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

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2021

OR

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

For the transition period from _____ to _____

Commission File Number: 001-39594

Spruce Biosciences, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**81-2154263**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
2001 Junipero Serra Boulevard, Suite 640	
Daly City, California	**94014**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (415) 655-4168

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	SPRB	Nasdaq Global Select Market

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

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

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

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

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

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

As of May 11, 2021, the registrant had 23,330,135 shares of common stock, $0.0001 par value per share, outstanding.

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SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESS

SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESSES

We face risks and uncertainties associated with our business, many of which are beyond our control. Some of the material risks associated with our business include the following:

- We have a limited operating history, have incurred significant net losses since our inception, and anticipate that we will continue to incur significant net losses for the foreseeable future. We expect these losses to increase as we continue our clinical development of, and seek regulatory approvals for, tildacerfont and any future product candidates.
- We will need substantial additional financing to develop tildacerfont and any future product candidates and implement our operating plans. If we fail to obtain additional financing, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- We currently depend entirely on the success of tildacerfont, which is our only product candidate. If we are unable to advance tildacerfont in clinical development, obtain regulatory approval, and ultimately commercialize tildacerfont, or experience significant delays in doing so, our business will be materially harmed.
- Our clinical trials may fail to adequately demonstrate the safety and efficacy of tildacerfont, which could prevent or delay regulatory approval and commercialization.
- Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs to us, delay or limit our ability to generate revenue, and adversely affect our commercial prospects.
- The U.S. Food and Drug Administration, or FDA, and comparable foreign regulatory authorities may require us to initiate one or more additional clinical trials for tildacerfont in adult patients with classic congenital adrenal hyperplasia, or CAH, including a Phase 3 clinical trial or trials. The estimated timing or scope of any such future clinical trials is not currently ascertainable. Even if regulatory approvals are obtained, we may never be able to successfully commercialize tildacerfont.
- Preclinical and clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of tildacerfont and any future product candidates.
- Our business has been and could continue to be adversely affected by the evolving and ongoing COVID-19 global pandemic in regions where we or third parties on which we rely have significant manufacturing facilities, concentrations of clinical trial sites or other business operations. The COVID-19 pandemic could adversely affect our operations, as well as the business or operations of our manufacturers, clinical research organizations, or CROs, or other third parties with whom we conduct business.
- Tildacerfont is, and any future product candidates will be, subject to extensive regulation and compliance obligations, which are costly and time-consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize tildacerfont and any future product candidates.
- If the market opportunities for tildacerfont and any future product candidates are smaller than we believe they are, our future revenue may be adversely affected, and our business may suffer.
- We may not be successful in our efforts to expand our pipeline by identifying additional indications and formulations for which to investigate tildacerfont in the future. We may expend our limited resources to pursue a particular indication or formulation for tildacerfont and fail to capitalize on product candidates, indications, or formulations that may be more profitable or for which there is a greater likelihood of success.
- We currently have no marketing and sales organization and have yet to commercialize a product. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell tildacerfont and any future product candidates, we may not be able to generate product revenues.
- We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize tildacerfont.
- If we are unable to obtain and maintain sufficient intellectual property protection for tildacerfont, any future product candidates, and other proprietary technologies we develop, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize tildacerfont, if approved, and any future product candidates, and other proprietary technologies if approved, may be adversely affected.
- Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

SPRUCE BIOSCIENCES, INC.
CONDENSED BALANCE SHEETS
(unaudited)
(in thousands, except share amounts)

	March 31, 2021	December 31, 2020
ASSETS		
Current assets:		
Cash and cash equivalents	$ 148,627	$ 157,150
Prepaid expenses	2,687	2,971
Other current assets	272	276
Total current assets	151,586	160,397
Restricted cash	216	216
Right-of-use assets	1,716	1,793
Other assets	473	477
Total assets	$ 153,991	$ 162,883
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,566	$ 3,628
Term loan, current portion	—	2,554
Accrued expenses and other current liabilities	3,558	2,496
Accrued compensation and benefits	727	1,085
Total current liabilities	6,851	9,763
Term loan, net of current portion	4,843	1,922
Lease liability, net of current portion	1,566	1,653
Other liabilities	8	118
Total liabilities	13,268	13,456
Commitments and contingencies (Note 7)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 10,000,000 shares authorized, and no shares issued and outstanding as of March 31, 2021 and December 31, 2020	—	—
Common stock, $0.0001 par value, 200,000,000 shares authorized, 23,301,872 and 23,260,399 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	2	2
Additional paid-in capital	211,449	210,266
Accumulated deficit	(70,728)	(60,841)
Total stockholders' equity	140,723	149,427
Total liabilities and stockholders' equity	$ 153,991	$ 162,883

See accompanying notes to the condensed financial statements.

SPRUCE BIOSCIENCES, INC.
CONDENSED STATEMENTS OF OPERATIONS
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2021	2020
Operating expenses:		
Research and development	$ 6,714	$ 4,610
General and administrative	3,103	523
Total operating expenses	9,817	5,133
Loss from operations	(9,817)	(5,133)
Interest expense	(89)	(74)
Other income, net	19	39
Net loss	$ (9,887)	$ (5,168)
Net loss per share, basic and diluted	$ (0.42)	$ (6.76)
Weighted-average shares of common stock outstanding, basic and diluted	23,283,658	764,408

See accompanying notes to the condensed financial statements.

SPRUCE BIOSCIENCES, INC.
CONDENSED STATEMENT OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance as of January 1, 2021	23,260,399	$ 2	$ 210,266	$ (60,841)	$ 149,427
Exercise of stock options	41,473	—	63	—	63
Stock-based compensation	—	—	1,120	—	1,120
Net loss	—	—	—	(9,887)	(9,887)
Balance as of March 31, 2021	23,301,872	$ 2	$ 211,449	$ (70,728)	$ 140,723

See accompanying notes to the condensed financial statements.

SPRUCE BIOSCIENCES, INC.
CONDENSED STATEMENT OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(unaudited)
(in thousands, except share amounts)

	Redeemable Convertible Preferred Stock								
	Series A		Series B		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance as of January 1, 2020	28,000,000	$27,813	—	$ —	764,408	$ 1	$ 664	$ (31,302)	$ (30,637)
Issuance of Series B redeemable convertible preferred stock, net of issuance costs of $345	—	—	36,666,665	43,655	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—	32	—	32
Net loss	—	—	—	—	—	—	—	(5,168)	(5,168)
Balance as of March 31, 2020	28,000,000	$27,813	36,666,665	$43,655	764,408	$ 1	$ 696	$ (36,470)	$ (35,773)

See accompanying notes to the condensed financial statements.

SPRUCE BIOSCIENCES, INC.
CONDENSED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	Three Months Ended March 31,	
	2021	2020
Cash flows from operating activities		
Net loss	$ (9,887)	$ (5,168)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	1,120	32
Depreciation and amortization	13	1
Non-cash lease expense	77	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	291	56
Accounts payable and accrued expenses	(82)	(269)
Accrued compensation and benefits	(358)	(685)
Other assets	16	36
Other liabilities	23	41
Net cash used in operating activities	(8,787)	(5,956)
Cash flows from investing activities		
Purchases of property and equipment	(19)	-
Net cash used in investing activities	(19)	-
Cash flows from financing activities		
Proceeds from issuance of Series B redeemable convertible preferred stock, net of issuance costs	—	43,655
Proceeds from exercise of stock options	63	—
Proceeds from issuance of long-term debt, net of issuance costs of $10	4,990	—
Repayment of term loan	(4,770)	—
Net cash provided by financing activities	283	43,655
Net increase/(decrease) in cash, cash equivalents and restricted cash	(8,523)	37,699
Cash, cash equivalents, and restricted cash at beginning of period	157,366	3,924
Cash, cash equivalents, and restricted cash at end of period	$ 148,843	$ 41,623
Supplemental cash flow data:		
Cash paid for interest	$ 54	$ 46

See accompanying notes to the condensed financial statements.

SPRUCE BIOSCIENCES, INC.
NOTES TO THE CONDENSED FINANCIAL STATEMENTS
(unaudited)

1. Organization and Principal Activities

Description of Business

Spruce Biosciences, Inc. (the Company) is a late-stage biopharmaceutical company focused on developing and commercializing novel therapies for rare endocrine disorders with significant unmet medical need. The Company is initially developing its wholly-owned product candidate, tildacerfont, as the potential first non-steroidal therapy for patients suffering from classic congenital adrenal hyperplasia (CAH). The Company is also developing tildacerfont for females suffering from a rare form of polycystic ovary syndrome (PCOS) with primary adrenal androgen excess, representing 3-5% of females with PCOS. The Company is located in Daly City, California and was incorporated in the state of Delaware in April 2016.

Initial Public Offering

In October 2020, the Company consummated its initial public offering (IPO) and issued a total of 6,900,000 shares of common stock, which includes 900,000 shares issued pursuant to the exercise of the underwriters' option to purchase additional shares, at an offering price of $15.00 per share. In aggregate, the Company received net proceeds of $93.4 million, after deducting underwriting discounts and commissions and offering expenses. Upon the closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock automatically converted into 15,492,019 shares of common stock.

Liquidity and Capital Resources

As of March 31, 2021, the Company had cash and cash equivalents of $148.6 million, which is sufficient to fund its planned operations for a period of at least twelve months following the issuance of the accompanying condensed financial statements.

The Company has incurred significant losses and negative cash flows from operations. During the three months ended March 31, 2021, the Company incurred a net loss of $9.9 million and used $8.8 million of cash in operations. As of March 31, 2021, the Company had an accumulated deficit of $70.7 million and does not expect positive cash flows from operations in the foreseeable future. The Company has funded its operations primarily through the issuance and sale of equity securities and debt.

The Company anticipates that it will need to raise substantial additional financing in the future to fund its operations. In order to meet these additional cash requirements, the Company may seek to sell additional equity or issue debt, convertible debt or other securities that may result in dilution to its stockholders. If the Company raises additional funds through the issuance of debt or convertible debt securities, these securities could have rights senior to those of its shares of common stock and could contain covenants that restrict its operations. There can be no assurance that the Company will be able to obtain additional equity or debt financing on terms acceptable to it, if at all. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting the Company's ability to take specific actions such as incurring debt, making capital expenditures or declaring dividends. The Company's failure to obtain sufficient funds on acceptable terms when needed could have a material adverse effect on its business, results of operations, and financial condition.

2. Summary of Significant Accounting Policies

Interim Condensed Financial Statements

The financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (U.S. GAAP) and applicable rules and regulations of the U.S. Securities and Exchange Commission (SEC) for interim reporting. As permitted under those rules and regulations, certain notes or other financial information normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted. The condensed balance sheet as of March 31, 2021, the condensed statements of operations for the three months ended March 31, 2021 and 2020, the condensed statement of stockholders' equity for the three months ended March 31, 2021, the condensed statement of redeemable convertible preferred stock and stockholders' deficit for the three months ended March 31, 2020, and the condensed statement of cash flows for the three months ended March 31, 2021 and 2020 are unaudited. The interim condensed financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal, recurring adjustments that are necessary to present fairly the Company's results for the interim periods presented. The condensed balance sheet as of December 31, 2020, is derived from the Company's audited financial statements. The results of operations for the three months ended March 31, 2021, are not necessarily indicative of the results to be expected for the year ending December 31, 2021, or for any other future annual or interim period.

These interim condensed financial statements should be read in conjunction with the Company's Annual Report on Form 10-K for the year ended December 31, 2020 filed with the SEC on March 22, 2021 (Annual Report).

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities and expenses as well as related disclosure of contingent assets and liabilities. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, accrued research and development expenses, valuation of common stock and stock-based compensation and income tax and uncertain tax positions. The Company bases its estimates on its historical experience and on assumptions that it believes are reasonable; however, actual results could significantly differ from those estimates.

Risks and Uncertainties

Any product candidates developed by the Company will require approvals from the U.S. Food and Drug Administration (FDA) or foreign regulatory agencies prior to commercial sales. There can be no assurance that the Company's current and future product candidates will meet desired efficacy and safety requirements to obtain the necessary approvals. If approval is denied or delayed, it may have a material adverse impact on the Company's business and its financial statements.

The Company is subject to a number of risks similar to other late-stage biopharmaceutical companies including, but not limited to, dependency on the clinical and commercial success of the Company's product candidate, tildacerfont, ability to obtain regulatory approval of tildacerfont, the need for substantial additional financing to achieve its goals, uncertainty of broad adoption of its approved products, if any, by physicians and consumers, significant competition and untested manufacturing capabilities, and dependence on key individuals and sole source suppliers.

The Company's business has been and could continue to be adversely affected by the evolving COVID-19 pandemic. For example, the COVID-19 pandemic has resulted in and could result in delays to the Company's clinical trials for numerous reasons including additional delays or difficulties in enrolling patients, diversion of healthcare resources away from the conduct of clinical trials, interruption or delays in the operations of the FDA or other regulatory authorities, and delays in clinical sites receiving the supplies and materials to conduct our clinical trials. At this time, the extent to which the COVID-19 pandemic impacts the Company's business will depend on future developments, which are highly uncertain. The Company will continue to evaluate the impact that these events could have on its future operations, financial position, and results of operations and cash flows.

Significant Accounting Policies

There have been no significant changes to the accounting policies during the three months ended March 31, 2021, as compared to the significant accounting policies described in our Annual Report.

Cash and Cash Equivalents

The Company considers all highly liquid investments with remaining maturities at the date of purchase of three months or less to be cash equivalents. Cash equivalents consist of amounts invested in money market funds and are stated at fair value. There are no unrealized gains or losses on the cash equivalents for the periods presented.

Restricted Cash

The Company has cash in a collateral account related to a letter of credit issued on behalf of the Company for the security deposit on the non-cancelable operating lease for an office facility. The collateralized cash in connection with the letter of credit was classified as restricted cash on the balance sheet as of March 31, 2021 and December 31, 2020 based on the terms of the lease agreement, which expires in 2025, unless extended.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the condensed statements of cash flows (in thousands):

	March 31,	
	2021	2020
Cash and cash equivalents	$ 148,627	$ 41,407
Restricted cash	216	216
Total cash, cash equivalents and restricted cash	$ 148,843	$ 41,623

Fair Value of Financial Instruments

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability, or an exit price, in the principal or most advantageous market for that asset or liability in an orderly transaction between market participants on the

measurement date. Fair value measurement establishes a fair value hierarchy that requires an entity to maximize the use of observable inputs, where available, and minimize the use of unobservable inputs when measuring fair value.

The Company determined the fair value of financial assets and liabilities using the fair value hierarchy that describes three levels of inputs that may be used to measure fair value, as follows:

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

The Company's financial instruments primarily consist of cash and cash equivalents, prepaid expenses, accounts payable, term loan, and accrued expenses. The carrying value of cash and cash equivalents, prepaid expenses, accounts payable, and accrued expenses are generally considered to be representative of their respective fair values because of the short-term nature of those instruments. The estimated fair value of the term loan is based on estimated interest rates currently available to the Company for debt with similar terms.

