FDA for a Type C meeting.

In the third quarter of 2022, we had a Type-C meeting with the FDA. The purpose of the Type C Meeting was to obtain FDA feedback on the information needed to resolve CTI-1601's current clinical hold in full or in part, as well as to discuss a proposed change in CTI-1601’s clinical development plan to introduce a Phase 2 dose exploration study to precede initiation of an open label extension study. We plan to submit a complete response to the CTI-1601 clinical hold later in the third quarter of 2022. In conjunction with the complete response, we are proposing as CTI-1601’s next clinical trial, a Phase 2, four-week dose exploration study in FA patients starting at the lower dose levels tested in the Company’s Phase 1 multiple-ascending dose clinical trial. This study will provide data on extended dosing of lower doses of CTI-1601, providing insights into safety and tolerability as well as whether lower doses for longer periods of time can increase frataxin levels, while still maintaining acceptable exposures. Throughout the course of the interactions with the FDA, we intend to continue to work with the Agency to enable resolution of the clinical hold and to agree on the study design and timing of the next proposed clinical trial. We do not know when, or if the clinical hold will be lifted.

Financing Activities

We have funded our operating and capital requirements to date primarily with proceeds from sales of common stock and prefunded warrants for the purchase of common stock and, prior to our May 2020 merger with Zafgen, with capital contributions from Chondrial Holdings, LLC ("Holdings").

In August 2020, we entered into an Equity Distribution Agreement ("the ATM Agreement") with an investment bank in connection with the establishment of an “at-the-market” offering program under which we could sell up to an aggregate of $50,000,000 of shares of our common stock from time to time through this investment bank as sales agent.

In July 2021, we sold 2,342,720 shares pursuant to the ATM Agreement for net proceeds of $19.9 million, after issuance costs. As of June 30, 2022, $29.2 million of additional shares of common stock remained available for sale by us under the ATM Agreement.

COVID-19 Update

In late 2019, an outbreak of a novel strain of the Coronavirus 2019 Disease (COVID-19) was identified and infections have been found in a number of countries around the world, including the United States. Our business, results of operations, financial condition and cash flows could be materially and adversely affected. Specifically, we could experience additional delays in future clinical trial timelines as a result of additional travel and hospital restrictions related to the COVID-19 pandemic and the efforts to mitigate it which may be imposed or could

experience supply shortages or manufacturer shutdowns impacting the manufacture of drug substance or drug product, which could also impact clinical trial timelines.

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales, and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts result in clinical success and regulatory approval or collaboration agreements with third parties for our product candidates, we may generate revenue from those product candidates or collaborations.

Operating Expenses

The majority of our operating expenses since inception have consisted primarily of research and development and general and administrative costs.

Research and Development Expenses

Research and development expenses, which consist primarily of costs associated with our product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

- third-party contract costs relating to research, formulation, manufacturing, non-clinical studies, and clinical trial activities;
- employee related costs, including salaries, benefits and stock-based compensation expenses for employees engaged in scientific research and development functions;
- external costs of outside consultants and vendors;
- payments made under our third-party licensing agreements;
- sponsored research agreements;
- laboratory consumables; and
- allocated facility-related costs.

Costs for certain activities, such as manufacturing, non-clinical studies and clinical trials are generally recognized based on the evaluation of the progress of completion of specific tasks using information and data provided by our vendors and collaborators. Research and development activities are central to our business. We expect to increase our investment in research and development in order to advance CTI-1601 through additional clinical trials. As a result, we expect that our research and development expenses will increase in the foreseeable future as we pursue clinical development of CTI-1601 and/or any other product candidates we develop.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the clinical and commercial development of CTI-1601 or any other product candidates we develop. We are also unable to predict when, if ever, material net cash inflows will commence from sales of our product candidates. The duration, costs, and timing of clinical trials and development of CTI-1601 or any other product candidates we develop will depend on a variety of factors, including:

- the scope, rate of progress and expense of clinical trials and other research and development activities, including the ongoing impact of COVID-19 on these activities;
- clinical trial results;
- uncertainties in clinical trial enrollment rate or design;
- significant and changing government regulation;
- the timing and receipt of any regulatory approvals;
- the influence of the FDA’s or other regulatory authority’s on our clinical trial design and timing;

- establishing manufacturing capabilities or making arrangements with third-party manufacturers and risk involved with development of manufacturing processes, FDA pre-approval inspection practices and successful completion of manufacturing batches for clinical development and other regulatory purposes;
- the impact of the on-going COVID-19 pandemic including the variants of the original virus that may prove more contagious and deadly;
- our ability to obtain and maintain patent and trade secret protection and regulatory exclusivity for our product candidates; and
- our ability to recruit and retain key research and development personnel.

