UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K/A

(Amendment No. 1)

CURRENT REPORT
Pursuant to Section 13 or 15(d) of
the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): May 28, 2020

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

Delaware	**001-36510**	**20-3857670**
(State or other jurisdiction of incorporation or organization)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

Three Bala Plaza East, Suite 506
Bala Cynwyd, Pennsylvania **19004**
(Address of principal executive offices) **(Zip Code)**

Registrant's telephone number, including area code: (844) 511-9056

Zafgen, Inc.
3 Center Plaza, Suite 610
Boston, Massachusetts 02108
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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**	**Nasdaq Global Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

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

Explanatory Note

This Current Report on Form 8-K/A (this "Amendment") is being filed by Larimar Therapeutics, Inc. (f/k/a Zafgen, Inc.), a Delaware corporation (the "Company"), to amend its Current Report on Form 8-K (the "Prior 8-K") filed with the Securities and Exchange Commission (the "SEC") on June 2, 2020, in connection with the consummation on May 28, 2020 of the transactions contemplated by that certain Agreement and Plan of Merger and Reorganization, dated December 17, 2019 (the "Merger Agreement"), by and among the Company, Zordich Merger Sub, Inc. ("Zordich"), Chondrial Therapeutics, Inc. ("Chondrial") and Chondrial Therapeutics Holdings, LLC ("Holdings"), pursuant to which Zordich merged with and into Chondrial, with Chondrial surviving as a wholly-owned subsidiary of the Company (the "Merger").

The Company is filing this Amendment solely to provide (i) certain voluntary disclosures concerning the risk factors and financial condition of the Company, as permitted by Item 8.01; (ii) the historical audited financial statements of Chondrial as of and for the years ended December 31, 2019 and 2018, and the unaudited condensed consolidated financial statements as of March 31, 2020 and for the three month periods ended March 31, 2020 and 2019, referred to in Item 9.01(a) below; and (iii) the unaudited pro forma condensed combined financial statements as of and for the three month period ended March 31, 2020 and for the year ended December 31, 2019, referred to in Item 9.01(b) below. Except for the foregoing, this Amendment does not modify or update any other disclosure contained in the Prior 8-K. Such financial information was excluded from the Prior 8-K in reliance on the instructions to such items.

Item 8.01 Other Events.

The Company's Risk Factors and Chondrial's Management's Discussion and Analysis of Financial Condition and Results of Operations as of March 31, 2020 and for the three month periods ended March 31, 2020 and 2019, are filed herewith and attached hereto as Exhibits 99.1 and 99.2, respectively, and incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(a) ***Financial Statements of Business Acquired***

The audited financial statements of Chondrial as of and for the years ended December 31, 2019 and 2018, and the unaudited condensed consolidated financial statements as of March 31, 2020 and for the three month periods ended March 31, 2020 and 2019, are filed herewith as Exhibits 99.3 and 99.4, respectively, and are incorporated herein by reference. The consent of PricewaterhouseCoopers LLP, the Company's independent registered public accounting firm, is attached as Exhibit 23.1 to this Current Report on Form 8-K/A.

(b) ***Pro Forma Financial Information***.

The unaudited pro forma condensed combined financial statements of the Company and Chondrial as of and for the three month period ended March 31, 2020 and for the year ended December 31, 2019, filed herewith and attached hereto as Exhibit 99.5, are incorporated herein by reference.

(d) Exhibits

Below is a list of exhibits included with this Current Report on Form 8-K.

Exhibit No.	Document
23.1	Consent of PricewaterhouseCoopers LLP, the Company's independent registered public accounting firm.
99.1	Management's Discussion and Analysis of Financial Condition and Results of Operations of the Chondrial as of March 31, 2020 and for the three month periods ended March 31, 2020 and 2019.
99.2	Risk Factors of the Company.
99.3	Audited financial statements of Chondrial as of and for the years ended December 31, 2019 and 2018.
99.4	Unaudited condensed consolidated financial statements of Chondrial as of March 31, 2020 and for the three month periods ended March 31, 2020 and 2019.
99.5	Unaudited pro forma condensed combined financial statements of the Company and Chondrial as of and for the three month period ended March 31, 2020 and for the year ended December 31, 2019.

SIGNATURES

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

Larimar Therapeutics, Inc.

By: /s/ Carole S. Ben-Maimon, M.D.
Name: Carole S. Ben-Maimon, M.D.
Title: President and Chief Executive Officer

Date: June 26, 2020

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-8 (Nos. 333-196900, 333-204931, 333-210216, 333-216602, 333-223561, 333-230291, 333-234579, 333-236917, and 333-228326) and Form S-3 (Nos. 333-219834 and 333-228328) of Larimar Therapeutics, Inc. of our report dated March 6, 2020 relating to the financial statements of Chondrial Therapeutics, Inc., which appears in this Current Report on Form 8-K/A.

/s/ PricewaterhouseCoopers LLP
Philadelphia, Pennsylvania
June 26, 2020

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Explanatory Note:

On May 28, 2020, Larimar Therapeutics, Inc., formerly known as Zafgen, Inc. (the "Company" or "Larimar"), completed its business combination with Chondrial Therapeutics, Inc., a Delaware corporation ("Chondrial"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated December 17, 2019 (as amended, the "Merger Agreement"), by and among Larimar, Zordich Merger Sub, Inc. ("Merger Sub"), Chondrial and Chondrial Therapeutics Holdings, LLC ("Holdings"), pursuant to which Merger Sub merged with and into Chondrial, with Chondrial surviving as a wholly-owned subsidiary of the Company (the "Merger"). In connection with, and immediately prior to the completion of the Merger, the Company effected a reverse stock split of the Company's Common Stock at a ratio of 1-for-12 (the "Reverse Stock Split"). Following completion of the Merger, the Company changed its name from "Zafgen, Inc." to "Larimar Therapeutics, Inc." (the "Name Change"), Chondrial was determined to be the accounting acquirer, the historical financials of Larimar will be those of Chondrial and the business conducted by the Company became primarily the business conducted by Chondrial.

You should read the following discussion and analysis of Chondrial's financial condition and results of operations together with Chondrial's condensed consolidated financial statements and the related notes included in Exhibit 99.4 of the Company's Amendment No. 1 to the Current Report on Form 8-K filed with the Securities and Exchange Commission ("SEC") on June 26, 2020. Some of the information contained in this discussion and analysis including information with respect to Chondrial's plans and strategy for Chondrial's business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" included in Exhibit 99.2 of the Company's Amendment No. 1 to the Current Report on Form 8-K filed with the SEC on June 26, 2020, Chondrial's actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

Chondrial 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. Chondrial's lead product candidate, CTI-1601, is a subcutaneously administered, recombinant fusion protein intended to deliver 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 enough FXN due to a genetic abnormality. There is currently no effective therapy for Friedreich's Ataxia. CTI-1601 is currently being evaluated in Phase 1 clinical trials in patients with Friedreich's Ataxia. Chondrial has received orphan drug status, fast track designation and rare pediatric disease designation, from the FDA for CTI-1601. The receipt of such designations may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA.

Chondrial's 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. Chondrial intends to use its proprietary platform to target additional orphan indications characterized by deficiencies in or alterations of intracellular protein content or activity.

Since Chondrial's inception in November 2016, Chondrial has devoted substantially all of its resources to developing CTI-1601, building its intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, and providing general and administrative support for such operations. From inception through March 31, 2020, Chondrial received gross proceeds of $35.6 million from the sale of Series A convertible preferred units by its parent company, Chondrial Therapeutics Holdings, LLC, or Holdings. Additionally, from inception through March 31, 2020, Chondrial received gross proceeds of $13.0 million from the sale of Series B Bridge Units by Holdings.

Chondrial has never generated any revenue and has incurred net losses in each year since inception. Chondrial has an accumulated deficit of $29.8 million as of March 31, 2020. Chondrial's net loss was $6.7 million for the three months ended March 31, 2020 and $4.7 million for the three months ended March 31, 2019. These losses have resulted principally from costs incurred in connection with research and development activities, in-licensing of technology and general and administrative costs associated with Chondrial's operations. Chondrial expects to incur significant expenses and operating losses for the foreseeable future.

Chondrial expects to continue to incur expenses in connection with its ongoing activities, if and as Chondrial:

- Continues to advance the development of CTI-1601 through additional clinical trials;
- Seeks to identify and advance development of additional product candidates into clinical development and indications for its product candidates;
- Seeks to obtain regulatory approvals for its product candidates;
- Identifies, acquires or in-licenses other product candidates and technologies;
- Maintains, leverages and expands its intellectual property portfolio; and
- Expands its operational, financial and management systems and personnel, including personnel to support its clinical development and future commercialization efforts and its operations as a public company.

As a result, Chondrial will need additional financing to support its continuing operations. Until such time that Chondrial can generate significant revenue from product sales, if ever, Chondrial expects to finance its operations through a combination of public equity, private equity, debt financings, or other sources, which may include collaborations with third parties. Arrangements with collaborators or others may require Chondrial to relinquish rights to certain of its technologies or product candidates. In addition, Chondrial may never successfully complete development of any of its product candidates, obtain adequate patent protection for its technology, obtain necessary regulatory approval for its product candidates or achieve commercial viability for any approved product candidates. Adequate additional financing may not be available to Chondrial on acceptable terms, or at all. Chondrial's failure to raise capital as and when needed would have a negative impact on its financial condition and ability to pursue its business strategy. Chondrial will need to generate significant revenue to achieve profitability, and may never do so.

Chondrial expects to continue to generate operating losses for the foreseeable future. As of June 26, 2020, the issuance date of the condensed consolidated financial statements for the three months March 31, 2020, Chondrial completed its merger with Zafgen Inc., which, upon closing, provided incremental net cash of approximately $40.0 million concurrent with a private placement which provided additional net proceeds of $75.5 million. Chondrial believes that, based on its current operating plan, its cash and cash equivalents as of the filing date will enable it to fund operations for at least twelve months from the issuance of these interim financial statements.

Merger with Zafgen

On December 17, 2019, Larimar, Merger Sub, Holdings and Chondrial entered into the Merger Agreement, pursuant to which the Merger Sub would merge with and into Chondrial, with Chondrial surviving the merger as a wholly owned subsidiary of Zafgen. The Merger was completed on May 28, 2020 pursuant to the terms of the Merger Agreement.

Pursuant to the terms of the Merger Agreement, upon closing of the Merger, all of Chondrial's outstanding common stock was exchanged for common stock of Zafgen and all outstanding options exercisable for units of Holdings were exchanged for options to purchase common stock of Larimar. In addition, immediately following the closing of the merger, the combined organization effected the Reverse Stock Split and the Name Change. Except as noted otherwise, the unaudited condensed consolidated financial statements and notes included in Exhibit 99.4 of Larimar's Amendment No. 1 to the Current Report on Form 8-K filed on June 26, 2020 do not give effect to the Reverse Stock Split.

The business combination was accounted for as a reverse acquisition in accordance with GAAP. Under this method of accounting, Chondrial was deemed to be the accounting acquirer for financial reporting purposes. This determination was primarily based on the facts that, immediately following the merger: (i) Chondrial's stockholders own a substantial majority of the voting rights in the combined organization, (ii) the majority of the board of directors of the combined company is composed of directors designated by Chondrial under the terms of the Merger Agreement and (iii) existing members of Chondrial management will be the management of the combined company. Accordingly, for accounting purposes, the business combination was treated as the equivalent of Chondrial issuing stock to acquire the net assets of Larimar. As a result, as of the closing date of the Merger, the net assets of Larimar were recorded at their acquisition-date fair values in the financial statements of Chondrial and the reported operating results prior to the business combination will be those of Chondrial.

Financial Operations Overview

Revenue

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

Operating Expenses

The majority of Chondrial's operating expenses since inception have consisted primarily of research and development activities, and general and administrative costs.

Research and Development Expenses. Research and development expenses, which consist primarily of costs associated with Chondrial's product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

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

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing, preclinical 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 Chondrial's vendors and collaborators. Research and development activities are central to Chondrial's business. Chondrial expects to increase its investment in research and development in order to advance CTI-1601 through additional clinical trials. As a result, Chondrial expects that its research and development expenses will increase in the foreseeable future as it pursues clinical development of its product candidates.

At this time, Chondrial cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of its product candidates. Chondrial is also unable to predict when, if ever, material net cash inflows will commence from sales of its product candidates. The duration, costs, and timing of clinical trials and development of Chondrial's product candidates will depend on a variety of factors, including:

- the scope, rate of progress and expense of clinical trials and other research and development activities;
- clinical trial results, including the ongoing impact of COVID-19;
- uncertainties in clinical trial enrollment rate or design;
- significant and changing government regulation;
- the timing and receipt of any regulatory approvals;
- the FDA's or other regulatory authority's influence on clinical trial design;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- commercializing Chondrial's product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for Chondrial's product candidates;
- continued applicable safety profiles of the products following approval; and
- retention of 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 Chondrial to conduct clinical trials beyond those that Chondrial currently anticipates will be required for the completion of clinical development of a product candidate, or if Chondrial experiences significant delays in enrollment in any of its clinical trials, Chondrial 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, consisting of salaries, related benefits and stock-based compensation, of Chondrial's executive, finance, information technology, and other administrative functions. General and administrative expenses also include travel expenses, allocated facility-related costs not otherwise included in research and development expenses, insurance expenses, and professional fees for auditing, tax and legal services, including legal expenses to pursue patent protection of Chondrial's intellectual property. Chondrial expects that its general and administrative expenses will increase in the foreseeable future as it hires additional employees to implement and improve its operational, financial and management systems. Additionally, as a publicly-traded company, Chondrial will incur significant additional legal, accounting and other expenses that Chondrial did not incur as a privately-held company.

Critical Accounting Policies and Significant Judgments and Estimates

Chondrial's condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States of America. The preparation of Chondrial's condensed consolidated financial statements and related disclosures requires Chondrial to make estimates and assumptions that affect the reported amount of assets, liabilities, costs and expenses, and related disclosures. Chondrial believes that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on Chondrial's condensed consolidated financial statements and, therefore, consider these to be Chondrial's critical accounting policies. Chondrial evaluates these estimates and assumptions on an ongoing basis. Chondrial's actual results may differ from these estimates under different assumptions and conditions. See also Note 3 of Chondrial's unaudited condensed consolidated financial statements included in Exhibit 99.4 of Larimar's Amendment No. 1 to the Current Report on Form 8-K filed on June 26, 2020.

Research and Development Expenses

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

- contract research organizations, or CROs, in connection with clinical trials;
- vendors in connection with nonclinical development activities; and
- vendors related to product candidate manufacturing, development and distribution of clinical supplies.

Chondrial bases its expenses related to clinical trials on its estimates of the services received and efforts expended pursuant to contracts with multiple CROs that conduct and manage clinical trials on Chondrial's 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 Chondrial's vendors will exceed the level of services provided and result in a prepayment of the clinical expense, nonclinical 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, Chondrial estimates 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. If the actual timing of the performance of services or the level of effort varies from Chondrial's estimate, Chondrial adjusts the accrual or prepaid accordingly. Although Chondrial does not expect its estimates to be materially different from amounts actually incurred, its 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

Chondrial has historically issued equity awards to employees in the form of options to purchase common units of Holdings and, to a lesser extent, restricted common units of Holdings. Chondrial measures equity-based awards granted to employees and directors at fair value on the date of grant and recognizes the corresponding compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. Generally, Chondrial issues common unit options and restricted common unit awards with only service-based vesting conditions and record the expense for these awards using the straight-line method. Stock-based compensation costs for non-employees are recognized as expense over the vesting period on a graded vesting basis.

The fair value of each service-based option grant to employees, and restricted common unit grants to non-employees, is estimated on the date of grant using the Black-Scholes option-pricing model. Chondrial estimates its expected volatility using a weighted average of the historical volatility of publicly-traded peer companies, and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price for the duration of the expected term. The expected term of Chondrial's 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 is based on the fact that Chondrial has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

As of March 31, 2020, Chondrial had unrecognized equity-based compensation expense related to Chondrial's unvested service-based option awards of less than $0.1 million, which is expected to be recognized over the remaining weighted-average vesting period of 1.5 years.

COVID-19 Update

The COVID-19 outbreak has caused significant business disruption around the globe. The extent of the impact of COVID-19 on Chondrial's operational and financial performance will depend on certain developments, including the duration and spread of the outbreak and the impact on Chondrial's clinical trials, employees and vendors. At this point, the degree to which COVID-19 may impact the Company's financial condition or results of operations is uncertain. A prolonged outbreak could have a material and adverse impact on financial results and business operations of Chondrial, including the timing and ability of Company to complete its clinical trials and other efforts required to advance the development of CTI-1601. For example, due to the continued impact of COVID-19, Chondrial has delayed initiation of the next cohort in Chondrial's single ascending dose ("SAD") clinical trial. Chondrial is conducting the clinical trial at one clinical trial site in New Jersey. Because Friedreich's Ataxia is a rare disease, there are a limited number of patients in close proximity to the clinical trial site and clinical trial patients travel from throughout the United States to the clinical trial site to participate. The travel advisories and risk of infection related to COVID-19 have presented increased risks to patients traveling to Chondrial's clinical trial site for dosing. Due to the uncertainty surrounding COVID-19, Chondrial cannot estimate when the next cohort of patients will begin the clinical trial. While top line results from the SAD and the planned multiple ascending dose clinical trials were originally expected by the end of 2020, the delay in the clinical trial timeline caused by the ongoing impact of COVID-19 resulted in top line results being expected in the first half of 2021.

Results of Operations

Comparison of the three months ended March 31, 2020 and 2019

The following table summarizes Chondrial's results of operations for the three months ended March 31, 2020 and 2019:

	Three months ended March 31,		
	2020	2019	Increase (Decrease)
	(in thousands)		
Statement of Operations Data:			
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	5,007	4,222	785
General and administrative	1,667	502	1,165
Total operating expenses	6,674	4,724	1,950
Loss from operations	(6,674)	(4,724)	(1,950)
Other income	—	—	—
Net loss	$(6,674)	$(4,724)	$ (1,950)

Research and development expenses

Research and development expenses increased by $0.8 million from $4.2 million for the three months ended March 31, 2019 to $5.0 million for the three months ended March 31, 2020. The increase was primarily due to a $0.5 million increase in external development costs for CTI-1601 and a $0.3 million increase in personnel related costs due to headcount additions in Chondrial's research and development functions. The $0.5 million increase in external development costs was primarily attributed to incremental costs incurred for the further development of CTI-1601. Specifically, during late 2019 the first patients were dosed in a Phase 1 SAD clinical trial of CTI-1601. The trial was ongoing during the first quarter of 2020. Chondrial is working to initiate the third cohort in the SAD clinical trial, which is delayed due to the continued impact of COVID-19. The $0.9 million increase from the clinical trial was offset by a reduction in costs for third-party manufacturing of CTI-1601 and timing of toxicology studies for CTI-1601.

General and administrative expenses

General and administrative expense increased by $1.2 million from $0.5 million for the three months ended March 31, 2019 to $1.7 million for the three months ended March 31, 2020. The increase was primarily due to a $1.0 million increase in professional fees that is primarily due to a $0.8 million increase in accounting and audit fees due to the Merger, and a $0.2 million increase in legal fees associated with Chondrial becoming a public company.

Liquidity and Capital Resources

Since its inception in November 2016, Chondrial has not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from its operations. Chondrial has devoted substantially all of its resources to developing CTI-1601, building its intellectual property portfolio, developing third-party manufacturing capabilities, business planning, raising capital, and providing general and administrative support for such operations. From inception through March 31, 2020, Chondrial received gross proceeds of $45.6 million from the sale of Series A convertible preferred units and Series B Bridge units by its parent company, Holdings.

Cash Flows

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

	Three Months Ended March 31,	
	2020	2019
	(in thousands)	
Net cash used in operating activities	$ (8,921)	$ (5,815)
Net cash used in investing activities	(778)	(15)
Net cash provided by financing activities	9,577	3,000
Net decrease in cash and cash equivalents	$ (122)	$ (2,830)

Net cash used in operating activities

During the three months ended March 31, 2020, operating activities used $8.9 million of cash, resulting from Chondrial's net loss of $6.7 million and changes in operating assets and liabilities of $2.3 million. Chondrial's net loss was primarily attributed to research and development activities related to its CTI-1601 program and its general and administrative expenses. Net cash used in changes in Chondrial's operating assets and liabilities during the three months ended March 31, 2020, consisted primarily of a $2.3 million decrease in account payable and accrued expenses.

During the three months ended March 31, 2019, operating activities used $5.8 million of cash, resulting from net loss of $4.7 million and changes in operating assets and liabilities of $1.1 million. Chondrial's net loss was primarily attributed to research and development activities related to Chondrial's CTI-1601 program and general and administrative expenses.

Net cash used in investing activities

During the three months ended March 31, 2020, investing activities used $0.8 million of cash, resulting from Chondrial's transaction costs for the acquisition of Zafgen of $0.7 million and $0.1 million from the purchase of equipment.

During the three months ended March 31, 2019, investing activities used less than $0.1 million of cash, respectively resulting from purchases of laboratory equipment.

Net cash provided by financing activities

During the three months ended March 31, 2020, net cash provided by financing activities of $9.6 million was the result of contributions from Holdings. Of the gross proceeds received by Holdings, $6.6 million was from the sale of 1,323 Series B Bridge Units and $3.0 million from the sale of 600 Second Series B Convertible Preferred Units.

During the three months ended March 31, 2019, net cash provided by financing activities of $3.0 million was the result of contributions from Holdings of $3.0 million from the sale of 150,000 shares of Series A Preferred Units.

Operating Capital Requirements

CTI-1601 is currently in Phase 1 clinical development, therefore Chondrial expects to continue to incur significant expenses and operating losses for the foreseeable future. Chondrial anticipates that it will continue to incur expenses, if and as it seeks to:

- 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 indications for Chondrial's product candidates;
- Seek to obtain regulatory approvals for its product candidates;

- Identify, acquire or in-license other product candidates and technologies;
- Maintain, leverage and expand its intellectual property portfolio; and
- Expand its operational, financial and management systems and personnel, including personnel to support its clinical development and future commercialization efforts and its operations as a public company.

Chondrial expects to continue to generate operating losses for the foreseeable future. The additional funding through Holding's Second Series B Bridge Unit Purchase Agreement entered into in January 2020 funded operations until the completion of the Merger. Chondrial completed the Merger on May 28, 2020 which, upon closing, provided incremental net cash of approximately $40.0 million concurrent with a private placement which provided additional net proceeds of $75.5 million. Chondrial believes that, based on its current operating plan, its cash, cash equivalents and marketable securities as of the filing date will enable it to fund operations for at least twelve months from the issuance of its interim financial statements.

Until such time, if ever, as the Chondrial can generate substantial revenue, Chondrial expects to seek additional funding through equity financings, debt financings, or other capital sources, which may include collaborations with other companies, government funding arrangements or other strategic transactions. Chondrial may not be able to obtain financing on acceptable terms, or at all, and Chondrial may not be able to enter into collaborations or other arrangements. The terms of any financing may adversely affect the holdings or rights Chondrial's existing stockholders' rights. If Chondrial is unable to obtain additional funding, Chondrial will be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect its business, or Chondrial may be unable to continue operations.

Contractual Obligations and Commitments

During the three months ended March 31, 2020, there have been no material changes to Chondrial's contractual obligations and commitments outside the ordinary course of business from those described under the heading "*Chondrial's Management's Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations and Commitments*" in the Company's Definitive Proxy Statement, as filed with the SEC on April 29, 2020.

Off-Balance Sheet Arrangements

During the periods presented Chondrial did not have and Chondrial does not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission 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 its balance sheets.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact Chondrial's financial position and results of operations is disclosed in Note 3 to Chondrial's unaudited condensed consolidated financial statements included as Exhibit 99.4 of Larimar's Amendment No. 1 to the Current Report on Form 8-K filed on June 26, 2020.

RISK FACTORS

On May 28, 2020, we completed our business combination with Chondrial Therapeutics, Inc., or Chondrial, in accordance with the terms of the Agreement and Plan of Merger, dated as of December 17, 2019, as amended, or the Merger Agreement, by and among us, Chondrial, a wholly-owned subsidiary of ours, or Merger Sub, and Chondrial Holdings, LLC, or Holdings, the sole stockholder of Chondrial, pursuant to which Merger Sub merged with and into Chondrial, with Chondrial surviving as a wholly-owned subsidiary of ours, or the Merger.

In connection with, and immediately prior to the completion of the Merger, we effected a reverse stock split of our common stock, at a ratio of 1-for-12, or the Reverse Stock Split. Under the terms of the Merger Agreement, we issued common stock to Holdings at an exchange ratio of 60,912.5005 shares of common stock, after taking into account the Reverse Stock Split, for each share of Chondrial's common stock outstanding immediately prior to the Merger. Holdings subsequently distributed the shares of our common stock it received in the Merger to its members. Immediately after the completion of the Merger, we changed our name from "Zafgen, Inc." to "Larimar Therapeutics, Inc.," Chondrial was determined to be the accounting acquirer, our historical financials will be those of Chondrial and the business conducted by us became the business conducted by Chondrial.

Unless the context otherwise requires, references in this prospectus to "Larimar," the "Company," "we," "our" or "us" refer to Larimar Therapeutics, Inc. (formerly known as Zafgen, Inc.) and its subsidiaries, references to "Zafgen" refer to the Company prior to the completion of the Merger, references to "Chondrial" refer to Chondrial Therapeutics, Inc., a privately held corporation prior to the completion of the Merger, and references to "Merger Subsidiary" refer to Zordich Merger Sub, Inc., the Company's wholly owned subsidiary following the Merger.

Investing in our securities involves a high degree of risk. You should carefully consider the risk factors set forth below and under "Risk Factors" in (i) our Annual Report on Form 10-K for the year ended December 31, 2019 and (ii) our Quarterly Report on Form 10-Q for the three months ended March 31, 2020, as updated by our subsequent filings under the Securities Exchange Act of 1934, as amended, before deciding whether to purchase our securities. The risks and uncertainties we describe below and in the documents mentioned above are not the only ones we face. Additional risks and uncertainties not presently known to us could adversely affect our business, operating results and financial condition, as well as adversely affect the value of an investment in our securities, and the occurrence of any of these risks might cause you to lose all or part of your investment.

Risks Related to Our Financial Position and Need for Capital

We have incurred significant losses since our inception and anticipate that we will incur continued losses for the foreseeable future.