Emerging Growth Company Status

The Company is an emerging growth company (EGC) as defined in the Jumpstart Our Business Startups Act of 2012 (JOBS Act) and may take advantage of reduced reporting requirements that are otherwise applicable to public companies. Section 107 of the JOBS Act exempts emerging growth companies from being required to comply with new or revised financial accounting standards until private companies are required to comply with those standards. The Company has elected to use the extended transition period for complying with new or revised accounting standards.

Recent Accounting Pronouncements Not Yet Adopted

In June 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-13, Measurement of Credit Losses on Financial Instruments (Topic 326): Measurement of Credit Losses on Financial Instruments (ASU 2016-13). ASU 2016-13 requires companies to measure credit losses utilizing a methodology that reflects expected credit losses and requires a consideration of a broader range of reasonable and supportable information to inform credit loss estimates. ASU 2016-13 is effective for non-EGC's electing to use the extended transition period for complying with new or revised accounting standards for fiscal years beginning after December 15, 2019, and for EGC's for fiscal years beginning after December 15, 2022, with early adoption permitted. The Company expects to adopt this ASU on January 1, 2023. The Company is currently assessing the impact of adopting this standard, but based on a preliminary assessment, does not expect the adoption of this guidance to have a material impact on its financial statements.

Recently Adopted Accounting Pronouncements

In December 2019, the FASB issued Accounting Standards Update (ASU) 2019-12, Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes. The amendments in ASU 2019-12 are intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in Topic 740 and also clarifies and amends existing guidance to improve consistent application. ASU 2019-12 is effective for public business entities for fiscal years beginning after December 15, 2020 and the Company adopted on January 1, 2021. The adoption did not have any impact on the Company's condensed financial statements as of and for the three months ended March 31, 2021.

3. Fair Value Measurements

The Company measures and reports certain financial instruments as assets and liabilities at fair value on a recurring basis. Certain of the Company's financial instruments are not measured at fair value on a recurring basis but are recorded at amounts that approximate their fair value due to their liquid or short-term nature, such as cash, prepaid expenses, accounts payable and accrued expenses.

As of March 31, 2021, cash equivalents comprised of money market funds. The carrying amounts of cash equivalents approximate their fair value based upon quoted market prices and are classified as Level 1. The fair value of money market funds as of March 31, 2021 was $123.0 million.

The estimated fair value of the term loan was $5.0 million as of March 31, 2021 and was based on estimated interest rates currently available to the Company for debt with similar terms, a Level 3 input.

The Company did not have any financial assets measured nor liabilities recorded at fair value on a recurring or non-recurring basis as of December 31, 2020.

4. Balance Sheet Components

Accrued Expenses and Other Current Liabilities

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

	March 31, 2021	December 31, 2020
Accrued research and development expenses	$ 2,864	$ 2,002
Accrued general and administrative expenses	362	245
Lease liability, current portion	332	249
Total accrued expenses and other current liabilities	$ 3,558	$ 2,496

Accrued research and development expenses were primarily related to clinical trials, preclinical studies, and manufacturing.

5. Leases

The Company leases space under non-cancelable operating leases which require the Company to pay base rent, real estate taxes, insurance, general repairs, and maintenance. The Company does not have finance leases.

In February 2020, the Company entered into a non-cancelable operating lease for office space with a commencement date of September 2020. The monthly payments escalate over the 63-month term with total gross commitments of $2.3 million. The lease includes an option to renew the lease term for an additional period of 60 months. The renewal option is not included in the lease term or minimum lease payments disclosures below as the Company is not reasonably certain to exercise the option. Lease incentives, which relate to rent abatement, were considered in the calculation of the lease liability and right-of-use asset. Lease expense for the three months ended March 31, 2021 and 2020 was $110 thousand and nil, respectively.

In February 2019, the Company entered into a short-term lease for office space with a commencement date of March 2019. Total gross commitments over the 12-month term were $0.2 million. In February 2020, the Company extended the lease for an additional three months for a total of $42 thousand in additional commitments. The lease was terminated in May 2020. Short-term lease expense for the three months ended March 31, 2021 and 2020 was nil and $42 thousand, respectively.

Lease right-of-use assets and liabilities are recognized at the commencement date based on the present value of the remaining minimum lease payments over the lease term, with certain adjustments. As the leases do not provide an implicit rate, the Company uses a collateralized incremental borrowing rate based on the information available at the commencement date to determine the lease liability. As of March 31, 2021, the weighted-average remaining lease term for operating leases was 4.7 years and the weighted-average discount rate was 7.0%. Cash paid for amounts included in the measurement of lease liabilities was $37 thousand for the three months ended March 31, 2021.

The Company recognizes monthly operating lease expense on a straight-line basis over the term of the lease. Variable lease expense relates primarily to office lease common area maintenance, insurance, and property taxes, is expensed as incurred, and is excluded from the calculation of the lease liability and right-of-use-asset. Variable lease expense for the three months ended March 31, 2021 was $2 thousand.

6. Term Loan

In September 2019, the Company entered into a Loan and Security Agreement (the Loan Agreement) with Silicon Valley Bank (SVB) providing for a term loan (the Term Loan). In April 2020, the Company and SVB entered into an agreement (the Deferral Agreement) whereby the parties agreed to extend the interest-only period, repayment dates of all monthly payments of principal due and the maturity date with respect to the Term Loan by six months. All other terms under the Loan Agreement remained unchanged. The Deferral Agreement was determined to be a debt modification, resulting in a prospective yield adjustment based on the revised terms.

In connection with the Loan Agreement, the Company issued a warrant to purchase up to an aggregate of 49,609 shares of common stock at $1.44 per share. The Company determined the initial fair value of the warrant to be $0.1 million using the Black-Scholes option-pricing model. The fair value of the warrant was recorded to equity and as a debt discount, which was being amortized to interest expense using the effective interest method over the term of the Term Loan. In November 2020, the warrant was net-exercised for 46,358 shares of common stock.

In March 2021, the Company entered into a First Amendment to Loan and Security Agreement (the First Amendment), pursuant to which the Company and SVB amended the Loan Agreement. The First Amendment increased the aggregate principal amount of the Term Loan commitment by SVB to up to $30.0 million. Up to $20.0 million is available under the first tranche of the Term Loan (First Tranche), $5.0 million of which was advanced immediately to repay the outstanding obligations under the Term Loan prior to

the First Amendment with the remainder of the First Tranche commitment available through December 31, 2021, and up to $10.0 million is available under the second tranche (Second Tranche), subject to the completion of certain clinical and financial milestones, which Second Tranche commitment is available through December 31, 2022. Pursuant to the First Amendment, the Term Loan will mature on January 1, 2026. The First Amendment was determined to be a debt modification.

Pursuant to the First Amendment, the Company is required to make monthly payments of interest only commencing on the first day of the month following the funding date of each respective tranche and continuing thereafter through December 31, 2022 to the extent that the Company does not borrow any part of the Second Tranche or December 31, 2023 if the Company has borrowed some or all of the Second Tranche (the Interest-Only Period). Outstanding principal balances under the Term Loan, as amended by the First Amendment, bear interest at a floating per annum rate equal to (A) if the Company does not borrow under the Second Tranche, the greater of (x) 1% above the prime rate or (y) 4.25%; or (B) if the Company does borrow under the Second Tranche, the greater of (x) 3% above the prime rate or (y) 6.25%.

Following the interest-only period, the outstanding Term Loan balance will be payable in (i) 37 consecutive monthly payments (or 25 if the Company borrows under the Second Tranche) after the end of the Interest-Only Period and continuing on the same day of each month thereafter, in amounts that would fully amortize such Term Loan balance, as of the first business day of the first month following the interest-only period, over the repayment period, plus (ii) monthly payments of accrued but unpaid interest. The final payment due on the maturity date shall include all outstanding principal and all accrued unpaid interest and an End of Term Payment totaling (x) 6% of the original funded principal amount of the First Tranche if the Company does not borrow under the Second Tranche, or (y) 9.5% of the total original funded principal amount under the First and Second Tranche if the Company does borrow under the Second Tranche (the End of Term Payment). The Company may prepay amounts outstanding under the Term Loan at any time provided certain notification conditions are met, in which case, all outstanding principal plus accrued and unpaid interest, the End of Term Payment, a prepayment fee between 1% and 3% of the commitment amount of the First and Second Tranches, and any bank expenses become due and payable.

The Company incurred $10 thousand of debt issuance costs in connection with the First Amendment and had unamortized issuance costs and unamortized debt discount under the Loan Agreement of $2 thousand and $16 thousand, respectively. The Company recorded these costs, including the unaccreted portion of the final payment obligation under the Loan Agreement as a discount from the carrying value of the Term Loan, which are being amortized using the effective interest method over the life of the First Amendment. The unamortized debt issuance costs and debt discount balance was $0.2 million as of March 31, 2021.

The Term Loan and unamortized discount and debt issuance costs balances as of March 31, 2021 are shown below (in thousands):

	March 31, 2021
Total Term Loan debt	$ 5,000
Less: unamortized discount and debt issuance costs	(157)
Total Term Loan, net	4,843
Less: Term Loan, current portion	—
Term loan, net of current portion	$ 4,843

The Company is subject to customary affirmative and restrictive covenants under the Loan Agreement, as amended. The Company's obligations under the Loan Agreement, as amended, are secured by a first priority security interest in substantially all of its current and future assets, other than intellectual property. The Company also agreed not to encumber its intellectual property assets, except as permitted by the Loan Agreement, as amended.

The Loan Agreement, as amended, also contains customary indemnification obligations and customary events of default, including, among other things, the Company's failure to fulfill certain obligations under the Loan Agreement, as amended, and the occurrence of a material adverse change in its business, operations, or condition (financial or otherwise), a material impairment of the prospect of repayment of any portion of the loan, or a material impairment in the perfection or priority of lender's lien in the collateral or in the value of such collateral. In the event of default by the Company under the Loan Agreement, as amended, the lender would be entitled to exercise their remedies thereunder, including the right to accelerate the debt, upon which the Company may be required to repay all amounts then outstanding under the Loan Agreement, as amended. As of March 31, 2021, management believes that the Company was in compliance with all financial covenants under the Loan Agreement, as amended, and there had been no material adverse change.

The Company made interest payments on the Term Loan of $54 thousand and $46 thousand during the three months ended March 31, 2021 and 2020, respectively.

7. Commitments and Contingencies

From time to time, the Company may have certain contingent liabilities that arise in the ordinary course of business. The Company evaluates the likelihood of an unfavorable outcome in legal or regulatory proceedings to which it is a party and records a loss contingency on an undiscounted basis when it is probable that a liability has been incurred and the amount of the loss can be reasonably estimated. These judgments are subjective and based on the status of such legal proceedings, the merits of the Company's defenses, and consultation with legal counsel. Actual outcomes of these legal proceedings may differ materially from the Company's estimates.

License Agreement

In May 2016, the Company entered into a license agreement (the License Agreement), with Eli Lilly and Company (Lilly). Pursuant to the terms of the License Agreement, Lilly granted the Company an exclusive, worldwide, royalty bearing, sublicensable license under certain technology, patent rights, know-how and proprietary materials related to certain compounds, to research, develop, and commercialize such compounds for all pharmaceutical uses. The Company is also required to pay Lilly up to an aggregate of $23.0 million upon the achievement, during the time the License Agreement remains in effect, of certain milestones relating to the clinical development and commercial sales of products licensed under the License Agreement. Such payments are for predetermined fixed amounts, are paid only upon the first occurrence of each event and are due shortly after achieving the applicable milestone. In addition, the Company is required to pay Lilly tiered royalties on annual worldwide net sales with rates ranging from mid-single digits to sub-teens. No additional amounts were paid by the Company to Lilly during any of the periods presented, nor were due as of such dates pursuant to the License Agreement.

Legal Matters

The Company's industry is characterized by frequent claims and litigation, including claims regarding intellectual property. As a result, the Company may be subject to various legal proceedings from time to time. The results of any future litigation cannot be predicted with certainty, and regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors. The Company was not subject to any material legal proceedings during the three months ended March 31, 2021.

8. Capital Structure

Common Stock

As of March 31, 2021 and December 31, 2020, the Company was authorized to issue 200,000,000 shares of common stock $0.0001 par value per share. Holders of the Company's common stock are entitled to dividends if and when declared by the Board of Directors of the Company (Board of Directors). The holder of each share of common stock is entitled to one vote. As of March 31, 2021, no dividends were declared.

Shares reserved for future issuance

Common stock reserved for future issuance, on an as converted basis, consisted of the following:

	March 31, 2021	December 31, 2020
Stock options, issued and outstanding	2,588,298	2,278,771
Stock options, available for future issuance	3,449,095	2,637,076
Employee stock purchase plan, available for future issuance	453,243	220,640
Total shares reserved	6,490,636	5,136,487

Redeemable Convertible Preferred Stock

Immediately prior to the closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock converted into 15,492,019 shares of common stock and the related carrying value was reclassified to common stock and additional paid-in capital. There was no redeemable convertible preferred stock issued or outstanding as of March 31, 2021.

9. Equity Incentive Plans and Stock-Based Compensation Expense

Equity Incentive Plans

In October 2020, the Company adopted the 2020 Equity Incentive Plan (the 2020 Plan), which is a successor to and continuation of the Company's Amended and Restated 2016 Equity Incentive Plan (the 2016 Plan). Total shares reserved under the 2020 Plan was 2,647,684, inclusive of the shares that remained available for issuance under the 2016 Plan at the time the 2020 Plan became effective. Following the effectiveness of the 2020 Plan, no further grants will be made under the 2016 Plan; however, shares subject to awards granted under the 2016 Plan continue to be governed by the 2016 Plan. Any shares subject to outstanding stock options or other stock awards that were granted under the 2016 Plan that terminate or expire prior to exercise or settlement; are settled in cash; are forfeited or repurchased because of the failure to vest; or are reacquired or withheld to satisfy a tax withholding obligation or the purchase or exercise price in accordance with the terms of the 2016 Plan will also be reserved for issuance under the 2020 Plan.

Under the 2020 Plan and the 2016 Plan, individuals can be granted the ability to early exercise their options. There were no shares, related to the early exercise of options, subject to repurchase by the Company as of March 31, 2021.

A summary of the Company's stock option activity and related information is as follows (in thousands, except share and per share amounts):

	Outstanding Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (Years)	Aggregate Intrinsic Value
Balance as of December 31, 2020	2,278,771	$ 2.78	8.6	$ 49,059
Granted	355,000	20.31	—	—
Exercised	(41,473)	1.50	—	—
Forfeited/Cancelled	(4,000)	20.90	—	—
Balance as of March 31, 2021	2,588,298	$ 5.18	8.7	$ 30,984
Vested and expected to vest as of March 31, 2021	2,569,295	$ 5.18	8.7	$ 30,812
Vested and exercisable as of March 31, 2021	683,689	$ 1.93	6.8	$ 10,079

Stock options vested and expected to vest differs from total stock options outstanding as it excludes performance-based stock options for which the performance criteria has not been achieved and achievement is not expected as of March 31, 2021.