A change in the outcome of any of these variables with respect to the development of a product candidate could significantly change the costs, timing and viability associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct additional non-clinical or clinical trials beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, composed of salaries, related benefits and stock-based compensation, costs related to our executive, finance, information technology, and costs related to other administrative functions. General and administrative expenses also include insurance expenses and professional fees for auditing, tax, and legal services, including legal expenses to pursue patent protection for our intellectual property. We expect that our general and administrative expenses will increase in the foreseeable future as we hire additional employees to implement, improve and scale our operational, financial, commercial and management systems.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with GAAP. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, costs and expenses, and related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our condensed consolidated financial statements and, therefore, consider these to be our critical accounting policies. We evaluate these estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

Research and Development Expenses

As part of the process of preparing our condensed consolidated financial statements, we are required to estimate our accrued research and development expenses and evaluate payments made to vendors in advance of actual work activities being performed. This process involves reviewing open contracts and purchase orders, communicating with our personnel and outside vendors to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our condensed consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to:

- CROs, in connection with clinical trials;
- vendors in connection with non-clinical development activities;
- contract manufacturing organizations in connection with the production of non-clinical and clinical trial materials; and
- vendors related to product candidate manufacturing, development, and distribution of clinical supplies.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple CROs or CMOs that conduct and manage clinical trials or manufacture clinical trial material on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense, non-clinical expense, or manufacturing activities. Payments under some of these contracts depend on factors such as the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed, enrollment of patients, number of sites activated and the level of effort to be expended in each period. In accruing CMO costs, we estimate the time period that manufacturing will be completed, the achievement of milestones and the percentage of completion of each specific CMO agreement. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us recognizing adjustments in future periods as additional information becomes available.

Stock-Based Compensation

We measure all stock-based awards granted to employees, directors and non-employee consultants based on the fair value on the date of grant using the Black-Scholes option-pricing model. Compensation expense of those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. Typically, we issue awards with only service-based vesting conditions and record the expense for these awards using the straight-line method. We account for forfeitures as they occur.

We classify stock-based compensation expense in our condensed consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Prior to May 28, 2020, we were a private company and lacked company-specific historical and implied volatility information for our common stock. Therefore, we estimate our expected common stock price volatility based on the historical volatility of publicly traded peer companies and expect to continue to do so until we have adequate historical data regarding the volatility of our own traded stock price. The expected term of our stock options have been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield considers the fact that we have never paid cash dividends on common stock and do not expect to pay any cash dividends in the foreseeable future.

Results of Operations

Comparison of three months ended June 30, 2022 and 2021

The following table summarizes our results of operations for the three months ended June 30, 2022 and 2021:

	Three Months Ended June 30,		
	2022	2021	Increase (Decrease)
		(in thousands)	
Statement of Operations Data:			
Operating expenses:			
Research and development	$ 5,644	$ 9,102	$ (3,458)
General and administrative	3,043	3,441	(398)
Total operating expenses	8,687	12,543	(3,856)
Loss from operations	(8,687)	(12,543)	3,856
Other income (expense), net	20	(66)	86
Net loss	$ (8,667)	$ (12,609)	$ 3,942

Research and development expenses

Research and development expenses for the three months ended June 30, 2022 decreased $3.5 million compared to the three months ended June 30, 2021. The decrease in research and development expenses was primarily driven by a decrease in nonclinical costs of $2.5 million and a decrease of $1.1 million in clinical trial costs, partially offset by an increase of $0.2 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

General and administrative expenses

General and administrative expenses for the three months ended June 30, 2022 decreased $0.4 million compared to the three months ended June 30, 2021. The decrease was primarily driven by a decrease in professional fees associated with legal and accounting services of $0.5 million, partially offset by an increase of $0.1 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