Since our inception we have devoted substantially all of our resources to the development of CTI-1601. We have incurred significant losses in each year of operation since our inception. For the year ended December 31, 2019, we had net losses of $23.1 million and as of December 31, 2019, had an accumulated deficit of $23.1 million and we expect to continue to incur significant expenses and net operating losses for the foreseeable future. Our financial statements for the years ended December 31, 2019 and December 31, 2018 include disclosures regarding management's assessment of our ability to continue as a going concern and a report from our independent registered public accounting firm that includes an explanatory paragraph regarding going concern, describing conditions that raise substantial doubt about Chondrial Therapeutics, Inc's ability to continue as a going concern due to our liquidity position and recurring losses from operations since inception and negative cash flows from operating activities.

We have devoted substantially all of our financial resources and efforts to research and development, including nonclinical studies and our clinical development program as well as the development of manufacturing processes. We expect to incur significant losses for the foreseeable future to further develop and commercialize our lead drug candidate.

We expect that our expenses will increase substantially if and as we:

- continue clinical development efforts for CTI-1601;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish sales, marketing, distribution and other commercial infrastructure to commercialize various products for which we may obtain marketing approval, if any;
- contract for the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio; and
- hire and retain additional personnel, such as clinical, quality control, regulatory, finance, and compliance personnel.

Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholder's (deficit) equity and working capital.

We have no commercial revenue and may never become profitable.

To date, we have not generated any commercial revenue. Our ability to generate revenue and become profitable depends upon our ability to obtain regulatory approval for, and successfully commercialize, CTI-1601 or other product candidates that we may develop, in-license or acquire in the future.

This will require success in a range of challenging activities, including completing clinical trials of CTI-1601 or any future product candidates, obtaining marketing approval for CTI-1601 and any future product candidates, manufacturing, marketing and selling those products for which we, or any future collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Even if we are able to successfully achieve the above, we do not know what the reimbursement status of CTI-1601 or any other future product candidates will be or when any of these products will generate revenue for us, if at all. We have not generated, and do not expect to generate, any product revenue for the foreseeable future, and expect to continue to incur significant operating losses for the foreseeable future due to the cost of research and development, nonclinical studies and clinical trials and the regulatory approval process for CTI-1601 and any future product candidates.

Our ability to generate revenue from CTI-1601 or any future product candidates also depends on a number of additional factors, including our ability to:

- successfully complete development activities, including the remaining nonclinical studies and planned clinical trials for our product candidates;
- complete and submit New Drug Applications, or NDAs, and Biologics License Applications, or BLAs, to the U.S. Food and Drug Administration, or FDA, and Marketing Authorisation Applications, or MAAs, to the European Medicines Agency, or EMA,, and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit applications to, and obtain regulatory approval from, other foreign regulatory authorities;
- manufacture any approved products in commercial quantities and on commercially reasonable terms;
- develop a commercial organization, or find suitable partners, to market, sell and distribute approved products in the markets in which we have retained commercialization rights;

- achieve acceptance among patients, clinicians and advocacy groups for any products we develop;
- obtain coverage and adequate reimbursement from third parties, including government payors; and
- set a commercially viable price for any products for which we may receive approval.

Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of increased expenses, and if or when we might achieve or maintain profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we are able to complete the processes described above, we anticipate incurring significant costs associated with commercializing CTI-1601 or any of our future product candidates. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods.

We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We expect to continue to spend substantial and increasing amounts to conduct clinical trials of CTI-1601 and further research and development activities for CTI-1601, and for any additional product candidates that we may develop, in-license or acquire in the future. In addition, our expenses will increase as we expand, through development, in-license or acquisition, our pipeline of product candidates. If we obtain marketing approval for any of our product candidates, we will likely incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future collaborator. Accordingly, we will need to obtain additional funding in connection with our continuing operations.

As of December 31, 2019, our existing cash and cash equivalents were $1.0 million. This amount, combined with the cash and cash equivalents of Zafgen that were acquired in the Merger, the additional $15.0 million of funding received through the Series B bridge convertible preferred units offering and the aggregate gross proceeds of approximately $80.0 million received in the private placement of shares of our common stock completed in May 2020, or the Private Placement, will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of CTI-1601. Accordingly, we will be required to obtain further funding through 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, 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. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. 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.

If we are unable to obtain funding on a timely basis, or on acceptable terms, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate. Our failure to raise capital as and when needed would have a negative impact on our business, financial condition and results of operations and our ability to pursue the development of CTI-1601 or future product candidates.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies, CTI-1601 or other product candidates that we may develop, in-license or acquire in the future.

We may seek additional capital through a combination of private and public equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent we raise additional capital through the sale of equity or convertible debt securities, existing ownership interests will be diluted and the terms of such financings may include liquidation or other preferences that adversely affect the rights of existing stockholders. Debt financings may be coupled with an equity component, such as warrants to purchase shares, which could also result in dilution of our existing stockholder's ownership.

If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to CTI-1601 or other product candidates that we may develop, in-license or acquire in the future, or grant licenses on terms that are not favorable to us.

Our ability to use our NOLs and certain other tax attributes may be limited.

Under Section 382 of the Internal Revenue Code, or the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOLs and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We have completed several financings since our inception, which we believe have resulted in a change in control as defined by Section 382 of the Code. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. The Tax Cuts and Jobs Act of 2017, or Tax Act, which significantly reformed the Code, also reduced the corporate income tax rate to 21%, from a prior rate of 35%. This may cause a reduction in the economic benefit of our NOLs and other deferred tax assets available to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed and would adversely affect our business, financial condition and results of operations.

Risks Related to Our Product Development and Regulatory Approvals

Our success is currently dependent upon the success of our sole product candidate, CTI-1601, which is currently in Phase 1 clinical trials. We cannot be certain that we will be successful with our clinical development or that we will be able to obtain regulatory approval for CTI-1601.

We currently have no drug products for sale and our business is currently dependent on our successful clinical development, regulatory approval and commercialization of CTI-1601, which is in Phase 1 clinical trials.

If our efforts to develop and commercialize CTI-1601 for the treatment of Friedreich's Ataxia are unsuccessful, or we experience significant delays in doing so, our business could also be substantially harmed. The success of CTI-1601 will depend on several factors, including the following:

- maintaining our IND application with the FDA in order to continue to conduct clinical trials in the United States;
- successfully recruiting, enrolling and retaining patients in and completing our Phase 1 clinical trials and any clinical trials we may conduct in the future;
- demonstrating safety, tolerability and efficacy profiles that are satisfactory to the FDA, EMA and other comparable regulatory authorities for marketing approval;
- successfully completing all necessary toxicology studies to support clinical development and regulatory approval for CTI-1601;

- receiving timely marketing approvals from applicable regulatory authorities;
- managing the extent and cost of any required post-marketing approval commitments to applicable regulatory authorities;
- establishing and maintaining arrangements with third-party manufacturers for CTI-1601, including developing, validating and maintaining a commercially viable manufacturing process that is compliant with current good manufacturing practices, referred to as cGMPs;
- obtaining, maintaining and protecting our patents, trade secrets and regulatory exclusivity in the United States and other countries;
- successfully launching commercial sales following any marketing approval, including establishing a specialty sales organization, if applicable;
- obtaining commercial acceptance of our products, if approved, by patients, the medical community and third-party payors and obtaining and maintaining healthcare coverage and adequate reimbursement;
- maintaining an acceptable safety profile following any marketing approval; and
- competing with other therapies.

Many of these factors are outside of our control, including the clinical development and regulatory approval processes, results of nonclinical and toxicology studies and clinical trials, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts, respectively, of any current or future third-party contractors. The process of obtaining regulatory approval is expensive and time consuming. The FDA and foreign regulatory authorities may never approve CTI-1601 for sale and marketing, and even if CTI-1601 is ultimately approved, regulatory approval may be delayed or limited in the United States or in other jurisdictions. Even if we are authorized to sell and market CTI-1601 in one or more markets, there is no assurance that we will be able to successfully market CTI-1601 or that CTI-1601 will achieve market acceptance sufficient to generate profits. If we are unable to successfully develop and commercialize CTI-1601 due to failure to obtain regulatory approval for CTI-1601, to successfully market CTI-1601, to generate profits from the sale of CTI-1601, or due to other risk factors outlined in this report, it would have material adverse effects on our business, financial condition, and results of operations as CTI-1601 is currently our sole product candidate.

Clinical development is a lengthy and expensive process with an uncertain outcome, and the results of nonclinical studies, toxicology studies or clinical trials may not be predictive of future nonclinical studies, toxicology studies or clinical trial results.

Clinical development is expensive and can take many years to complete, and its outcome is inherently uncertain. We cannot guarantee that any nonclinical studies, toxicology studies or clinical trials will be conducted as planned or completed on schedule, if at all, and failure can occur at any time during the nonclinical study, toxicology study or clinical trial process. Despite promising nonclinical, toxicology or clinical results, any product candidate can unexpectedly fail at any stage of nonclinical, toxicology or clinical development. The historical failure rate for product candidates in our industry is high, especially for products in early stages of development.

The results from nonclinical studies, toxicology studies or clinical trials of a product candidate may not predict the results of later nonclinical or clinical trials of the product candidate, and interim results of a clinical trial are not necessarily indicative of final results. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy characteristics despite having progressed through nonclinical studies and initial clinical trials. It is not uncommon to observe results in clinical trials that are unexpected based on nonclinical studies and early clinical trials, and many product candidates fail in clinical trials despite very promising early results.

Moreover, this and any future nonclinical and clinical data may be susceptible to varying interpretations and analyses. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving promising results in earlier studies. Furthermore, we cannot assure you that we will be able to successfully progress any future nonclinical programs from candidate identification to Phase 1 clinical development. As is typical in candidate development, we have a program of ongoing toxicology studies in animals for CTI-1601 and cannot provide assurance that the findings from such studies or any ongoing or future clinical trials will not adversely affect the clinical development of CTI-1601. For the foregoing reasons, we cannot be certain that our ongoing and planned nonclinical studies and clinical trials will be successful. If nonclinical or clinical trials for CT1-1601 or any future product candidates or indications fail to demonstrate safety or efficacy to the satisfaction of the FDA or the equivalent regulatory authorities in other countries, the FDA or equivalent regulatory authority will not approve our product candidates in those and other indications, which could have a material adverse effect on our business, financial condition and results of operations.

We do not know whether any clinical trials for CTI-1601 will be completed on schedule, if at all, as the commencement and completion of clinical trials can be delayed, prevented or terminated for a number of reasons, including as a result of ambiguous or negative interim results. In addition, a clinical trial may be suspended or terminated by us, the FDA, other regulatory authorities, the institutional review boards, or IRBs**,** or ethics committees, a data monitoring committee, or safety review committee, overseeing the clinical trial at issue or other regulatory authorities due to a number of factors, including, among others:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or trial sites by the FDA, the EMA, or other applicable regulatory authorities that reveals deficiencies or violations that require us to undertake corrective action, including the imposition of a partial clinical hold or a full clinical hold;
- unforeseen safety issues, including any that could be identified in our prior or ongoing toxicology studies, adverse events or lack of effectiveness;
- changes in government regulations or administrative actions;
- problems with clinical supply materials;
- lack of adequate funding to continue the clinical trial;
- challenges in recruiting and enrolling patients to participate in clinical trials, including the size and nature of the patient population, the proximity of patients to clinical trial sites, eligibility criteria for the clinical trial, the nature of the clinical trial protocol, the availability of approved effective treatments for the relevant disease and competition from other clinical trial programs for similar indications;
- difficulties in retaining or recruiting clinical investigators in our ongoing or future clinical trials;
- difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to rigors of the clinical trial, perceived lack of efficacy, side effects, screening and monitoring measures, personal issues or loss of interest;
- severe or unexpected drug-related adverse events experienced by patients in a clinical trial;
- the FDA, the EMA, or other applicable regulatory authorities may disagree with our clinical trial designs, our interpretation of data from clinical trials, or may change the requirements for approval even after it has reviewed and commented on the design for our clinical trials; and
- reports from nonclinical studies or clinical testing of other therapies that raise safety or efficacy concerns.

Failures or delays in the completion of our clinical trials could result in increased costs and could delay, prevent or limit our ability to generate revenue and continue our business.

Our lead product candidate CTI-1601 is currently in Phase 1 clinical trials, and there are a number of FDA regulatory requirements that we must satisfy before we can commence late-stage clinical trials of CTI-1601. To receive approval of CTI-1601 in other countries we will also have to satisfy their regulatory requirements. Satisfaction of these requirements will entail substantial time, effort and financial resources. We may never satisfy these requirements. Clinical trials may also be delayed or terminated as result of ambiguous or negative interim results or events outside of our control. We are currently evaluating CTI-1601 in a SAD Phase 1 clinical trial in patients with Friedreich's Ataxia. The first two cohorts of patients have completed the SAD clinical trial; however, due to the continued impact of COVID-19, we have delayed initiation of the next cohort in the SAD clinical trial. We are conducting the clinical trial at one clinical trial site in New Jersey. Because Friedreich's Ataxia is a rare disease, there are a limited number of patients in close proximity to the clinical trial site and clinical trial patients travel from throughout the United States to the clinical trial site to participate. The travel advisories and risk of infection related to COVID-19 have presented increased risks to patients traveling to our clinical trial site for dosing. Due to the uncertainty surrounding COVID-19, we cannot estimate when the next cohort of patients will begin the clinical trial. While top line results from the SAD and MAD clinical trials were originally expected by the end of 2020, the delay in the clinical trial timeline caused by the ongoing impact of COVID-19 has resulted in top line results being delayed until the first half of 2021. If Phase 1 clinical trials of CTI-1601 fail or further delays occur, we may not be able to develop and commercialize CTI-1601 and could fail to realize the potential advantages of doing so, and it could materially adversely affect our business, financial condition and results of operations.

We may not be successful in our efforts to identify, discover or acquire additional product candidates.

We only have one product candidate CTI-1601, which is in Phase 1 clinical trials in the United States. Therefore, the success of our business largely depends upon our ability to identify, develop, in-license or acquire and commercialize products targeting rare diseases. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. In addition, our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business, financial condition and results of operations.

We have no marketed proprietary products and have not yet advanced a product candidate beyond Phase 1 clinical trials, which makes it difficult to assess our ability to develop CTI-1601 or any future product candidates and commercialize any resulting products independently.

We have no experience in Phase 2 and later stage clinical development, and related regulatory requirements or the commercialization of products. As a result, we have not yet demonstrated our ability to independently and repeatedly conduct clinical development after Phase 1, which has not yet been successfully completed, successfully conduct an international multi-center clinical trial, complete a clinical trial, conduct a pivotal clinical trial, obtain regulatory approval, manufacture drug product on a commercial scale or arrange for a third party to do so on our behalf, and commercialize therapeutic products. We will need to develop such abilities if we are to execute on our business strategy to develop and independently commercialize product candidates for orphan and niche indications. To execute on our business plan for the development of independent programs, we will need to successfully:

- execute our clinical development plans for later-stage product candidates;
- obtain required regulatory approvals in each jurisdiction in which we will seek to commercialize products;
- build and maintain appropriate sales, distribution and marketing capabilities;
- gain market acceptance for our future products, if any; and
- manage our spending as costs and expenses increase due to clinical trials, regulatory approvals and commercialization activities.

If we are unsuccessful in accomplishing these objectives, we will not be able to develop and commercialize any product candidates independently and could fail to realize the potential advantages of doing so, and it would materially adversely affect our business, financial condition and results of operations.

We cannot be certain that we will be able to successfully complete clinical trials for CTI-1601 or any other product candidates.

We currently have only one product candidate in clinical development, CTI-1601, which is in Phase 1 clinical trials in the United States. Our business currently depends primarily on CTI-1601's successful clinical development, regulatory approval and commercialization. We submitted our IND and our application has gone into effect, permitting the conduct of clinical trials. However, the outcome of toxicology studies and early clinical trials may not be positive and may not be predictive of the success of later nonclinical studies or clinical trials, and interim results of clinical trials do not necessarily predict success in those or future clinical trials.

Published clinical data or case reports from third parties or early clinical trial data of CTI-1601 or any future product candidates may not be predictive of the results of later-stage clinical trials. Interpretation of results from early, usually smaller, studies that suggest a clinically meaningful response in some patients, requires caution. Results from later stages of clinical trials enrolling more patients may fail to show the desired safety or efficacy results or otherwise fail to be consistent with the results of earlier trials of the same product candidate. Later clinical trial results may not replicate earlier clinical trials for a variety of reasons, including differences in trial design, different trial endpoints (or lack of trial endpoints in exploratory studies), patient population, number of patients, patient selection criteria, trial duration, drug dosage and formulation and lack of statistical power in the earlier trials. These uncertainties are enhanced where the diseases under study lack established clinical endpoints, validated measures of efficacy, as is often the case with orphan diseases for which no drugs have been developed previously and where the product candidates target novel mechanisms. For example, to our knowledge, CTI-1601 is the only protein replacement therapy being developed for the treatment of Friedreich's Ataxia and therefore nonclinical studies may not be adequate to predict efficacy in a clinical trial due to our novel protein replacement therapy platform.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, variability of the disease being studied, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of CTI-1601, the development timeline and regulatory approval and commercialization prospects for CTI-1601, and, correspondingly, our business, financial prospects and results of operation would be negatively impacted.

Further, CTI-1601 or any future product candidates may not be approved even if they achieve their primary endpoint in clinical trials. The FDA, EMA or foreign regulatory authorities may disagree with our trial design and our interpretation of data from nonclinical studies and clinical trials. In addition, any of these regulatory authorities may change its requirements for the approval of a product candidate even after reviewing and providing comments or advice on a protocol for a pivotal clinical trial that, if successful, would potentially form the basis for an application for approval by the FDA, EMA or another regulatory authority. Furthermore, any of these regulatory authorities may also approve CTI-1601 or any future product candidates for a narrower indication than we may request or may grant approval contingent on the performance of costly post-marketing clinical trials. Any of the above could materially adversely affect our business, financial condition and results of operations.

Unforeseen safety issues or adverse events, including any that may be identified in our ongoing toxicology studies or clinical trials, may delay or prevent the development and regulatory approval of CTI-1601, damage public perception of the safety of CTI-1601 or increase government regulation of CTI-1601.

We are collecting data about CTI-1601 from ongoing Phase 1 clinical trials and toxicology studies and unforeseen safety issues or adverse events caused by, or other unexpected properties of, CTI-1601 could be identified. Such safety issues or adverse events could cause us or regulatory authorities to interrupt, delay or halt clinical trials of CTI-1601 or could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities for CTI-1601, which would materially adversely affect our business, financial condition and results of operations.

Even if we were to receive regulatory approval for CTI-1601 following such events, these events could damage public perception of the safety of CTI-1601 and prevent us from achieving or maintaining market acceptance of CTI-1601 and significantly impact our ability to successfully commercialize CTI-1601 and generate revenues, all of which would materially adversely affect our business, financial condition and results of operations.

We may experience difficulties identifying and enrolling patients in our clinical trials given the limited number of patients who have the diseases for which CTI-1601 is being studied or for any other product candidate we may study in the future. Difficulty in enrolling patients could delay or prevent clinical trials of CTI-1601 or any future product candidate.

Identifying and qualifying patients to participate in clinical trials of CTI-1601 is critical to our success. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing CTI-1601, and we may experience delays in our clinical trials if we encounter difficulties in enrollment.

The conditions for which we are planning to evaluate CTI-1601, and any product candidates we may evaluate in the future, are rare genetic diseases. Accordingly, there are limited patient pools from which to draw for clinical trials. We are investigating our product candidate in Friedreich's Ataxia, a rare disease. Arranging for investigative sites and recruiting patients for clinical trials in this disease may be very difficult. In addition, if other companies are investigating their investigational products in Friedreich's Ataxia, it may be more difficult to enroll eligible patients into our clinical trials. If the actual number of patients with Friedreich's Ataxia is lower than we believe or if any approval that we obtain is based on a narrower definition of these patient populations, then the potential market for CTI-1601 will be smaller than we anticipate.

In addition to the rarity of Friedreich's Ataxia and other diseases that we are studying, the eligibility criteria of our clinical trials will further limit the pool of available study participants as it will require patients to have specific characteristics that we can measure to assure their disease is either severe enough or not too advanced to include them in a clinical trial. The process of finding and diagnosing patients may prove costly, especially since the diseases we are studying are rare. We also may not be able to identify, recruit, and enroll a sufficient number of appropriate patients to complete our clinical trials because of demographic criteria for prospective patients, the perceived risks and benefits of the product candidate under study, the proximity and availability of clinical trial sites for prospective patients, and the patient referral practices of physicians. The availability and efficacy of competing therapies and clinical trials can also adversely impact enrollment. If patients are unwilling to participate in our trials for any reason, the timeline for recruiting patients, conducting trials, and obtaining regulatory approval of potential products may be delayed, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. Furthermore, our inability to enroll a sufficient number of patients for our clinical trials could result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for CTI-1601 or any future product candidates, and jeopardize our ability to achieve our clinical development timeline and goals, including the dates by which we will commence, complete and receive results from clinical trials. Enrollment delays in our clinical trials may also jeopardize our ability to commence sales of and generate revenues from CTI-1601, which could cause the value of our company to decline and limit our ability to obtain additional financing, if needed. Any of these occurrences may harm our business, financial condition, and prospects significantly.

Friedreich's Ataxia has no FDA-approved treatments, and clinical endpoints required to obtain approval are not well defined.

There are currently no therapies approved to treat Friedreich's Ataxia. We have concentrated our research and development efforts on developing a novel therapeutic for the treatment of Friedreich's Ataxia, and our future success depends on the success of this therapeutic approach. The clinical trial requirements of the FDA and other comparable regulatory agencies and the criteria these regulators use to determine the safety and efficacy of any product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential product. Given the nature of Friedreich's Ataxia, we may have to devise novel clinical endpoints to be tested in our clinical trials, which can lead to some subjectivity in interpreting trial results and could result in regulatory agencies not agreeing with the validity of our endpoints, or our interpretation of the clinical data, and therefore denying approval, which would materially adversely affect our business, financial condition and results of operations. As a result, the design and conduct of clinical trials for a therapeutic product candidate such as CTI-1601 that is intended to deliver human FXN through a subcutaneously administered, recombinant fusion protein in Friedreich's Ataxia patients is subject to unknown risks, and we may experience setbacks with our ongoing or planned clinical trials of CTI-1601 in Friedreich's Ataxia because of the limited clinical experience with our mechanism of action in these patients.

In particular, regulatory authorities in the United States and the European Union, or the EU, have not issued definitive guidance as to how to measure and achieve efficacy in treatments for Friedreich's Ataxia. As a result, the design and conduct of clinical trials of CTI-1601 may take longer, be more costly or be less effective as part of the novelty of development in Friedreich's Ataxia. We may use new or novel endpoints or methodologies, and the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results. Even if applicable regulatory authorities do not object to our proposed endpoints in an earlier stage clinical trial, such regulatory authorities may require evaluation of additional or different clinical endpoints in later-stage clinical trials.

CTI-1601 may cause adverse events or undesirable side effects that could delay or prevent its regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

We are collecting data about CTI-1601 from ongoing Phase 1 clinical trials and toxicology studies and any adverse events or undesirable side effects caused by, or other unexpected properties of, CTI-1601 could cause us, any future collaborators, an IRB or ethics committee or regulatory authorities to interrupt, delay or halt clinical trials of our product candidate and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other regulatory authorities. It is possible that as we progress CTI-1601 through clinical trials and toxicology studies, or as the use of CTI-1601 becomes more widespread if it receives regulatory approval, illnesses, injuries, discomforts and other adverse events that were not observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. If such side effects become known later in development or after approval, such findings may harm our business, financial condition and prospects significantly. Further, if a serious safety issue is identified in connection with the use of CTI-1601 commercially or in third-party clinical trials elsewhere, such issues may adversely affect the development potential of CTI-1601 elsewhere or result in regulatory authorities restricting our ability to develop or commercialize CTI-1601.

Further, if CTI-1601 were to receive marketing approval and we or others identify undesirable side effects caused by the product (or any other product) after the approval, a number of potentially significant negative consequences could result, including:

- regulatory authorities may request that we recall or withdraw the product from the market or may limit the approval of the product through labeling or other means;

- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication or a precaution;
- we may be required to change the way the product is distributed or administered, conduct additional clinical trials or change the labeling of the product;
- we may decide to recall or remove the product from the marketplace;
- we could be sued and/or held liable for injury caused to individuals exposed to or taking our product candidates; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidate and could substantially increase the costs of commercializing our product candidates and significantly impact our ability to successfully commercialize our product candidates and generate revenues, all of which would materially adversely affect our business, financial condition and results of operations.

Our approach to discover and develop fusion proteins for delivering proteins is novel and may never lead to marketable products.

We have concentrated our efforts and research and development activities on delivering proteins (FXN or other) to intracellular targets. Our future success depends on the successful development and manufacturing of such therapeutics and the effectiveness of our platform. The scientific discoveries that form the basis for our research are relatively new.

CTI-1601 uses a novel and unproven approach and mechanism to treat Friedreich’s Ataxia and therefore its efficacy and safety are difficult to predict, and there is no guarantee that CTI-1601 will be approved by the FDA.

If our lead product candidate proves to be ineffective, unsafe or commercially unviable, it is possible that our platform and pipeline would have little, if any, value, which would substantially harm our business, financial condition, results of operations and prospects. In addition, our approach may expose us to additional financial risks and make it more difficult to raise additional capital than other, more advanced proven technologies, which would materially adversely affect our business, financial condition and results of operations.

Protein replacement therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our protein replacement therapy platform or product candidates or otherwise harm our business.

The manufacture of fusion proteins, such as CTI-1601 and any fusion protein candidates, is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for clinical trials and commercial products for CTI-1601 or any fusion protein product that may receive regulatory approval in the future. Additionally, because biologic products are complex, the manufacture of such products and product candidates is more difficult and costly. We may not be able to have such products reliably manufactured in accordance with the applicable regulatory requirements in sufficient quantities to support our development programs and, if ultimately approved, commercial supply. We contract with third parties for the manufacturing of program materials for CTI-1601.

There are a limited number of contract manufacturers who specialize in the manufacture of biologic products and those that do may still be developing appropriate processes, controls and facilities for large-scale production. While we believe that there will be sufficient sources of supply that can satisfy our clinical and commercial requirements, we cannot be certain that we will be able to identify and establish additional relationships with such sources, if necessary, in a timely manner or at all, and what the terms and costs of such new arrangements would be, or that such suppliers would be able to supply our potential commercial needs. Furthermore, in the event our primary manufacturer cannot meet our needs, any switch to an alternative manufacturer would result in a significant delay, would require FDA approval, and cause material additional costs.