The aggregate intrinsic values of options outstanding and exercisable were calculated as the difference between the exercise price of the options and the estimated fair value of the Company's common stock as of the respective balance sheet date. The total intrinsic value of options exercised were $0.9 million for the three months ended March 31, 2021.

As of March 31, 2021, a total of 3,810,703 shares were authorized for issuance under the 2020 Plan and 3,449,095 shares remained available for issuance, and a total of 2,226,690 shares were authorized for issuance under the 2016 Plan and no shares remained available for issuance.

For the three months ended March 31, 2021, the weighted-average fair value of options granted was $15.89 per share.

Stock-Based Compensation Expense

The following table summarizes the components of stock-based compensation expense recognized in the Company's statement of operations and comprehensive loss during the three months ended March 31, 2021 and 2020 (in thousands):

	Three Months Ended March 31,	
	2021	2020
Research and development	$ 264	$ 10
General and administrative	856	22
Total stock-based compensation expense	$ 1,120	$ 32

During 2020, the Company granted options to purchase 127,042 shares of common stock to certain executive officers with performance criteria stipulating that no shares will vest unless certain financing and other related milestones are achieved. In January 2021, the Board of Directors determined that the performance-based vesting criteria of such options had been satisfied, which was deemed a modification. The fair value of the modified awards was $2.4 million, which will be recognized over the requisite service period. For the three months ended March 31, 2021, the Company recorded stock-based compensation of $0.5 million in connection with the modification.

Unrecognized stock-based compensation expense as of March 31, 2021 was approximately $11.9 million, which is expected to be recognized over a weighted-average vesting term of 3.5 years.

Employee Stock Purchase Plan

The Board of Directors adopted and the Company's stockholders approved the 2020 Employee Stock Purchase Plan (the ESPP) in October 2020. The ESPP allows eligible employees to purchase shares of the Company's common stock at a discount through payroll deductions of up to 15% of their eligible compensation. At the end of each purchase period, employees are able to purchase shares at 85% of the lower of the fair market value of the Company's common stock as of the offering date or the applicable purchase date. A total of 220,640 shares of common stock were approved to be initially reserved for issuance under the ESPP. In addition, the number of shares of common stock available for issuance under the ESPP will be automatically increased on the first day of each calendar year during the first ten-years of the term of the ESPP, beginning with January 1, 2021 and ending with January 1, 2030, by an amount equal to the lessor of (i) 1% of the outstanding number of shares of the Company's common stock on December 31st of the preceding calendar year, (ii) 441,280 shares of common stock or (iii) such lesser amount as determined by the Board of Directors. As of March 31, 2021, 453,243 shares of common stock remained available for issuance under the ESPP.

10. Income Taxes

The Company accounts for income taxes under ASC Topic 740, Income Taxes. Under this standard, deferred tax assets and liabilities are recognized for future tax benefits or consequences attributable to temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured 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.

A valuation allowance is provided for significant deferred tax assets when it is more likely than not that such assets will not be realized through future operations. No provision for income taxes has been recorded due to the available net operating loss carry forwards. Future tax benefits which may arise as a result of these losses have not been recognized in these financial statements, as their realization is determined not likely to occur and accordingly, the Company has recorded a valuation allowance for the future deferred tax assets.

11. Net Loss Per Share

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

	Three Months Ended March 31,	
	2021	2020
Numerator:		
Net loss	$ (9,887)	$ (5,168)
Denominator:		
Weighted-average common shares outstanding	23,283,658	764,408
Net loss per share, basic and diluted	$ (0.42)	$ (6.76)

Basic net loss per share was the same as diluted net loss per share for all periods as the inclusion of potentially dilutive securities would have been anti-dilutive. Potentially dilutive securities that were not included in the diluted per share calculations were as follows:

	March 31,	
	2021	2020
Series A redeemable convertible preferred stock (on an if-converted basis)	—	4,280,690
Series B redeemable convertible preferred stock (on an if-converted basis)	—	5,605,661
Shares subject to outstanding common stock options	2,588,298	790,060
Estimated shares issuable under the ESPP	24,118	—
Shares subject to common stock warrants	—	49,609
Total	2,612,416	10,726,020

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed financial statements and related notes to those statements included elsewhere in this Quarterly Report on Form 10-Q and our audited financial statements and notes thereto and the related Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the Securities and Exchange Commission, or SEC, on March 22, 2021, or the Annual Report. Unless otherwise indicated, all references in this Quarterly Report on Form 10-Q to "Spruce," the "company," "we," "our," "us" or similar terms refer to Spruce Biosciences, Inc.

Forward-Looking Statements

In addition to historical financial information, this discussion contains forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the section titled "Risk Factors" under Part II, Item 1A below. In some cases, you can identify forward-looking statements by terminology such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potentially," "predict," "should," "will" or the negative of these terms or other similar expressions.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

Overview

We are a late-stage biopharmaceutical company focused on developing and commercializing novel therapies for rare endocrine disorders with significant unmet medical need. We are initially developing our wholly-owned product candidate, tildacerfont, as the potential first non-steroidal therapy to offer markedly improved disease control and reduce steroid burden for patients suffering from classic congenital adrenal hyperplasia, or CAH. Classic CAH is a serious and life-threatening disease with no known novel therapies approved in approximately 50 years. In a 12-week Phase 2a proof-of-concept clinical trial, tildacerfont-treated adult patients suffering from classic CAH who had poor disease control despite being on standard of care therapy achieved approximately 80% reductions in hormones that are key indicators of poor disease control. Furthermore, we are developing tildacerfont for females suffering from a rare form of polycystic ovary syndrome, or PCOS, with primary adrenal androgen excess, representing 3-5% of females with PCOS (estimated to be 150,000 to 200,000 patients in the United States). 171 subjects across seven clinical trials to date have been administered tildacerfont with no drug-related serious adverse events, or SAEs, reported.

We have initiated CAHmelia-203, a placebo-controlled, double-blind Phase 2b clinical trial in adult patients with classic CAH with poor disease control and anticipate topline results in the first half of 2022. We have also initiated CAHmelia-204, a second Phase 2b clinical trial in adult patients with classic CAH with good disease control focused on glucocorticoid reduction and anticipate topline results in the second half of 2022. Based on post-hoc analyses of our clinical data to date, we have chosen to target two distinct groups of classic CAH patients with either good disease control or poor disease control. These two groups, which together make up the entire classic CAH patient population, have differing disease challenges centered on excessive adrenal androgen levels or excessive glucocorticoid usage, both of which have the potential to be addressed by treatment with tildacerfont, if approved. We believe our strategy to study CAH patients in these two enriched sub-populations may enable us to observe clinically meaningful outcomes. Additionally, we believe these two clinical trials will provide sufficient patient exposures for our registrational safety database. Assuming positive results in CAHmelia-204, we plan to meet with the U.S. Food and Drug Administration, or FDA, and comparable foreign regulatory authorities to discuss registration.

We also plan to investigate tildacerfont for the treatment of classic CAH in children as young as two years of age, and plan to initiate the clinical development program for tildacerfont in the pediatric classic CAH population in the second half of 2021. We have received feedback from the FDA and European Medicines Agency, or EMA, on our planned Phase 2 clinical trial of tildacerfont in children with classic CAH. We have also submitted a pediatric investigational plan, or PIP, to the Pediatric Committee of the EMA regarding a registrational program in children with classic CAH. Beyond classic CAH, we believe tildacerfont has potential utility in a range of diseases where the underlying biology supports a need to reduce excess secretion of or hyperresponsiveness to adrenocorticotropic hormone, or ACTH. We are committed to leveraging our deep scientific knowledge of the biology of rare endocrine disorders, the benefits of tildacerfont, and our commercial expertise to dramatically transform the lives of individuals living with these devastating disorders.

PCOS is a hormonal disorder common among females of reproductive age affecting nearly five million females in the United States and approximately 115 million females worldwide. PCOS is characterized by elevated levels of androgens, cysts in the ovaries, and irregular periods. We have identified a subpopulation of patients where elevated levels of adrenal androgens are the cause of disease. We believe that tildacerfont may present a novel mechanism to reduce ACTH and provide a therapeutic option for females with this rare form of PCOS, representing 3-5% of females with PCOS (estimated to be 150,000 to 200,000 patients in the United States). We plan to file an investigational new drug application, or IND, to study tildacerfont in this patient population in the first half of 2021 and are pursuing orphan drug designation in this patient population. By leveraging our existing Phase 1 program, which includes safety, tolerability, and pharmacokinetics of tildacerfont, subject to the clearance of our planned IND, we plan to initiate a Phase 2 proof-of-concept clinical trial in the second half of 2021.

Since our inception in November 2014, we have focused primarily on raising capital, establishing and protecting our intellectual property portfolio, organizing and staffing our company, business planning, and conducting preclinical and clinical development of, and manufacturing development for, our product candidate, tildacerfont. We have no products approved for commercial sale and have not generated any revenue to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. Our ability to generate product revenue sufficient to achieve profitability, if ever, will depend on the successful development of tildacerfont and any future product candidates. We intend to build a highly specialized commercial organization to support the commercialization of tildacerfont, if approved, in the United States and Europe. Given a relatively small number of endocrinologists and specialists treat a large proportion of patients with classic CAH, we believe this market can be effectively addressed with a modest-sized targeted commercial sales force, alongside various high-touch patient initiatives. If tildacerfont is approved for additional indications, we plan to leverage our rare disorder commercial infrastructure and expertise to efficiently address those patient populations. We may also either build a commercial infrastructure or opportunistically seek strategic collaborations to benefit from the resources of biopharmaceutical companies specialized in either relevant disease areas or geographies.

We rely, and expect to continue to rely, on third parties for the manufacture of tildacerfont for preclinical studies and clinical trials, as well as for commercial manufacture if tildacerfont obtains marketing approval. We also rely, and expect to continue to rely, on third parties to package, label, store, and distribute tildacerfont, if marketing approval is obtained. We believe that this strategy allows us to maintain a more efficient infrastructure by eliminating the need for us to invest in our own manufacturing facilities, equipment, and personnel while also enabling us to focus our expertise and resources on the development of tildacerfont.

Since inception, we have incurred significant losses and negative cash flows from operations. During the three months ended March 31, 2021, we incurred a net loss of $9.9 million and used $8.8 million of cash in operations. As of March 31, 2021, we had an accumulated deficit of $70.7 million, and we do not expect positive cash flows from operations for the foreseeable future. We expect to continue to incur significant and increasing losses for the foreseeable future, and our net losses may fluctuate significantly from period to period, depending on the timing of expenditures on our planned research and development activities.

In October 2020, we consummated our initial public offering, or IPO, and issued 6,900,000 shares of common stock for net proceeds of $93.4 million, after deducting underwriting discounts and commissions and offering expenses. Since inception through March 31, 2021, we have raised aggregate gross proceeds of $224.5 million, including $103.5 million from our IPO in October 2020, $116.0 million from the sale of our redeemable convertible preferred stock and $5.0 million from the issuance of debt. As of March 31, 2021, we had cash and cash equivalents of $148.6 million. We believe, based on our current operating plan, that our cash and cash equivalents as of March 31, 2021 will be sufficient to fund our operations for at least the next 12 months. We have based this projection on assumptions that may be inaccurate and as a result, we may utilize our capital resources sooner than we expect. We expect our expenses will increase significantly in connection with our ongoing activities, as we:

- advance tildacerfont through our ongoing Phase 2b clinical trials in adult patients with classic CAH;
- pursue regulatory approvals of tildacerfont in adult patients with classic CAH;
- advance clinical development of tildacerfont in additional indications, including pediatric classic CAH and a subpopulation of females with a rare form of PCOS;
- build a highly specialized commercial organization to support the commercialization of tildacerfont, if approved, in the United States and Europe;
- build a commercial infrastructure or opportunistically seek strategic collaborations to benefit from the resources of biopharmaceutical companies specialized in either relevant disease areas or geographies, if tildacerfont is approved for additional indications;
- identify additional indications and formulations for which to investigate tildacerfont in the future and expand our pipeline of product candidates;
- implement operational, financial, and management information systems;

- hire additional personnel;
- operate as a public company; and
- obtain, maintain, expand, and protect our intellectual property portfolio.

Our business has been and could continue to be adversely affected by the evolving COVID-19 pandemic. For example, the COVID-19 pandemic has resulted in and could result in delays to our clinical trials for numerous reasons including additional delays or difficulties in enrolling patients, diversion of healthcare resources away from the conduct of clinical trials, interruption or delays in the operations of the FDA or other regulatory authorities, and delays in clinical sites receiving the supplies and materials to conduct our clinical trials. At this time, the extent to which the COVID-19 pandemic impacts our business will depend on future developments, which are highly uncertain and cannot be predicted.

License Agreement with Eli Lilly and Company

In May 2016, we entered into a license agreement, or the License Agreement, with Eli Lilly and Company, or Lilly. Pursuant to the terms of the License Agreement, Lilly granted us an exclusive, worldwide, royalty bearing, sublicensable license under certain technology, patent rights, know-how, and proprietary materials, which we refer to collectively as the Lilly IP, and such patents, the Lilly Licensed Patents, relating to the CRF1 receptor antagonist compounds either listed in the License Agreement or covered by patent rights controlled by Lilly, which we refer to collectively as the Lilly Compounds, to research, develop, commercialize, make, have made, use, sell, offer to sell, and import the Lilly Compounds and any products containing a Lilly Compound, including any products containing a Lilly Compound and one or more additional active pharmaceutical ingredients, or APIs, other than a Lilly Compound, which we refer to collectively as the Lilly Licensed Products, for all pharmaceutical uses, including all diagnostic, therapeutic, and prophylactic uses, for human or animal administration. Lilly retained rights under the Lilly IP and the Lilly Licensed Patents for internal research purposes.

As partial consideration for the rights granted to us under the License Agreement, we made a one-time upfront payment to Lilly of $0.8 million. We are also required to pay Lilly up to an aggregate of $23.0 million upon the achievement, during the time the License Agreement remains in effect, of certain milestones relating to the clinical development and commercial sales of the Lilly Licensed Products. Such payments are for predetermined fixed amounts, are paid only upon the first occurrence of each such event, and are due shortly after achieving the applicable milestone. In addition, we are required to pay Lilly tiered royalties on annual worldwide net sales of Lilly Licensed Products, with rates ranging from mid-single-digits to sub-teens, or the Lilly Royalties. The Lilly Royalties shall commence on a country-by-country basis on the date of the first commercial sale of Lilly Licensed Product in such country, and shall expire on a country-by-country basis on the latest of the following dates: (i) the tenth anniversary of the date of first commercial sale in such country, (ii) the expiration in such country of the last-to-expire Lilly Licensed Patent having a valid claim covering the manufacture, use, or sale of the Lilly Licensed Product as commercialized in such country, and (iii) the expiration of any data or regulatory exclusivity period for the Lilly Licensed Product in such country. Upon such expiration, the license granted to us with respect to such country shall become fully paid-up, royalty-free, perpetual and irrevocable. In addition, the Lilly Royalties may be reduced upon the occurrence of certain events.