Results of Operations

Comparison of six months ended June 30, 2022 and 2021

The following table summarizes our results of operations for the six months ended June 30, 2022 and 2021:

	Six Months Ended June 30,		
	2022	2021	Increase (Decrease)
		(in thousands)	
Statement of Operations Data:			
Operating expenses:			
Research and development	$ 11,450	$ 18,076	$ (6,626)
General and administrative	6,124	6,573	(449)
Total operating expenses	17,574	24,649	(7,075)
Loss from operations	(17,574)	(24,649)	7,075
Other income (expense), net	(36)	(48)	12
Net loss	$ (17,610)	$ (24,697)	$ 7,087

Research and development expenses

Research and development expenses for the six months ended June 30, 2022 decreased $6.6 million compared to the six months ended June 30, 2021. The decrease in research and development expenses compared to the prior year period was primarily driven by a decrease of $3.0 million in clinical trial costs, a decrease in clinical supply manufacturing costs of $2.3 million, a decrease in nonclinical costs of $2.3 million, partially offset by an increase of $0.4 million in personnel related costs due to annual compensation increases and an increase of $0.4 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

General and administrative expenses

General and administrative expenses for the six months ended June 30, 2022 decreased $0.4 million compared to the six months ended June 30, 2021. The decrease in general and administrative expenses was primarily driven by a decrease in professional fees associated with legal and accounting services of $0.6 million, a decrease in operational expenses of $0.3 million, partially offset by an increase of $0.4 million in stock-based compensation expense associated with stock option grants made in 2021 and 2022.

Liquidity and Capital Resources

Since our inception, we have not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from our operations. We have devoted substantially all of our resources to developing CTI-1601, building our intellectual property portfolio, developing third-party manufacturing capabilities, business planning, capital raising, and providing general and administrative support for such operations.

Cash Flows

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

	Six Months Ended June 30,	
	2022	2021
	(in thousands)	
Net cash used in operating activities	$ (15,019)	$ (21,988)
Net cash provided by (used in) investing activities	(35,342)	24,470
Net cash provided by financing activities	—	—
Net increase in cash, cash equivalents and restricted cash	$ (50,361)	$ 2,482

Net cash used in operating activities

During the six months ended June 30, 2022, operating activities used $15.0 million of cash, resulting from our net loss of $17.6 million, adjusted for noncash expenses of $3.5 million and changes in our operating assets and liabilities of $0.9 million. Our net loss was primarily attributed to research and development activities related to our CTI-1601 program and our general and administrative expenses as described above. Noncash expenses are primarily stock-based compensation expense. The change in operating assets and liabilities was primarily due to decreases in prepaid expenses and accounts payable.

During the six months ended June 30, 2021, operating activities used $22.0 million of cash, resulting from our net loss of $24.7 million, adjusted for noncash expenses of $2.7 million. Our net loss was primarily attributable to research and development activities related to our CTI-1601 program and our general and administrative expenses as described above. Noncash expenses primarily include stock-based compensation expense. The change in operating assets and liabilities was primarily due to decreases in prepaid expenses and accounts payable.

Net cash provided by (used in) investing activities

During the six months ended June 30, 2022, investing activities used $35.3 million of cash, including $35.2 million in purchases of new marketable debt securities and $0.1 million in purchases of property and equipment.

During the six months ended June 30, 2021, investing activities provided $24.5 million of cash, resulting from a $26.3 million increase from maturities of marketable debt securities, partially offset by $1.7 million in purchases of new marketable debt securities.

Net cash provided by financing activities

During the six months ended June 30, 2022 and 2021 there were no financing activities.

Operating Capital Requirements

We have not yet commercialized any products and do not expect to generate revenue from the commercial sale of any products for several years, if at all.

We have to date incurred net losses. We incurred net losses of approximately $17.6 million and $24.7 million for the six months ended June 30, 2022 and 2021, respectively. As of June 30, 2022, we had an accumulated deficit of $133.9 million and cash, cash equivalents and marketable debt securities of $54.9 million, excluding restricted cash of $1.3 million.