As further described in these risk factors, the manufacturers of biologic products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical trials or commercial use, among other consequences. If we or our manufacturers fail to comply with the FDA, EMA, or other regulatory authorities, it could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, clinical holds or termination of clinical trials, warning or untitled letters, regulatory communications warning the public about safety issues with a product, import or export refusals, license revocation, seizures, detentions, or recalls of product candidates or product, operating restrictions, criminal prosecutions or debarment, suits under the civil False Claims Act, corporate integrity agreements, or consent decrees any of which could significantly and adversely affect supplies of our product candidates and our business, financial conditions and results of operations could be materially adversely affected.

Our dependence upon others for the manufacture of our product candidates may also adversely affect our business, results of operations, financial condition and results of operations, and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Fast track designation by the FDA or any future designations may not lead to a faster development, regulatory review or approval process and it does not increase the likelihood that any of our product candidates will receive marketing approval.

We have received fast track therapy designation for CTI-1601 for the treatment of Friedreich's Ataxia. We may, in the future, apply for other accelerated programs from the FDA (such as breakthrough therapy or accelerated approval) for CTI-1601 or future product candidates. Designation for these programs is within the discretion of the FDA. Accordingly, even if we believe CTI-1601 or a future product candidate meets the criteria for designation, the FDA may disagree. In any event, the receipt of a designation may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and, in any event, does not assure ultimate approval by the FDA. In addition, even though CTI-1601 has been designated as fast track, the FDA may later decide that it no longer meets the criteria for designation and revoke it. If we apply for designation to additional accelerated programs from the FDA for CTI-1601 or future product candidates, the FDA might not grant the designation. If we apply for any similar programs in foreign countries for CTI-1601 or future product candidates, those designations also might not be granted by the regulatory authorities of those countries. Any of the above could adversely affect our business, financial condition and results of operations.

If we fail to maintain orphan drug designation or other regulatory exclusivity for CTI-1601 or obtain such exclusivity for any of our other product candidates in the future, our competitive position would be harmed

We received orphan drug designation from the FDA for CTI-1601 in July 2017. In the United States, orphan drug designation entitles a party to financial incentives such as tax advantages and user-fee waivers. In addition, if a product candidate that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including an NDA, to market the same drug for the same indication for seven years, except in limited circumstances, including if the FDA concludes that the later drug is clinically superior to the approved drug. A drug is clinically superior if it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We may lose orphan drug exclusivity if we are unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Moreover, orphan drug exclusivity may not effectively protect our product candidates from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same condition if such regulatory authority concludes that the later drug is clinically superior if it is shown to be safer, more effective or makes a major contribution to patient care. Loss of orphan drug designation for CTI-1601 or the failure to obtain such designation in other countries or for any future product candidates could adversely affect our business, financial condition and results of operation.

Although we have obtained rare pediatric disease designation for CTI-1601, we may not be eligible to receive a priority review voucher in the event the FDA determines we no longer meet the criteria for designation, revokes the designation or that FDA approval does not occur prior to October 1, 2022.

We received rare pediatric disease designation from the FDA for CTI-1601 in 2019. We may, in the future, apply for rare pediatric disease designation from the FDA for future product candidates that may qualify for designation. Vouchers for rare pediatric disease drugs are awarded when the designated drug receives approval. CTI-1601 may not receive approval and therefore, we may not receive a voucher. In addition, even though CTI-1601 has been designated as a drug for a rare pediatric disease, the FDA may later decide that it no longer meets the criteria for designation, revoke the designation or not award the voucher. If we apply for designation for future product candidates as drugs for rare pediatric diseases, the FDA may not grant the designation. In addition, the current law authorizing the rare pediatric disease program contains sunset provisions such that the FDA cannot award a voucher after September 30, 2020 unless the designation is granted by September 30, 2020 and the application is approved by September 30, 2022. Therefore, if we do not receive approval for CTI-1601 by September 30, 2022, or the legislation is not extended, we would not be able to receive a voucher. Furthermore, if the legislation is not extended, we would not be able to request designation for future product candidates or be eligible to receive vouchers for future product candidates. The failure to maintain rare pediatric disease designation for CTI-1601 or if FDA approval does not occur prior to October 1, 2022 could result in the inability to receive a priority review voucher which could adversely affect our business, financial condition and results of operations.

Changes in regulatory requirements, FDA guidance, guidance from other regulatory authorities or unanticipated events during our clinical trials of CTI-1601 may result in changes to clinical trial protocols or additional clinical trial requirements, which could result in increased costs to us and could delay our development timeline.

Changes in regulatory requirements, FDA guidance or guidance from EMA or unanticipated events during our clinical trials may force us to terminate or adjust our clinical program. The FDA, or the applicable regulatory authorities may impose additional clinical trial and/or nonclinical study requirements. Amendments to our clinical trial protocols would require resubmission to the FDA, or the applicable regulatory authorities as well as IRBs and ethics committees for review and approval, which may adversely impact the cost, timing or successful completion of a clinical trial. If we experience delays completing, or if we terminate, any of our clinical trials, or if we are required to conduct additional clinical trials and/or nonclinical studies, the commercial prospects for CTI-1601 or any other potential product candidates may be harmed and our ability to generate product revenue will be delayed, and it would materially adversely affect our business, financial condition and results of operations.

Regulatory requirements governing biologic products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to perform additional nonclinical studies or clinical trials, and increase our costs, or may force us to delay, limit or terminate certain of ours programs.

Regulatory requirements governing biologic drug products are still evolving and may continue to change in the future. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for CTI-1601 for the treatment of Friedreich's Ataxia or any other future protein replacement therapy product candidates in any indication, if at all. Regulatory review agencies and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval studies, limitations or restrictions. Delays, failure or unexpected costs in obtaining, the regulatory approval necessary to bring our product candidates to market could have a material adverse effect on our business, results of operations, financial condition and prospects.

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates.

The clinical trials of CTI-1601 and any future product candidates are, and the manufacturing and marketing of CTI-1601 and any future product candidates will be subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries, such as within the EU, where we intend to seek regulatory approval of, and market, any product candidate.

Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through nonclinical testing and clinical trials that the product candidate is safe and effective for use in each target indication. This process can take many years. If marketing approval is obtained, it will likely include post-marketing studies, and other post-marketing requirements, and surveillance such as Risk Evaluation and Mitigation Strategies, or REMS, which will require the expenditure of substantial resources beyond the proceeds we currently have on hand.

Furthermore, we are not permitted to market CTI-1601 in the United States or the EU until we receive approval of a BLA from the FDA or a MAA from the EMA, or in any other foreign countries until we receive the requisite marketing approval from such countries. The development of drugs for Friedreich's Ataxia or other rare diseases may require initial nonclinical studies, early and usually smaller, clinical trials and randomized, double-blind placebo controlled long-term safety and efficacy trials in order to test the safety and efficacy of the drug.

CTI-1601 is currently in Phase 1 clinical trials in the United States and will require substantial further clinical development before we can submit a BLA to the FDA. Development and/or regulatory programs for CTI-1601 in any countries other than the United States (such as a MAA to the EMA) is only in very preliminary stages and may require substantial further development in those countries prior to regulatory submissions seeking regulatory approval for marketing.

Even after successful completion of clinical trials, there is a risk that the FDA or other regulatory agencies may request further information from us, disagree with our findings or otherwise undertake a lengthy review of our submission.

The FDA and certain European regulatory authorities may delay, limit or deny testing or approval of CTI-1601 for many reasons, including, among others:

- we may not be able to demonstrate that CTI-1601 is safe and effective to the satisfaction of the FDA or the EMA;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA or the EMA for marketing approval;
- the FDA or the EMA may disagree with the number, design, size, duration, conduct or implementation of our clinical trials;
- the FDA or the EMA may require that we conduct additional nonclinical studies or clinical trials;
- the FDA or the EMA may not approve the formulation, manufacturing, labeling or specifications of CTI-1601;
- the contract research organizations, or CROs, that we retain to conduct our clinical trials may take actions outside of our control that materially adversely impact our clinical trials;
- the FDA or the EMA may find the data from nonclinical studies and clinical trials insufficient to demonstrate that CTI-1601's clinical and other benefits outweigh its safety risks;

- the FDA or the EMA may disagree with our interpretation of data from our nonclinical studies or clinical trials;
- the FDA or the EMA may not accept data generated at our clinical trial sites;
- if and when our BLA is submitted, the FDA could require an FDA advisory committee assessment, or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional nonclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- the FDA could require development of a REMS as a condition of approval or post-approval, or may not agree with our proposed REMS, or may impose additional requirements that limit the promotion, advertising, distribution, or sales of CTI-1601;
- the FDA or the EMA may find deficiencies with or not approve the manufacturing processes or facilities of third-party manufacturers with which we contract; or
- the FDA or the EMA may change their approval policies or adopt new regulations.

Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain and/or maintain regulatory approval for and successfully market CTI-1601. Any delay or failure in obtaining required approvals could have a material adverse effect on our business, financial condition and results of operations. This process can take many years and will likely require the expenditure of substantial resources. Of the large number of drugs in development in the United States, only a small percentage will successfully complete the FDA regulatory approval process and be commercialized. It is possible that the FDA or other regulatory agencies will not approve any application that we submit. It is possible that our product candidates may not obtain appropriate regulatory approvals necessary for us to commence clinical trials for our product candidates. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development and clinical trials, we cannot assure that CTI-1601, or any other of our potential product candidates will be successfully developed or commercialized.

We are subject to healthcare laws and regulations, and health information privacy and security laws, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of CTI-1601 or any potential product candidates, if approved. Our future arrangements with third-party payors will expose us broadly to applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute CTI-1601 or potential product candidates, if we obtain marketing approval. In addition, we may be subject to patient privacy regulation by both the federal government and the states or other countries in which we conduct our business. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- the federal false claims laws impose criminal and civil penalties, including those from civil whistleblower or qui tam actions pursuant to the federal False Claims Act, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements, sometimes referred to as the "Sunshine Act," under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or ACA, require manufacturers of drugs, devices, biologics, and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests;
- HIPAA and its implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws and transparency laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures and drug pricing.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including anticipated activities to be conducted by our sales team, were found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines and exclusion from government funded healthcare programs, such as Medicare and Medicaid, any of which could substantially disrupt our operations and would materially adversely affect our business, financial condition and results of operations. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain regulatory approval of and commercialize our product candidates and affect the prices we may obtain.

The commercial potential for our approved products, if any, could be affected by changes in healthcare spending and policy in the United States and abroad. We operate in a highly regulated industry. New laws, regulations or judicial decisions or new interpretations of existing laws, regulations or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could adversely affect our business, operations and financial condition.

For example, the ACA, has a significant impact on the healthcare industry. The ACA is a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The current presidential administration has indicated that enacting changes to the ACA is a legislative priority and has alternatively discussed repealing and replacing the ACA. While Congress has not passed repeal legislation to date, the Tax Act includes a provision that repealed the individual mandate, effective January 1, 2019. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 13, 2017, President Trump signed an Executive Order terminating the cost-sharing subsidies that reimburse insurers under the ACA. In addition, the Centers for Medicare and Medicaid Services, or CMS, has proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through these marketplaces. Congress will likely consider other legislation to replace elements of the ACA. We do not know at this time what implications these changes and other, proposed changes, if enacted, would have on the ACA's current requirements or on our future business. Changes to the ACA or other existing health care regulations could significantly impact our business and the pharmaceutical industry.

In addition, on January 31, 2020, the United Kingdom exited from the European Union, or Brexit. The effects of Brexit will depend on any agreements the United Kingdom makes to retain access to European Union markets either during a transitional period or more permanently. Brexit could lead to legal uncertainty and potentially divergent national laws and regulation as the United Kingdom determines which European Union laws to replace or replicate.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we or our collaborators are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or our collaborators are not able to maintain regulatory compliance, CTI-1601 or any future product candidates may lose any marketing approval that may have been obtained and we may not achieve or sustain profitability, which would materially adversely affect our business, financial condition and results of operations.

Even if approved, reimbursement policies could limit our ability to sell product candidates that we elect to sell on our own.

If approved by regulatory authorities, market acceptance and sales of product candidates that we elect to sell on our own will depend on reimbursement policies and may be affected by healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels for those medications. Cost containment is a primary concern in the U.S. healthcare industry and elsewhere. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for CTI-1601 or future product candidates that we elect to sell on our own and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, product candidates that we elect to sell on our own. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize product candidates that we elect to sell on our own.

In some foreign countries, particularly in Canada and European countries, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of product candidates that we elect to sell on our own with other available therapies. If reimbursement for product candidates that we elect to sell on our own is unavailable in any country in which we seek reimbursement, if it is limited in scope or amount, if it is conditioned upon our completion of additional clinical trials, or if pricing is set at unsatisfactory levels, our business, financial conditions and results of operations could be materially adversely affected.

Even if we obtain regulatory and marketing approval for a product candidate, our product candidates will remain subject to regulatory oversight.

Even if we receive marketing and regulatory approval for CTI-1601 or a future product candidate, regulatory authorities may still impose significant restrictions on the indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies. CTI-1601 or future product candidates will also be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. The FDA has significant post-market authority, including, for example, the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate serious safety risks related to the use of a drug. Any regulatory approvals that we receive for CTI-1601 may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including post-approval clinical trials, and surveillance to monitor the quality, safety and efficacy of the product, all of which could lead to lower sales volume and revenue. For example, the holder of an approved BLA or NDA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA or NDA. The holder of an approved BLA or NDA also must submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or NDA or foreign marketing application. If we, or a regulatory authority, discover(s) previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we or our contractors fail to comply with applicable regulatory requirements following approval of CTI-1601, a regulatory authority may:

- issue a warning letter asserting that we are in violation of the law;
- request voluntary product recalls;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize CTI-1601 and adversely affect our business, financial condition, results of operations and prospects.

In addition, the FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of CTI-1601. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would materially and adversely affect our business, financial condition, results of operations and prospects.

Even if we receive marketing approval for CTI-1601 in the United States, we may never receive regulatory approval to market CTI-1601 outside of the United States.

We may pursue marketing approval for CTI-1601 in the United States, the European Union and in other countries worldwide. In order to market any product outside of the United States, we must establish and comply with the numerous and varying safety, efficacy and other regulatory requirements of other countries, including potential additional clinical trials and/or nonclinical studies. Approval procedures vary among countries and can involve additional testing and additional administrative review periods. The time required to obtain approvals in other countries might differ from that required to obtain FDA approval. The marketing approval processes in other countries may implicate all of the risks detailed above regarding FDA approval in the United States as well as other risks. In particular, in many countries outside of the United States, products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval can result in substantial delays in bringing products to market in such countries. Marketing approval in one country does not necessarily ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process or commercial activities in others. Failure to obtain marketing approval in other countries or any delay or other setback in obtaining such approval would impair our ability to market a product candidate in such foreign markets. Any such impairment would reduce the size of our potential market, which could have a material adverse impact on our business, financial condition, results of operations and prospects.

Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties.

Our future profitability will depend, in part, on our ability to commercialize CTI-1601 and future product candidates in foreign markets for which we may rely on collaborations with third parties. If we commercialize a product candidate in foreign markets, we would be subject to additional risks and uncertainties, including:

- our customers' ability to obtain reimbursement for a product candidate in foreign markets;
- compliance with the Foreign Corrupt Practices Act of 1977, or FCPA;
- our inability to directly control commercial activities because we are relying on third parties;
- the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements;
- different medical practices and customs in foreign countries affecting acceptance in the marketplace;
- import or export licensing requirements;
- longer accounts receivable collection times;

- longer lead times for shipping;
- language barriers for technical training;
- reduced protection of intellectual property rights in some foreign countries;
- foreign currency exchange rate fluctuations; and
- the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute.

Foreign sales of a product candidate could also be adversely affected by the imposition of governmental controls, political and economic instability, trade restrictions and changes in tariffs.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell CTI-1601, we may not be able to generate any revenue.

We do not currently have an established infrastructure for the sales, marketing and distribution of biologic or drug products in the United States or foreign countries. In order to market a product candidate, if approved by the FDA or any other regulatory authority, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, or if we are unable to do so on commercially reasonable terms, our business, results of operations, financial condition and prospects will be materially adversely affected.

Even if we receive marketing approval for CTI-1601, we may not achieve broad market acceptance, which would limit the revenue that we generate from our sales.

The commercial success of CTI-1601, if developed and approved for marketing by the FDA or EMA or other applicable regulatory authorities, will depend upon the awareness and acceptance of CTI-1601 among the medical community, including physicians, patients, advocacy groups and healthcare payors. Market acceptance of CTI-1601, if approved, will depend on a number of factors, including, among others:

- the relative convenience and ease of subcutaneous injections as the necessary method of administration;
- the prevalence and severity of any adverse side effects associated with CTI-1601;
- limitations or warnings contained in the labeling approved for CTI-1601 by the FDA, EMA, or other regulatory authorities, such as a “black box” warning;
- availability of alternative treatments, including any competitive Friedreich’s Ataxia therapies in development that could be approved or commercially launched prior to approval of CTI-1601;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments;
- pricing;

- payor acceptance;
- increased political pressure on pharmaceutical pricing;
- increased pressure on orphan drug pricing for affected patient groups;
- the impact of any future changes in U.S. healthcare, including medical financial assistance or a transition to a single-payer system;
- the effectiveness of our sales and marketing strategies;
- our ability to increase awareness of CTI-1601 through marketing efforts;
- our ability to obtain sufficient third-party coverage or reimbursement;
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage; and
- the likelihood that the FDA may require development of a REMS, as a condition of approval or post-approval or may not agree with our proposed REMS or may impose additional requirements that limit the promotion, advertising, distribution or sales of our product candidates.

If CTI-1601 is approved but does not achieve an adequate level of acceptance by patients, advocacy groups, physicians and payors, we may not generate sufficient revenue from CTI-1601 to become or remain profitable and our business, financial condition and results of operations could be materially adversely affected. Our efforts to educate the medical community and third-party payors about the benefits of CTI-1601 may require significant resources and may never be successful.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as CTI-1601 or any potential product candidates, if approved. If we receive marketing approval for CTI-1601, or any potential product candidates, physicians may prescribe our product candidates to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion and required that they enter into corporate integrity agreements with the Office of Inspector General of the Department of Health and Human Services. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of CTI-1601 or any potential product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business, financial condition and results of operations.

Competing technologies could emerge, adversely affecting our opportunity to generate revenue from the sale of CTI-1601.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Any new product that competes with an approved product may need to demonstrate compelling advantages in efficacy, convenience, tolerability and safety to be commercially successful. Other competitive factors, including biosimilar and gene therapy competition, could force us to lower prices or could result in reduced sales. In addition, new products developed by others could emerge as competitors to CTI-1601 or any other potential product candidates. If we are not able to compete effectively against our current and future competitors, our business will not grow and our financial condition and results of operations will be adversely affected.

We may face competition from biosimilars and may face increasing competition over time.

We may face competition from biosimilars in both the United States and Europe, and over time we may face increasing biosimilar competition. To the extent that governments adopt more permissive approval frameworks and competitors are able to obtain broader or expedited marketing approval for biosimilars, the rate of increased competition for our biologic drug products could accelerate. Expiration or successful challenge of applicable patent rights could trigger such competition, and we could face more litigation regarding the validity and/or scope of our patents. Our products may also experience greater competition from lower-cost biosimilars or generics that come to market when branded products that compete with our products lose their own patent protection.

In the EU, the European Commission has granted marketing authorizations for biosimilars pursuant to a set of general and product class-specific guidelines for biosimilar approvals issued in 2005. In addition, in an effort to spur biosimilar utilization and/or increase potential healthcare savings, some EU countries have adopted biosimilar uptake measures such as requiring physician prescribing quotas or promoting switching or pharmacy substitution of biosimilars for the corresponding reference products, and other countries may adopt similar measures. Some EU countries may impose automatic price reductions upon market entry of the second or third biosimilar competitor.

In the United States, in 2010 the ACA authorized the FDA to approve biosimilars via a separate, abbreviated pathway. A growing number of companies have announced that they are in varying stages of development of biosimilar versions of existing biotechnology products. Some companies pursuing development of biosimilars may challenge our patents well in advance of the expiration of our material patents. The U.S. pathway includes the option for biosimilar products meeting certain criteria to be approved as interchangeable with their reference products. Some companies developing biosimilars may seek to register their products as interchangeable biologics, which could make it easier for prescribers or pharmacists to substitute those biosimilars for our products. In addition, critics of the 12-year exclusivity period in the biosimilar pathway law will likely continue to seek to shorten the data exclusivity period and/or to encourage the FDA to interpret narrowly the law's provisions regarding which new products receive data exclusivity. While we are unable to predict the precise impact of biosimilars, we expect in the future for there to be greater competition in the United States as a result of biosimilars and downward pressure on product prices and sales. This additional competition could have a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Business

If we are unable to manage expected growth in the scale and complexity of our operations, including attracting and hiring additional qualified management, our performance may suffer.

We are an early-stage clinical biotechnology company with a small number of employees, and our management systems currently in place are not likely to be adequate to support our future growth plans. As a result, we are highly dependent on our management and scientific personnel. The loss of the services of any of our executive officers, other key employees or consultants and other scientific advisors in the foreseeable future, might impede the achievement of our research, development and commercialization objectives. We rely on consultants and advisors, including scientific, nonclinical and clinical advisors, to assist us in formulating our development and commercialization strategy. These consultants and advisors may be employed by other employers and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the competition for talent, particularly with the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products.

Recruiting and retaining qualified scientific, medical clinical, manufacturing, quality assurance, regulatory, legal, public company financial, business, sales, marketing and commercial personnel and implementing and improving our operational, financial and management systems will be critical to our ability to grow and succeed. These demands also will require the hiring of additional executive or management-level personnel or the development of additional expertise by our senior management personnel. Hiring a significant number of additional

employees, particularly those at the executive or management level, would increase our expenses significantly. In addition, we may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical companies for similar personnel. We also experience competition for the hiring of scientific personnel from universities and research institutions. Moreover, delays or failures in clinical trials may also make it more challenging to recruit and retain qualified scientific personnel. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our business strategy will be limited and our business, financial condition and results of operations would be adversely affected.

Further, if we fail to expand and enhance our operational, financial and management systems in conjunction with potential future growth, such failure could have a material adverse effect on our business, financial condition and results of operations. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development, business and growth goals.

We have previously identified material weaknesses in our internal control over financial reporting. If we are unable to remediate these material weaknesses, or if we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect our business.

We have previously identified material weaknesses in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our financial statements will not be prevented or detected on a timely basis. If we are unable to remediate these material weaknesses, or if we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations.

The material weaknesses we identified were as follows:

- We did not maintain an effective control environment commensurate with our financial reporting requirements. We lacked a sufficient number of professionals with an appropriate level of accounting and controls knowledge, training and experience to appropriately analyze, record and disclose accounting matters timely, completely and accurately. Additionally, the limited personnel resulted in our inability to consistently establish appropriate authorities and responsibilities in pursuit of our financial reporting objectives, as demonstrated by, amongst other things, our insufficient segregation of duties in our finance and accounting functions. This material weakness contributed to the following material weakness.
- We did not design and maintain adequate controls over the preparation and review of certain account reconciliations and journal entries. Specifically, we did not design and maintain controls to ensure (i) appropriate segregation of duties in the preparation and review of account reconciliations and journal entries, and (ii) account reconciliations and journal entries were reviewed at the appropriate level of precision. This material weakness resulted in adjustments to prepaid expenses and accrued expenses which were identified and recorded as part of the audit of our consolidated financial statements as of and for the years ended December 31, 2019 and 2018.

Each of these control deficiencies could result in a misstatement of our accounts or disclosures that would result in a material misstatement of our consolidated financial statements that would not be prevented or detected, and accordingly, we determined these control deficiencies constitute material weaknesses.

We are in the process of implementing measures designed to improve our internal control over financial reporting and remediate the control deficiencies that led to this material weakness, including hiring additional finance and accounting personnel and initiating design and implementation of our financial control environment, including the establishment of formal accounting policies and procedures and period-end financial reporting controls. We cannot assure you that the measures we have taken to date, and actions we may take in the future, will be sufficient to remediate the control deficiencies that led to our material weaknesses in our internal control over financial reporting or that they will prevent or avoid potential future material weaknesses. In addition, neither our

management nor our independent registered public accounting firm has performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act of 2002, or Sarbanes-Oxley Act, because no such evaluation has been required. Had we or our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, additional material weaknesses may have been identified. If we are unable to successfully remediate our existing or any future material weaknesses in our internal control over financial reporting, or identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements and investors may lose confidence in our financial reporting.

Our internal computer systems, or those of any contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of ours product development programs.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Any system failure, accident or security breach that causes interruptions in our operations could result in a material disruption of our product development programs and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. For example, the loss of clinical trial data from completed clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liabilities and the further development of our product candidates may be delayed. In addition, we may not have adequate insurance coverage to provide compensation for any losses associated with such events.

We could be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in the information systems and networks of our company, including personal information of our employees. In addition, outside parties may attempt to penetrate our systems or those of our vendors or fraudulently induce our employees or employees of our vendors to disclose sensitive information in order to gain access to our data. Like other companies, we may experience threats to our data and systems, including malicious codes and viruses, and other cyber-attacks. The number and complexity of these threats continue to increase over time. If a material breach of our security or that of our vendors occurs, the market perception of the effectiveness of our security measures could be harmed, we could lose business and our reputation and credibility could be damaged, all of which would materially adversely affect our business, financial condition and results of operations. We could be required to expend significant amounts of money and other resources to repair or replace information systems or networks. Although we develop and maintain systems and controls designed to prevent these events from occurring, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Moreover, despite our efforts, the possibility of these events occurring cannot be eliminated entirely.

We may acquire businesses or products, or form strategic alliances, in the future, and we may not realize the benefits of such acquisitions or alliances.

We may acquire additional businesses or products, form strategic alliances or create joint ventures with third parties that we believes will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate such businesses with our existing operations and company culture.

We may encounter numerous difficulties in developing, manufacturing and marketing any new products resulting from a strategic alliance or acquisition that delays or prevents us from realizing their expected benefits or enhancing our business. We cannot be certain that, following any such transaction, we will achieve the expected synergies to justify the transaction and it could adversely affect our business, financial condition and results of operations.

We may seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans or expand our internal efforts and growth.