Components of Results of Operations

Operating Expenses

We classify operating expenses into two main categories: (i) research and development expenses and (ii) general and administrative expenses.

Research and Development Expenses

Our research and development expenses consist of external and internal expenses incurred in connection with our research activities and development programs.

These expenses include:

- external expenses, consisting of:
  - clinical development—expenses associated with clinical research organizations, or CROs, engaged to manage and conduct clinical trials;
  - preclinical studies—expenses associated with preclinical studies performed by vendors;
  - manufacturing—expenses associated with contract manufacturing; labeling, packaging, and distribution of clinical trial supplies; and consulting;
  - other research and development—expenses associated with quality and regulatory compliance; and

- internal expenses, consisting of personnel, including expenses for salaries, bonuses, benefits, stock-based compensation, as well as allocation of certain expenses.

To date, most of these expenses have been incurred to advance tildacerfont. These expenses will primarily consist of expenses for the administration of clinical trials as well as manufacturing costs for clinical drug supply. We expect that significant additional spending will be required to progress tildacerfont through clinical development and regulatory approval.

Research and development expenses are recognized as they are incurred. If deposits are required by external vendors, a portion of the deposit is included as a prepaid expense until sufficient progress has occurred to amortize the deposit to expense in the statement of operations.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs (including salaries, bonuses, benefits, and stock-based compensation expense) for personnel in executive, finance, and other administrative functions. General and administrative expenses also include legal fees, professional fees, insurance costs, facility costs not otherwise included in research and development expenses, and public company expenses such as costs associated with compliance with the rules and regulations of the SEC, and those of the Nasdaq Stock Market, Inc., or Nasdaq, listing rules.

We expect that our general and administrative expenses will continue to increase in the foreseeable future as additional administrative personnel and services are required to manage these functions of a public company, as we advance tildacerfont through clinical development and regulatory approval.

Interest Expense

Interest expense consists of interest incurred and non-cash amortization of debt discount and issuance costs in connection with the term loan, or Term Loan, provided pursuant to the Loan and Security Agreement entered into with Silicon Valley Bank in September 2019, or the Loan Agreement, as amended by a First Amendment to Loan and Security Agreement entered into with Silicon Valley Bank in March 2021, or the First Amendment.

Other Income, Net

Other income, net primarily consists of interest income earned on our cash and cash equivalents.

Results of Operations

Comparisons of the Three Months Ended March 31, 2021 and 2020

The following table summarizes our results of operations for the periods presented (in thousands):

	Three Months Ended March 31,		
	2021	2020	Change
Operating expenses:			
Research and development	$ 6,714	$ 4,610	$ 2,104
General and administrative	3,103	523	2,580
Total operating expenses	9,817	5,133	4,684
Loss from operations	(9,817)	(5,133)	(4,684)
Interest expense	(89)	(74)	(15)
Other income, net	19	39	(20)
Net loss	$ (9,887)	$ (5,168)	$ (4,719)

Research and Development Expenses

Research and development expenses were $6.7 million for the three months ended March 31, 2021, compared to $4.6 million for the three months ended March 31, 2020. The overall increase in research and development expenses was primarily related to an increase in clinical development, manufacturing, and personnel costs, associated with progressing clinical development. The following table sets forth the primary external and internal research and development expenses for the periods presented below (in thousands).

	Three Months Ended March 31,		
	2021	2020	Change
External expenses:			
Clinical development	$ 3,644	$ 2,257	$ 1,387
Manufacturing	545	696	(151)
Preclinical studies	581	350	231
Other research and development	267	73	194
Internal expenses:			
Personnel	1,601	1,185	416
Allocated overhead	76	49	27
Total research and development expenses	$ 6,714	$ 4,610	$ 2,104

General and Administrative Expenses

General and administrative expenses were $3.1 million for the three months ended March 31, 2021, compared to $0.5 million for the three months ended March 31, 2020. The overall increase in general and administrative expenses was primarily related to an increase of $1.3 million in personnel-related expenses, an increase of $0.7 million in legal and professional fees primarily related to operating as a public company, and an increase of $0.4 million in directors' and officers' liability insurance premiums.

Interest Expense

Interest expense was $0.1 million for the three months ended March 31, 2021, compared to $0.1 million interest expense for the three months ended March 31, 2020. Interest expense incurred relate to the Term Loan with Silicon Valley Bank.

Other Income, Net

Other income, net was minimal for the three months ended March 31, 2021 and 2020.

Liquidity and Capital Resources

Liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from operations. We anticipate that we will continue to incur net losses for the foreseeable future. As of March 31, 2021, we had an accumulated deficit of $70.7 million. As of March 31, 2021, we had cash and cash equivalents of $148.6 million. In October 2020, we consummated our IPO and issued 6,900,000 shares of common stock for net proceeds of approximately $93.4 million, after deducting underwriting discounts and commissions and offering expenses. We believe, based on our current operating plan, that our cash and cash equivalents as of March 31, 2021 will be sufficient to fund our operations for at least the next 12 months.

Contractual Obligations and Commitments

During the three months ended March 31, 2021, there were no material changes to our contractual obligations and commitments from those described under "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report.

We enter into contracts in the normal course of business with third-party contract manufacturing organizations and CROs for clinical trials, non-clinical studies and testing, drug substance and product manufacturing and other services for operating purposes. These contracts are generally cancelable by us upon prior written notice after a certain period. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation.

We have also entered into the License Agreement under which we are obligated to make aggregate milestone payments upon the achievement of specified milestones as well as royalty payments. The payment obligations under the License Agreement are contingent upon future events, such as our achievement of specified milestones or generating product sales. As of March 31, 2021, we were unable to estimate the timing or likelihood of achieving these milestones or generating future product sales. See the section titled “License Agreement with Lilly and Company” above.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Loan Agreement

In September 2019, we entered into the Loan Agreement with Silicon Valley Bank providing for the Term Loan. Pursuant to the Loan Agreement, we requested $2.5 million from the first tranche in connection with the entry into the Loan Agreement and drew the second tranche of $2.0 million in December 2019.

In April 2020, we and Silicon Valley Bank entered into the Deferral Agreement whereby we and Silicon Valley Bank agreed to extend the repayment dates of all monthly payments of principal due and the maturity date with respect to the Term Loan by six months. In March 2021, we entered into the First Amendment with Silicon Valley Bank. The First Amendment increased the aggregate principal amount of the Term Loan commitment by Silicon Valley Bank to up to $30.0 million. Up to $20.0 million is available under the first tranche of the Term Loan, or the First Tranche, $5.0 million of which was advanced immediately to repay the outstanding obligations under the Term Loan prior to the First Amendment with the remainder of the First Tranche commitments available through December 31, 2021, and up to $10.0 million is available under the second tranche, or the Second Tranche, subject to the completion of certain clinical and financial milestones which Second Tranche commitment is available through December 31, 2022. Pursuant to the First Amendment, the Term Loan will mature on January 1, 2026. As of March 31, 2021, $5.0 million was outstanding under the Term Loan.

The Loan Agreement, as amended by the Deferral Agreement and the First Amendment, provides for monthly cash interest-only payments following the funding date of each respective tranche and continuing thereafter through December 31, 2022 to the extent that the Company does not borrow any part of the Second Tranche or December 31, 2023 if the Company has borrowed some or all of the Second Tranche. Outstanding principal balances under the Term Loan, as amended by the First Amendment, bear interest at a floating per annum rate equal to (A) if the Company does not borrow under the Second Tranche, the greater of (x) 1% above the prime rate or (y) 4.25%; or (B) if the Company does borrow under the Second Tranche, the greater of (x) 3% above the prime rate or (y) 6.25%.

Following the interest-only period, the outstanding Term Loan balance will be payable in (i) 37 consecutive monthly payments (or 25 if the Company borrows under the Second Tranche) after the end of the interest-only period and continuing on the same day of each month thereafter, in amounts that would fully amortize such Term Loan balance, as of the first business day of the first month following the amendment interest-only period, over the repayment period, plus (ii) monthly payments of accrued but unpaid interest. The final payment due on the maturity date shall include all outstanding principal and all accrued unpaid interest and an end of term payment totaling (x) 6% of the original funded principal amount of the First Tranche if the Company does not borrow under the Second Tranche, or (y) 9.5% of the total original funded principal amount under the First and Second Tranche if the Company does borrow under the Second Tranche (the End of Term Payment).

We may prepay amounts outstanding under the Term Loan at any time provided certain notification conditions are met, in which case, all outstanding principal plus accrued and unpaid interest, the End of Term Payment, a prepayment fee between 1% and 3% of the principal amount of the first and second tranches, and any bank expenses become due and payable.

The Loan Agreement, as amended, contains certain covenants that limit our ability to engage in certain transactions that may be in our long-term best interest, including entering into a change in control transaction. The Loan Agreement, as amended, also contains certain covenants that limit our ability to obtain additional debt financing, including incurring debt from third parties not permitted under the Loan Agreement, as amended, or incurring liens or encumbrances on our property. While we have not previously breached and are currently in compliance with the covenants contained in the Loan Agreement, as amended, we may breach these covenants in the future. Our ability to comply with these covenants may be affected by events and factors beyond our control. In the event that we breach one or more covenants, Silicon Valley Bank may choose to declare an event of default and require that we immediately repay all amounts outstanding under the Loan Agreement, terminate any commitment to extend further credit and foreclose on the collateral. In addition, if an event of default occurs under the Loan Agreement, as amended, Silicon Valley Bank may, among other things, accelerate the Term Loan or do any acts it considers necessary or reasonable to protect its security interest in the collateral under the Term Loan. Events of default include the occurrence of a material adverse change in our business, operations, or condition (financial

or otherwise). The occurrence of any of these events could have a material adverse effect on our business, financial condition, and results of operations.

In connection with the first and second tranches under the Loan Agreement prior to the First Amendment, we issued a warrant to purchase up to an aggregate of 49,609 shares of common stock at $1.44 per share. We determined the initial fair value of the warrant to be $0.1 million using the Black-Scholes option-pricing model. The fair value of the warrant was recorded to equity and also as a debt discount, which is amortized to interest expense using the effective interest method over the term of the Term Loan. The warrant was net-exercised for 46,358 shares of our common stock in November 2020.

Funding Requirements

To date, we have not generated any revenue. We do not expect to generate any meaningful revenue unless and until we obtain regulatory approval and commercialize tildacerfont or any future product candidates, and we do not know when, or if at all, that will occur. We will continue to require additional capital to develop tildacerfont and fund operations for the foreseeable future. Our primary uses of cash are to fund our operations, which consist primarily of research and development expenses related to our clinical development programs, and to a lesser extent, general and administrative expenses.

At this time, we cannot reasonably estimate or know the nature, timing, and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, tildacerfont or any of our future product candidates. We expect our research and development expenses to increase significantly in the foreseeable future as we continue to invest in research and development activities related to developing tildacerfont, as tildacerfont continues advancing in late stage studies for the treatment of classic CAH in adult patients, as we conduct clinical trials of tildacerfont in additional indications beyond classic CAH in adult patients, as we seek regulatory approvals for tildacerfont, and incur expenses associated with hiring additional personnel to support our research and development efforts. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, the successful development of tildacerfont is highly uncertain, and we may never succeed in achieving regulatory approval for tildacerfont in classic CAH in adult patients or other indications. In addition, we expect to incur additional costs associated with operating as a public company.

We may seek to raise capital through equity or debt financings, collaborative agreements or other arrangements with other companies, or through other sources of financing. Adequate additional funding may not be available to us on acceptable terms or at all. Our failure to raise capital as and when needed could have a negative impact on our financial condition and our ability to pursue our business strategies. We anticipate that we will need to raise substantial additional capital, the requirements of which will depend on many factors, including:

- the progress, costs, trial design, results of, and timing of our ongoing and planned clinical trials of tildacerfont;
- the outcome, costs and timing of seeking and obtaining FDA and any other regulatory approvals;
- the number and characteristics of product candidates that we may pursue;
- our ability to manufacture sufficient quantities of tildacerfont;
- our plan to expand our research and development activities;
- the costs associated with manufacturing tildacerfont and establishing commercial supplies and sales, marketing, and distribution capabilities;
- the costs associated with securing and establishing commercialization;
- the costs of acquiring, licensing, or investing in product candidates;
- our ability to maintain, expand, and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense, and enforcement of any patents or other intellectual property rights;
- our need and ability to retain key management and hire scientific, technical, business, and medical personnel;
- the effect of competing products and product candidates and other market developments;
- the timing, receipt, and amount of sales from tildacerfont and any future product candidates, if approved;
- our need to implement additional internal systems and infrastructure, including financial and reporting systems;
- the economic and other terms, timing of, and success of any collaboration, licensing, or other arrangements which we may enter in the future; and
- the effects of the disruptions to and volatility in the credit and financial markets in the United States and worldwide from the COVID-19 pandemic.

If we raise additional funds by issuing equity securities, our stockholders will experience dilution. If we raise additional capital through debt financing, we may be subject to covenants that restrict our operations including limitations on our ability to incur liens or additional debt, pay dividends, repurchase our common stock, make certain investments, and engage in certain merger, consolidation, or asset sale transactions. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders.

We may be unable to raise additional funds or to enter into such agreements or arrangements on favorable terms, or at all. Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and the disruptions to, and volatility in, the credit and financial markets in the United States and worldwide resulting from the COVID-19 pandemic and actions taken to slow its spread, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back, or discontinue the development or commercialization of tildacerfont or other research and development initiatives. We also could be required to seek collaborators for tildacerfont and any future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to tildacerfont and any future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Summary Statement of Cash Flows

The following table sets forth the primary sources and uses of cash, cash equivalents, and restricted cash for the periods presented below (in thousands):

	Three Months Ended March 31,	
	2021	2020
Net cash used in operating activities	$ (8,787)	$ (5,956)
Net cash used in investing activities	(19)	-
Net cash provided by financing activities	283	43,655
Net increase in cash, cash equivalents, and restricted cash	$ (8,523)	$ 37,699

Cash Used in Operating Activities

For the three months ended March 31, 2021, net cash used in operating activities was $8.8 million, which consisted of a net loss of $9.9 million, partially offset by and a net change of $0.1 million in our net operating assets and liabilities and by $1.2 million in non-cash charges. The net change in our operating assets and liabilities was primarily due to a decrease in prepaid expenses and other current assets of $0.3 million, offset by a decrease in accounts payable and accrued expenses of $0.1 million, and a decrease in accrued compensation and noncurrent assets and liabilities of $0.3 million. The non-cash charges of $1.2 million consisted of stock-based compensation expense, non-cash lease expense, depreciation and amortization expense.