Losses have resulted principally from costs incurred in connection with research and development activities, and general and administrative costs associated with the development of CTI-1601 and our operations. We expect to incur significant expenses and operating losses for the foreseeable future as we expect to continue to incur expenses in connection with our ongoing activities, if and as we:

- continue to advance the development of CTI-1601 through additional clinical trials;
- seek to identify and advance development of additional product candidates into clinical development and identify additional indications for our product candidates;
- seek to obtain regulatory approvals for our product candidates;
- identify, acquire or in-license other product candidates and technologies;
- maintain, leverage and expand our intellectual property portfolio; and
- expand our operational, financial, commercial and management systems and personnel, including personnel to support our clinical development and future commercialization efforts and our operations as a public company.

We believe that based on our current operating plan our cash, cash equivalents and marketable debt securities will be able fund operating expenses and capital expenditure requirements for at least the next twelve months from the filing of these financial statements with the SEC. If we encounter unexpected delays in our clinical trials or if there are other unanticipated changes to our operating plan from our current assumptions that negatively impact our operations, we may reduce expenditures in order to further extend our existing cash resources. Until we can generate substantial revenue, if ever, we expect to seek additional funding through a combination of public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, minimum cash balances, 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. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms, if at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, or we do not have sufficient authorized shares, we may be required to delay, limit, or eliminate the development of business opportunities and our ability to achieve our business objectives, our competitiveness, and our business, financial condition, and results of operations will be materially adversely affected. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects. In addition, geopolitical unrest including the potential impact of the Russian invasion of Ukraine and the possibility that the conflict could expand beyond eastern Europe, the impact of the COVID-19 pandemic and/or other health crises on the global financial markets may reduce our ability to access capital, which could negatively affect our liquidity and ability to continue as a going concern.

If we are unable to obtain funding when needed and/or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research and development programs, the manufacture of clinical and commercial supplies, product portfolio expansion or pre commercialization efforts, which could adversely affect our business prospects, or we may be unable to continue operations. Certain 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. Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates.

Off-Balance Sheet Arrangements

During the periods presented we did not have, and we currently do not have, any off-balance sheet arrangements, as defined under applicable SEC rules, such as relationships with unconsolidated entities or financial partnerships, which are often referred to as structured finance or special purpose entities, established for the purpose of facilitating financing transactions that are not required to be reflected on our balance sheets.

Recently Issued Accounting Pronouncements

Please read Note 2 to our condensed consolidated financial statements included in Part I of Item 1 of this Quarterly Report on Form 10-Q for a description of recent accounting pronouncements applicable to our business.

Other Company Information

None.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

We are a "smaller reporting company" as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and are not required to provide the information under this item.

Item 4. Controls and Procedures

We maintain “disclosure controls and procedures,” as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, to allow timely decisions regarding required disclosure.

The design of any disclosure controls and procedures also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

With respect to the quarter ended June 30, 2022, under the supervision and with the participation of our management, we conducted an evaluation of the effectiveness of the design and operations of our disclosure controls and procedures. Based upon this evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures are effective.

Management does not expect that our internal control over financial reporting will prevent or detect all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control systems are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in a cost-effective control system, no evaluation of internal control over financial reporting can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been or will be detected.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the fiscal quarter ended June 30, 2022 which have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II - OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we are subject to claims in legal proceedings arising in the normal course of business. To our knowledge, during the three months ended June 30, 2022, there were no, and as of the date of this Quarterly Report, there are no, threatened or pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

Item 1A. Risk Factors

You should carefully consider the risk factors described in our 2021 Annual Report under the caption "Item 1A. Risk Factors." The risks described in our 2021 Annual Report are not the only risks facing our company. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial also may materially adversely affect our business, financial condition or future results.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report are set forth on the Exhibit Index, which is incorporated herein by reference.

EXHIBIT INDEX

Exhibit No.	Description
10.1*	Second Amendment to License Agreement, by and between the Company and The Trustees of Indiana University, effective as of May 28, 2020.
10.2*	Third Amendment to License Agreement, by and between the Company and The Trustees of Indiana University, effective as of June 9, 2022.
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	Inline XBRL Instance Document- the instance document does not appear in the Interactive Data File because its XBRL tag re embedded within the Inline XBRL document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.
101.CAL*	Inline XBRL Taxonomy Extension Calculation 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 Link Document.
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Filed herewith.