Our development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For CTI-1601, and any future product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates in some or all markets.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration for CTI-1601 or other potential product candidates will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the applicable product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such collaboration could be more attractive than the one with us for our product candidate. The terms of any collaboration or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing license agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable or unwilling to do so, we may have to curtail the development potential product candidates for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay potential commercialization in some or all markets or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense, including potentially increasing our infrastructure and investment outside the United States. If we elect to increase our expenditures to fund development or commercialization activities on our own, we will need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue. In addition, such efforts may require diversion of a disproportionate amount of our attention away from other day-to-day activities and require devotion of a substantial amount of our time to managing these activities.

In addition, any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration could adversely affect our business, financial condition, results of operations and could harm our business reputation.

We face risks related to health epidemics and other outbreaks of communicable diseases, which could significantly disrupt our operations and may materially and adversely affect our business and financial conditions.

Our business could be adversely impacted by the effects of the coronavirus or other epidemics. In December 2019, a novel strain of the coronavirus, or COVID-19, emerged in China and the virus has now spread to several other countries. In an effort to halt the outbreak of COVID-19, governments of countries around the world, including the United States, China and several European Union member states, have placed significant travel restrictions or advisories on travel within their respective borders and have instituted shelter-in-place policies that have led to extended business closures. The extent to which the coronavirus and global efforts to contain its spread will impact our operations will depend on future developments, which are highly uncertain and cannot be predicted at this time, and include the duration, severity and scope of the outbreak and the actions taken to contain or treat the coronavirus outbreak. The continued spread of the coronavirus globally could materially and adversely impact our operations, including without limitation, our manufacturing and supply chain for CTI-1601 and ongoing and planned Phase 1 clinical trials, which are facing, and could continue to face, enrollment difficulties as hospitals or clinical trials sites experience closures. We are currently evaluating CTI-1601 in a SAD Phase 1 clinical trial in patients with Friedreich's Ataxia. The first two cohorts of patients have completed the SAD clinical trial; however, due to the continued impact of COVID-19, we have delayed initiation of the next cohort in the SAD clinical trial. We are conducting the clinical trial at one clinical trial site in New Jersey. Because Friedreich's Ataxia is a rare disease, there are a limited number of patients in close proximity to the clinical trial site and clinical trial patients travel from throughout the United States to the clinical trial site to participate. The travel advisories and risk of infection related to COVID-19 have presented increased risks to patients traveling to our clinical trial site for dosing. Due to the uncertainty surrounding COVID-19, we cannot estimate when the next cohort of patients will begin the clinical trial. While top line results from the SAD and MAD clinical trials were originally expected by the end of 2020, the delay in the clinical trial timeline caused by the ongoing impact of COVID-19 has resulted in top line results being delayed until the first half of 2021. In addition, employee health and availability could be impacted, which may have a material and adverse effect on our business, financial condition and results of operations. A significant outbreak of coronavirus could also result in widespread global health crisis that could adversely affect global economies and financial markets resulting in an economic downturn that could have a material adverse effect on our business and prospects.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we may operate has established its own data security and privacy frameworks with which we must comply. For example, the European Union's General Data Protection Regulation 2016/679, or GDPR, imposes strict obligations on the processing of personal data, including personal health data, and the free movement of such data. The GDPR applies to any company established in the European Union as well as any company outside the European Union that processes personal data in connection with the offering of goods or services to individuals in the European Union or the monitoring of their behavior. The GDPR enhances data protection obligations for processors and controllers of personal data, including, for example, obligations relating to: processing health and other sensitive data; obtaining consent of individuals; providing notice to individuals regarding data processing activities; responding to data subject requests; taking certain measures when engaging third-party processors; notifying data subjects and regulators of data breaches; implementing safeguards to protect the security and confidentiality of personal data; and transferring personal data to countries outside the European Union, including the United States. The GDPR imposes additional obligations and risks upon our business and substantially increases the penalties to which we could be subject in the event of any non-compliance, including fines of up to €20 million or 4% of total worldwide annual turnover, whichever is higher. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages. Given the breadth and depth of changes in data protection obligations, if we are required to comply with the GDPR's requirements, we will be required to spend significant time and resources to review our technologies, systems and practices, as well as those of any third-party service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices or lead to government enforcement actions, private litigation or significant fines and penalties against us, reputational harm and could have a material adverse effect on our business, financial condition or results of operations.

We face potential product liability exposure, and, if claims are brought against us, we may incur substantial liability.

The use of CTI-1601 and other potential product candidates in clinical trials, if any, and the sale of CTI-1601 and other potential product candidates, if developed and approved, exposes us to the risk of product liability claims. Product liability claims might be brought against us by patients, healthcare providers or others selling or otherwise coming into contact with CTI-1601 or other potential product candidates. For example, we may be sued if any product we develop allegedly causes injury or death or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, including as a result of interactions with alcohol or other drugs, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we become subject to product liability claims and cannot successfully defend ourselves against them, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

- withdrawal of patients from our clinical trials;
- substantial monetary awards to patients or other claimants;
- decreased demand for CTI-1601 or ours other potential product candidates following marketing approval, if obtained;
- damage to our reputation and exposure to adverse publicity;
- increased FDA warnings on product labels;
- voluntary product recalls, withdrawals, or labeling restrictions;
- litigation costs;
- distraction of management’s attention from our primary business;
- loss of revenue; and
- the inability to successfully commercialize CTI-1601 or other potential product candidates, if approved.

We maintain product liability insurance coverage for our clinical trials with a $5 million aggregate coverage limit. Nevertheless, our insurance coverage may be insufficient to reimburse us for any expenses or losses we may suffer. Moreover, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses, including if insurance coverage becomes increasingly expensive. If we obtain marketing approval for CTI-1601 or other potential product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may not be able to obtain this product liability insurance on commercially reasonable terms. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. The cost of any product liability litigation or other proceedings, even if resolved in our favor, could be substantial, particularly in light of the size of our business and financial resources. A product liability claim or series of claims brought against us could cause our stock price to decline and, if we are unsuccessful in defending such a claim or claims and the resulting judgments exceed our insurance coverage, our financial condition, business, results of operations and prospects could be materially adversely affected.

Our employees may engage in misconduct or other improper activities, including violating applicable regulatory standards and requirements or engaging in insider trading, which could significantly impact our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA and applicable non-U.S. regulators, provide accurate information to the FDA and applicable non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. Employees may also unintentionally or willfully disclose our proprietary and/or confidential information to competitors. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of, including trading on, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We are expected to adopt a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may be ineffective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Risks Related to Ours Reliance on Third Parties

We have limited experience in conducting or supervising clinical trials and must outsource all clinical trials. As a result, many important aspects of our drug development programs are outside of our direct control.

We have limited experience in conducting or supervising clinical trials that must be performed to obtain data to submit in concert with applications for approval by the FDA or the EMA. As a result, we expect to continue to rely on CROs, clinical data management organizations and consultants to design, conduct, supervise and monitor our nonclinical studies and clinical trials. We and our CROs are required to comply with various regulations, including the FDA's regulations commonly referred to as good clinical practices, or GCPs, which are enforced by regulatory agencies, including the FDA, and comparable foreign regulatory authorities to ensure the health, safety and rights of patients are protected in clinical development and clinical trials, and that trial data integrity is assured. Regulatory authorities ensure compliance with these requirements through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Our expected reliance on third parties that we does not control does not relieve us of these responsibilities and requirements. If we or any of our CROs fail to comply with applicable requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or other comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with such requirements. In addition, our clinical trials must be conducted with products produced under cGMP requirements, which mandate, among other things, the methods, facilities and controls used in manufacturing, processing and packaging of a drug product to ensure its safety and identity. Failure to comply with these regulations may require us to repeat nonclinical studies and/or clinical trials, which would delay the regulatory approval process, and could also subject us to enforcement action, up to and including, civil and criminal penalties, which would materially adversely affect our business, financial condition and results of operations.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed or reduced. In addition, operations of our CROs could be affected by earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions. If their facilities are unable to operate because of an accident or incident, even for a short period of time, some or all of our research and development programs may be harmed or delayed and our operations and financial condition could suffer.

We have less direct control over the conduct, timing and completion of these clinical trials and the management of data developed through the clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical trials and may subject us to unexpected cost increases that are beyond our control. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on CROs does not relieve us of our regulatory responsibilities.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. We currently have a small number of employees, which limits the internal resources we have available to engage new third-party providers, if necessary, and monitor existing third-party providers. To the extent we are unable to engage new third-party providers, if necessary, and successfully manage the performance of third-party service providers in the future, our business may be adversely affected. Though we carefully manage our relationships with CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, results of operation and prospects.

We rely on third-party supply and manufacturing partners for drug supplies for our research and development, nonclinical activities, and clinical activities, and may do the same for any commercial supplies of our product candidates.

We rely on third-party supply and manufacturing partners to supply the materials and components for, and manufacture, our research and development, nonclinical and clinical study drug substance and product. We have not yet manufactured or formulated any product candidate on a commercial scale and may not be able to do so for any of our product candidates. We will work to develop and optimize our manufacturing process, however we cannot be sure that the process will result in therapies that are safe, potent or effective.

We do not own manufacturing facilities or supply sources for such components, nonclinical and clinical study drug substance, product and materials, including devices that may be required for administration, but may develop these capabilities in the future. There can be no assurance that our supply of research and development, nonclinical and clinical development of drugs and other materials will not be limited, interrupted, restricted in certain geographic regions or will be of satisfactory quality or continue to be available at acceptable prices. In particular, replacement of any product formulation manufacturer we may engage could require significant effort and expertise because there may be a limited number of qualified replacements.

In the event that any of our suppliers or manufacturers fails to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third party manufacture our product candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

We also rely on third parties to store master and working cell banks. We currently have one master cell bank and one working cell bank for CTI-1601 and believe we would have adequate backup should any cell bank be lost in a catastrophic event. However, it is possible that we could lose multiple cell banks and have our manufacturing severely impacted by the need to replace the cell banks, which could materially and adversely affect our business, financial condition and results of operations

We may rely on third party manufacturers if we receive regulatory approval for any product candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third party's failure to execute on our manufacturing requirements could adversely affect our business, financial condition and results of operations in a number of ways, including:

- an inability to initiate or continue clinical trials of product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for product candidates;
- loss of the cooperation of a collaborator;
- subjecting our product candidates to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of our product candidates; and
- in the event of approval to market and commercialize a product candidate, an inability to meet commercial demands for our products.

Our and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for CTI-1601, are subject to extensive regulation. Some components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA or NDA on a timely basis and where required, must adhere to the FDA's or other regulator's good laboratory practices, or GLPs, and cGMP regulations enforced by the FDA or other regulator through facilities inspection programs. The facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of CTI-1601 or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of CTI-1601 or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA or other regulatory approval of the products will not be granted.

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

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA or other regulators can impose regulatory sanctions including, among other things, refusal to approve a pending application for a biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be materially harmed.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. The number of manufacturers with the necessary manufacturing capabilities is limited. In addition, an alternative manufacturer would need to be qualified through a BLA or NDA supplement or similar regulatory submission which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

These factors could also cause the delay of manufacturing development, clinical trials, regulatory submissions, required approvals or commercialization of CTI-1601 or any other product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenues. Any of the above would materially adversely affect our business, financial condition and results of operations.

We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, it could materially increase our costs and potential liability.

In the normal course of business, we periodically enter into academic, commercial, service, collaboration, licensing, consulting and other agreements that contain indemnification provisions. With respect to our academic and other research agreements, we typically indemnify the institution and related parties from losses arising from claims relating to the products, processes or services made, used, sold or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to our collaboration and contract service agreements, we indemnify our collaborators from any third-party product liability claims that could result from the production, use or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party. With respect to consulting agreements, we indemnify consultants from claims arising from the good faith performance of their consulting services.

Should our obligation under an indemnification provision exceed applicable insurance coverage or should we be denied insurance coverage, our business, financial condition and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds the applicable insurance coverage, and if the collaborator does not have other assets available to indemnify us, our business, financial condition and results of operations could be adversely affected.

To the extent we are able to enter into collaborative arrangements or strategic alliances, we may be exposed to risks related to those collaborations and alliances.

Biotechnology companies sometimes become dependent upon collaborative arrangements or strategic alliances to complete the development and commercialization of product candidates. If we elect to enter into collaborative arrangements or strategic alliances, these arrangements may place the development of our product candidates outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us, which could adversely affect our business, financial condition and results of operations.

Dependence on collaborative arrangements or strategic alliances would subject us to a number of risks, including the risk that:

- we may not be able to control the amount and timing of resources that our collaborators may devote to the relevant product candidates;
- our collaborators may experience financial difficulties;
- we may be required to relinquish important rights, such as marketing and distribution rights;
- business combinations or significant changes in a collaborator's business strategy may also adversely affect a collaborator's willingness or ability to complete its obligations under any arrangement;
- a collaborator could independently move forward with a competing drug candidate developed either independently or in collaboration with others, including ours competitors; and
- collaborative arrangements are often terminated or allowed to expire, which would delay the development and may increase the cost of developing our drug candidates.

Risks Related to Our Intellectual Property Rights

If we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect CTI-1601 or potential product candidates, third parties could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects.

Our commercial success will depend in part on our success in obtaining and maintaining issued patents and other intellectual property rights in the United States and elsewhere and protecting our proprietary technology. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability.

With respect to our patent portfolio, we in-license from WFUHS certain issued U.S. patents that relate to CTI-1601 and its use for treating Friedreich's Ataxia. We also in-license from IU certain pending U.S. provisional patent applications that relate to the composition of CTI-1601 and methods of use, and certain U.S. patents relating to materials and methods of use relating to the development of CTI-1601. We also own or co-own pending U.S. provisional applications relating to methods of use of CTI-1601, biomarkers and to our platform technology.

In some cases, we have only filed provisional patent applications on certain aspects of our technologies and each of these provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of the applicable provisional patent application. Any failure to file a non-provisional patent application within this timeline could cause us to lose the ability to obtain patent protection for the inventions disclosed in the associated provisional patent applications.

With respect to both in-licensed and owned intellectual property, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties.

We cannot provide any assurances that any of our pending patent applications that mature into issued patents will include claims with a scope sufficient to protect CTI-1601, or other potential product candidates. Other parties have developed technologies that may be related or competitive to our approach and may have filed or may file patent applications and may have received or may receive patents that may overlap or conflict with our patent applications, either by claiming the same methods or formulations or by claiming subject matter that could dominate our patent position. The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, the issuance, scope, validity and enforceability of any patent claims that we may obtain cannot be predicted with certainty. Patents, if issued, may be challenged, deemed unenforceable, invalidated, or circumvented. U.S. patents and patent applications may also be subject to interference proceedings, ex parte reexamination, or inter partes review proceedings, supplemental examination and

challenges in district court. Patents may be subjected to opposition, post-grant review, or comparable proceedings lodged in various foreign, both national and regional, patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. In addition, such proceedings may be costly. Thus, any patents that we may own or exclusively license may not provide any protection against competitors. Furthermore, an adverse decision in an interference proceeding can result in a third party receiving the patent right sought by us, which in turn could affect our ability to develop, market or otherwise commercialize CTI-1601, and other potential product candidates.

Furthermore, though an issued patent is presumed valid and enforceable, its issuance is not conclusive as to its validity or its enforceability and it may not provide us with adequate proprietary protection or competitive advantages against competitors with similar products. Competitors may also be able to design around our patents. Other parties may develop and obtain patent protection for more effective technologies, designs or methods. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the United States, and we may encounter significant problems in protecting our proprietary rights in these countries. If these developments were to occur, they could have a material adverse effect on our potential future sales.

Our ability to enforce our patent rights depends on our ability to detect infringement. It is difficult to detect infringers who do not advertise the components that are used in their products. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product. Any litigation to enforce or defend our patent rights, even if we were to prevail, could be costly and time-consuming and would divert the attention of our management and key personnel from our business operations. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If any of our patents covering CTI-1601 are invalidated or found unenforceable, our financial position and results of operations would be materially and adversely impacted. In addition, if a court found that valid, enforceable patents held by third parties covered CTI-1601, our financial position and results of operations would also be materially and adversely impacted.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- any of our patents, or any of our pending patent applications, if issued, will include claims having a scope sufficient to protect CTI-1601 or any other products or product candidates;
- any of our pending patent applications will issue as patents;
- we will be able to successfully develop and, if approved, commercialize CTI-1601 before our relevant patents expire;
- we were the first to make the inventions covered by each of our patents and pending patent applications;
- we were the first to file patent applications for these inventions;
- others will not develop similar or alternative technologies that do not infringe our patents;
- any of our patents will be found to ultimately be valid and enforceable;
- any patents issued to us will provide a basis for an exclusive market for our commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;

- we will develop additional proprietary technologies or product candidates that are separately patentable; or
- that our commercial activities or products will not infringe upon the patents of others.

We rely upon unpatented trade secrets, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us and have non-compete agreements with some, but not all, of our consultants. It is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees and consultants who are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could otherwise become known or be independently discovered by our competitors. If we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect CTI-1601 or potential future product candidates, third parties could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects.

Over time, we will lose our ability to rely upon the intellectual property we currently own to prevent competing products, which may impair our ability to generate revenue.

We have in-licensed certain patents relating to CTI-1601 from WFUHS. The U.S. patents relating to CTI-1601 and its use for the treatment of Friedreich's Ataxia expire in 2024 and 2025, respectively. We have also in-licensed certain provisional patent applications relating to the composition of CTI-1601 and methods of use from IU, which, if issued as a patent, would expire at the earliest in 2040. We cannot predict whether these provisional patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors or other third parties. When these various patents expire, we will be unable to use the patents to try to block others from marketing CTI-1601 in the United States.

In addition, given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Once our patents expire, we will be subject to competition from third parties who will be able to use the intellectual property covered by these patents, which could impair our ability to generate revenue and could adversely affect our business, financial condition and results of operations.

We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing CTI-1601 or other potential product candidates, if approved.

Our success will depend in part on our ability to operate without infringing the intellectual property and proprietary rights of third parties. We cannot ensure that our business, products and methods do not or will not infringe the patents or other intellectual property rights of third parties.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Third parties may allege that CTI-1601 or our other potential product candidates or the use of our technologies infringes patent claims or other intellectual property rights held by them or that we are employing their proprietary technology without authorization. Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. Any claim relating to intellectual property infringement that is successfully asserted against us may require us to pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing another party's patents, for past use of the asserted intellectual property and royalties and other consideration going forward if we are forced to take a license.

In addition, if any such claim were successfully asserted against us and we could not obtain such a license, we may be forced to stop or delay developing, manufacturing, selling or otherwise commercializing CTI-1601.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or redesign our products. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, intellectual property litigation or claims could force us to do one or more of the following:

- cease developing, selling or otherwise commercializing CTI-1601;
- cease preparations or development of our other potential product candidates;
- pay substantial damages for past use of the asserted intellectual property;
- obtain a license from the holder of the asserted intellectual property, which license may not be available on reasonable terms, if at all; and
- in the case of trademark claims, redesign or rename the trademarks or trade names of our product candidates to avoid infringing the intellectual property rights of third parties, which may not be possible and, even if possible, could be costly and time-consuming.

Any of these risks coming to fruition could have a material adverse effect on our business, results of operations, financial condition and prospects.

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

We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business, financial condition and results of operations. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

The U.S. Patent and Trademark Office, or U.S. PTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case, which would adversely affect our business, financial condition and results of operations.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe on our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of our or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the

grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing which could materially adversely affect our business, financial condition and results of operations.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to us from the prevailing party. Our business, financial condition and results of operations could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States, which could adversely affect our business, financial condition and results of operations.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

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

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

We do not seek to protect our intellectual property rights in all jurisdictions throughout the world and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection.

Filing, prosecuting and defending patents on product candidates in all countries and jurisdictions throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. For example, an April 2014 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license, which would materially adversely affect our business, financial condition and results of operations.

We are dependent on licensed intellectual property for CTI-1601. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing CTI-1601, if approved.

We have an exclusive license with WFUHS, pursuant to which we exclusively license certain patent rights relating to the TAT-frataxin fusion protein and its use, on a worldwide basis. We have an exclusive license with IU, pursuant to which we exclusively license certain patent rights relating to CTI-1601 and its use for the treatment of mitochondrial diseases, on a worldwide basis. We may enter into additional licenses for third-party intellectual property that are necessary or useful to our business. Current or future licensors may also allege that we have breached our license agreement and may accordingly seek to terminate our license with them. In addition, current or future licensors may decide to terminate our license at will. If successful, this could result in loss of our right to use the licensed intellectual property, which could materially adversely affect our ability to develop and commercialize CTI-1601, if approved, as well as harm our competitive business position, our business prospects, financial condition and results of operations.

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

Our license agreements with WFUHS and IU impose, and we expect our future license agreements will impose, various development, diligence, commercialization, and other obligations on us in order to maintain the licenses. In spite of our efforts, WFUHS, IU, or a future licensor might conclude that we have materially breached our obligations under such license agreements and seek to terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If these licenses are terminated, or if the underlying patent rights licensed thereunder fail to provide the intended exclusivity, competitors or other third parties would have the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of certain of our product candidates or of CTI-1601. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

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

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;

- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners;
- whether and the extent to which inventors are able to contest the assignment of their rights to our licensors; and
- the priority of invention of patented technology.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize CTI-1601, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

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

Our in-licensed patent rights from WFUHS and IU were funded in part by the U.S. government and are therefore subject to certain federal regulations. When new technologies are developed with U.S. government funding, the U.S. government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. The U.S. government's rights may also permit it to disclose the funded inventions and technology to third parties and to exercise march-in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, or because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States in certain circumstances and if this requirement is not waived. Any exercise by the U.S. government of such rights or by any third party of its reserved rights could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects.

We have not yet registered trademarks for a commercial trade name for CTI-1601 or other potential product candidates and failure to secure such registrations could adversely affect our business, financial condition and results of operations.

We have not yet registered trademarks for a commercial trade name for CTI-1601 or other potential product candidates. Any future trademark applications may be rejected during trademark registration proceedings. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, the U.S. PTO and comparable agencies in many foreign jurisdictions give third parties an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we propose to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation by extending the patent terms and obtaining data exclusivity for CTI-1601, our business may be materially harmed.

Depending upon the timing, duration and specifics of development and FDA marketing approval of CTI-1601 or our other potential product candidates, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain a patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our ability to generate revenues, business, financial condition and results of operations could be materially adversely affected.

Our proprietary rights may not adequately protect our technologies, which may adversely affect our position in the market, business, financial condition and results of operations.

We rely on unpatented trade secrets, know-how, and technology, which are difficult to protect, especially in the pharmaceutical industry, where much of the information about a product must be made public during the regulatory approval process. We seek to protect trade secrets, in part, by entering into confidentiality agreements with employees, consultants and others. These parties may breach or terminate these agreements or may refuse to enter into such agreements with us, and we may not have adequate remedies for such breaches. Furthermore, these agreements may not provide meaningful protection for our trade secrets or other proprietary information or result in the effective assignment to us of intellectual property and may not provide an adequate remedy in the event of unauthorized use or disclosure of confidential information or other breaches of the agreements. Despite our efforts to protect our trade secrets, we or our board members, employees, consultants, contractors or scientific and other advisors may unintentionally or willfully disclose our proprietary information to competitors.

If we fail to maintain trade secret protection, our competitive position may be adversely affected. Competitors may also independently discover our trade secrets. Enforcement of claims that a third party has illegally obtained and is using trade secrets is expensive, time consuming and uncertain. If ours competitors independently develop equivalent knowledge, methods and know-how, we would not be able to assert our trade secrets against them and our business, financial condition and results of operations could be harmed.

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

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. Patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or the Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. For example, the Leahy-Smith Act allows third-party submission of prior art to the U.S. PTO during patent prosecution and additional procedures to attack the validity of a patent by U.S. PTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. In addition, the Leahy-Smith Act has transformed the U.S. patent system from a “first-to-invent” system to a “first-to-file” system in which, assuming that other requirements for patentability are met, the first inventor to file a patent

application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our or our collaboration partners' patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations, financial condition and prospects.

In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the U.S. PTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of our employees' former employers.

Our employees have been previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we are not aware of any claims currently pending against us, we may be subject to claims that we or our employees inadvertently or otherwise used or disclosed the trade secrets or other proprietary information of our employees' former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management. If we fail in defending such claims, in addition to paying money claims, we may lose valuable intellectual property rights or personnel. A loss of key personnel or their work product could hamper or prevent our ability to develop and commercialize CTI-1601 or our other potential product candidates, which would materially adversely affect our business, financial condition and results of operations.

Risks Related to Our Common Stock

Our stock price could be highly volatile, and purchasers of our common stock could incur substantial losses.

The market price of our common stock could be subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology, and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- Our ability to obtain regulatory approvals for product candidates, and delays or failures to obtain such approvals;
- the results of current, and any future, nonclinical or clinical trials of CTI-1601 or any of our future product candidates;
- the entry into, or termination of, key agreements, including key licensing or collaboration agreements;
- the failure of CTI-1601 or any of our future product candidates, if approved for marketing and commercialization, to achieve commercial success;
- issues in manufacturing our approved products, if any, or product candidates;
- the initiation of material developments in, or conclusion of, disputes or litigation to enforce or defend any of our intellectual property rights or defend against the intellectual property rights of others;

- announcements by commercial partners or competitors of new commercial products, clinical progress (or the lack thereof), significant contracts, commercial relationships, or capital commitments;
- adverse publicity relating to our markets, including with respect to other products and potential products in such markets;
- the introduction of technological innovations or new therapies competing with our potential products;
- the loss of key employees;
- general and industry-specific economic conditions potentially affecting our research and development expenditures;
- changes in the structure of health care payment systems;
- adverse regulatory decisions;
- trading volume of our common stock; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies or the biotechnology sector. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management's attention and resources, which could significantly impact our profitability and reputation.

We must maintain effective internal controls over financial reporting, and if we are unable to do so, the accuracy and timeliness of our financial reporting may be adversely affected, which could have a material adverse effect on our business and stock price.

Until December 31, 2019, Zafgen was an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, and took advantage of certain exemptions from various reporting requirements that are applicable to other companies that are not "emerging growth companies" including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act.

We must maintain effective internal control over financial reporting in order to accurately and timely report our results of operations and financial condition. In addition, as a public company, the Sarbanes-Oxley Act requires, among other things, that we assess the effectiveness of our disclosure controls and procedures quarterly and the effectiveness of our internal control over financial reporting at the end of each fiscal year.

The rules governing the standards that must be met for our management to assess our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act are complex and require significant documentation, testing and possible remediation. These stringent standards require that our audit committee be advised and regularly updated on management's review of internal control over financial reporting.