For the three months ended March 31, 2020, net cash used in operating activities was $6.0 million, which consisted of a net loss of $5.2 million, partially offset by and a net change of $0.8 million in our net operating assets and liabilities and by $33 thousand in non-cash charges. The net change in our operating assets and liabilities was primarily due to a decrease in prepaid expenses and other current assets of $56 thousand, offset by a decrease in accounts payable and accrued expenses of $0.3 million, and a decrease in accrued compensation and noncurrent assets and liabilities of $0.7 million. The non-cash charges of $33 thousand consisted of stock-based compensation expense and depreciation and amortization expense.

Cash Used in Investing Activities

For the three months ended March 31, 2021 and 2020, cash used in investing activities was less than $0.1 million and related to the purchase of property and equipment.

Cash Provided by Financing Activities

For the three months ended March 31, 2021, cash provided by financing activities was $0.3 million, consisting primarily of net proceeds from the issuance of long-term debt and proceeds from exercise of stock options.

For the three months ended March 31, 2020, cash provided by financing activities was $43.7 million primarily from the issuance and sale of Series B redeemable convertible preferred stock.

Critical Accounting Policies, Significant Judgments and Use of Estimates

Our condensed financial statements have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these condensed financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities and expenses, as well as the related disclosure of contingent assets and liabilities as of the date of the financial statements. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

Our critical accounting policies are described in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies, Significant Judgments and Use of Estimates" in the Annual Report and the notes to our condensed financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. During the three months ended March 31, 2021, there were no material changes to our critical accounting policies from those discussed in the Annual Report.

JOBS Act

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or JOBS Act. The JOBS Act permits emerging growth companies to take advantage of an extended transition period to comply with new or revised accounting standards, delaying the adoption of these accounting standards until they would apply to private companies. We have elected to use this extended transition period under the JOBS Act until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, our financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We could be an emerging growth company until December 31, 2025, although circumstances could cause us to lose that status earlier, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

Our cash and cash equivalents as of March 31, 2021 consisted of readily available checking and money market funds. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition and/or results of operations. We do not believe that our cash or cash equivalents have significant risk of default or illiquidity. While we believe our cash and cash equivalents do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash and cash equivalents at one financial institution that is in excess of federally insured limits.

Additionally, the interest rate for borrowings under the Loan Agreement is variable. A hypothetical 10% relative change in interest rates during any of the periods presented would not have had a material impact on our financial statements. We do not currently engage in hedging transactions to manage our exposure to interest rate risk.

Foreign Currency Rate Risk

Our operations include activities in the United States. In addition, we contract with vendors that are located outside of the United States and certain invoices are denominated in foreign currencies. While our operating results are exposed to changes in foreign currency exchange rates between the U.S. dollar and various foreign currencies, we do not believe that inflation, interest rate changes, or exchange rate fluctuations had a significant impact on our results of operations for any periods presented herein.

There was no material foreign currency risk for the three months ended March 31, 2021.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our Chief Executive Officer and our Chief Financial Officer, have evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that, as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls and procedures are effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) during the quarter ended March 31, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitation on the Effectiveness of Disclosure Controls and Procedures

In designing and evaluating our disclosure controls and procedures and internal control over financial reporting, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable, not absolute, assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures and internal control over financial reporting must reflect the fact that there are resource constraints and our management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs. The design of any disclosure controls and procedures and internal control over financial reporting also are based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently a party to any material legal proceedings. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity, reputational harm and other factors.

Item 1A. Risk Factors

An investment in shares of common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this Quarterly Report on Form 10-Q, including our unaudited condensed financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the risks described below could harm our business, financial condition, results of operations, growth prospects, and/or stock price or cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business. We have marked with an asterisk (*) those risk factors that reflect changes from the similarly titled risk factors included in the Annual Report.

Risks Related to Our Business and Industry

We have a limited operating history, have incurred significant net losses since our inception, and anticipate that we will continue to incur significant net losses for the foreseeable future.*

We are a late-stage biopharmaceutical company founded in 2014, and our operations to date have focused primarily on raising capital, establishing and protecting our intellectual property portfolio, organizing and staffing our company, business planning, and conducting preclinical and clinical development of, and manufacturing development for, our only product candidate, tildacerfont. Additionally, as an organization, we have not yet demonstrated an ability to successfully complete clinical development, obtain regulatory approvals, manufacture a commercial-scale product, or conduct sales and marketing activities necessary for successful commercialization. As we build our capabilities and expand our organization, we have not yet demonstrated an ability to overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing biopharmaceutical products.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effectiveness in the targeted indication or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred significant net losses since our inception. If tildacerfont is not successfully developed and approved in the United States or Europe, we may never generate any revenue. For the three months ended March 31, 2021, we reported a net loss of $9.9 million. As of March 31, 2021, we had an accumulated deficit of $70.7 million.

We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our clinical development of, seek regulatory approvals for, and commercially launch tildacerfont and any future product candidates, if approved. We may encounter unforeseen expenses, difficulties, complications, delays, and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. Our prior net losses and expected future net losses have had and will continue to have an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses, or when, if at all, we will be able to achieve profitability.

We will need substantial additional financing to develop tildacerfont and any future product candidates and implement our operating plan. If we fail to obtain additional financing, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.*

Our operations have consumed substantial amounts of cash since our inception. We expect to continue to spend substantial amounts to continue the clinical development of, and seek regulatory approval for, tildacerfont and any future product candidates. We will require significant additional amounts in order to prepare for commercialization, and, if approved, to launch and commercialize tildacerfont.

As of March 31, 2021, we had cash and cash equivalents of $148.6 million. In October 2020, we consummated our IPO and issued 6,900,000 shares of common stock for net proceeds of $93.4 million, after deducting underwriting discounts and commissions and offering expenses. We believe, based on our current operating plan, that our cash and cash equivalents as of March 31, 2021 will be sufficient to fund our operations for at least the next 12 months.

However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. For example, as a result of the COVID-19 pandemic, we have amended our clinical trial protocols to enable remote visits to mitigate any potential impacts. As a result of this home health component, the overall costs of our Phase 2b clinical trials have increased and may continue to increase in the future.

We will require additional capital for the further development and commercialization of tildacerfont and any future product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

Additional funding may not be available on acceptable terms, or at all. As a result of the COVID-19 pandemic and actions taken to slow its spread, the global credit and financial markets have experienced volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly or more dilutive. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back, or discontinue the development or commercialization of tildacerfont or other research and development initiatives. We also could be required to seek collaborators for tildacerfont and any future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to tildacerfont and any future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Any of the above events could significantly harm our business, prospects, financial condition, and results of operations and cause the price of our common stock to decline.

We currently depend entirely on the success of tildacerfont, which is our only product candidate. If we are unable to advance tildacerfont in clinical development, obtain regulatory approval, and ultimately commercialize tildacerfont, or experience significant delays in doing so, our business will be materially harmed.*

We currently only have one product candidate, tildacerfont, and our business and future success depends entirely on our ability to develop, obtain regulatory approval for, and then successfully commercialize, tildacerfont, which is currently in clinical development for adult patients with classic CAH. This may make an investment in our company riskier than similar companies that have multiple product candidates in active development that may be able to better sustain failure of a lead product candidate. We have initiated CAHmelia-203, a placebo-controlled, double-blind Phase 2b clinical trial in adult patients with classic CAH with poor disease control and anticipate topline results in the first half of 2022. We have also initiated CAHmelia-204, a second Phase 2b clinical trial in adult patients with classic CAH with good disease control focused on glucocorticoid reduction and anticipate topline results in the second half of 2022.

Assuming positive results in CAHmelia-204, we plan to meet with the FDA and comparable foreign regulatory authorities to discuss registration. While we believe these two clinical trials will provide sufficient patient exposures for our registrational safety database, the FDA and comparable foreign regulatory authorities may not agree and may require us to enroll additional patients or initiate one or more additional clinical trials, including a Phase 3 clinical trial or trials. If the FDA or comparable foreign regulatory authorities require us to conduct one or more clinical trials, including a Phase 3 clinical trial or trials, the design, duration, and scope of such clinical trials will be decided upon after further discussions with the FDA or comparable foreign regulatory authorities, and at this time are not ascertainable. As a result, we are unable to predict with certainty the estimated timing or scope of any future clinical trials of tildacerfont we may be required to conduct.

In addition, we have received feedback from the FDA and EMA on our planned Phase 2 clinical trial of tildacerfont in children with classic CAH in order to initiate the clinical development program for tildacerfont in the pediatric classic CAH population in the second half of 2021. We also plan to file an IND to study tildacerfont in a subpopulation of females with a rare form of PCOS in the first half of 2021 and plan to pursue orphan drug designation in this patient population. By leveraging our existing Phase 1 program, which includes safety, tolerability, and pharmacokinetics of tildacerfont, subject to the clearance of our planned IND, we plan to initiate a Phase 2 proof-of-concept clinical trial in the second half of 2021. The COVID-19 pandemic continues to evolve and any impacts on these projected milestones are highly uncertain and cannot be predicted with confidence.

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

- successful enrollment in our ongoing and planned clinical trials and completion of such clinical trials with favorable results;
- acceptance by the FDA and EMA of data from our ongoing Phase 2b clinical trials in adult patients with classic CAH;
- demonstrating safety and efficacy to the satisfaction of applicable regulatory authorities;
- the outcome, timing, and cost of meeting regulatory requirements established by the FDA, EMA, and other comparable foreign regulatory authorities;
- receipt of marketing approvals from applicable regulatory authorities, including one or more NDAs from the FDA, and maintaining such approvals;
- establishing commercial manufacturing capabilities and receiving/importing commercial supplies approved by the FDA and other regulatory authorities from any future third-party manufacturer;
- establishing sales, marketing, and distribution capabilities and commercializing tildacerfont, if approved, whether alone or in collaboration with others;
- establishing and maintaining patent and trade secret protection and regulatory exclusivity for tildacerfont;
- maintaining an acceptable safety profile of tildacerfont following approval; and
- maintaining and growing an organization of people who can develop and, if approved, commercialize, market, and sell tildacerfont to physicians, patients, healthcare payors, and others in the medical community.

If we do not achieve one or more of these factors, many of which are beyond our control, in a timely manner or at all, we could experience significant delays or an inability to obtain regulatory approvals or commercialize tildacerfont.

Even if regulatory approvals are obtained, we may never be able to successfully commercialize tildacerfont. In addition, we will need to transition at some point from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. Accordingly, we may not be able to generate sufficient revenue through the sale of tildacerfont to continue our business.

Our clinical trials may fail to adequately demonstrate the safety and efficacy of tildacerfont, which could prevent or delay regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of a product candidate, we must demonstrate through lengthy, complex, and expensive preclinical testing and clinical trials that a product candidate is both safe and effective for use in each target indication. Clinical trials often fail to demonstrate safety and efficacy of the product candidate studied for the target indication. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. We are seeking to develop treatments for rare endocrine disorders for which there is limited clinical experience, and our two ongoing Phase 2b clinical trials use novel endpoints that do not have regulatory precedent in classic CAH due to the lack of clinical trials in classic CAH, which add complexity to the conduct and analysis of data from our clinical trials and may delay or prevent regulatory approval. Additionally, any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of tildacerfont in other indications.

Preclinical and clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of tildacerfont and any future product candidates.

Preclinical and clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. A failure of one or more preclinical or clinical trials can occur at any stage of testing. The results of preclinical studies and early clinical trials of tildacerfont may not be predictive of the results of later-stage clinical trials. However, product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. For example, tildacerfont has not yet been evaluated in pediatric patients with classic CAH, and the results may not be similar to the results observed in clinical trials of adult patients. In addition, we intend to use doses in our two Phase 2b clinical trials that may not be safe or efficacious doses. As such, our hypotheses of efficacy may not show the desired clinical results. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or safety profiles, notwithstanding promising results in earlier trials. Moreover, preclinical and clinical data is often susceptible to varying interpretations and analyses. We may face significant setbacks as we conduct our two Phase 2b clinical trials in adult patients with classic CAH, which may delay or prevent regulatory approval of tildacerfont.

Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits, or otherwise fail to follow clinical trial protocols, whether as a result of the COVID-19 pandemic, actions taken to slow the spread of COVID-19 or otherwise, the integrity of data from our clinical trials may be compromised or not accepted by the FDA or other regulatory authorities, which would represent a significant setback for the applicable program.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may not be able to initiate or continue our clinical trials for tildacerfont and any future product candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA and comparable foreign regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the clinical trial, the design of the clinical trial, competing clinical trials, and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

In particular, each indication for which we are evaluating tildacerfont is a rare endocrine disorder with limited patient populations from which to draw participants in clinical trials. For example, we estimate the total classic CAH populations are approximately 20,000 to 30,000 people in the United States and approximately 50,000 people in the European Union, or EU. We will be required to identify and enroll a sufficient number of patients with the disorder under investigation for our clinical trials of tildacerfont. Potential patients may not be adequately diagnosed or identified with the disorders which we are targeting or may not meet the entry criteria for our clinical trials. Additionally, other pharmaceutical companies with more resources and greater experience in drug development and commercialization are targeting these same endocrine disorders and are recruiting clinical trial patients from these patient populations, which may delay or make it more difficult to fully enroll our clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

Our clinical trials have been, and may in the future be, affected by the COVID-19 pandemic. For example, the COVID-19 pandemic may impact patient enrollment in our two ongoing Phase 2b clinical trials. In particular, some sites may pause enrollment to focus on, and direct resources to, COVID-19, while at other sites, patients may choose not to enroll or continue participating in the clinical trial as a result of the pandemic. In addition, patient visits to endocrinologists in the United States have slowed as a result of the COVID-19 pandemic. Further, according to the Centers for Disease Control and Prevention, people who have serious chronic medical conditions, including those such as classic CAH, are at higher risk of getting very sick from COVID-19. As a result, current or potential patients in our ongoing and planned clinical trials may choose to not enroll, not participate in follow-up clinical visits, or drop out of the trial as a precaution against contracting COVID-19. Further, some patients may not be able or willing to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services.

We are unable to predict with confidence the likelihood or duration of such patient enrollment delays and difficulties, whether related to COVID-19 or otherwise. If patient enrollment is delayed for an extended period of time, our Phase 2b clinical trials could be delayed or otherwise adversely affected.

Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs to us, delay or limit our ability to generate revenue, and adversely affect our commercial prospects.

Before we can initiate clinical trials for tildacerfont or any future product candidates, we must submit the results of preclinical studies to the FDA, or comparable foreign regulatory authorities, along with other information, including information about chemistry, manufacturing and controls, and our proposed clinical trial protocol, as part of an IND or similar regulatory filing under which we must receive authorization to proceed with clinical development.