** Furnished herewith.

SIGNATURES

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

LARIMAR THERAPEUTICS, INC.

Date: August 11, 2022

By: /s/ Carole S. Ben-Maimon, M.D.
Carole S. Ben-Maimon, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 11, 2022

By: /s/ Michael Celano
Michael Celano
Chief Financial Officer
(Principal Financial and Accounting Officer)

SECOND AMENDMENT
TO
LICENSE AGREEMENT

This Second Amendment (the "Second Amendment") is made and entered into as of May 28, 2020 (the "Second Amendment Effective Date") by and between:

The Trustees of Indiana University ("IU"), a body politic and corporate of the State of Indiana, having its principal offices at 107 S. Indiana Ave., Bloomington, IN 47405; and

Larimar Therapeutics, Inc. ("Larimar"), a corporation organized under the laws of Delaware, having its principal offices at Three Bala Plaza East, Suite 506, Bala Cynwyd, PA 19004

(each referred to individually as a "Party" and collectively as the "Parties").

The Parties hereby agree:

1 **Background:** IU and Chondrial Therapeutics IP, LLC ("Chondrial") entered into the License Agreement dated November 30, 2016 with IU Agreement No. 2017-0063 as amended by the first amendment dated August 16, 2019 with IU Agreement No. 2019-0070 (collectively, the "Agreement"). Chondrial subsequently entered into an Agreement and Plan of Merger with Larimar pursuant to which Chondrial merged with and into Larimar with Larimar being the survivor as of May 28, 2020 (the "Merger"). In connection with the Merger and pursuant to Article 14 of the Agreement, Larimar provided IU with written notice dated June 29, 2021 of Chondrial's intent to assign the Agreement to Larimar and Larimar's agreement to assume all obligations and liabilities of Chondrial under the Agreement. The Parties desire to enter into this Second Amendment to amend the Agreement in consideration of the foregoing premises and the mutual promises, covenants, and agreements hereinafter set forth.

2 **Amendments:**

2.1 Larimar represents and warrants that Larimar has read the Agreement and agrees to abide by all its terms and conditions.

2.2 Larimar is substituted for Chondrial and assumes all liability and obligation under the Agreement and is bound by all its terms in all respects as if Larimar were the original licensee of the Agreement in place of Chondrial.

2.3 Replace Chondrial's notice address in Paragraph 18.2 with the following:

If to Larimar:
Larimar Therapeutics, Inc.
Three Bala Plaza East, Suite 506
Bala Cynwyd, PA 19004
Attn: Legal
Email: info@larimartx.com

3 Except as provided in this Second Amendment, all other terms and conditions of the Agreement remain unmodified and in full force and effect.

4 This Second Amendment may be executed in counterparts, each of which will be deemed an original and all of which when taken together will be deemed one instrument. Facsimile, Portable Document Format (PDF) or photocopied signatures of the Parties will have the same legal validity as original signatures.

Witness: The Parties have caused this Second Amendment to be executed by their duly authorized representatives as of the Second Amendment Effective Date.

The Trustees of Indiana University	**Larimar Therapeutics, Inc.**
By: Simran Trana	By: Jennifer Johansson
Name: Simran Trana	Name: Jennifer S. Johansson
Title: Associate VP, ICO	Title: VP, Regulatory Affairs and Counsel
Date: Dec 10, 2021	Date: December 9, 2021

Approved By: WL

THIRD AMENDMENT
TO
LICENSE AGREEMENT

This Third Amendment (the "Third Amendment") is made and entered into as of June 9, 2022 (the "Third Amendment Effective Date") by and between:

The Trustees of Indiana University ("IU"), a body politic and corporate of the State of Indiana, having its principal offices at 107 S. Indiana Ave., Bloomington, IN 47405; and

Larimar Therapeutics, Inc. ("Larimar"), a corporation organized under the laws of Delaware, having its principal offices at Three Bala Plaza East, Suite 506, Bala Cynwyd, PA 19004

(each referred to individually as a "Party" and collectively as the "Parties").