We have previously identified material weaknesses in our internal control over financial reporting. See "*Risk Factors—Risks Related to Our Business— We have identified material weaknesses in our internal control over financial reporting. If we are unable to remediate these material weaknesses, or if we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to*

accurately or timely report our financial condition or results of operations, which may adversely affect our business." We are in the process of implementing measures designed to improve our internal control over financial reporting and remediate the control deficiencies that led to this material weakness, including hiring additional finance and accounting personnel and initiating design and implementation of our financial control environment, including the establishment of formal accounting policies and procedures and period-end financial reporting controls. We will continue this process.

Our management may not be able to effectively and timely implement controls and procedures that adequately remediate our material weaknesses and respond to the increased regulatory compliance and reporting requirements that are applicable to us as a public company. If we fail to staff our accounting, finance and information technology functions adequately or maintain internal control over financial reporting adequate to meet the demands that are placed upon us as a public company, including the requirements of the Sarbanes-Oxley Act, or to otherwise remediate our existing or any future material weaknesses in internal control over financial reporting, or identify any additional material weaknesses, our business and reputation may be harmed and our stock price may decline. Furthermore, investor perceptions of us may be adversely affected, which could cause a decline in the market price of our common stock.

Our failure to meet the continued listing requirements of The Nasdaq Stock Market LLC could result in a delisting of our Common Stock.

If we fail to satisfy the continued listing requirements of The Nasdaq Stock Market LLC, or Nasdaq, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase shares of common stock when you wish to do so. In the event of a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would allow the common stock to become listed again, stabilize the market price or improve the liquidity of the common stock, prevent the common stock from dropping below the Nasdaq minimum bid price requirement or prevent future noncompliance with Nasdaq's listing requirements.

Financial reporting obligations of being a public company in the United States are expensive and time-consuming, and our management will be required to devote substantial time to new compliance matters.

As a publicly-traded company, we will incur significant additional legal, accounting and other expenses that Chondrial did not incur as a privately-held company prior to the Merger. The obligations of being a public company in the United States requires significant expenditures and will place significant demands on our management and other personnel, including costs resulting from public company reporting obligations under the Securities Exchange Act of 1934, as amended, and the rules and regulations regarding corporate governance practices, including those under the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, or the Dodd-Frank Act, and the listing requirements of Nasdaq on which our securities are listed. These rules require the establishment and maintenance of effective disclosure and financial controls and procedures, internal control over financial reporting and changes in corporate governance practices, among many other complex rules that are often difficult to implement, monitor and maintain compliance with. Moreover, despite recent reforms made possible by the Tax Act, the reporting requirements, rules, and regulations will make some activities more time-consuming and costly. In addition, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage that Chondrial had as a privately-held company. For example, Chondrial's management had identified material weaknesses in Chondrial's internal control over financial reporting prior to the Merger. As a result, our management and other personnel will need to devote a substantial amount of time to remedy the identified material weaknesses and otherwise ensure that we comply with all of these requirements and to keep pace with new regulations, otherwise we may fall out of compliance and risk becoming subject to litigation or being delisted, among other potential problems.

The sale or availability for sale of a substantial number of shares of our common stock after the expiration of applicable lock-up periods could adversely affect the market price of such shares.

Sales of a substantial number of shares of our common stock in the public market after expiration of applicable lock-up periods and other legal restrictions on resale, or the perception that these sales could occur, could adversely affect the market price of such shares and could materially impair our ability to raise capital through equity offerings in the future. We are unable to predict what effect, if any, market sales of securities held by significant stockholders, directors or officers or the availability of these securities for future sale will have on our market price.

Ownership of our common stock is highly concentrated, and it may prevent other stockholders from influencing significant corporate decisions.

Holdings' members beneficially own or control approximately 39.8% of our outstanding common stock (assuming no exercise of outstanding options) as of June 1, 2020, on a fully-diluted basis. Accordingly Holdings' members have substantial influence over the outcome of a corporate action by us requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transaction. These stockholders also may exert influence in delaying or preventing a change in control of the combined company, even if such change in control would benefit our other stockholders.

We are a smaller reporting company. We cannot be certain whether the reduced disclosure requirements applicable to smaller reporting companies will make our common shares less attractive to investors or otherwise limit our ability to raise additional funds.

We are currently a "smaller reporting company" as defined in the Securities Exchange Act of 1934, as amended, or the Exchange Act, and have elected to take advantage of certain of the scaled disclosures available to smaller reporting companies, including simplified executive compensation disclosures in our filings, exemption from the provisions of Section 404(b) of the Sarbanes-Oxley Act requiring that an independent registered accounting firm provide an attestation report on the effectiveness of internal control over financial reporting and certain other decreased disclosure obligations in our SEC filings, including, among other things, only being required to provide two years of audited financial statements in annual reports. Reduced disclosure in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects. We cannot predict whether investors will find our common stock less attractive because of our reliance on any of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our certificate of incorporation and bylaws may delay or prevent an acquisition or a change in management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders, and the ability of the board of directors to issue preferred stock without stockholder approval. In addition, because we are in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporations Law, or DGCL, which prohibits stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain, if any, for the foreseeable future.

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

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including due to the impact of any potential new outbreaks related to the COVID-19 pandemic, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

CHONDRIAL'S AUDITED CONSOLIDATED FINANCIAL STATEMENTS

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONSOLIDATED FINANCIAL STATEMENTS

YEARS ENDED DECEMBER 31, 2019 AND 2018

CHONDRIAL'S AUDITED CONSOLIDATED FINANCIAL STATEMENTS

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Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Chondrial Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Chondrial Therapeutics, Inc. and its subsidiary (the "Company") as of December 31, 2019 and 2018, and the related consolidated statements of operations, changes in stockholder's (deficit) equity and cash flows for the years then ended, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2019 and 2018, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Substantial Doubt About the Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the consolidated financial statements, the Company has suffered recurring losses from operations since its inception and has an accumulated deficit that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 2. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Change in Accounting Principle

As discussed in Note 3 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/PricewaterhouseCoopers LLP
Philadelphia, Pennsylvania
March 6, 2020

We have served as the Company's auditor since 2020.

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS

DECEMBER 31, 2019 AND 2018
(In thousands, except share data)

	December 31, 2019	December 31, 2018
ASSETS		
Current assets		
Cash	$ 1,009	$ 4,356
Restricted cash equivalents	—	40
Prepaid expenses and other assets	3,741	456
Total current assets	4,750	4,852
Fixed assets, net	274	269
Other assets	90	26
Operating lease right-of-use assets	87	—
Total assets	$ 5,201	$ 5,147
LIABILITIES AND STOCKHOLDER'S (DEFICIT) EQUITY		
Current liabilities		
Accounts payable	$ 3,539	$ 909
Accrued expenses	2,259	1,324
Operating lease liability, current	97	—
Total liabilities	5,895	2,233
Commitments (See note 11)		
Stockholder's (deficit) equity		
Common stock, 5,000 shares authorized and 100 shares issued, par value $0.01	1	1
Additional paid-in capital	22,437	2,913
Accumulated deficit	(23,132)	—
Total stockholder's (deficit) equity	(694)	2,914
Total liabilities and stockholder's (deficit) equity	$ 5,201	$ 5,147

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

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF OPERATIONS

FOR THE YEARS ENDED DECEMBER 31, 2019 AND 2018
(In thousands)

	December 31, 2019	December 31, 2018
Revenue	$ —	$ —
Operating expenses:		
Research and development	20,790	9,609
General and administrative	2,424	1,583
Total operating expenses	23,214	11,192
Loss from operations	(23,214)	(11,192)
Other income	82	—
Loss before provision for income taxes	(23,132)	(11,192)
Provision for income taxes	—	—
Net Loss	$ (23,132)	$ (11,192)
Total Comprehensive Loss	$ (23,132)	$ (11,192)

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

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDER'S (DEFICIT) EQUITY

YEARS ENDED DECEMBER 31, 2019 AND 2018
(In thousands, except share data)

	Common		Combined Equity	Additional paid in capital	Accumulated Earnings (Deficit)	Total
	Shares	Amount				
Balance as of December 31, 2017	—	$ —	$ 1,850	$ —	$ —	$ 1,850
Stock-based compensation expense	—	—	173	—	—	173
Capital contribution from related party (See Note 12)	—	—	12,082	—	—	12,082
Net loss	—	—	(11,192)	—	—	(11,192)
Reorganization of entities under common control (See Note 8)	100	1	(2,913)	2,913	—	1
Balance as of December 31, 2018	100	1	—	2,913	—	2,914
Stock-based compensation expense	—	—	—	129	—	129
Capital contribution from related party (See Note 12)	—	—	—	19,395	—	19,395
Net loss	—	—	—	—	(23,132)	(23,132)
Balance as of December 31, 2019	100	$ 1	$ —	$ 22,437	$ (23,132)	$ (694)

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

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CASH FLOWS

YEARS ENDED DECEMBER 31, 2019 AND 2018
(In thousands)

	December 31, 2019	December 31, 2018
Cash flows used in operating activities		
Net loss	$ (23,132)	$ (11,192)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	129	173
Depreciation	78	60
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(3,285)	(116)
Other assets	(64)	(10)
Accounts payable	2,630	810
Accrued expenses	945	770
Net cash used in operating activities	(22,699)	(9,505)
Cash flows used in investing activities		
Purchase of equipment	(83)	(93)
Net cash used in investing activities	(83)	(93)
Cash flows provided by financing activities		
Capital contribution from related party	19,395	12,082
Net cash provided by financing activities	19,395	12,082
Net (decrease) increase in cash	(3,387)	2,484
Cash and restricted cash equivalents, beginning of period	4,396	1,912
Cash and restricted cash equivalents, end of period	$ 1,009	$ 4,396
Reconciliation of cash and restricted cash equivalents:		
Cash	$ 1,009	$ 4,356
Restricted cash equivalents	—	40
Cash and restricted cash equivalents, end of period	$ 1,009	$ 4,396

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

(1) Nature of the Business and Basis of Presentation

Chondrial Therapeutics, Inc. ("Chondrial Inc."), a Delaware corporation, was formed on November 22, 2016, as a wholly owned subsidiary of Chondrial Therapeutics Holdings, LLC ("Holdings"). Holdings was also formed on November 22, 2016, in the state of Delaware as a limited liability company ("LLC"). Chondrial Therapeutics, LLC ("Old Chondrial"), an Indiana LLC, was formed on September 4, 2013.

On November 30, 2016 (the "Transaction Date"), Old Chondrial filed a certificate of conversion in the state of Delaware, pursuant to which it changed its name to Chondrial Therapeutics IP, LLC ("IP LLC"), a Delaware LLC, and became another wholly owned subsidiary of Holdings. On the Transaction Date, the members of Old Chondrial contributed their member units to Holdings in exchange for Common Units in Holdings (the "Transaction"). As of the Transaction Date, Old Chondrial had limited assets, primarily consisting of an option agreement to license technology (see Note 11) from two institutions for use in the treatment of a mitochondrial disorder. IP LLC holds the Company's material patents and intellectual property license agreements. On December 31, 2018, the membership units of IP LLC were contributed by Holdings to Chondrial Inc. and IP LLC became a wholly-owned subsidiary of Chondrial Inc. (collectively, "the Company") (see Note 8).

The Company is a clinical stage biopharmaceutical company leveraging its proprietary knowledge to develop a therapeutic treatment for mitochondrial disorders which currently have no cure. The Company has focused on Friedreich's ataxia ("Friedreich's Ataxia"), which is a progressive disease that affects multiple body systems, particularly the brain and heart. CTI-1601, the Company's lead product candidate, utilizes a cell penetrant peptide to deliver frataxin, the protein deficient in "Friedreich's Ataxia", to the mitochondria where it is believed to be processed into mature frataxin and becomes active in mitochondrial metabolism. In July 2017, the Company received orphan drug designation for CTI-1601 from the Food and Drug Administration ("FDA"). This makes CTI-1601 eligible for orphan product exclusivity lasting seven years starting at FDA approval. On September 27, 2019, the Company submitted an Investigational New Drug ("IND") application for CTI-1601 to the Center for Drug Evaluation and Research ("CDER") of the FDA as well as a "Fast Track" designation request, and on October 9, 2019, the Company requested designation for CTI-1601 as a drug for a rare pediatric disease. Fast Track designation is designed to facilitate the development of, and expedite the review of, drugs to treat serious conditions and fill unmet medical needs, the purpose being to make important new drugs available to patients earlier. Rare pediatric disease designation incentivizes companies to develop drugs to treat rare pediatric diseases and drugs with this designation can become eligible upon approval for a voucher entitling another drug to priority review by FDA. On October 25, 2019, the FDA informed the Company that it may proceed with its clinical investigation for the treatment of "Friedreich's Ataxia" and on November 20, 2019, the Company was granted Fast Track Designation for CTI-1601. On December 5, 2019, the FDA granted the Company designation for CTI-1601 as a drug for a rare pediatric disease, and that same month, the Company dosed its first human patient in its Phase I clinical trial.

On December 17, 2019 the Company entered into an Agreement and Plan of Merger ("Merger Agreement") with Zordich Merger Sub, Inc., ("Merger Sub") a wholly owned subsidiary of Zafgen, Inc. ("Zafgen"), a publicly traded company on the NASDAQ Global Market. Pursuant to the Merger Agreement, the Company will be merged with and into Merger Sub at the effective time of the merger, with the Company continuing after the merger as the surviving company (the "Merger"). The surviving company will be named Larimar Therapeutics, Inc. Under the exchange ratio formula in the Merger Agreement, immediately after the Merger, the Company's shareholders are expected to own approximately 60% of the outstanding common stock on a fully-diluted basis, and shareholders of Zafgen are expected to own approximately 40% of the outstanding shares on a fully-diluted basis. Zafgen's and the Company's obligations to consummate the Merger are subject to the satisfaction or waiver of customary closing conditions, including, among others, obtaining the requisite approvals of the stockholders of Zafgen, Holdings and the Company, including the approval of the charter amendments by the stockholders of Zafgen, as well as satisfaction of minimum net cash thresholds of $30.0 million by Zafgen and not less than zero by the Company. Holdings, in its capacity as the sole stockholder of Chondrial Inc., has approved the Merger Agreement by written consent.

The Zafgen board of directors has unanimously approved the Merger Agreement and the related transactions and has adopted resolutions recommending the requisite stockholder approval for the issuance of the shares of Zafgen common stock pursuant to the Merger. Zafgen has agreed to hold a stockholders' meeting to submit certain matters to its stockholders for their consideration.

The Merger will be accounted for as an asset acquisition under the provisions of Financial Accounting Standards Board Accounting Standards Codification Topic 805, *Business Combinations* ("ASC 805"). Because the Company has been determined to be the accounting acquirer in the Merger, but not the legal acquirer, the Merger is deemed a reverse acquisition under the guidance of ASC 805. As a result, upon consummation of the Merger, the historical financial statements of the Company will become the historical financial statements of the combined company and the Company will record the assets acquired and liabilities assumed of Zafgen in the Merger at their fair values as of the acquisition date.

The consolidated financial statements include the accounts of Chondrial Inc. and its wholly owned subsidiary, IP LLC. All intercompany balances and transactions have been eliminated. The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

(2) Going Concern, Liquidity and Uncertainties

In accordance with Accounting Standards Update ("ASU") 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the consolidated financial statements are issued.

From its inception through December 31, 2019, the Company has received funding from Holdings which originated from Holdings sale of Series A Preferred Units and Series B convertible preferred units to Deerfield Private Design Fund IV, L.P., Deerfield Private Design Fund III, L.P., Deerfield Health Innovations Fund, L.P. (together the "Deerfield Funds") and certain other purchasers during 2019, 2018, 2017 and 2016. The Company has incurred recurring losses since its inception, including net losses of $23.1 million and $11.2 million for the years ended December 31, 2019 and 2018, respectively. In addition, as of December 31, 2019, the Company had an accumulated deficit of $23.1 million. The Company expects to continue to generate operating losses for the foreseeable future.

As of March 6, 2020, the issuance date of the consolidated financial statements for the year ended December 31, 2019, the Company expects that its cash balance at December 31, 2019, funding from the issuances of Series B Bridge Units (see Note 12) and Second Series B Bridge Units (see Note 13) by Holdings, would enable it to fund its operating expense and capital requirements into the second quarter of 2020. The future viability of the Company is largely dependent on its ability to generate cash from operating activities and to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed will have a negative impact on its financial condition and its ability to continue to pursue its business strategies.

The Company believes that, based on its current operating plan, its cash and cash equivalents as of December 31, 2019, and funding received from the issuance of Series B Bridge Units by Holdings in January and February 2020, will enable it to fund its operating expenses and capital expenditure requirements into March 2020. Accordingly, there is substantial doubt about the Company's ability to continue as a going concern as the Company does not believe that its cash, cash equivalents and investments will be sufficient to fund operations for at least twelve months from the date of issuance of these financial statements.

The Company expects to receive an additional $15.0 million of funding through the Second Series B Bridge Unit agreement entered into in January 2020, which will be used to fund operations until the Merger. However, receipt of the funds cannot be considered probable, as defined in accounting standards update ASU No. 2014-15 (subtopic 205-40), until the closing occurs and the funds are received. This funding is expected to occur as cash requirements are needed to fund operations and is expected to be able to allow the Company to fund operations into the second quarter of 2020 if it were received.

The Company is seeking to complete the Merger, which upon closing, would provide the Company minimum incremental net cash of $30.0 million. The Company can provide no assurances that the Merger will be consummated. In the event the Company does not complete the Merger, the Company expects to seek additional funding through private equity financings, debt financings, or other capital sources, which may include collaborations with other companies, government funding arrangements or other strategic transactions. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaborations or other arrangements. The terms of any financing may adversely affect the holdings or rights of the Company's stockholders.

If the Company is unable to obtain funding, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect its business prospects, or the Company may be unable to continue operations. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

Based on its recurring losses from operations incurred since inception, expectation of continuing losses for the foreseeable future and need to raise additional capital to finance its future operations, as of March 6, 2020, the issuance date of the consolidated financial statements for the year ended December 31, 2019, the Company has concluded that there is substantial doubt about its ability to continue as a going concern.

The accompanying consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

(3) Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expense and the fair value of equity instruments. Actual results could differ from those estimates.

Cash and Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of ninety days or less at acquisition date to be cash equivalents. Restricted cash equivalents represent amounts held as collateral pursuant to the Company's corporate credit card program. Cash and restricted cash equivalents are stated at fair value.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company has all cash and cash equivalents balances at one accredited financial institution in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply batches of CTI-1601 for research and development activities. The Company's development efforts could be adversely affected by a significant interruption in the supply of CTI-1601. The Company is also dependent on third-parties to conduct its clinical research programs. The Company's development efforts could be adversely affected if these clinical research organizations are unable to conduct the Company's clinical trials.

Fixed Assets, net

Fixed assets consist of furniture and fixtures, computers and laboratory equipment which are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over a five-year estimated useful life for both computer and laboratory equipment and a seven-year estimated useful life for furniture and fixtures. Expenditures for repairs and maintenance are expensed as incurred.

Impairment of Long Lived Assets

Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable.

If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses on long-lived assets.

Research and Development Costs

Research and development costs are expensed as incurred. Included in research and development expenses are wages, stock-based compensation and benefits of employees, third-party license fees and milestones and other operational costs related to the Company's research and development activities, including facility-related expenses and external costs of outside vendors engaged to conduct nonclinical studies, manufacturing activities, and clinical trials.

Acquired In-process Research and Development

In-process research and development ("IPR&D") purchased in asset acquisition transactions are expensed as research and development unless the assets acquired have an alternative future use. The IPR&D acquired in connection with the Transaction in 2016 (see Note 1) was expensed on the Transaction Date due to uncertain future economic benefit of the acquired research and development.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies both inside and outside of the United States. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates and these differences could be material.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are recorded as general and administrative expenses as incurred, as recoverability of such expenditures is uncertain.

Income Taxes

The Company accounts for income taxes using the asset and liability method, whereby deferred tax assets and liabilities are recognized for the expected future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax basis, operating loss and tax credit carryforwards. Deferred tax assets and liabilities are determined based on differences between financial statement carrying amounts of existing assets and their respective tax basis using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled.

The Company provides a valuation allowance on its net deferred tax assets to the extent that realization of such benefits is more likely than not to occur.

The accounting for uncertainty in income tax positions prescribes a recognition threshold and measurement of a tax position taken or expected to be taken in an income tax return. It also provides guidance in de-recognition, classification, interest and penalties, accounting in interim periods, disclosures and transition. The Company recognizes such tax benefits based upon the tax position being more-likely-than-not to be sustained upon examination by taxing authorities. There were no uncertain tax positions as of December 31, 2019 and 2018.

Accounting for Stock-Based Compensation

The Company measures all stock-based awards granted to employees and directors 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, the Company issues awards with only service-based conditions and records the expense for these awards using the straight-line method. The Company accounts for forfeitures as they occur.

The Company adopted ASU 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting*, or ASU 2018-07, effective January 1, 2017. The adoption of ASU 2018-07 did not have a material impact on the Company's financial position, results of operations or cash flows. After the adoption of ASU 2018-07, the measurement date for non-employee awards is the later of the adoption date of ASU 2018-07 or the date of grant, without recognition for changes in the fair value of the award. Stock-based compensation costs for non-employees are recognized as expense over the vesting period on a graded vesting basis.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company is a clinical stage biopharmaceutical company leveraging its proprietary knowledge to develop a therapeutic treatment for mitochondrial disorders. No revenue has been generated since inception, and all tangible assets are held in the United States.

Recently Issued and Adopted Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board (the "FASB") issued ASU 2016-02, *Leases* and in July 2018, the FASB issued ASU 2018-11, *Leases (Topic 842): Targeted Improvements*. The new leasing standards generally require lessees to recognize operating and financing lease liabilities and corresponding right-of-use assets on the consolidated balance sheet and to provide enhanced disclosures surrounding the amount, timing and uncertainty of cash flows arising from leasing arrangements.

The Company adopted the new leasing standards using the modified retrospective transition approach, as of January 1, 2019, with no restatement of prior periods or cumulative adjustment to retained earnings. Upon adoption, the Company elected the package of transition practical expedients, which allowed us to carry forward prior conclusions related to whether any expired or existing contracts are or contain leases, the lease classification for any expired or existing leases and initial direct costs for existing leases. In addition, the Company elected the hindsight practical expedient to determine the lease term for existing leases. The Company elected not to record leases with an initial term of 12 months or less on the balance sheet and recognize the associated lease payments in the consolidated statements of operations on a straight-line basis over the lease term. Rent expense associated with leases with an initial term of 12 months or less was $0.1 million for the year ended December 31, 2019. Upon adoption of the new leasing standards the Company recognized an operating lease asset of approximately $0.2 million and a corresponding operating lease liability of approximately $0.2 million. The adoption of the new leasing standards did not have an impact on the Company's consolidated statements of operations or cash flows.

The Company determines if an arrangement is a lease at contract inception. Operating lease assets represent the Company's right to use an underlying asset for the lease term and operating lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease assets and liabilities are recognized at the commencement date of the lease based upon the present value of lease payments over the lease term. When determining the lease term, the Company includes options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. The Company uses the implicit rate when readily determinable and uses the incremental borrowing rate when the implicit rate is not readily determinable based upon the information available at the commencement date in determining the present value of the lease payments. The Company's incremental borrowing rate was estimated based on the respective weighted average term of the agreements. As the Company does not have outstanding collateralized borrowings, the rate was determined using market comparisons.

The lease payments used to determine the Company's operating lease assets may include lease incentives, stated rent increases and escalation clauses linked to rates of inflation when determinable and are recognized in the Company's operating lease assets in our consolidated balance sheets.

The Company's operating leases are reflected in operating lease right-of-use assets and in current operating lease liabilities in the Company's consolidated balance sheets. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term.

For additional information on the adoption of the new leasing standards, see Note 11, Commitments, to these consolidated financial statements.

(4) Fair Value Measurements

The Company's assets and liabilities that are measured at fair value on a recurring basis as of December 31, 2019 and 2018 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 upon 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 cash is carried at fair value and is comprised of a checking account and money market account which totaled $1.0 million and $4.4 million at December 31, 2019 and 2018, respectively, and are classified as Level 1 investments.

The Company's financial instruments consist primarily of cash and cash equivalents, accounts payable and accrued liabilities. For accounts payable and accrued liabilities, the carrying amounts of these financial instruments as of December 31, 2019 and 2018 were considered representative of their fair values due to their short term to maturity.

(5) Prepaid Expenses and Other Assets

Prepaid expenses and other assets consisted of the following as of December 31, 2019 and 2018.

	December 31, 2019	2018
	(In thousands)	
Payroll tax receivable	$ 76	$ 17
Research and development tax credit sale receivable	82	—
Prepaid research and development expenses	3,099	416
Capitalized transaction costs	419	—
Other prepaid expenses and other assets	65	23
	$3,741	$456

Capitalized transaction costs as of December 31, 2019, consists of capitalized legal fees incurred by the Company during the year ended December 31, 2019, related to the Merger. These costs will be included in the purchase price allocation when accounting for the Merger. In the event the Merger Agreement is terminated, such costs will be expensed in the period in which such termination occurs.

(6) Fixed Assets, net

Fixed assets, net consisted of the following as of December 31, 2019 and 2018:

	Useful Life	December 31, 2019	2018
		(In thousands)	
Computer equipment	5 years	$ 14	$ 14
Lab equipment	5 years	389	356
Furniture and fixtures	7 years	50	—
		453	370
Less: Accumulated depreciation		(179)	(101)
		$ 274	$ 269

Depreciation expense for the years ended December 31, 2019 and 2018 was approximately $0.1 million and $0.1 million respectively.

(7) Accrued Expenses

Accrued expenses consisted of the following as of December 31, 2019 and 2018.

	December 31, 2019	2018
	(In thousands)	
Accrued expenses—research and development	$1,295	$ 807
Accrued expenses—professional services	337	22
Accrued bonuses	508	406
Accrued payroll and related expense	119	89
	$2,259	$1,324

(8) Stockholder's (Deficit) Equity

On November 22, 2016, Holdings purchased 100 shares of the Company's common stock, par value of $0.01 per share for proceeds of $1.00. The Company has 5,000 common shares authorized for issuance.

See Note 12 for related party transactions and capital contributions associated with Holdings funding of the Company.

On December 31, 2018 the Board of Managers approved the Contribution Agreement between Holdings, IP LLC and Chondrial Inc. Pursuant to this agreement, Holdings transferred and contributed 100 Series A-1 units of IP LLC, which constitutes all of the outstanding equity interests of IP LLC, to Chondrial Inc. for no consideration. This transaction was considered a transaction between entities under common control. The consolidation of Chondrial Inc. and its subsidiary (IP LLC) has been accounted for at historical cost and on the basis as if the aforementioned transaction had become effective on the Transaction Date, the date on which common control was established.