Before obtaining marketing approval from regulatory authorities for the sale of tildacerfont or any future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of tildacerfont and any future product candidates in humans. Clinical testing is expensive, time-consuming, and uncertain as to outcome. In addition, we may rely in part on preclinical, clinical and quality data generated by CROs and other third parties for regulatory submissions for tildacerfont and any future product candidates. While we have or will have agreements governing these third parties' services, we have limited influence over their actual performance. If these third parties do not make data available to us, or, if applicable, do not make regulatory submissions in a timely manner, in each case pursuant to our agreements with them, our development programs may be significantly delayed, and we may need to conduct additional clinical trials or collect additional data independently. In either case, our development costs would increase.

We do not know whether our current or any future clinical trials will begin on time or be completed on schedule, if at all. The commencement and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- the FDA or comparable foreign regulatory authorities' failure to accept our proposed manufacturing processes and suppliers and/or requirement to provide additional information regarding our manufacturing processes before providing marketing authorization;
- obtaining regulatory authorizations to commence a clinical trial or reaching a consensus with regulatory authorities on clinical trial design or implementation;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining approval from one or more institutional review boards, or IRBs, or Ethics Committees, or ECs;
- IRBs or ECs refusing to approve, suspending or terminating the clinical trial at an investigational site, precluding enrollment of additional subjects, or withdrawing their approval of the clinical trial;
- changes to clinical trial protocols;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- sites deviating from clinical trial protocol or dropping out of a clinical trial;
- manufacturing sufficient quantities of tildacerfont or any future product candidates or obtaining sufficient quantities of combination therapies for use in clinical trials;
- subjects failing to enroll or remain in our trials at the rate we expect, or failing to return for post-treatment follow-up;
- subjects choosing an alternative treatment for the indications for which we are developing tildacerfont and any future product candidates, or participating in competing clinical trials;
- lack of adequate funding to continue the clinical trial;
- subjects experiencing severe or unexpected drug-related adverse effects;
- occurrence of SAEs in clinical trials of the same class of agents conducted by other companies;
- a facility manufacturing tildacerfont or any of its components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice, or cGMP, regulations or other applicable requirements, or infections or cross-contaminations of tildacerfont in the manufacturing process;
- any changes to our manufacturing process, suppliers or formulation that may be necessary or desired;
- third-party vendors not performing manufacturing and distribution services in a timely manner or to sufficient quality standards;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, good clinical practice, or GCP, or other regulatory requirements;
- third-party contractors not performing data collection or analysis in a timely or accurate manner;
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications; or
- the impacts of the COVID-19 pandemic on our ongoing and planned clinical trials.

In addition, disruptions caused by the COVID-19 pandemic may increase the likelihood that we encounter such difficulties or delays in initiating, enrolling, conducting, or completing our planned and ongoing clinical trials. We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs or ECs of the institutions in which such trials are being conducted or by the FDA or comparable foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, changes in regulatory

requirements and policies may occur, and we have amended, and may need to further amend, clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing, or successful completion of a clinical trial.

Further, conducting clinical trials in foreign countries, which we are doing for tildacerfont and may do for any future product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

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

If we experience delays in the completion of, or termination of, any clinical trial of tildacerfont or any future product candidates, the commercial prospect of tildacerfont or any future product candidates will be harmed, and our ability to generate product revenue will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of tildacerfont or any future product candidates. Further, delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize tildacerfont and our competitors may be able to bring products to market before we do, and the commercial viability of tildacerfont could be significantly reduced. Any of these occurrences may harm our business, financial condition, and prospects significantly.

Tildacerfont is, and any future product candidates will be, subject to extensive regulation and compliance obligations, which are costly and time-consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize tildacerfont and any future product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing, and distribution of tildacerfont is subject to extensive regulation by the FDA in the United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market tildacerfont and any future product candidates until we receive regulatory approval from the FDA. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity, and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and the FDA has substantial discretion in the drug approval process, including the ability to delay, limit, or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed. Neither we nor any future collaborator is permitted to market tildacerfont and any future product candidates in the United States until we receive approval of an NDA from the FDA. We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities.

Prior to obtaining approval to commercialize a product candidate in the United States or in foreign markets, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from non-clinical studies and clinical trials can be interpreted in different ways. Even if we believe the non-clinical or clinical data for tildacerfont are promising, such data may not be sufficient to support approval by the FDA and comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for tildacerfont and any future product candidates either prior to or post-approval, or may object to elements of our clinical development program.

Tildacerfont and any future product candidates could fail to receive regulatory approval for many reasons, including the following:

- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by people using drugs similar to tildacerfont and any future product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;

- the FDA or comparable foreign regulatory authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the United States;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for any of its proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of tildacerfont and any future product candidates may not be sufficient to satisfy the FDA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submissions in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere, requiring, in the case of adult patients with classic CAH, additional clinical trials beyond our two ongoing Phase 2b clinical trials prior to any such approval;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Any of the above events could prevent us from achieving market approval of tildacerfont or any future product candidates and could substantially increase the costs of commercializing tildacerfont or any future product candidates. The demand for tildacerfont or any future product candidates could also be negatively impacted by any adverse effects of a competitor's product or treatment.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market tildacerfont and any future product candidates, which would significantly harm our business, financial condition, results of operations, and prospects.

Even if we eventually complete clinical trials and receive approval of an NDA or foreign marketing application for tildacerfont and any future product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a risk evaluation and mitigation strategy, or REMS, which may be required to ensure safe use of the drug after approval. The FDA or the comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or comparable foreign regulatory authority may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

Our business has been and could continue to be adversely affected by the evolving and ongoing COVID-19 global pandemic in regions where we or third parties on which we rely have significant manufacturing facilities, concentrations of clinical trial sites or other business operations. The COVID-19 pandemic could adversely affect our operations, as well as the business or operations of our manufacturers, CROs, or other third parties with whom we conduct business.

Our business has been and could continue to be adversely affected by the evolving COVID-19 pandemic, which was declared by the World Health Organization as a global pandemic. As COVID-19 continues to spread, we may experience ongoing disruptions that could severely impact our business and clinical trials, including:

- delays or difficulties in enrolling patients in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site; investigators and clinical site staff;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;
- changes in local regulations as part of a response to the COVID-19 outbreak which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;

- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- interruptions or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events;
- refusal of the FDA to accept data from clinical trials in affected geographies; and
- increased costs relating to mitigating the impact of COVID-19 on any of the foregoing factors.

These and other disruptions in our operations and the global economy could negatively impact our business, operating results and financial condition.

Our clinical trials have been, and may in the future be, affected by the COVID-19 pandemic. For example, the COVID-19 pandemic may impact patient enrollment in our two ongoing Phase 2b clinical trials. In particular, some sites may pause enrollment to focus on, and direct resources to, COVID-19, while at other sites, patients may choose not to enroll or continue participating in the clinical trial as a result of the pandemic. In addition, patient visits to endocrinologists in the United States have slowed as a result of the COVID-19 pandemic. Further, according to the Centers for Disease Control and Prevention, people who have serious chronic medical conditions, including those such as classic CAH, are at higher risk of getting very sick from COVID-19. As a result, current or potential patients in our ongoing and planned clinical trials may choose to not enroll, not participate in follow-up clinical visits, or drop out of the trial as a precaution against contracting COVID-19. Further, some patients may not be able or willing to comply with clinical trial protocols if quarantines impede patient movement or interrupts healthcare services.

If patient enrollment is delayed for an extended period of time, our ongoing and planned clinical trials could be delayed or otherwise adversely affected. Similarly, our ability to recruit and retain principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, may be adversely impacted.

In addition, ongoing or planned clinical trials may also be impacted by interruptions or delays in the operations of the FDA and comparable foreign regulatory agencies. For example, we and our CROs have also made certain adjustments to the operation of our trials in an effort to ensure the monitoring and safety of patients and minimize risks to trial integrity during the pandemic in accordance with the guidance issued by the FDA, and may need to make further adjustments in the future. For example, we have amended our clinical trial protocols to enable remote visits to mitigate any potential impacts as a result of the COVID-19 pandemic. As a result of this home health component, the overall costs of our Phase 2b clinical trials have increased and may continue to increase in the future. Many of these adjustments are new and untested, may not be effective, may affect the integrity of data collected, and may have unforeseen effects on the progress and completion of our clinical trials and the findings from such clinical trials.

In addition, we may encounter a shortage in supplies of, or in delays in shipping, our study drug or other components of the clinical trial vital for successful conduct of the trial. For example, in our two ongoing Phase 2b clinical trials, patients will continue to use their steroid regimen for the duration of the clinical trial. In particular, we have experienced a shortage of supply of hydrocortisone as a result of the COVID-19 pandemic, which if continued indefinitely, could adversely affect the timing and ultimately success of our clinical trials. Further, the successful conduct of our clinical trials depends on retrieving laboratory data from patients. Any failure by the laboratories with which we work to send us such data could impair the progress of such clinical trials. These events could delay our clinical trials, increase the cost of completing our clinical trials, and negatively impact the integrity, reliability, or robustness of the data from our clinical trials.

In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns, or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our CROs or third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for tildacerfont. To the extent our suppliers and service providers are unable to comply with their obligations under our agreements with them or they are otherwise unable to deliver or are delayed in delivering goods and services to us due to the COVID-19 pandemic, our ability to continue meeting clinical supply demand for tildacerfont or otherwise advancing development of tildacerfont may become impaired.

The spread of COVID-19 and actions taken to reduce its spread may also materially affect us economically. While the potential economic impact brought by, and the duration of, the COVID-19 pandemic may be difficult to assess or predict, there could be a significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity and financial position. In addition, the trading prices for other companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms.

COVID-19 and actions taken to reduce its spread continue to evolve. The extent to which COVID-19 may impede the development of tildacerfont, reduce the productivity of our employees, disrupt our supply chains, delay our clinical trials, reduce our access to capital or limit our business development activities, will depend on future developments, which are highly uncertain and cannot be predicted with confidence.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

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

From time to time, we may publicly disclose interim, topline, or preliminary data from our clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, topline, or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Interim, topline, and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, such data should be viewed with caution until the final data are available. Adverse differences between preliminary, interim, or topline data and final data could significantly harm our business prospects.

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

If the market opportunities for tildacerfont and any future product candidates are smaller than we believe they are, our future revenue may be adversely affected, and our business may suffer.

If the size of the market opportunities in each of our target indications for tildacerfont and any future product candidates is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus our clinical development of tildacerfont on treatments for rare endocrine disorders with relatively small patient populations. For example, we believe that tildacerfont has the potential to bring therapeutic benefit to patients suffering from endocrine disorders where the underlying disease biology supports a need to reduce excess secretion of or hyperresponsiveness to ACTH, including, but not limited to, a severe form of non-classic CAH in adults and a subpopulation of females with a rare form of PCOS with primary adrenal androgen excess, representing 3-5% of females with PCOS (estimated to be 150,000 to 200,000 patients in the United States). Given the relatively small number of patients who have the disorders that we are targeting and intend to target with tildacerfont, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare endocrine disorders. In addition, our estimates of the patient populations for our target indications have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these disorders. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. For example, while classic CAH is usually detected at birth through required newborn screening programs in most developed countries, new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. In addition, the potentially addressable patient population for classic CAH may be limited or may not be amenable to treatment with tildacerfont, if approved. Further, even if we obtain significant market share for tildacerfont in

classic CAH, we may never achieve profitability despite obtaining such significant market share, as other pharmaceutical companies with more resources and greater experience in drug development and commercialization are targeting this same endocrine disorder.

We may not be successful in our efforts to expand our pipeline by identifying additional indications and formulations for which to investigate tildacerfont in the future. We may expend our limited resources to pursue a particular indication or formulation for tildacerfont and fail to capitalize on product candidates, indications, or formulations that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we are focused on specific indications and formulations for tildacerfont. As a result, we may fail to generate additional clinical development opportunities for tildacerfont for a number of reasons, including, tildacerfont may in certain indications, on further study, be shown to have harmful side effects, limited to no efficacy, or other characteristics that suggest it is unlikely to receive marketing approval and achieve market acceptance in such additional indications.

We plan to conduct several clinical trials for tildacerfont in parallel over the next several years, including multiple clinical trials in adult and pediatric patients with classic CAH and in a subpopulation of females with a rare form of PCOS. As a result, we may forgo or delay pursuit of opportunities with other indications that could have had greater commercial potential or likelihood of success. However, we may focus on or pursue one or more of our target indications over other potential indications and such development efforts may not be successful, which would cause us to delay the clinical development and approval of tildacerfont. Furthermore, research programs to identify additional indications for tildacerfont require substantial technical, financial, and human resources. We may also pursue additional formulations for tildacerfont, including transitioning from a tablet formulation to a granulate formulation. However, we may not successfully develop these additional formulations for chemistry-related, stability-related, or other reasons. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for specific indications may not yield any commercially viable products.

Additionally, we may pursue additional in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, potentially resulting in a diversion of our management's time and the expenditure of our resources with no resulting benefit.

For example, if we are unable to identify programs that ultimately result in approved products, we may spend material amounts of our capital and other resources evaluating, acquiring and developing products that ultimately do not provide a return on our investment.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not mean that we will be successful in obtaining regulatory approval for that product candidate in other jurisdictions.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for tildacerfont is also subject to approval.

We expect to submit a Marketing Authorization Application, or MAA, to the EMA for approval of tildacerfont in the EU for the treatment of classic CAH. As with the FDA, obtaining approval of an MAA from the EMA is a similarly lengthy and expensive process and the EMA has its own procedures for approval for product candidates. Regulatory authorities in jurisdictions outside of the United States and the EU also have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of tildacerfont in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of tildacerfont will be harmed, which would adversely affect our business, prospects, financial condition, and results of operations.

We currently have no marketing and sales organization and have yet to commercialize a product. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell tildacerfont and any future product candidates, we may not be able to generate product revenues.

We currently do not have a commercial organization for the marketing, sales, and distribution of pharmaceutical products. To commercialize tildacerfont and any future product candidates, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We intend to build a highly specialized commercial organization to support the commercialization of tildacerfont, if approved, in the United States and Europe.

The establishment and development of our own sales force or the establishment of a contract sales force to market tildacerfont and any future product candidates will be expensive and time-consuming and could delay any commercial launch. Moreover, we may not be able to successfully develop this capability. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train, and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of tildacerfont. To the extent we rely on third parties to commercialize tildacerfont, if approved, we may have little or no control over the marketing and sales efforts of such third parties and our revenues from product sales may be lower than if we had commercialized tildacerfont and any future product candidates ourselves. In the event we are unable to develop our own marketing and sales force or collaborate with a third-party marketing and sales organization, we would not be able to commercialize tildacerfont or any future product candidates.

Use of tildacerfont or any future product candidates could be associated with side effects, adverse events or other properties that could delay or prevent regulatory approval or result in significant negative consequences following marketing approval, if any.