The Parties hereby agree:

1 **Background:** IU and Chondrial Therapeutics IP, LLC ("Chondrial") entered into the License Agreement dated November 30, 2016 with IU Agreement No. 2017-0063 as amended by the first amendment dated August 16, 2019 with IU Agreement No. 2019-0070; and further amended by the second amendment with IU Agreement No. 2020-0002 between IU and Larimar Therapeutics, Inc. (formerly Chondrial) dated May 28, 2020 (collectively, the "Agreement"). The Parties desire to enter into this Third Amendment to amend the Agreement in consideration of the foregoing premises and the mutual promises, covenants, and agreements hereinafter set forth.

2 **Amendments:**

2.1 Delete Section 5.2 and its subsections in their entirety and replace with the following:

5.2 Without limiting the generality of the diligence provisions of Section 5.1 above, Larimar will achieve (either itself or through its affiliates and/or its Sublicensees) the following milestones by the following dates ("Milestones"):

(a) Larimar will have at least two full-time equivalent personnel working on the development, manufacturing and marketing of the License Products within the twelve (12) month period from the Effective Date and each subsequent year thereafter;

(b) Enrollment of the first patient in the first Phase I (or its non-U.S. equivalent) clinical trial of a Licensed Product by June 30, 2020;

(c) Enrollment of the first patient in the first Phase II (or its non-U.S. equivalent) clinical trial of a Licensed Product by December 31, 2023.

5.2.1 Larimar will provide to IU (at the time of the next due report under Section 8) written notice of the achievement of each Milestone in this Section 5.2 (and continued confirmation of Larimar's sustained achievement of Section 5.2. (a)).

5.2.1 Notwithstanding the foregoing, IU shall not unreasonably withhold its assent to any revisions of such Milestones if requested in writing by Larimar and supported by evidence of continued diligence and/or difficulties or delays that Larimar could not have reasonably avoided.

2.2 Replace IU's notice address in Paragraph 18.2 with the following:

The Trustees of Indiana University
Attn: Innovation and Commercialization Office IU Agreement No. number
107 S. Indiana Ave., Bryan Hall 211 Bloomington, IN 47405

With copy to:
The Trustees of Indiana University
Attn: Innovation and Commercialization Office IU Agreement No. number
1220 Waterway Blvd.
Indianapolis, IN 46202

3 Except as provided in this Third Amendment, all other terms and conditions of the Agreement remain unmodified and in full force and effect.

4 This Third Amendment may be executed in counterparts, each of which will be deemed an original and all of which when taken together will be deemed one instrument. Facsimile, Portable Document Format (PDF) or photocopied signatures of the Parties will have the same legal validity as original signatures.

Witness: The Parties have caused this Third Amendment to be executed by their duly authorized representatives as of the Third Amendment Effective Date.

The Trustees of Indiana University		**Larimar Therapeutics, Inc.**	
By:	Simran Trana Simran Trana (Jun 10, 2022 08:46 EDT)	By:	Jennifer Johansson Jennifer Johansson (Jun 23, 2022 10:23 EDT)
Name:	Simran Trana	Name:	Jennifer S. Johansson
Title:	Associate VP, ICO	Title:	Vice President, Regulatory Affairs and Counsel
Date:	Jun 10, 2022	Date:	Jun 23, 2022

Exhibit 31.1

CERTIFICATION

I, Carole S. Ben-Maimon, M.D., certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 11, 2022
/s/ Carole S. Ben-Maimon, M.D.

Carole S. Ben-Maimon, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Michael Celano, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 11, 2022
/s/ Michael Celano

Michael Celano
Chief Financial Officer
(Principal Financial Officer and Accounting Officer)

Exhibit 32.1

CERTIFICATION
Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
(Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code)

Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (subsections (a) and (b) of section 1350, chapter 63 of title 18, United States Code), each of the undersigned officers of Larimar Therapeutics, Inc. (the "Company"), does hereby certify, to the best of such officer's knowledge, that:

(1) The Quarterly Report on Form 10-Q for the quarter ended June 30, 2022 (the "Report") of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: August 11, 2022

/s/ Carole S. Ben-Maimon, M.D.
Carole S. Ben-Maimon, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 11, 2022

/s/ Michael Celano
Michael Celano
Chief Financial Officer
(Principal Financial and Accounting Officer)