Restricted Common Units

In November 2016, Holdings granted 123,853 restricted Common Units to its Chief Scientific Officer with an aggregate grant date fair value of approximately $0.5 million. Thirty percent (30%) of the award vested upon issuance with the remaining seventy percent (70%) vesting ratably over the next 48 months as long as services were continued to be provided as stipulated in the consulting agreement. The Company has recognized

compensation expense on a graded vesting basis in research and development expense of approximately $0.1 million and $0.1 million in the years ended December 31, 2019 and 2018, respectively. The Company expects to recognize less than $0.1 million over the remaining eleven month vesting period. In accordance with Topic 718, *Compensation—Stock Compensation*, the Company has recorded costs incurred as stock-based compensation with a corresponding capital contribution from Holdings as such employees are working on behalf of the Company.

Common Unit Options

Under the 2016 Equity Incentive Plan adopted by Holdings on November 30, 2016 (the "2016 Equity Incentive Plan"), the Board of Managers or committee thereof was authorized to issue 122,133 Common Units or combination of Common Units, Common Unit options or profit interest units. On March 23, 2018, the Board of Managers increased the number of Common Units reserved for grant and issuance pursuant to the 2016 Equity Incentive Plan from 122,133 to 138,133 and on April 29, 2019 increased the number of Common Units reserved for grant and issuance pursuant to the 2016 Equity Incentive Plan by an additional 101,500 to 239,633.

During 2019 and 2018, Holdings issued 73,986 and 11,500 options, respectively, to purchase Common Units to certain employees of the Company. These options vest 25% on the first anniversary date of the grant date and the remaining 75% vest ratably over a 3 year period and are exercisable over a 10 year period at a weighted average exercise price of $11.00 and $10.00 per Common Unit option, respectively.

A summary of the Company's Common Unit option activity in Holdings is as follows:

	December 31, 2019		December 31, 2018	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Outstanding, beginning of year	129,606	$ 10.00	118,106	$ 10.00
Granted	73,986	11.00	11,500	10.00
Exercised	—	—	—	—
Forfeited	(1,200)	10.00	—	—
Expired	—	—	—	—
Outstanding, end of year	202,392	$ 10.36	129,606	$ 10.00
Exercisable, end of year	95,684		63,632	
Weighted average grant date fair value	$ 0.55		$ 1.60	

The following table summarizes information about Common Unit options exercisable, and vested and expected to vest as of December 31, 2019:

	Units	Weighted Average Exercise Price	Aggregate Intrinsic Value	Weighted Average Remaining Contractual Term (in years)
Vested and expected to vest	104,937	$ 10.03	$ —	7.5
Exercisable	95,684	$ 10.00	$ —	7.3

The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the Common Unit for the options as of December 31, 2019. The Common Unit options had no intrinsic value as of December 31, 2019, as the exercise price of such Common Unit options exceeded the fair value of a Common Unit on such date.

The Company has recorded costs incurred as stock-based compensation with a corresponding capital contribution from Holdings. Total share-based expense associated with Common Unit options and restricted Common Units was reflected in the Statement of Operations as follows:

	December 31, 2019	December 31, 2018
	(In thousands)	
Research and development	$ 63	$ 111
General and administrative	66	62
	$ 129	$ 173

The fair value of each Common Unit option granted is estimated on the grant date using the Black-Scholes stock option pricing model. The following assumptions were made in estimating fair value:

Assumption	December 31, 2019	December 31, 2018
Dividend yield	0.00%	0.00%
Expected term	6.25 years	6.25 years
Risk-free interest rate	1.60-2.00%	1.80%
Expected volatility	77%	80%
Fair Value of common unit	$0.98 - $1.75	$ 3.17

The dividend yield is based upon the assumption that Holdings will not declare a dividend over the life of the options. The Company is unable to use historical employee exercise and option expiration data to estimate the expected term assumption and has therefore utilized the “simplified” method, as prescribed by the SEC’s Staff Accounting Bulletin No. 107, *Share-Based Payment*, as such options are considered to have “plain vanilla” characteristics. The risk-free interest rate is based on valuations used to derive the unit price. The expected volatility was based on the historical volatility of peer company data.

In the absence of a public trading market, the Company determined a reasonable estimate of the then-current fair value of Holding’s Common Units for purposes of granting equity-based compensation. The Company determined the fair value of Holding’s Common Units utilizing methodologies, approaches and assumptions from a third-party valuation firm. In addition, the Company exercised judgment in evaluating and assessing the foregoing based on several factors including:

- the nature and history of the Company’s business;
- the market value of companies that are engaged in a similar business to the Company;
- the lack of marketability of the Company’s Common Units;
- the price at which shares of the Company’s other equity instruments have been sold;
- the overall inherent risks associated with the Company’s business at the time common unit option grants were approved; and
- the overall equity market conditions and general economic trends.

Unrecognized compensation expense related to non-vested employee Common Unit options amounted to approximately $0.1 million as of December 31, 2019. Such compensation expense is expected to be recognized over a weighted-average period of 1.8 years.

(9) Income Taxes

During the years ended December 31, 2019 and 2018, the Company recorded no income tax benefits for the net operating losses incurred in each year due to its uncertainty of realizing a benefit from those items.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	2019	2018
Federal statutory income tax rate	21.0%	21.0%
State taxes, net of federal benefit	7.9%	7.9%
Change in deferred tax asset valuation allowance	(29.0)%	(29.1)%
Other	0.1%	0.2%
Effective income tax rate	0.0%	0.0%

Net deferred tax assets as of December 31, 2019 and 2018 consisted of the following:

	2019	2018
	(In thousands)	
Noncurrent deferred tax assets:		
Net operating loss carryforwards	$ 10,983	$ 4,702
Research and development tax credit carryforwards	62	39
Other temporary differences	690	559
Total noncurrent deferred tax assets	11,735	5,300
Total gross deferred tax assets	11,735	5,300
Valuation allowance	(11,735)	(5,300)
Net deferred tax assets	$ —	$ —

As of December 31, 2019, the Company had net operating loss carryforwards for federal and state income tax purposes of $38.0 million and $38.0 million, respectively, which begin to expire in 2036 and 2036, respectively. The losses arising in taxable years beginning after December 31, 2017 do not expire, but the allowable federal net operating loss deduction in a particular tax period is limited to 80% of federal taxable income. The Company has $33.2 million of federal net operating loss carryforward subject to this limitation. As of December 31, 2019, the Company also had available tax credit carryforwards for state income tax purposes of approximately $0.1 million which begin to expire in 2027. Utilization of the net operating loss carryforwards and tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income.

In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards or tax credit carryforwards would be subject to an annual limitation under Section 382, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or tax credit carryforwards before utilization. Further, until a study is completed and any limitation is known, no amounts are being presented as an uncertain tax position.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and its lack of commercialization of any products or generation of any revenue from product sales since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2019 and 2018. Management reevaluates the positive and negative evidence at each reporting period.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2019 or 2018.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's tax years are still open under statute from 2016 to the present. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

(10) Retirement Plan

Effective May 1, 2017, the Company became a party to the TriNet 401(k) Plan, a multiple employer 401(k) plan offered through the Company's third-party payroll and benefits provider. Under the terms of the plan, eligible employees may choose to contribute between 1% and 100% of their total pay subject to Internal Revenue Service limitations. The TriNet 401(k) Plan is a safe harbor plan whereby the Company matches 100% of employee contributions up to the first 4% of employee pay contributed to the plan via salary deferral and all such company matching contributions are immediately vested.

Effective December 31, 2018, the Company terminated is participation in the TriNet 401(k) Plan and on January 1, 2019, the Company adopted the Chondrial Therapeutics, Inc. 401(k) Plan (the "Chondrial 401(k)"). Under the terms of the plan, eligible employees of the Company may choose to contribute between 1% and 100% of their total pay subject to Internal Revenue Service limitations. The Chondrial 401(k) is a safe harbor plan whereby the Company matches 100% of employee contributions up to the first 4% of employee pay contributed to the plan via salary deferral and all such company matching contributions are immediately vested.

For the years ended December 31, 2019 and 2018, the Company recognized approximately $0.1 million and $0.1 million of expense related to its contributions.

(11) Commitments

Intellectual Property Licenses

Old Chondrial entered into an Option Agreement dated February 14, 2014 with Wake Forest University Health Sciences ("WFUHS") and Indiana University Research and Technology Corporation ("IURTC") which provided a non-transferable, worldwide exclusive option (the "Option") to license certain patent rights regarding technology for the use in the treatment of Friedreich's Ataxia. The Option could be exercised during a period extending 18 months from February 14, 2014. As consideration for the rights granted under the Option Agreement, Holdings agreed that upon exercise of the Option, it would grant WFUHS 4.0% and IURTC 1.0% of the equity of Holdings on a fully diluted basis.

On July 30, 2015, Old Chondrial informed WFUHS and IURTC of its intent to exercise the Option and on November 30, 2016, the Company entered into separate License Agreements with both WFUHS and IURTC. Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology for the use in the diagnosis, treatment, or prevention of mitochondrial diseases, including without limitation Friedreich's Ataxia (pursuant to the IURTC license) and for the use in the diagnosis, treatment or prevention of

any disease that benefits from the treatment with TAT-Frataxin, including without limitation Friedreich's Ataxia (pursuant to the WFUHS license) for the respective patent periods (together "Licensed Product"). In addition, the agreements provide full rights to sublicense through multiple tiers of sub licensees any and all such rights. Pursuant to the terms of the Option Agreement, upon exercise of the Option and the entering into formal License Agreements, Holdings issued 14,622 Common Units to WFUHS and 3,647 Common Units to IURTC with such amount being recorded as research and development expense in the period ended December 31, 2016.

In partial consideration for the right and license granted under these agreements, the Company will pay each of WFUHS and IURTC a royalty of a low single digit percentage of net sales of the Licensed Products depending on whether there is a valid patent covering the Licensed Products. As additional consideration for these agreements, the Company is obligated to pay each of WFUHS and IURTC certain milestone payments of up to $2.2 million in the aggregate upon the achievement of certain developmental milestones.

In the event that the Company is required to pay IURTC consideration, then the Company may deduct 20% of such IURTC 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 IURTC, as amended on August 16, 2019, as described below.

In December 2019, the Company recognized milestone expenses of twenty-eight thousand dollars, after taking into account the potential deductions indicated above, which is included in research and development expense in the accompany consolidated Statement of Operations for the year ended December 31, 2019. None of the milestones were achieved in 2018 or prior years and therefore no such expense was recognized in 2018.

The Company is required to utilize commercially reasonable efforts to bring the Licensed Products to market through the exploitation of the licensed patents and commercialization of the Licensed Products. Additionally, the Company is required to have at least two full-time equivalent employees working on the development, manufacturing and marketing of the Licensed Products within the 12 month period following the effective date and each subsequent year thereafter. The Company is also required to enroll the first patient in the first Phase I (or its non-U.S. equivalent) clinical trial of Licensed Product within 30 months of the effective date and to enroll the first patient in the first Phase II (or its non-US equivalent) clinical trial of a Licensed Product within 60 months of the effective date as amended on August 16, 2019, as described below. Pursuant to amendments to extend these dates, the Company believes it is in compliance with each of the above noted requirements as of December 31, 2019.

The WFUHS License Agreement was terminable in the event the Company failed to raise $1.0 million by December 31, 2016, $2.0 million by December 31, 2017 and $4.0 million by December 31, 2018. The Company successfully raised the required proceeds in the respective periods.

In addition, under the IURTC License Agreement, the Company has a non-transferable, exclusive option to negotiate in good faith for a royalty-bearing, worldwide, exclusive license (including the right to sublicense) to certain new inventions that may be developed by Indiana University pursuant to a sponsored research agreement between Indiana University and the Company for research conducted in an Indiana Laboratory for a period of six months from IURTC disclosure of such invention to the Company.

Both agreements continue from their effective date through the last to expire of the licensed patents unless earlier terminated. Either party may terminate upon 60 days written notice if there is a material breach of the terms of the license that has not been cured within such 60 day period. IURTC and WFUHS may terminate the license if at any time the Company ceases to carry required Director's and Officer's insurance coverage, ceases to have at least one employee or consultant who is devoting at least 20 hours a week to the affairs of the Company or in the event the Company files for bankruptcy or is insolvent. The Company may terminate the licenses with or without cause on 30 day's written notice to WFUHS and 60 day's written notice to IURTC.

The Company may assign the WFUHS license to an affiliate or in conjunction with the sale of the business, but may not assign the licenses to another third-party without prior written consent. The Company may assign the IURTC license to an affiliate or a third party as long as the Company is not in breach of the license at the time of assignment, the successor is not materially insolvent and the successor agrees in writing to assume all obligations and liabilities of the Company to IURTC.

On August 16, 2019, the Company entered into a First Amendment of its License Agreement with IURTC. The First Amendment transfers all rights and obligations under the License Agreement to the Trustees of Indiana University ("IU") and releases IURTC from all liability and obligations under the License Agreement which arose before or after the First Amendment effective date. The First Amendment also expanded the definition of Licensed Patents and expanded the option to license additional technologies developed by IU. The date by which the Company was required to enroll the first patient in a Phase I clinical trial of licensed product was extended to June 30, 2020 and the date by which the Company is required to enroll the first patient in a Phase II clinical trial of licensed product was extended to June 30, 2022. Additionally, it added milestone payments aggregating up to less than $0.1 million upon the issuance of a U.S. Patent and up to less than $0.1 million upon the issuance of certain ex-US patents. It reduced the amount the Company may deduct from consideration payable to IU for payments made to WFUHS from 80% to 60%. The Company also agreed to pay to IU a minimum annual royalty of five-thousand dollars per annum starting the 2020 calendar year for the term of the agreement. In consideration for the First Amendment the Company paid IU a ten-thousand dollar fee which is included in research and development expense and in the accompanying Statement of Operations for the year ended December 31, 2019.

Leases

On November 30, 2016, the Company entered into an operating lease for office and lab space in Philadelphia, Pennsylvania, effective as of December 1, 2016 and expiring on November 30, 2017, subject to thirty day extensions. The lease terminated in January 2019.

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 in December 31, 2020 with an option to extend the lease for two additional years.

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 possession of the leased property and the lease term did not commence until February 15, 2020.

Expense arising from operating leases was $0.1 million and $0.1 million and for the year ended December 31, 2019 and 2018 respectively. The Company made $0.1 million of lease payments for the period ended December 31, 2019. The weighted-average remaining lease term and the weighted average discount rate for leases accounted for in accordance with ASU 2018-11 at December 31, 2019 was 1 year and 12.0%, respectively. The Company has not entered into any financing leases.

Future minimum lease payments due under operating lease agreements as of December 31, 2018 were as follows:

Year Ended December 31,	(In thousands)
2019	$ 81
2020	100
2021	—
	$ 181

Maturities of lease liabilities due under these lease agreements as of December 31, 2019 are as follows:

Year Ended December 31,	(In thousands)
2020	$ 100
2021	—
2022	—
Total lease payments	$ 100
Less imputed interest	(3)
Present value of lease liabilities	$ 97

Employment Agreements

On December 31, 2016, the Company entered into an employment agreement with its CEO for a term of four years, a salary of $0.4 million, an annual bonus of up to 40% of her base pay and severance equal to 12 months of base salary.

(12) Related Party Transactions

In November 2016, the Company entered into a consulting agreement with R. Mark Payne, M.D. to serve in the capacity of Chief Scientific Officer. Dr. Payne is a director of the Company, 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 IURTC License. Pursuant to the terms of his consulting agreement the Company agreed to pay Dr. Payne ten-thousand dollars per month over the term of the agreement and granted Dr. Payne 123,853 restricted Common Units in Holdings of which 30% was associated with the Transaction and expensed as research and development in 2016 and the remaining 70% associated with future services (See Note 8) vesting ratably over the next 48 months. The consulting agreement has a four year term, subject to earlier termination. For each of the years ended December 31, 2019 and 2018, the Company recognized $0.1 million of expense related to this consulting agreement, recorded as research and development expense in the Statement of Operations.

The funding to the Company originated from Holdings sale of Series A Preferred Units and Series B Bridge Units to the Deerfield Funds, and certain other purchasers, from inception through December 31, 2019 and the contribution of the proceeds received by Holdings on such sales to the Company in order to fund the Company’s operations.

Under a November 30, 2016 Series A Preferred Unit Purchase Agreement, as amended on September 8, 2017, November 15, 2017, November 14, 2018 and April 29, 2019, Holdings sold an aggregate of 1,780,000 Series A Preferred Units at a purchase price of $20.00 per unit for gross proceeds of $35.6 million. Of the aggregate Series A Preferred Units sold, 604,333 Series A Preferred Units were sold during the year-ended December 31, 2018 for aggregate gross proceeds of $12.1 million and 799,779 Series A Preferred Units were sold during the year-ended December 31, 2019 for aggregate gross proceeds of $16.0 million.

On November 21, 2019 (as amended on December 20, 2019), Holdings entered into a Second Amended and Restated LLC Agreement and entered into a Series B Bridge Unit Purchase Agreement with the Deerfield Funds and certain other purchasers to sell up to 2,004 Series B convertible preferred units (“Series B Bridge Units”) at a purchase price of $5,000.00 per unit for gross proceeds of up to $10.0 million. On November 21, 2019, Holdings sold 681 Series B Bridge Units and on December 20, 2019 Holdings sold an additional 700 Series B Bridge Units for aggregate gross proceeds of approximately $6.9 million.

During the years ended December 31, 2019 and 2018, Holdings provided the Company non-interest bearing, permanent funding from the above Series A and Series B preferred unit transactions, totaling $19.4 million and $12.1 million, respectively, which has been recorded as capital contributions with the balance of combined equity and additional paid in capital on the Consolidated Balance Sheets and Consolidated Statements of Changes in Stockholders' equity for each respective period.

(13) Subsequent Events

For its consolidated financial statements as of December 31, 2019 and for the year then ended, the Company evaluated subsequent events through March 6, 2020, the date on which those financial statements were issued.

On January 15, 2020, Holdings sold the final 621 Series B Bridge Units available for sale under the Series B Bridge Unit Purchase Agreement for gross proceeds of approximately $3.1 million and contributed such amount to the Company.

On January 16, 2020, Holdings entered into a Third Amended and Restated LLC Agreement and entered into a Second Series B Bridge Unit Purchase Agreement with the Deerfield Funds and certain other purchasers to sell up to 3,000 Second Series B Bridge convertible preferred units ("Second Series B Bridge Units") at a purchase price of $5,000.00 per unit, for gross proceeds of up to $15.0 million. Amounts raised by Holdings pursuant to the sale of Series B Bridge Units will be utilized to fund the Company. On February 26, 2020, Holdings sold 600 Second Series B Bridge Units for gross proceeds of approximately $3.0 million and contributed such amount to the Company.

Exhibit 99.4

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

THREE MONTHS ENDED MARCH 31, 2020 AND 2019

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

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CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

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

	March 31, 2020	December 31, 2019
ASSETS		
Current assets		
Cash	$ 887	$ 1,009
Prepaid expenses and other current assets	5,116	3,741
Total current assets	6,003	4,750
Fixed assets, net	309	274
Other assets	147	90
Operating lease right-of-use assets	533	87
Total assets	$ 6,992	$ 5,201
LIABILITIES AND STOCKHOLDER'S EQUITY (DEFICIT)		
Current liabilities		
Accounts payable	$ 1,659	$ 3,539
Accrued expenses	2,527	2,259
Operating lease liability, current	209	97
Total current liabilities	4,395	5,895
Operating lease liability, noncurrent	341	—
Total liabilities	4,736	5,895
Commitments (See Note 10)		
Stockholder's equity (deficit)		
Common stock, 5,000 shares authorized;100 shares issued and outstanding, par value $0.01	1	1
Additional paid-in capital	32,061	22,437
Accumulated deficit	(29,806)	(23,132)
Total stockholder's equity (deficit)	2,256	(694)
Total liabilities and stockholder's equity	$ 6,992	$ 5,201

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

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

FOR THE THREE MONTHS ENDED MARCH 31, 2020 AND 2019
(In thousands)
(Unaudited)

	March 31, 2020	March 31, 2019
Revenue	$ —	$ —
Operating expenses:		
Research and development	5,007	4,222
General and administrative	1,667	502
Total operating expenses	6,674	4,724
Loss from operations	(6,674)	(4,724)
Other income	—	—
Net Loss	$ (6,674)	$ (4,724)
Total Comprehensive Loss	$ (6,674)	$ (4,724)

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDER'S EQUITY (DEFICIT)

THREE MONTHS ENDED MARCH 31, 2020 AND 2019
(In thousands, except share data)
(Unaudited)

	Common		Additional paid in capital	Accumulated deficit	Total
	Shares	Amount			
Balance as of December 31, 2018	100	$ 1	$ 2,913	$ —	$ 2,914
Stock-based compensation expense	—	—	34	—	34
Capital contributions from related party (See Note 11)	—	—	3,000	—	3,000
Net loss	—	—	—	(4,724)	(4,724)
Balance as of March 31, 2019	100	1	5,947	(4,724)	1,224
Balance as of December 31, 2019	100	1	22,437	(23,132)	(694)
Stock-based compensation expense	—	—	29	—	29
Capital contributions from related party (See Note 11)	—	—	9,595	—	9,595
Net loss	—	—	—	(6,674)	(6,674)
Balance as of March 31, 2020	100	$ 1	$ 32,061	$ (29,806)	$ 2,256

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

CHONDRIAL THERAPEUTICS INC. AND SUBSIDIARY

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

THREE MONTHS ENDED MARCH 31, 2020 AND 2019
(In thousands)
(Unaudited)

	March 31, 2020	March 31, 2019
Cash flows used in operating activities		
Net loss	$ (6,674)	$ (4,724)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	29	34
Depreciation	23	19
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(11)	(837)
Accounts payable	(2,524)	23
Accrued expenses	230	(353)
Right-of-use assets	9	18
Operating lease liabilities	(2)	(4)
Other assets	(1)	9
Net cash used in operating activities	(8,921)	(5,815)
Cash flows used in investing activities		
Purchase of equipment	(58)	(15)
Merger transaction costs	(720)	—
Net cash used in investing activities	(778)	(15)
Cash flows provided by financing activities		
Capital contribution, related party—Series A Preferred Unit proceeds	—	3,000
Capital contributions, related party—Series B Bridge Unit proceeds	6,595	—
Capital contributions, related party—Second Series B Bridge Unit proceeds	3,000	—
Offering costs	(18)	—
Net cash provided by financing activities	9,577	3,000
Net decrease in cash and restricted cash equivalents	(122)	(2,830)
Cash and restricted cash equivalents, beginning of period	1,009	4,396
Cash and restricted cash equivalents, end of period	$ 887	$ 1,566
Supplemental disclosure of non-cash investing and financing activities:		
Offering costs included in accounts payable and accrued expenses	$ 38	$ —
Merger transaction costs included in accounts payable and accrued expenses	$ 644	$ —
Leased assets obtained in exchange for new operating lease liabilities	$ 448	$ —
Payments for leasehold improvements on right-of-use assets included in operating lease liability, current	$ 7	$ —

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

(1) Organization, Nature of the Business and Basis of Presentation

Chondrial Therapeutics, Inc. (“Chondrial Inc.”), a Delaware corporation, was formed on November 22, 2016, as a wholly owned subsidiary of Chondrial Therapeutics Holdings, LLC (“Holdings”). Holdings was also formed on November 22, 2016, in the state of Delaware as a limited liability company (“LLC”). Chondrial Therapeutics, LLC (“Old Chondrial”), an Indiana LLC, was formed on September 4, 2013.

On November 30, 2016 (the “Transaction Date”), Old Chondrial filed a certificate of conversion in the state of Delaware, pursuant to which it changed its name to Chondrial Therapeutics IP, LLC (“IP LLC”), a Delaware LLC, and became another wholly owned subsidiary of Holdings. On the Transaction Date, the members of Old Chondrial contributed their member units to Holdings in exchange for Common Units in Holdings (the “Transaction”). As of the Transaction Date, Old Chondrial had limited assets, primarily consisting of an option agreement to license technology (see Note 10) from two institutions for use in the treatment of a mitochondrial disorder. IP LLC holds the Company’s material patents and intellectual property license agreements. On December 31, 2018, the membership units of IP LLC were contributed by Holdings to Chondrial Inc. and IP LLC became a wholly-owned subsidiary of Chondrial Inc. (collectively, “the Company” or “Chondrial”).

The Company is a clinical stage biopharmaceutical company leveraging its proprietary knowledge to develop a therapeutic treatment for mitochondrial disorders which currently have no cure. The Company has focused on Friedreich’s Ataxia, which is a progressive disease that affects multiple body systems, particularly the brain and heart. CTI-1601, the Company’s lead product candidate, utilizes a cell penetrant peptide to deliver frataxin, the protein deficient in Friedreich’s Ataxia, to the mitochondria where it is believed to be processed into mature frataxin and becomes active in mitochondrial metabolism. In July 2017, the Company received orphan drug designation for CTI-1601 from the Food and Drug Administration (“FDA”). This makes CTI-1601 eligible for orphan product exclusivity lasting seven years starting at FDA approval.

On October 25, 2019, the FDA informed the Company that it may proceed with its clinical investigation for the treatment of Friedreich’s Ataxia with CTI-1601 and on November 20, 2019, the Company was granted Fast Track Designation for CTI-1601. Fast Track Designation is designed to facilitate the development of, and expedite the review of, drugs to treat serious conditions and fill unmet medical needs, the purpose being to make important new drugs available to patients earlier. On December 5, 2019, the FDA granted the Company designation for CTI-1601 as a drug for a rare pediatric disease, and that same month, the Company dosed its first human patient in its Phase 1 clinical trial (see Note 2 Other Risks and Uncertainties) ..

On December 17, 2019 the Company entered into an Agreement and Plan of Merger (“Merger Agreement”) with Zordich Merger Sub, Inc., (“Merger Sub”) a wholly owned subsidiary of Zafgen, Inc. (“Zafgen”), a publicly traded company on the NASDAQ exchange. Pursuant to the Merger Agreement, the Company will be merged with and into Merger Sub at the effective time of the merger, with the Company continuing after the merger as the surviving company (the “Merger”). Holdings, in its capacity as the sole stockholder of Chondrial Inc., has approved the Merger Agreement by written consent. The Zafgen board of directors unanimously approved the Merger Agreement and the related transactions and the requisite stockholder approval was obtained at the May 28, 2020 Zafgen shareholders meeting. Accordingly, the Merger closed on May 28, 2020 and the combined companies became Larimar Therapeutics, Inc. The Company’s shareholders owned approximately 66% of the outstanding common stock following the Merger (see Note 12).