As is the case with biopharmaceuticals generally, it is likely that there may be side effects and adverse events associated with the use of tildacerfont and any future product candidates. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by tildacerfont and any future product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. For example, although tildacerfont has been assessed in 171 patients across seven clinical trials in which it has been well tolerated with no drug-related SAEs, in our proof-of-concept, dose-escalating Phase 2a clinical trial in adults with classic CAH, one patient experienced a grade one liver-related adverse event after 14 days of treatment at 1,000mg once daily. This patient had elevated levels of alanine transaminase, or ALT, between five and nine times the upper limit of normal, or ULN, elevations in aspartate aminotransferase, or AST, less than five times the ULN, and no increases in bilirubin. The event resolved on its own without additional medical intervention. No cases of liver enzyme elevations above three times the ULN were observed in any patient receiving total daily doses of 600mg, which is approximately three times the proposed therapeutic dose for adults with classic CAH, and below. If drug-related SAEs are observed, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval for tildacerfont for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition, and prospects significantly.

Furthermore, only adults have been treated with tildacerfont, and the safety profile in pediatric patients is unknown and may be different than that observed in previous clinical trials. Results of our trials could reveal a high and unacceptable severity and prevalence of side effects.

Additionally, if tildacerfont and any future product candidates receive marketing approval, and we or others later identify undesirable side effects caused by such product candidate, a number of potentially significant negative consequences could result, including:

- we may be forced to suspend marketing of that product, or decide to remove the product form the marketplace;
- regulatory authorities may withdraw approvals or change their approvals of such product;
- regulatory authorities may require additional warnings on the label or limit access of that product to selective specialized centers with additional safety reporting and with requirements that patients be geographically close to these centers for all or part of their treatment;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way the product is administered;
- we could be subject to fines, injunctions, or the imposition of criminal or civil penalties, or to sued and held liable for harm caused to subjects or patients;
- we could be sued and held liable for harm caused to patients; and
- the product may become less competitive, and our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of tildacerfont and any future product candidates, if approved, and could significantly harm our business, results of operations, and prospects.

If we receive regulatory approval for tildacerfont and any future product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with any product.

Any regulatory approvals that we receive may be subject to 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-market studies or clinical trials, and surveillance to monitor safety and effectiveness. The FDA may also require us to adopt a REMS to ensure that the benefits of treatment with such product candidate outweigh the risks for each potential patient, which may include, among other things, a communication plan to health care practitioners, patient education, extensive patient monitoring, or distribution systems and processes that are highly controlled, restrictive and more costly than what is typical for the industry. We or our collaborators may also be required to adopt a REMS or engage in similar actions, such as patient education, certification of health care professionals, or specific monitoring, if we or others later identify undesirable side effects caused by any product that we develop alone or with collaborators.

In addition, if the FDA or a comparable foreign regulatory authority approves a product candidate, the manufacturing, quality control, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export, and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. The FDA also requires submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP requirements and GCP for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product candidate, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- issue warning letters or untitled letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners, or require other restrictions on the labeling or marketing of such products;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend, withdraw or modify regulatory approval;
- suspend or modify any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize tildacerfont and any future product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

Advertising and promotion of any product candidate that obtains approval in the United States will be heavily scrutinized by the FDA, the U.S. Federal Trade Commission, the Department of Justice, or the DOJ, the Office of Inspector General of the U.S. Department of Health and Human Services, or HHS, state attorneys general, members of the U.S. Congress, and the public. Additionally, advertising and promotion of any product candidate that obtains approval outside of the United States will be heavily scrutinized by comparable foreign entities and stakeholders. Violations, including actual or alleged promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries, and investigations, and civil and criminal sanctions by the FDA, DOJ, or comparable foreign bodies. Any actual or alleged failure to comply with labeling and promotion requirements may result in fines, warning letters, mandates to corrective information to healthcare practitioners, injunctions, or civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for tildacerfont and any future product candidates. 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 adversely affect our business, prospects, financial condition, and results of operations.

Changes in funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

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

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Separately, in response to the global COVID-19 pandemic, in March 2020 the FDA announced its intention to postpone most foreign inspections of manufacturing facilities and products and routine surveillance inspections of domestic manufacturing facilities. In July 2020, the FDA restarted routine pre-announced surveillance inspections of domestic manufacturing facilities on a risk-based basis. Regulatory authorities outside the United States have adopted similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Even if we obtain regulatory approval for tildacerfont and any future product candidates, tildacerfont and any future product candidates may not gain market acceptance among physicians, patients, healthcare payors and others in the medical community.

Tildacerfont and any future product candidates may not be commercially successful. The commercial success of tildacerfont or any future product candidates, if approved, will depend significantly on the broad adoption and use of such product by physicians and patients for approved indications. The degree of market acceptance of tildacerfont or any future products, if approved, will depend on a number of factors, including:

- the clinical indications for which such product candidate is approved;
- physicians and patients considering the product as a safe and effective treatment;
- the potential and perceived advantages of the product over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;
- the timing of market introduction of the product as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement by third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage and adequate reimbursement by third-party payors and government authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies; and
- the effectiveness of our sales and marketing efforts and those of any collaboration or distribution partner on whom we rely for sales in foreign jurisdictions.

If tildacerfont and any future product candidate is approved but fails to achieve market acceptance among physicians, patients, healthcare payors or others in the medical community, we will not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition, and results of operations. In addition, even if tildacerfont and any future product candidate gains acceptance, the markets for the treatment of patients with our target indications may not be as significant as we estimate.

If tildacerfont and any future product candidate is approved for marketing, and we are found to have improperly promoted off-label uses, or if physicians prescribe or use tildacerfont and any future product candidates off-label, we may become subject to

prohibitions on the sale or marketing of tildacerfont and any future product candidates, significant fines, penalties, sanctions, or product liability claims, and our image and reputation within the industry and marketplace could be harmed.

The FDA, DOJ, and comparable foreign authorities strictly regulate the marketing and promotional claims that are made about pharmaceutical products, such as tildacerfont, if approved. In particular, a product may not be promoted for uses or indications that are not approved by the FDA or comparable foreign authorities as reflected in the product's approved labeling. However, if we receive marketing approval for tildacerfont and any future product candidates, physicians can prescribe such product to their patients in a manner that is inconsistent with the approved label based on the physician's independent medical judgement. If we are found to have promoted such off-label uses, we may receive warning letters from the FDA and comparable foreign authorities and become subject to significant liability, which would materially harm our business. 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. If we become the target of such an investigation or prosecution based on our marketing and promotional practices, we could face similar sanctions, which would materially harm our business. In addition, management's attention could be diverted from our business operations, significant legal expenses could be incurred, and our reputation could be damaged. The FDA and other governmental authorities have also required that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed in order to resolve enforcement actions. If we are deemed by the FDA, DOJ, or other governmental authorities to have engaged in the promotion of tildacerfont or any future product candidate for off-label use, we could be subject to certain prohibitions or other restrictions on the sale or marketing and other operations or significant fines and penalties, and the imposition of these sanctions could also affect our reputation and position within the industry.

Coverage and reimbursement may be limited or unavailable in certain market segments for tildacerfont and any future product candidates, which could make it difficult for us to sell tildacerfont and any future product candidates profitably.

Successful sales of tildacerfont and any future product candidates, if approved, depend on the availability of coverage and adequate reimbursement from third-party payors. Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance, and we may not obtain such coverage or adequate reimbursement. Moreover, we focus our clinical development of tildacerfont on treatments for rare endocrine disorders with relatively small patient populations. As a result, we must rely on obtaining appropriate coverage and reimbursement for these populations.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will cover and the amount of reimbursement they will provide. Reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. We may not be able to provide data sufficient to obtain coverage and adequate reimbursement. Assuming we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use tildacerfont or any future product candidate unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost. Additionally, the reimbursement rates and coverage amounts may be affected by the approved label for tildacerfont or any future product candidate. If coverage and reimbursement of our future products are unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability.

In addition, the market for tildacerfont and any future product candidates will depend significantly on access to third-party payors' drug formularies or lists of medications for which third-party payors provide coverage and reimbursement. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access through formulary controls or otherwise to a branded drug when a less costly generic equivalent or other alternative is available.

In the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. Therefore, coverage and reimbursement for

drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of tildacerfont and any future product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

We intend to seek approval to market tildacerfont in the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for tildacerfont, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of prescription pharmaceuticals and biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval for a drug candidate. In addition, market acceptance and sales of a product will depend significantly on the availability of coverage and adequate reimbursement from third-party payors for a product and may be affected by existing and future health care reform measures.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to obtain marketing approval for and commercialize tildacerfont and any future product candidates and may affect the prices we may set.*

In the United States and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably sell any product candidates for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the Affordable Care Act, was enacted in the United States. Among the provisions of the Affordable Care Act of importance to our potential product candidates are that it established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; expands eligibility criteria for Medicaid programs; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; created a new Medicare Part D coverage gap discount program; established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare and Medicaid Innovation at the Centers for Medicare & Medicaid Services, or CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

There have been executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. By way of example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the Affordable Care Act-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminated the health insurer tax. On December 14, 2018, a Texas U.S. District Court Judge ruled that the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit ruled that that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the Affordable Care Act are invalid as well. The United States Supreme Court is currently reviewing this case, although it is unknown when a decision will be made. On February 10, 2021, the Biden administration withdrew the federal government support for overturning the Affordable Care Act. Although the Supreme Court has not yet ruled on the constitutionality of the Affordable Care Act, on January 28, 2021, President Biden issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace, which began on February 15, 2021 and will remain open through August 15, 2021. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. It is unclear how the Supreme Court ruling, other such litigation, and the healthcare reform measures of the Biden administration will impact the Affordable Care Act and our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, included reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030, with the exception of a temporary suspension from May 1, 2020 through December 31, 2021 due to COVID-19 relief legislation, unless additional Congressional action is taken. In addition, on January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products.

At the federal level, the previous Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, the Trump administration announced several executive orders related to drug pricing that attempted to implement several of the Trump administration's proposals. As a result, the FDA released a final rule on September 24, 2020, effective November 30, 2020, implementing a portion of the importation executive order. Further, on November 20, 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Medicare Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed until January 1, 2023. On November 20, 2020, CMS issued an interim final rule implementing the Trump administration's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. It is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives.

At the state level, individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition, and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for tildacerfont, if approved, or put pressure on our product pricing, which could negatively affect our business, results of operations, financial condition, and prospects.

We expect that the Affordable Care Act, these new laws, and other healthcare reform measures that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. In addition, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from third-party payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize tildacerfont, if approved.

A variety of risks associated with marketing tildacerfont and any future product candidates internationally could materially adversely affect our business.

We plan to seek regulatory approval for tildacerfont and any future product candidates internationally and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, including differing reimbursement, pricing and insurance regimes;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls, and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration, and labor laws for employees living or traveling internationally;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;

- potential liability under the U.S. Foreign Corrupt Practices Act of 1977, or FCPA, or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities internationally; and
- business interruptions resulting from geo-political actions, including war and terrorism.

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

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business.

We may seek to in-license or acquire development-stage product candidates in endocrine disorders that have the potential to complement our existing portfolio. If we decide to pursue the development and commercialization of any additional product candidates, we may be required to invest significant resources to acquire or in-license the rights to such product candidates or to conduct drug discovery activities. We do not currently have the necessary drug discovery personnel or expertise adequate to discover and develop an additional product candidate on our own. Any other product candidates will require additional, time-consuming development efforts, and significant financial resources, prior to commercial sale, including preclinical studies, extensive clinical trials, and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. In addition, we may not be able to acquire, discover, or develop any additional product candidates, and any additional product candidates we may develop may not be approved, manufactured, or produced economically, successfully commercialized or widely accepted in the marketplace, or be more effective than other commercially available alternatives. Research programs to identify new product candidates require substantial technical, financial, and human resources whether or not we ultimately identify any candidates. If we are unable to develop or commercialize any other product candidates, our business and prospects will suffer.

If we fail to develop tildacerfont for additional indications, our commercial opportunity may be limited.

One of our strategies is to pursue clinical development of tildacerfont in additional endocrine disorders, including, but not limited to, pediatric classic CAH and a subpopulation of females with a rare form of PCOS. The endocrine disorders we are targeting are all rare disorders and, as a result, the market size for the treatment of patients with such disorders is limited. In addition, CRF1 receptor antagonism may not be an appropriate or effective mechanism in indications where disease biology supports a need to reduce ACTH. Due to these factors, our ability to grow revenue may be dependent on our ability to successfully develop and commercialize tildacerfont for the treatment of additional indications. Developing, obtaining regulatory approval and commercializing tildacerfont for additional indications will require substantial additional funding and is prone to the risks of failure inherent in drug development. We may not be able to successfully advance any of these indications through the development process. Even if we receive regulatory approval to market tildacerfont for the treatment of any of these additional indications, any such additional indications may not be successfully commercialized, widely accepted in the marketplace, or more effective than other commercially available alternatives. If we are unable to successfully develop and commercialize tildacerfont for these additional indications, our commercial opportunity may be limited.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.*

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Although we believe that we hold a leading position in our focus on rare endocrine disorders, our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis drug products that are more effective or less costly than tildacerfont. We believe the key competitive factors that will affect the development and commercial success of tildacerfont are efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement.

Although classic CAH is part of the newborn screening program in most developed countries, there are no known novel therapies that have been approved in approximately 50 years. We are aware of three other companies actively developing treatments for patients with classic CAH. Neurocrine Biosciences, Inc., or Neurocrine, is developing a CRF1 receptor antagonist and has completed a two-week Phase 2 clinical trial in adults with classic CAH. Neurocrine has initiated a Phase 2 clinical trial in a pediatric classic CAH population and a registrational trial for adult patients with classic CAH. Neurocrine has also initiated a registrational program in pediatric classic CAH in 2021. BridgeBio Pharma, Inc. plans to evaluate an AAV5 gene therapy product candidate to treat classic CAH in a Phase 1/2 proof of concept clinical trial in 2021. In addition, Crinetics Pharmaceuticals, Inc. has initiated a Phase 1 clinical trial in 2021 to evaluate the ability of an oral ACTH antagonist to suppress ACTH-stimulated secretion in healthy volunteers.

In addition, while tildacerfont ultimately seeks to significantly reduce steroid use for patients with classic CAH, patients will continue use of their steroid regimen. As corticosteroids are the current standard of care for the treatment of classic CAH, in the United States alone, there are more than two dozen companies manufacturing steroid-based products. One such company is Diurnal Group PLC, or Diurnal, which is developing an exogenous cortisol treatment with a modified release intended to more closely match the physiological release profile of cortisol but recently announced a failed Phase 3 clinical trial and placed its U.S. development activities on hold. Diurnal submitted a MAA to the EMA in December of 2019. In March 2021, the EMA announced a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) on granting marketing approval for its exogenous cortisol treatment with a modified release for the treatment of adults and adolescents aged 12 and over with CAH. Diurnal also announced that it has withdrawn its application for maintenance of orphan drug designation in Europe and anticipates commercial launch in the third quarter of 2021.