Basis of Presentation

The condensed consolidated financial statements include the accounts of Chondrial Inc. and its wholly owned subsidiary, IP LLC. All intercompany balances and transactions have been eliminated. The accompanying condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

Unaudited Interim Financial Information

The condensed consolidated balance sheet as of December 31, 2019 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 March 31, 2020 and for the three months ended March 31, 2020 and 2019, 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, 2019. 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 March 31, 2020 and condensed consolidated results of operations and cash flows for the three months ended March 31, 2020 and 2019 have been made. The results of operations for the three months ended March 31, 2020 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2020.

(2) Liquidity and Other Risks and Uncertainties

Liquidity

The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the condensed consolidated financial statements are issued.

From its inception through March 31, 2020, the Company has received funding from Holdings which originated from Holdings sale of Series A Preferred Units and Series B convertible preferred units to Deerfield Private Design Fund IV, L.P., Deerfield Private Design Fund III, L.P., Deerfield Health Innovations Fund, L.P. (together the "Deerfield Funds") and certain other purchasers. The Company has incurred recurring losses since its inception, including net losses of $6.7 million and $4.7 million during the three months ended March 31, 2020 and 2019, respectively. In addition, as of March 31, 2020, the Company had an accumulated deficit of $29.8 million.

The Company expects to continue to generate operating losses for the foreseeable future. The additional funding through Holding's Second Series B Bridge Unit Purchase Agreement entered into in January 2020 (see Note 11) funded operations until the completion of the Merger. The Merger was completed on May 28, 2020 which, upon closing, provided incremental net cash of approximately $40.0 million concurrent with a private placement which provided additional net proceeds of $75.5 million (see Notes 1 and 12). The Company believes that, based on its current operating plan, its cash, cash equivalents and marketable securities as of the filing date will enable it to fund operations for at least twelve months from the issuance of these interim financial statements.

Until such time, if ever, as the Company can generate substantial revenue, the Company expects to seek additional funding through equity financings, debt financings, or other capital sources, which may include collaborations with other companies, government funding arrangements or other strategic transactions. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaborations or other arrangements. The terms of any financing may adversely affect the holdings or rights of the Company's stockholders. If the Company is unable to obtain additional funding, the Company will be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect its business, or the Company may be unable to continue operations.

Other Risks and Uncertainties

The Company is currently evaluating CTI-1601 in a single ascending dose (referred to as "SAD") Phase 1 clinical trial in patients with Friedreich's Ataxia. The first two cohorts of patients have completed the SAD clinical trial; however, due to the continued impact of coronavirus (referred to as "COVID-19"), the Company has delayed initiation of the next cohort in the SAD clinical trial. The Company is conducting the clinical trial at one clinical trial site. Because Friedreich's Ataxia is a rare disease, there are a limited number of patients in close proximity to the clinical trial site and clinical trial patients travel from throughout the United States to the clinical trial site to participate. The travel advisories and risk of infection related to COVID-19 have presented increased risks to patients traveling to the Company's clinical trial site for dosing. Due to the uncertainty surrounding COVID-19, the Company cannot estimate when the next cohort of patients will begin the clinical trial. While top line results from the SAD and the planned multiple ascending dose (referred to as "MAD") clinical trials were originally expected by the end of 2020, the delay in the clinical trial timeline caused by the ongoing impact of COVID-19 resulted in top line results being expected in the first half of 2021.

(3) Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expense and valuation of stock-based awards. Actual results could differ from those estimates.

Recently Issued and Adopted Accounting Pronouncements

In June 2016 the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*. The FASB subsequently issued amendments to ASU 2016-13. This standard requires entities to estimate an expected lifetime credit loss on financial assets ranging from short-term trade accounts receivable to long-term financings and report credit losses using an expected losses model rather than the incurred losses model that was previously used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, the standard now requires allowances to be recorded instead of reducing the amortized cost of

the investment. This standard limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and requires the reversal of previously recognized credit losses if fair value increases. The Company adopted the standard on January 1, 2020. The adoption of this standard did not have a material impact on the Company's condensed consolidated financial statements and related disclosures.

In August 2018 the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*. This standard modifies certain disclosure requirements on fair value measurements. This standard became effective for the Company on January 1, 2020. The adoption of this standard did not have a material impact on the Company's disclosures.

(4) Fair Value Measurements

The Company's assets and liabilities that are measured at fair value on a recurring basis as of March 31, 2020 and December 31, 2019 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 upon 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 cash is carried at fair value and is comprised of a checking account and money market account which totaled $0.9 million and $1.0 million at March 31, 2020 and December 31, 2019, respectively, and are classified as Level 1 investments.

The Company's financial instruments consist primarily of cash and cash equivalents, accounts payable and accrued liabilities. For accounts payable and accrued liabilities, the carrying amounts of these financial instruments as of March 31, 2020 and December 31, 2019 were considered representative of their fair values due to their short term to maturity.

(5) Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	March 31, 2020	December 31, 2019
	(In thousands)	
Prepaid research and development expenses	$ 3,573	$ 3,099
Capitalized transaction costs	1,364	419
Other prepaid expenses and other current assets	93	65
Research and development tax credit sale receivable	—	82
Payroll tax receivable	86	76
	$ 5,116	$ 3,741

Capitalized transaction costs as of March 31, 2020 and December 31, 2019, consists of capitalized legal and proxy fees incurred by the Company, related to the Merger. These costs will be included in the purchase price allocation when accounting for the Merger. In the event the Merger Agreement is terminated, such costs will be expensed in the period in which such termination occurs.

(6) Fixed Assets, net

Fixed assets, net consisted of the following:

	Useful Life	March 31, 2020	December 31, 2019
		(In thousands)	
Computer equipment	5 years	$ 14	$ 14
Lab equipment	5 years	389	389
Furniture and fixtures	7 years	108	50
		511	453
Less: Accumulated depreciation		(202)	(179)
		$ 309	$ 274

Depreciation expense during the three months ended March 31, 2020 and 2019 were less than $0.1 million.

(7) Accrued Expenses

Accrued expenses consisted of the following:

	March 31, 2020	December 31, 2019
	(In thousands)	
Accrued expenses – research and development	$ 830	$ 1,295
Accrued expenses – professional services	1,437	337
Accrued bonuses	164	508
Accrued payroll and related expense	96	119
	$ 2,527	$ 2,259

(8) Stockholders' Equity

On November 22, 2016, Holdings purchased 100 shares of the Company's common stock, par value of $0.01 per share for proceeds of $1.00. The Company has 5,000 common shares authorized for issuance.

See Note 11 for related party transactions and capital contributions associated with Holdings funding of the Company.

Restricted Common Units

In November 2016, Holdings granted 123,853 restricted Common Units to its Chief Scientific Officer with an aggregate grant date fair value of approximately $0.5 million. Thirty percent (30%) of the award vested upon issuance with the remaining seventy percent (70%) vesting ratably over the next 48 months as long as services were continued to be provided as stipulated in the consulting agreement. The Company has recognized compensation expense of less than $0.1 million on a graded vesting basis in research and development expense during each of the three months ended March 31, 2020 and March 31, 2019. As of March 31, 2020, the Company expects to recognize less than $0.1 million over the remaining eight month vesting period. In accordance with Topic 718, *Compensation—Stock Compensation*, the Company has recorded costs incurred as stock-based compensation with a corresponding capital contribution from Holdings as such employees are working on behalf of the Company.

Common Unit Options

Under the 2016 Equity Incentive Plan adopted by Holdings on November 30, 2016 (the "2016 Equity Incentive Plan"), the Board of Managers or committee thereof was authorized to issue 122,133 Common Units or combination of Common Units, Common Unit options or profit interest units. On March 23, 2018, the Board of Managers increased the number of Common Units reserved for grant and issuance pursuant to the 2016 Equity Incentive Plan from 122,133 to 138,133 and on April 29, 2019 increased the number of Common Units reserved for grant and issuance pursuant to the 2016 Equity Incentive Plan by an additional 101,500 to 239,633.

During the three months ended March 31, 2020 and 2019, Holdings did not issue options to purchase Common Units to employees of the Company.

The Company has recorded costs incurred as stock-based compensation with a corresponding capital contribution from Holdings. Total stock-based expense associated with Common Unit options and restricted Common Units was reflected in the Statement of Operations and consisted of the follow:

	March 31, 2020	March 31, 2019
	(In thousands)	
Research and development	$ 12	$ 19
General and administrative	17	15
	$ 29	$ 34

Unrecognized compensation expense related to non-vested employee Common Unit options was less than $0.1 million as of March 31, 2020. Such compensation expense is expected to be recognized over a weighted-average period of 1.5 years.

(9) Retirement Plan

Effective January 1, 2019, the Company adopted the Chondrial Therapeutics, Inc. 401(k) Plan (the “Chondrial 401(k)”). The Chondrial 401(k) is a safe harbor plan whereby the Company matches 100% of employee contributions up to the first 4% of employee pay contributed to the plan via salary deferral and all such company matching contributions are immediately vested.

During the three months ended March 31, 2020 and 2019, the Company recognized less than $0.1 million of expense related to its contributions.

(10) Commitments

Intellectual Property Licenses

Old Chondrial entered into an Option Agreement dated February 14, 2014 with Wake Forest University Health Sciences (“WFUHS”) and Indiana University Research and Technology Corporation (“IURTC”) which provided a non-transferable, worldwide exclusive option (the “Option”) to license certain patent rights regarding technology for the use in the treatment of Friedreich’s Ataxia. The Option could be exercised during a period extending 18 months from February 14, 2014. As consideration for the rights granted under the Option Agreement, Holdings agreed that upon exercise of the Option, it would grant WFUHS 4.0% and IURTC 1.0% of the equity of Holdings on a fully diluted basis.

On July 30, 2015, Old Chondrial informed WFUHS and IURTC of its intent to exercise the Option and on November 30, 2016, the Company entered into separate License Agreements with both WFUHS and IURTC. Such agreements provide for a transferable, worldwide license to certain patent rights regarding technology for the use in the diagnosis, treatment, or prevention of mitochondrial diseases, including without limitation Friedreich’s Ataxia (pursuant to the IURTC license) and for the use in the diagnosis, treatment or prevention of any disease that benefits from the treatment with TAT-Frataxin, including without limitation Friedreich’s Ataxia (pursuant to the WFUHS license) for the respective patent periods (together “Licensed Product”). In addition, the agreements provide full rights to sublicense through multiple tiers of sub licensees any and all such rights.

Pursuant to the terms of the Option Agreement, upon exercise of the Option and the entering into formal License Agreements, Holdings issued 14,622 Common Units to WFUHS and 3,647 Common Units to IURTC. In partial consideration for the right and license granted under these agreements, the

Company will pay each of WFUHS and IURTC a royalty of a low single digit percentage of net sales of the Licensed Products depending on whether there is a valid patent covering the Licensed Products. As additional consideration for these agreements, the Company is obligated to pay each of WFUHS and IURTC certain milestone payments of up to $2.2 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 will also pay each of WFUHS and IURTC 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 IURTC 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.

In the event that the Company is required to pay IURTC consideration, then the Company may deduct 20% of such IURTC 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 IURTC, as amended on August 16, 2019, as described below.

In December 2019, the Company recognized milestone expenses of less than $0.1 million, after taking into account the potential deductions indicated above. During the three months ended March 31, 2020 and 2019, no milestones were achieved and no expense was recognized. The Company is required to utilize commercially reasonable efforts to bring the Licensed Products to market through the exploitation of the licensed patents and commercialization of the Licensed Products. Additionally, the Company is required to have at least two full-time equivalent employees working on the development, manufacturing and marketing of the Licensed Products within the 12-month period following the effective date and each subsequent year thereafter. The Company is also required to enroll the first patient in the first Phase I (or its non-U.S. equivalent) clinical trial of Licensed Product within 30 months of the effective date and to enroll the first patient in the first Phase II (or its non-US equivalent) clinical trial of a Licensed Product within 60 months of the effective date as amended on August 16, 2019, as described below.

In addition, under the IURTC License Agreement, the Company has a non-transferable, exclusive option to negotiate in good faith for a royalty-bearing, worldwide, exclusive license (including the right to sublicense) to certain new inventions that may be developed by Indiana University pursuant to a sponsored research agreement between Indiana University and the Company for research conducted in an Indiana Laboratory for a period of six months from IURTC disclosure of such invention to the Company.

Both agreements continue from their effective date through the last to expire of the licensed patents unless earlier terminated. Either party may terminate upon 60 days written notice if there is a material breach of the terms of the license that has not been cured within such 60-day period. IURTC and WFUHS may terminate the license if at any time the Company ceases to carry required Director's and Officer's insurance coverage, ceases to have at least one employee or consultant who is devoting at least 20 hours a week to the affairs of the Company or in the event the Company files for bankruptcy or is insolvent. The Company may terminate the licenses with or without cause on 30 day's written notice to WFUHS and 60 day's written notice to IURTC.

The Company may assign the WFUHS license to an affiliate or in conjunction with the sale of the business but may not assign the licenses to another third-party without prior written consent. The Company may assign the IURTC license to an affiliate or a third party as long as the Company is not in breach of the license at the time of assignment, the successor is not materially insolvent and the successor agrees in writing to assume all obligations and liabilities of the Company to IURTC.

On August 16, 2019, the Company entered into a First Amendment of its License Agreement with IURTC. The First Amendment transfers all rights and obligations under the License Agreement to the Trustees of Indiana University ("IU") and releases IURTC from all liability and obligations under the License Agreement which arose before or after the First Amendment effective date. The First Amendment also expanded the definition of Licensed Patents and expanded the option to license additional technologies developed by IU. The date by which the Company was required to enroll the first patient in a Phase I clinical trial of licensed product was extended to June 30, 2020 and the date by which the Company is required to enroll the first patient in a Phase II clinical trial of licensed product was extended to June 30, 2022. Additionally, it added milestone payments aggregating up to less than $0.1 million upon the issuance of a U.S. Patent and up to less than $0.1 million upon the issuance of certain ex-US patents. It reduced the amount the Company may deduct from consideration payable to IU for payments made to WFUHS from 80% to 60%. The Company also agreed to pay to IU a minimum annual royalty of less than $0.1 million per annum starting the 2020 calendar year for the term of the agreement. During the three months ended March 31, 2020 and 2019, no expense was recognized in relation to the first amendment of its license agreement. Pursuant to amendments to extend these dates, the Company is in compliance with each of the above noted requirements as of March 31, 2020.

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 possession of the leased property and the lease term commenced on February 15, 2020. In the quarter ended March 31, 2020, the Company recorded operating lease right-of-use asset and operating lease liability of $0.4 million.

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.

Expense arising from operating leases were less than $0.1 million during the three-month periods ended March 31, 2020 and March 31, 2019. For operating leases, the weighted-average remaining lease term for leases at March 31, 2020 and December 31, 2019 was 3.05 and 3.30 years, respectively. For operating leases, the weighted average discount rate for leases at March 31, 2020 and December 31, 2019 was 12.0%. The Company has not entered into any financing leases.

Maturities of lease liabilities due under these lease agreements as of March 31, 2020 are as follows:

Year Ended December 31,	(In thousands)
2020 (April – December)	$ 217
2021	164
2022	167
2023	99
Total lease payments	647
Less imputed interest	(97)
Present value of lease liabilities	$ 550

(11) Related Party Transactions

In November 2016, the Company entered into a consulting agreement with R. Mark Payne, M.D. to serve in the capacity of Chief Scientific Officer. Dr. Payne was a director of the Company 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 IURTC 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 Holdings of which 30% was associated with the Transaction and expensed as research and development in 2016 and the remaining 70% associated with future services (see Note 8) vesting ratably over the next 48 months. The consulting agreement has a four-year term, subject to earlier termination. During the three months ended March 31, 2020 and March 31, 2019, the Company recognized less than $0.1 million, related to this consulting agreement, recorded as research and development expense in the Statement of Operations.

The funding to the Company originated from Holdings sale of Series A Preferred Units and Series B convertible preferred units to the Deerfield Funds, and certain other purchasers, from inception through March 31, 2020 and the contribution of the proceeds received by Holdings on such sales to the Company in order to fund the Company's operations.

Under a November 30, 2016 Series A Preferred Unit Purchase Agreement, as amended on September 8, 2017, November 15, 2017, November 14, 2018 and April 29, 2019, Holdings sold an aggregate of 1,780,000 Series A Preferred Units at a purchase price of $20.00 per unit for gross proceeds of $35.6 million. Of the aggregate Series A Preferred Units sold, 604,333 Series A Preferred Units were sold during the year-ended December 31, 2018 for aggregate gross proceeds of $12.1 million and 799,779 Series A Preferred Units were sold during the year-ended December 31, 2019 for aggregate gross proceeds of $16.0 million.

On November 21, 2019 (as amended on December 20, 2019), Holdings entered into a Second Amended and Restated LLC Agreement and entered into a Series B Bridge Unit Purchase Agreement with the Deerfield Funds and certain other purchasers to sell up to 2,004 Series B convertible preferred units ("Series B Bridge Units") at a purchase price of $5,000.00 per unit for gross proceeds of up to $10.0 million. On November 21, 2019, Holdings sold 681 Series B Bridge Units for aggregate gross proceeds of approximately $3.4 million which were immediately contributed to the Company. On December 20, 2019 Holdings sold 666 Series B Bridge Units from the second closing for aggregate gross proceeds of approximately $3.3 million which was later contributed to the Company during the three months ended March 31, 2020. On January 15, 2020, Holdings sold the final 657 Series B Bridge Units available for sale under the Series B Bridge Unit Purchase Agreement for gross proceeds of approximately $3.3 million and contributed such amount to the Company.

On January 16, 2020, Holdings entered into a Third Amended and Restated LLC Agreement and entered into a Second Series B Bridge Unit Purchase Agreement with the Deerfield Funds and certain other purchasers to sell up to 3,000 Second Series B convertible preferred units ("Second Series B Bridge Units") at a purchase price of $5,000.00 per unit, for gross proceeds of up to $15.0 million. Amounts raised by Holdings pursuant to the sale of Series B Bridge Units will be utilized to fund the Company. On February 26, 2020, Holdings sold 600 Second Series B Bridge Units for gross proceeds of approximately $3.0 million and contributed such amount to the Company.

During the three months ended March 31, 2020 and the year December 31, 2019, Holdings provided the Company non-interest bearing, permanent funding from the above Series A and Series B preferred unit transactions, totaling $9.6 million and $19.4 million, respectively, which has been recorded as capital contributions with the balance of combined equity and additional paid in capital on the condensed consolidated balance sheets and condensed consolidated statements of changes in stockholders' equity (deficit) for each respective period.

(12) Subsequent Events

For its condensed consolidated financial statements as of March 31, 2020 and for the quarter then ended, the Company evaluated subsequent events through June 26, 2020, the date on which those Condensed Consolidated financial statements were issued.

On April 10, 2020, Holdings sold 400 Second Series B Bridge Units for gross proceeds of approximately $2.0 million and contributed such amount to the Company.

On May 7, 2020, Holdings sold 800 Second Series B Bridge Units for gross proceeds of approximately $4.0 million and contributed such amount to the Company.

On May 28, 2020, the Company, Zafgen, and Merger Sub completed their merger transaction pursuant to the Merger Agreement with the Company becoming a wholly-owned subsidiary and the surviving corporation. As a result, Zafgen issued 6,091,250 million shares of common stock, after giving effect to the Reverse Stock Split described below, to the sole stockholder of the Company, Holdings, in exchange for common shares of the Company.

For accounting purposes, the Company is considered to be acquiring Zafgen which was determined based upon the terms of the Merger Agreement and other factors including: (i) Chondrial security holders own approximately 66% of the voting interests of the combined company immediately following the closing of the merger; (ii) directors appointed by Chondrial hold a majority of board seats in the combined company; and (iii) Chondrial management holds a majority of the key positions in the management of the combined company. Immediately following the closing of the Merger, Zafgen effected a 1-for-12 reverse stock split of the Zafgen Common Stock (the "Reverse Stock Split") and changed its name to Larimar Therapeutics, Inc. ("Larimar"). Immediately following the close of the Merger and after giving effect for the merger there were approximately 9.2 million shares of the Company's Common stock outstanding. In addition, pursuant to the terms of the Merger Agreement, Zafgen assumed all outstanding stock options to purchase shares of Holdings Common Units at the closing of the Merger. At the closing of the Merger, such stock options became options to purchase an aggregate of 330,818 shares of Zafgen Common Stock, after giving effect to the Reverse Stock Split.

The Merger will be accounted for as a reverse acquisition under the provisions of Financial Accounting Standards Board Accounting Standards Codification Topic 805, Business Combinations ("ASC 805"). As a result, upon consummation, the assets and liabilities of the Company will be recorded at their pre-combination carrying amounts and the Company's historical financial statements will become the financial statements of the combined company with the assets acquired of Zafgen being recorded at their fair values on the acquisition date.

On May 29, 2020, Larimar Therapeutics, Inc. entered into a Securities Purchase Agreement with certain accredited investors (the "Purchasers") for the sale by the Company in a private placement (the "Private Placement") of 6,105,359 shares of the Company's common stock, par value $0.001 per share ("Common Stock") and pre-funded warrants to purchase an aggregate of 628,403 shares of the Company's Common Stock ("Pre-Funded Warrants"), for a price of $11.88 per share of Common Stock and $11.87 per Pre-Funded Warrant. The Pre-Funded Warrants will be immediately exercisable at an exercise price of $0.01 and will be exercisable indefinitely. The Purchasers may exercise the Pre-Funded 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 Pre-Funded Warrants (the "Warrant Shares") 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 Shares and Pre-Funded Warrants were $80.0 million and, after deducting certain of the Company's expenses, the net proceeds received by the Company in the Private Placement were $75.5 million. The Company intends to use the net proceeds from the Private Placement for research and development of the Company's product candidates, working capital and general corporate purposes.

UNAUDITED PRO FORMA COMBINED FINANCIAL STATEMENTS

On December 17, 2019, Zafgen, Inc. ("Zafgen"), Chondrial Therapeutics Inc. ("Chondrial") and Zordich Merger Sub, Inc. ("Merger Sub"), entered into an Agreement and Plan of Merger, as amended on March 9, 2020 (the "Merger Agreement"), pursuant to which Merger Sub merged with and into Chondrial, with Chondrial surviving as a wholly owned subsidiary of Zafgen and the surviving corporation of the merger (the "Merger"). The Merger was completed on May 28, 2020 pursuant to the terms of the Merger Agreement. At the effective time of the Merger (the "Effective Time"), each share of Chondrial's common stock, par value $0.001 per share ("Chondrial Common Stock") outstanding immediately prior to the effective time was converted into the right to receive shares of Zafgen's common stock, par value $0.001 per share ("Zafgen Common Stock"), based on an exchange ratio set forth in the Merger Agreement ("Exchange Ratio"). At the effective time, the Exchange Ratio was determined to be 730,950.0000 shares of Zafgen Common Stock for each share of Chondrial Common Stock. At the closing of the Merger on May 28, 2020, Zafgen issued an aggregate of 73,095,000 shares of its common stock to Chondrial's sole stockholder, based on the Common Stock Exchange Ratio of 730,950.0000 shares of Zafgen Common Stock for each share of Chondrial Common Stock, before giving effect to the Reverse Stock Split described below. Immediately following the closing of the Merger, Zafgen effected a 1-for-12 reverse stock split of the Zafgen Common Stock (the "Reverse Stock Split") and changed its name to Larimar Therapeutics, Inc.

The following unaudited pro forma combined financial information gives effect to the Merger. Except as otherwise noted, the unaudited pro forma combined financial information also gives effect to the Reverse Stock Split. Amounts in the historical Zafgen and historical Chondrial columns of the unaudited pro forma combined financial statements do not give effect to the Reverse Stock Split.

In the unaudited pro forma combined financial statements, the Merger has been accounted for as a business combination using the acquisition method of accounting under the provisions of Financial Accounting Standards Board Accounting Standards Codification Topic 805, *Business Combinations* ("ASC 805"). The merger will be accounted for as a reverse acquisition with Chondrial being deemed the acquiring company for accounting purposes. Under ASC 805, Chondrial, as the accounting acquirer, will record the assets acquired and liabilities assumed of Zafgen in the merger at their fair values as of the acquisition date. As the merger has been accounted for as an asset acquisition, goodwill has not been recorded within the pro forma combined balance sheet as of March 31, 2020.

Chondrial was determined to be the accounting acquirer based on an analysis of the criteria outlined in ASC 805 and the facts and circumstances specific to the merger, including: (1) shareholders of Chondrial own a substantial majority of the voting rights of the combined company; (2) the majority of the board of directors of the combined company is composed of directors designated by Chondrial under the terms of the merger; and (3) existing members of Chondrial management will be the management of the combined company.

Because Chondrial has been determined to be the accounting acquirer in the Merger, but not the legal acquirer, the Merger is deemed a reverse acquisition under the guidance of ASC 805. As a result, upon consummation of the Merger, the historical financial statements of Chondrial will become the historical financial statements of the combined company.

The unaudited pro forma combined balance sheet as of March 31, 2020 gives effect to the merger as if it took place on March 31, 2020 and combines the historical balance sheets of Zafgen and Chondrial as of March 31, 2020. The unaudited pro forma combined statement of operations for the quarter ended March 31, 2020 and the year ended December 31, 2019 gives effect to the merger as if it took place on January 1, 2019 and combines the historical results of Zafgen and Chondrial for the quarter ended March 31, 2020 and the year ended December 31, 2019. The historical financial statements of Zafgen and Chondrial have been adjusted to give pro forma effect to events that are (1) directly attributable to the merger, (2) factually supportable, and (3) with respect to the unaudited pro forma combined statements of operations, expected to have a continuing impact on the combined results of operations of the combined company.

The unaudited pro forma combined financial information is based on assumptions and adjustments that are described in the accompanying notes. The application of the acquisition method of accounting is dependent upon certain valuations, such as leases, that have yet to be completed. Accordingly, the pro forma adjustments reflected in the unaudited pro forma combined financial statements are preliminary and based on estimates, subject to further

revision as additional information becomes available and additional analyses are performed, and have been made solely for the purpose of providing the unaudited pro forma combined financial information. Differences between the preliminary adjustments reflected in the unaudited pro forma combined financial information and the final application of the acquisition method of accounting, which is expected to be completed as soon as practicable after the closing of the Merger, may arise, and those differences could have a material impact on the accompanying unaudited pro forma combined financial information and the combined company's future results of operations and financial position. In addition, differences between the preliminary and final adjustments will likely occur as a result of the amount of cash used for Zafgen's operations and other changes in Zafgen's assets and liabilities between March 31, 2020 and the closing date of the Merger.

The unaudited pro forma combined financial information does not give effect to the potential impact of operating efficiencies or other savings or expenses that may be associated with the integration of the two companies. The unaudited pro forma combined financial information has been prepared for illustrative purposes only and is not necessarily indicative of the financial position or results of operations in future periods or the results that actually would have been realized had Zafgen and Chondrial been a combined company during the specified periods.

Concurrently with the closing of the Merger, the Company entered into a Securities Purchase Agreement ("Purchase Agreement") with certain accredited investors ("Purchasers") for the sale by the Company in a private placement (the "Private Placement") of 6,105,359 shares ("Shares") of the Company's common stock, par value $0.001 per share ("Common Stock") and pre-funded warrants to purchase an aggregate of 628,403 shares of the Company's Common Stock ("Pre-Funded Warrants"), for a price of $11.88 per share of Common Stock and $11.87 per Pre-Funded Warrant. The following unaudited pro forma combined financial information also gives effect to the Private Placement.

The Pre-Funded Warrants will be immediately exercisable at an exercise price of $0.01 and will be exercisable indefinitely. The Purchasers may exercise the Pre-Funded 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 Pre-Funded Warrants ("Warrant Shares") 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 Shares and Pre-Funded Warrants were $80.0 million and, after deducting certain of the Company's expenses, the net proceeds received by the Company in the Private Placement were $75.5 million.

The unaudited pro forma combined financial statements, including the notes thereto, should be read in conjunction with the separate historical consolidated financial statements of Zafgen and Chondrial. Zafgen's historical audited consolidated financial statements for the years ended December 31, 2019, 2018 and 2017 are included in Zafgen's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, as filed with the Securities and Exchange Commission (the "SEC") on March 5, 2020. In addition, the historical unaudited condensed consolidated financial statements for the quarter ended March 31, 2020 are included in Zafgen's Quarterly Report on Form 10-Q, as filed with the SEC on May 7, 2020. Chondrial's historical audited consolidated financial statements for the years ended December 31, 2019 and 2018 are included as Exhibit 99.3 in this Current Report on Form 8-K and Chondrial's historical unaudited condensed consolidated financial statements for the quarter ended March 31, 2020 are included as Exhibit 99.4 in this Current Report on Form 8-K.

Unaudited Pro Forma Combined Balance Sheet
For the quarter ended March 31, 2020
(in thousands)

	Historical		Merger Pro Forma		Pro Forma	Private		Pro Forma
	Chondrial	Zafgen	Adjustments	Note 6	Combined	Placement	Note 9	Combined
Assets								
Current assets:								
Cash and cash equivalents	$ 887	$ 38,980	$ (21,760)	**a**	$ 18,107	$ 75,502	**a**	$ 93,609
Marketable securities	—	24,905	—		24,905	—		24,905
Tax incentive receivable	—	214	—		214	—		214
Prepaid expenses and other current assets	5,116	581	(1,364)	**b**	4,333	—		4,333
Total current assets	6,003	64,680	(23,124)		47,559	75,502		123,061
Property and equipment, net	309	743	—		1,052	—		1,052
Operating lease right-of-use assets	533	6,928	(4,418)	**c**	3,043	—		3,043
Restricted cash	—	1,339	—		1,339	—		1,339
Other assets	147	12	—		159	—		159
Total assets	$ 6,992	$ 73,702	$ (27,542)		$ 53,152	$ 75,502		$128,654
Liabilities and Stockholders' Equity								
Current liabilities:								
Accounts payable	$ 1,659	$ 322	$ —		$ 1,981	$ —		$ 1,981
Accrued expenses	2,527	881	—		3,408	—		3,408
Accrued restructuring costs	—	981	—		981	—		981
Operating lease liabilities, current	209	410	58	**d**	677	—		677
Notes payable, current	—	7,273	(7,273)	**a**	—	—		—
Total current liabilities	4,395	9,867	(7,215)		7,047	—		7,047
Notes payable, noncurrent	—	6,747	(6,747)	**a**	—	—		—
Operating lease liabilities, noncurrent	341	6,346	66	**d**	6,753	—		6,753
Total liabilities	4,736	22,960	(13,896)		13,800	—		13,800
Stockholders' equity:								
Common stock—Chondrial	1	—	(1)	**g**	—	—		—
Common stock—Zafgen	—	37	(28)	**g**	9	6	**a**	15
Additional paid-in capital	32,061	450,623	(413,564)	**e & f**	69,149	75,496	**a**	144,645
			29	**g**		—		
Accumulated deficit	(29,806)	(399,949)	399,949	**f**	(29,806)	—		(29,806)
Accumulated other comprehensive income	—	31	(31)	**f**	—	—		—
Total stockholders' equity	2,256	50,742	(13,646)		39,352	75,502		114,854
Total liabilities and stockholders' equity	$ 6,992	$ 73,702	$ (27,542)		$ 53,152	$ 75,502		$128,654

The accompanying notes are an integral part of the unaudited pro forma combined financial statements.

Unaudited Pro Forma Combined Statements of Operations
For the quarter ended March 31, 2020
(in thousands, except share and per share amounts)

	Historical		Merger Pro Forma Adjustments	Note 7	Pro Forma Combined	Private Placement	Note 9	Pro Forma Combined
	Chondrial	Zafgen						
Operating expenses:								
Research and development	$ 5,007	$ (94)	$ —		$ 4,913	$ —		$ 4,913
General and administrative	1,667	3,647	(536)	a	4,778	—		4,778
Restructuring charges	—	10	—		10	—		10
Total operating expenses	6,674	3,563	(536)		9,701	—		9,701
Loss from operations	(6,674)	(3,563)	536		(9,701)	—		(9,701)
Other income (expense):								
Interest income	—	258	—		258	—		258
Interest expense	—	(301)	294	b	(7)	—		(7)
Other income	—	218	—		218	—		218
Foreign currency transaction losses, net	—	(210)	—		(210)	—		(210)
Total other (expense) income, net	—	(35)	294		259	—		259
Net loss	$ (6,674)	$ (3,598)	$ 830		$ (9,442)	$ —		$ (9,442)
Net loss per share, basic and diluted	$(66,740.00)	$ (0.10)			$ (1.02)			$ (0.59)
Weighted average common shares outstanding, basic and diluted	100	37,467,411	(28,253,795)	c	9,213,716	6,733,762	b	15,947,478

The accompanying notes are an integral part of the unaudited pro forma combined financial statements.

Unaudited Pro Forma Combined Statements of Operations
For the year ended December 31, 2019
(in thousands, except share and per share amounts)

	Historical		Merger Pro Forma Adjustments	Note 8	Pro Forma Combined	Private Placement	Note 9	Pro Forma Combined
	Chondrial	Zafgen						
Operating expenses:								
Research and development	$ 20,790	$ 23,886	$ —		$ 44,676	$ —		$ 44,676
General and administrative	2,424	16,215	(1,421)	a	17,218	—		17,218
Restructuring charges	—	5,553	—		5,553	—		5,553
Total operating expenses	23,214	45,654	(1,421)		67,447	—		67,447
Loss from operations	(23,214)	(45,654)	1,421		(67,447)	—		(67,447)
Other income (expense):								
Interest income	—	1,989	—		1,989	—		1,989
Interest expense	—	(1,766)	1,729	b	(37)	—		(37)
Other income	82	—	—		82	—		82
Foreign currency transaction gains, net	—	25	—		25	—		25
Total other income, net	82	248	1,729		2,059	—		2,059
Net loss	$ (23,132)	$ (45,406)	$ 3,150		$ (65,388)	$ —		$ (65,388)
Net loss per share, basic and diluted	$(231,320.00)	$ (1.22)			$ (7.10)			$ (4.10)
Weighted average common shares outstanding, basic and diluted	100	37,347,199	(28,133,583)	c	9,213,716	6,733,762	b	15,947,478

The accompanying notes are an integral part of the unaudited pro forma combined financial statements.

Notes to Unaudited Pro Forma Combined Financial Statements

(1) Description of the Transactions

Merger of Chondrial and Zafgen

On December 17, 2019, Zafgen, Chondrial and Merger Sub entered into the Merger Agreement pursuant to which Merger Sub merged with and into Chondrial, with Chondrial surviving as a wholly owned subsidiary of Zafgen and the surviving corporation of the Merger. The Merger was completed on May 28, 2020 pursuant to the terms of the Merger Agreement. At the Effective Time of the Merger, each share of Chondrial Common Stock outstanding immediately prior to the Effective Time was converted into the right to receive shares of Zafgen Common Stock based on the Common Stock Exchange Ratio. At the Effective Time, the Common Stock Exchange Ratio was determined to be 730,950 shares of Zafgen Common Stock for each share of Chondrial Common Stock. At the closing of the Merger on May 28, 2020, Zafgen issued an aggregate of 70,395,000 shares of its common stock to Chondrial's sole stockholder, based on the Common Stock Exchange Ratio of 730,950.0000 shares of Zafgen Common Stock for each share of Chondrial Common Stock before giving effect to the Reverse Stock Split described below. Immediately following the closing of the Merger, Zafgen effected a 1-for-12 reverse stock split of the Zafgen Common Stock and changed its name to Larimar Therapeutics, Inc.

Private Placement Offering

Concurrently with the closing of the Merger, the Company entered into a Purchase Agreement with certain Purchasers for the sale by the Company in a private placement of 6,105,359 shares of the Company's common stock, par value $0.001 per share, and Pre-Funded Warrants to purchase an aggregate of 628,403 shares of the Company's Common Stock, for a price of $11.88 per share of Common Stock and $11.87 per Pre-Funded Warrant.

The Pre-Funded Warrants will be immediately exercisable at an exercise price of $0.01 and will be exercisable indefinitely. The Purchasers may exercise the Pre-Funded 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 Warrant Shares 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 Shares and Pre-Funded Warrants were $80.0 million and, after deducting certain of the Company's expenses, the net proceeds received by the Company in the Private Placement were $75.5 million.

(2) Basis of Presentation

The accompanying unaudited pro forma combined financial information was prepared in accordance with Article 11 of SEC Regulation S-X. The unaudited pro forma combined balance sheet as of March 31, 2020 was prepared using the historical balance sheets of Chondrial and Zafgen as of March 31, 2020 and gives effect to the Merger and Private Placement as if each occurred on March 31, 2020. The unaudited pro forma combined statements of operations for the quarter ended March 31, 2020 and the year ended December 31, 2019 give effect to the Merger and Private Placement as if each occurred on January 1, 2019 and were prepared using:

- the historical audited consolidated financial statements of Chondrial for the year ended December 31, 2019;
- the historical audited consolidated financial statements of Zafgen for the year ended December 31, 2019;
- the historical unaudited condensed consolidated financial statements of Chondrial for the quarter ended March 31, 2020; and
- the historical unaudited condensed consolidated financial statements of Zafgen for the quarter ended March 31, 2020.

Except as otherwise noted, the unaudited pro forma combined financial information also gives effect to the Reverse Stock Split. Amounts in the historical Chondrial and historical Zafgen columns of the unaudited pro forma combined financial statements do not give effect to the Reverse Stock Split.

The unaudited pro forma combined financial information does not include the impacts of any revenue, cost or other operating synergies that may result from the Merger or any related restructuring costs that may be contemplated.

(3) Accounting Policies

During the preparation of the accompanying unaudited pro forma combined financial information, Chondrial was not aware of any material differences between Chondrial's accounting policies and the accounting policies of Zafgen. In the period prior to its reporting of the business combination in connection with the Merger, Chondrial will continue to conduct a more detailed review of Zafgen's accounting policies. As a result, Chondrial may identify differences between the accounting policies of the two companies that, when conformed, could have had a material impact on the accompanying unaudited pro forma combined financial information.

(4) Accounting for the Merger

Based on the Common Stock Exchange Ratio of 730,950 set forth above, immediately following the Merger, former Zafgen stockholders, Zafgen option holders and other persons holding securities or other rights directly or indirectly convertible, exercisable or exchangeable for Zafgen Common Stock (collectively, the "Zafgen Securityholders") owned approximately 34% of the outstanding capital stock of the combined company, and the former Chondrial stockholder, Chondrial Therapeutics Holdings, LLC ("Holdings"), owned approximately 66% of the outstanding capital stock of the combined company. At the closing of the Merger, all shares of Chondrial Common Stock were exchanged for an aggregate of 6,091,250 shares of Zafgen Common Stock, after giving effect to the Reverse Stock Split.

In addition, pursuant to the terms of the Merger Agreement, Zafgen assumed all outstanding options to purchase shares of Holdings Common Units at the closing of the Merger. At the closing of the Merger, such options became options to purchase an aggregate of 330,818 shares of Zafgen Common Stock, after giving effect to the Reverse Stock Split.

The estimated preliminary purchase price is calculated based on the fair value of the Zafgen common stock of the combined company that Zafgen stockholders will own as of the closing date of the transaction because, with no active trading market for shares of Chondrial, the fair value of the Zafgen common stock represents a more reliable measure of the fair value of consideration transferred in the merger. The following summarizes the preliminary estimate of the purchase price paid in the Merger (in thousands, except share and per share amounts):

Estimated number of shares of the combined company to be owned by Zafgen Stockholders(1)	3,122,466
Multiplied by the fair value per share of Zafgen common stock(2)	$ 11.88
Estimated fair value of Zafgen common stock	$ 37,096
Estimated Chondrial transaction costs(3)	$ 1,649
Estimated purchase price	$ 38,745

(1) For purposes of this unaudited pro forma combined financial information, the number of shares of 3,122,466 represents the historical 37,469,596 shares of Zafgen Common Stock outstanding immediately prior to the closing of the Merger, adjusted for the Reverse Stock Split. In addition, for purposes of this unaudited pro forma combined financial information, the estimated purchase price does not include the impact of the portion of the fair value of certain options to acquire shares of Zafgen Common Stock attributable to precombination employee services because the amount is not material.

(2) The estimated purchase price was based on the last reported sale price of Zafgen Common Stock on the Nasdaq Global Market on May 28, 2020, the closing date of the Merger, of $0.99 per share and gives effect to the Reverse Stock Split.

(3) The estimated Chondrial transaction costs consist primarily of legal and proxy related expenses incurred by Chondrial. The transaction costs have been reflected as an increase in the estimated purchase price.

The estimated fair value of the net assets of Zafgen adjusted for the excess of fair value over consideration transferred on a pro forma basis as of March 31, 2020, after giving effect of accruals of costs expected to be incurred in connection with the merger was $38.8 million. The preliminary purchase price assigned a value to the assets and liabilities acquired based on the accumulated cost of the acquisition and allocated based on the acquired assets and liabilities relative fair value adjusted for the excess of fair value over consideration transferred; refer to Note 6.

The following summarizes the preliminary allocation of the estimated purchase price paid in the Merger as if it had been completed on March 31, 2020 (in thousands):

	Purchase Price Allocation —Pro Forma
Cash and cash equivalents	$ 17,505
Marketable securities	24,905
Tax incentive receivable	214
Prepaid expenses and other current assets	581
Property and equipment, net	743
Operating lease right-of-use assets	2,510
Restricted cash	1,339
Other assets	12
Operating lease liabilities, current	(468)
Total other current liabilities	(2,184)
Operating lease liabilities, noncurrent	(6,412)
Net tangible assets acquired	$ 38,745

The application of the acquisition method of accounting is dependent upon certain valuations and other studies that have yet to be completed. The purchase price allocation will remain preliminary until Chondrial management determines the fair values of assets acquired and liabilities assumed upon the closing of the Merger. The final determination of the purchase price allocation is anticipated to be completed as soon as practicable after completion of the Merger and will be based on the fair values of the assets acquired and liabilities assumed as of the closing date of the Merger. Chondrial does not expect to acquire or assign any value to intangible assets. Operating lease liabilities have been measured at the present value of the remaining lease payments, as if the acquired leases were new leases of the acquirer at the acquisition date. The operating lease right-of-use assets have been measured at the same amount as the lease liability as adjusted to reflect terms of the leases compared to market terms. The excess of the purchase price over the fair value of the assets and liabilities was allocated to the operating right-of-use assets. The final amounts allocated to assets acquired and liabilities assumed could differ materially from the amounts presented in the unaudited pro forma combined financial statements.

(5) Shares of Zafgen Common Stock Issued to Chondrial Stockholder upon Closing of the Merger

As part of the Merger, all outstanding shares of Chondrial Common Stock were exchanged for shares of Zafgen Common Stock. Based on the historical 100 shares of Chondrial Common Stock, before giving effect to the Reverse Stock Split, and based on the Common Stock Exchange Ratio determined in accordance with the terms of the Merger Agreement of 730,950.0000, Zafgen issued 6,091,250 shares of Zafgen Common Stock in the Merger, after giving effect to the Reverse Stock Split, determined as follows:

Chondrial Common Stock outstanding immediately prior to the closing of the Merger (before giving effect to the Reverse Stock Split)	100
Common Stock Exchange Ratio	730,950.0000
Shares of Zafgen Common Stock issued to Chondrial’s sole stockholder upon closing of the Merger (before giving effect to the Reverse Stock Split)	73,095,000
Reverse Stock Split Ratio	12.0000
Shares of Zafgen Common Stock issued to Chondrial’s sole stockholder upon closing of the Merger (after giving effect to the Reverse Stock Split)	6,091,250

In addition, in connection with the Merger, Zafgen assumed all of the outstanding options to acquire Holdings stock and such options became exercisable for shares of Zafgen Common Stock following the Merger; refer to Note 4.

(6) Adjustments to Unaudited Pro Forma Combined Balance Sheet as of March 31, 2020

The unaudited pro forma combined balance sheet includes pro forma adjustments that are (1) directly attributable to the Merger and (2) factually supportable. The pro forma adjustments associated with the asset acquisition, based on preliminary estimates that may change significantly as additional information is obtained, are as follows:

(a) The adjustment to cash and cash equivalents represents (in thousands):

Repayment of Zafgen's outstanding term loan(1)	$14,385
Cash paid for transaction costs expected to be incurred through consummation of the Merger(2)	7,375
Total adjustment to cash and cash equivalents	$21,760

1. The repayment of Zafgen's term loan with Silicon Valley Bank ("Term Loan"), which was repaid as part of the Merger, and consists of the principle repayment of $12.7 million, a final payment equal to $1.6 million and a prepayment fee of $0.1 million; and
2. the cash paid for transaction costs expected to be incurred through the consummation of the merger that are not already included in accrued liabilities as of March 31, 2020. Of the $7.4 million of incremental transaction costs, approximately $0.3 million relate to Chondrial and have been reflected as an increase to the purchase price and allocated based on the acquired assets and liabilities relative fair value in the unaudited combined pro forma balance sheet. The remaining approximately $7.1 million of incremental transaction costs relate to Zafgen. The transaction costs of Zafgen include approximately $3.4 million in employee retention bonuses, severance and change-in-control obligations for Zafgen employees that will be reflected as pre-combination compensation expense of Zafgen. The retention bonuses were communicated by Zafgen in September 2019, prior to Zafgen entering into negotiations with Chondrial regarding the merger; as such, the retention bonuses were determined to be for the benefit of Zafgen. The remaining $3.7 million of estimated transaction costs consist primarily of banker fees, legal expenses, insurance, auditor and printer fees to be incurred by Zafgen. These pro forma adjustments are not reflected in the unaudited pro forma combined statement of operations as these amounts are not expected to have a continuing impact on the operating results of the combined company.

(b) The adjustment of $1.4 million to prepaid expenses and other current assets represents the prepaid transaction costs by Chondrial as of March 31, 2020 that are added to the consideration paid and included in the purchase price allocation..

(c) The adjustment to right-of-use assets represents an adjustment of $4.4 million to measure the fair value in accordance with ASC 805 and for the step-down associated with the excess fair value over the transaction price; refer to Note 4.

(d) The adjustment to operating lease liabilities, current, and operating lease liabilities, noncurrent, represents an adjustment of $0.1 million to measure the fair value in accordance with ASC 805.

(e) Represents an adjustment of $37.1 million to additional paid-in capital for the estimated fair value of the equity portion of the consideration transferred; refer to Note 4.

(f) Represents the elimination of Zafgen's accumulated deficit, historical additional paid-in capital and historical accumulate other comprehensive income.

(g) Represents the impact on the common stock par value from the exchange of all outstanding shares of Chondrial Common Stock into 6,091,250 shares of Zafgen Common Stock pursuant to the Merger Agreement, as if the Merger had occurred on March 31, 2020 and after giving effect to the Reverse Stock Split; refer to Note 5.

(7) Adjustments to Unaudited Pro Forma Combined Statement of Operations for the Quarter Ended March 31, 2020

The unaudited pro forma combined statements of operations include pro forma adjustments that are (1) directly attributable to the Merger, (2) factually supportable and (3) expected to have a continuing impact on the results of operations of the combined company. Based on Chondrial's management's review of Zafgen's summary of significant accounting policies, the nature and amount of any adjustments to the historical consolidated financial statements of Zafgen to conform to the accounting policies of Chondrial are not expected to be significant. The pro forma adjustments, based on preliminary estimates that could change materially as additional information is obtained, are as follows:

(a) Represents an adjustment to eliminate non-recurring transaction costs of $0.5 million incurred by Zafgen in connection with the merger and recorded as expense in Zafgen's historical consolidated statement of operations for the quarter ended March 31, 2020 as these expenses are not expected to have a continuing impact on the operating results of the combined company.

(b) Represents an elimination of interest expense due to the repayment of Zafgen's Term Loan, which was repaid as part of the Merger.

(c) To reflect an increase in the weighted average shares outstanding for the period after giving effect to the issuance of Zafgen common stock in connection with the merger. As the combined company is in a net loss position, any adjustment for potentially dilutive shares would be anti-dilutive, and as such basic and diluted loss per share are the same. The following table presents these pro forma adjustments without giving effect to the reverse stock split, as follows (presented on a weighted average basis):

	Quarter Ended March 31, 2020
Weighted average shares of Chondrial Common Stock outstanding (before giving effect to the Common Stock Exchange Ratio and the Reverse Stock Split)	100
Common Stock Exchange Ratio	730,950.0000
Subtotal	73,095,000
Reverse Stock Split Ratio	12.0000
Weighted average shares of Chondrial Common Stock outstanding immediately prior to the closing of the Merger (after giving effect to the Common Stock Exchange Ratio and the Reverse Stock Split)	6,091,250
Shares of Zafgen Common Stock outstanding immediately prior to the closing of the Merger (after giving effect to the Reverse Stock Split)	3,122,466
Pro forma combined weighted average number of common shares outstanding—basic and diluted	9,213,716

(8) Adjustments to Unaudited Pro Forma Combined Statement of Operations for the Year Ended December 31, 2019

The unaudited pro forma combined statements of operations include pro forma adjustments that are (1) directly attributable to the Merger, (2) factually supportable and (3) expected to have a continuing impact on the results of operations of the combined company. Based on Chondrial's management's review of Zafgen's summary of significant accounting policies, the nature and amount of any adjustments to the historical consolidated financial statements of Zafgen to conform to the accounting policies of Chondrial are not expected to be significant. The pro forma adjustments, based on preliminary estimates that could change materially as additional information is obtained, are as follows:

(a) Represents an adjustment to eliminate non-recurring transaction costs of $1.4 million incurred by Zafgen in connection with the merger and recorded as expense in Zafgen's historical consolidated statement of operations for the year ended December 31, 2019 as these expenses are not expected to have a continuing impact on the operating results of the combined company.

(b) Represents an elimination of interest expense due to the repayment of Zafgen's Term Loan, which was repaid as part of the Merger.

(c) To reflect an increase in the weighted average shares outstanding for the period after giving effect to the issuance of Zafgen common stock in connection with the merger. As the combined company is in a net loss position, any adjustment for potentially dilutive shares would be anti-dilutive, and as such basic and diluted loss per share are the same. The following table presents these pro forma adjustments without giving effect to the reverse stock split, as follows (presented on a weighted average basis):

	Year Ended December 31, 2019
Weighted average shares of Chondrial Common Stock outstanding (before giving effect to the Common Stock Exchange Ratio and the Reverse Stock Split)	100
Common Stock Exchange Ratio	730,950.0000
Subtotal	73,095,000
Reverse Stock Split Ratio	12.0000
Weighted average shares of Chondrial Common Stock outstanding immediately prior to the closing of the Merger (after giving effect to the Common Stock Exchange Ratio and the Reverse Stock Split)	6,091,250
Shares of Zafgen Common Stock outstanding immediately prior to the closing of the Merger (after giving effect to the Reverse Stock Split)	3,122,466
Pro forma combined weighted average number of common shares outstanding—basic and diluted	9,213,716

(9) Private Placement

The unaudited pro forma combined statements include pro forma adjustments associated with the Private placement that are (1) directly attributable to the private placement and (2) factually supportable. The pro forma adjustments are as follows:

(a) To record the sale of 6,105,359 shares of the Company's common stock and Pre-Funded Warrants to purchase an aggregate of 628,403 shares of the Company's Common Stock, for a price of $11.88 per share of Common Stock and $11.87 per Pre-Funded Warrant, resulting in net proceeds of $75.5 million.

(b) To reflect an increase in the weighted average shares outstanding for the period after giving effect to the issuance of 6,105,359 Shares of the Company's Common Stock and Pre-Funded warrants to purchase an aggregate of 628,403 shares of the Company's Common Stock for a price of $0.01 per share.

(10) Chondrial Loss Per Share

After the close of the Merger Chondrial's financials will be the historical financials of Larimar and the following table presents on a pro forma basis what the historical loss per share, basic and diluted, would be giving effect for the Common Stock Exchange Ratio and reverse stock split:

(in thousands, expect share and per share data)	Year Ended December 31, 2019	Year Ended December 31, 2018	Quarter Ended March 31, 2020	Quarter Ended March 31, 2019
Net loss per share, basic and diluted, as originally reported	$ (23,132)	$ (11,192)	$ (6,674)	$ (4,724)
Weighted average shares of Chondrial Common Stock outstanding	100	100	100	100
Common Stock Exchange Ratio	730,950.0000	730,950.0000	730,950.0000	730,950.0000
Subtotal	73,095,000	73,095,000	73,095,000	73,095,000
Reverse stock split ratio	12	12	12	12
Weighted average shares of Chondrial Common Stock outstanding immediately prior to the closing of the Merger	6,091,250	6,091,250	6,091,250	6,091,250
Net loss per share, basic and diluted	$ (3.80)	$ (1.84)	$ (1.10)	$ (0.78)