Our commercial potential could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market or make our development more complicated. We believe the key competitive factors affecting the success of tildacerfont are likely to be efficacy, safety, and convenience.

Even though we have obtained orphan drug designation for tildacerfont for the treatment of CAH, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the EMA Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the EU.

We have received orphan drug designation for tildacerfont for the treatment of patients with CAH in both the US and EU. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug and same indication for that time period. The applicable period is seven years in the United States and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency's agreed upon pediatric investigation plan. The exclusivity period in the EU can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA and the EMA can subsequently approve a different drug for the same condition or the same drug for a different condition, which may subject the orphan-exclusive product to off-label competition. As well, before the expiration of the orphan exclusivity period, the FDA or EMA may grant approval to a competitor if it concludes that a subsequent application for the same drug for the same indication is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the EU, the EMA may deny marketing approval for a product candidate if it determines such product candidate is structurally similar to an approved product for the same indication. In addition, if an orphan designated product receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to tildacerfont for the treatment of classic CAH, if we receive approval for tildacerfont for a modified or different indication, our current orphan designations may not provide us with exclusivity.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for tildacerfont, that exclusivity may not effectively protect us from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period.

We are currently pursuing orphan drug designation for tildacerfont for the treatment of a subpopulation of females with a rare form of PCOS. The incidence and prevalence of this target patient population is based on our estimates and third-party data. If the market opportunity for this target population is larger than we estimate, we may be unable to receive orphan drug designation. Additionally, if orphan drug designation is granted, we may be unable to maintain any benefits associated with orphan drug designation, including market exclusivity.

Periodically, we make estimates regarding the incidence and prevalence of target patient populations based on various third-party sources and internally generated analysis. Our estimates may be inaccurate or based on imprecise data. As described above, under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. If our incidence or prevalence estimates for the subpopulation of females with a rare form of PCOS are incorrect, we may be unable to receive orphan drug designation.

Even if the FDA grants orphan drug designation for tildacerfont for the treatment of a subpopulation of females with a rare form of PCOS, exclusive marketing rights in the United States may be limited if we seek FDA marketing approval for an indication broader than the orphan designated indication. Additionally, any product candidate that initially receives orphan drug status designation, may lose such designation if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, others may obtain orphan drug status for products addressing the same diseases or conditions as products we are developing, thus limiting our ability to compete in the markets addressing such diseases or conditions for a significant period of time. As a result, our business and prospects could suffer.

We may form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.

We may form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to tildacerfont and any future product candidates that we may develop. We intend to establish commercial partnerships outside of the United States in selected foreign markets. Any of these relationships may require us to incur non-recurring and other charges, increase our near-and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. Following a strategic transaction or license, we may not

achieve the revenues or cash flows that justifies such transaction. Any delays in entering into new strategic partnership agreements related to tildacerfont could delay the development and commercialization of tildacerfont in certain geographies for certain indications, which would harm our business prospects, financial condition, and results of operations.

Our failure to successfully in-license, acquire, develop, and market additional product candidates or approved products would impair our ability to grow our business.

Although a substantial amount of our efforts are focused on the clinical development, potential regulatory approval and commercialization of tildacerfont, a key element of our long-term strategy is to in-license, acquire, develop, market, and commercialize a portfolio of products to treat patients with endocrine disorders. Because we do not have the necessary internal research and development capabilities, unless we build such capabilities internally, we will be dependent upon pharmaceutical companies, academic scientists, and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify and select promising biopharmaceutical product candidates and products, negotiate licensing or acquisition agreements with their current owners, and finance these arrangements. The process of proposing, negotiating, and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing, sales, and other resources, may compete with us for the license or acquisition of product candidates and approved products. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses, and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional product candidates on terms that we find acceptable, or at all. Further, any product candidate that we acquire may require additional development efforts prior to commercial sale, including preclinical or clinical testing and approval by the FDA, the EMA and other similar regulatory authorities. All product candidates are prone to risks of failure during biopharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, any approved products that we acquire may not be manufactured or sold profitably or achieve market acceptance.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific, and medical personnel. We are highly dependent on our management, scientific, and medical personnel. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations.

We conduct our operations in Daly City, California. This region serves as the headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific, and development teams may terminate their employment with us on short notice. Although we have employment agreements and/or offer letters with our key employees, these arrangements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key man” insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

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

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.*

As of March 31, 2021, we had 19 employees, all of whom are full-time. As our development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional development, managerial, operational, financial, sales, marketing, and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining, and motivating additional employees;

- managing our internal development efforts effectively, including the clinical and regulatory review process for tildacerfont and any future product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

In addition, we initiated enrollment in CAHmelia-203, our ongoing placebo-controlled, double-blind Phase 2b clinical trial in adult patients with classic CAH with poor disease control, and in CAHmelia-204, our second Phase 2b clinical trial in adult patients with classic CAH with good disease control focused on glucocorticoid reduction. Our future financial performance and our ability to commercialize tildacerfont will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. To date, we have used the services of outside vendors to perform tasks including clinical trial management, manufacturing, statistics and analysis, regulatory affairs, formulation development, and other drug development functions. Our growth strategy may also entail expanding our group of contractors or consultants to implement these tasks going forward. Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed, or terminated, and we may not be able to obtain regulatory approval for tildacerfont and any future product candidates or otherwise advance our business. We may not be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize tildacerfont and any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our indebtedness to Silicon Valley Bank may limit our flexibility in operating our business and adversely affect our financial health and competitive position, and our obligations to Silicon Valley Bank are secured by substantially all of our assets, excluding our intellectual property assets. If we default on these obligations, Silicon Valley Bank could foreclose on our assets.*

In September 2019, we entered into the Loan Agreement providing for the Term Loan. In April 2020, we entered into a deferral agreement with Silicon Valley Bank, or the Deferral Agreement, whereby we and Silicon Valley Bank agreed to extend the repayment dates of all monthly payments of principal due and the maturity date with respect to the Term Loan by six months. In March 2021, we entered into the First Amendment with Silicon Valley Bank. The First Amendment increased the aggregate principal amount of the Term Loan commitment by Silicon Valley Bank to up to $30.0 million. As of December 31, 2020, we had $4.5 million outstanding under the Loan Agreement. Following the First Amendment, the principal amount due under the Loan Agreement was $5.0 million as of March 31, 2021.

All obligations under the Term Loan are secured by a first priority lien on substantially all of our assets, excluding intellectual property assets. We have agreed with Silicon Valley Bank not to encumber our intellectual property assets without its prior written consent unless a security interest in the underlying intellectual property is necessary to have a security interest in the accounts and proceeds that are part of the assets securing the Term Loan, in which case our intellectual property shall automatically be included within the assets securing the Term Loan. As a result, if we default on any of our obligations under the Loan Agreement, Silicon Valley Bank could foreclose on its security interest and liquidate some or all of the collateral, which would harm our business, financial condition, and results of operations and could require us to reduce or cease operations.

In order to service this indebtedness and any additional indebtedness we may incur in the future, we need to generate cash from our operating activities. Our ability to generate cash is subject, in part, to our ability to successfully execute our business strategy, as well as general economic, financial, competitive, regulatory, and other factors beyond our control. Our business may not be able to generate sufficient cash flow from operations, and future borrowings or other financings may not be available to us in an amount sufficient to enable us to service our indebtedness and fund our other liquidity needs. To the extent we are required to use cash from operations or the proceeds of any future financing to service our indebtedness instead of funding working capital, capital expenditures or other general corporate purposes, we will be less able to plan for, or react to, changes in our business, industry, and in the economy generally. This could place us at a competitive disadvantage compared to our competitors that have less indebtedness.

The Loan Agreement contains certain covenants that limit our ability to engage in certain transactions that may be in our long-term best interest, including entering into a change in control transaction. The Loan Agreement also contains certain covenants that limit our ability to obtain additional debt financing, including incurring debt from third parties not permitted under the Loan Agreement or incurring liens or encumbrances on our property. While we have not previously breached and are currently in compliance with the covenants contained in the Loan Agreement, we may breach these covenants in the future. Our ability to comply with these covenants may be affected by events and factors beyond our control. In the event that we breach one or more covenants, Silicon Valley Bank may choose to declare an event of default and require that we immediately repay all amounts outstanding under the Loan Agreement, terminate any commitment to extend further credit and foreclose on the collateral. In addition, if an event of default occurs under the Loan Agreement, Silicon Valley Bank may, among other things, accelerate the Term Loan or do any acts it considers necessary or reasonable to protect its security interest in the collateral under the Term Loan. Events of default include the occurrence of a material adverse change in our business, operations, or condition (financial or otherwise). The occurrence of any of these events could have a material adverse effect on our business, financial condition, and results of operations.

For a more detailed description of the terms of the Loan Agreement, see the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources—Loan Agreement” and Note 6 to our condensed financial statements, each included elsewhere in this Quarterly Report on Form 10-Q.

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

We may seek additional capital through a combination of equity offerings, debt financings, strategic partnerships and alliances, and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights, and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or current or future product candidates, or grant licenses on terms unfavorable to us.

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

We are exposed to the risk that employees, independent contractors, principal investigators, CROs, consultants, and vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (i) the rules of the FDA and other similar foreign regulatory bodies, including those rules that require the reporting of true, complete, and accurate information to the FDA and other similar foreign regulatory bodies; (ii) manufacturing standards; (iii) healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws or (iv) laws that require the true, complete, and accurate reporting of our financial information or data. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as proposed and future sales, marketing, and education programs. In particular, the promotion, sales, and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs, and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials.

If we obtain regulatory approval for tildacerfont and begin commercializing those products in the United States and in Europe, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. 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 civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our relationships with customers, physicians and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners, or vendors violate these laws, we could face substantial penalties.

Our relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. These laws may impact, among other things, our clinical research program, as well as our proposed and future sales, marketing, and education programs. In particular, the promotion, sales and marketing of healthcare items and services is subject to extensive laws and regulations designed to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive, and other business arrangements. We may also be subject to federal, state and foreign laws governing the privacy and security of identifiable patient information. The U.S. healthcare laws and regulations that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person or entity from knowingly and willfully, offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, the purchasing, leasing, ordering or arranging for the purchase, lease, or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term "remuneration" has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that may be alleged to be intended to induce prescribing, purchases or recommendations, include any payments of more than fair market value, and may be subject to scrutiny if they do not qualify for an exception or safe harbor. In addition, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation.
- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal government programs that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government, including federal healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act and the civil monetary penalties statute;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal civil and criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by any trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, which impose requirements on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, and their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information as well as their covered subcontractors; and
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Beginning in 2022, such reporting obligations will include payments and other transfers of value provided during the previous year to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified nurse anesthetists, and certified nurse-midwives.

We may also be subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope. For example, we may be subject to the following: state anti-kickback and false claims laws that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures, or drug pricing; state and local laws requiring the registration of pharmaceutical sales and medical representatives; and state and foreign laws, such as the European Union General Data Protection Regulation, or GDPR, governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Additionally, we may be subject to federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, or our arrangements with physicians, could be subject to challenge under one or more of such laws. It is not always possible to identify and deter employee misconduct or business noncompliance, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws, we may be subject to investigations, enforcement actions and/or significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of tildacerfont outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

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

Despite the implementation of security and back-up measures, our internal computer, server, and other information technology systems as well as those of our third-party collaborators, consultants, contractors, suppliers, and service providers, may be vulnerable to damage from physical or electronic break-ins, computer viruses, malware, ransomware, natural disasters, terrorism, war, telecommunication and electrical failure, denial of service, and other cyberattacks or disruptive incidents that could result in unauthorized access to, use or disclosure of, corruption of, or loss of sensitive, and/ or proprietary data, including personal information, including health-related information, and could subject us to significant liabilities and regulatory and enforcement actions, and reputational damage. For example, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in any regulatory approval or clearance efforts and significantly increase our costs to recover or reproduce the data, and subsequently commercialize the product. Additionally, theft of our intellectual property or proprietary business information could require substantial expenditures to remedy. If we or our third-party collaborators, consultants, contractors, suppliers, or service providers were to suffer an attack or breach, for example, that resulted in the unauthorized access to or use or disclosure of personal or health information, we may have to notify consumers, partners, collaborators, government authorities, and the media, and may be subject to investigations, civil penalties, administrative and enforcement actions, and litigation, any of which could harm our business and reputation. Likewise, we rely on our third-party research institution collaborators and other third parties to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. The COVID-19 pandemic has generally increased the risk of cybersecurity intrusions. For example, there has been an increase in phishing and spam emails as well as social engineering attempts from "hackers" hoping to use the recent COVID-19 pandemic to their advantage. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or systems, or inappropriate or unauthorized access to or disclosure or use of confidential, proprietary, or other sensitive, personal, or health information, we could incur liability and suffer reputational harm, and the development and commercialization of tildacerfont could be delayed.

Failure to comply with data protection laws and regulations could lead to government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business.*

We and our partners may be subject to federal, state, and foreign data protection laws and regulations, as well as other rules, standards, policies and contractual or other obligations, relating to the collection, use, retention, security, disclosure, transfer and other processing of personal data, collectively, Data Protection Requirements. If we fail, or are perceived to have failed, to address or comply with any such Data Protection Requirements, this could result in enforcement actions against us that could include investigations, fines, penalties, audits and inspections, additional reporting requirements and/or oversight, temporary or permanent bans on all or some processing of personal data or orders to destroy or not use personal data. Further, individuals or other relevant stakeholders could bring a variety of claims against us for our actual or perceived failure to comply with the Data Protection Requirements.

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws and regulations that govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our partners. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. If we violate HIPAA, we may subject to significant administrative and civil penalties. Additionally, depending on the facts and circumstances, we could be subject to criminal penalties if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

Even when HIPAA does not apply, according to the Federal Trade Commission, or the FTC, failing to take appropriate steps to keep consumers' personal information secure may constitute unfair acts or practices in or affecting commerce in violation of the Federal Trade Commission Act. The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards.

In addition, California recently enacted the California Consumer Privacy Act, or CCPA, which creates new individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling certain personal data of consumers or households. The CCPA requires covered companies to provide new disclosure to consumers about such companies' data collection, use and sharing practices, provide such consumers new ways to opt-out of certain sales or transfers of personal information, and provide consumers with additional causes of action. The CCPA became effective January 1, 2020, and the California Attorney General may bring enforcement actions for violations beginning July 1, 2020. The CCPA has been amended from time to time, and it remains unclear what, if any, further modifications will be made to this legislation or how it will be interpreted. As currently written, the CCPA may impact our business activities and exemplifies the vulnerability of our business to the evolving regulatory environment related to personal data and protected health information.

Foreign data protection laws, including the GDPR may also apply to health-related and other personal data we process in respect of certain of our operations. The GDPR applies to any processing operations carried out in the context of the activities of an establishment in the EEA as well as any processing operations relating to the offering of goods or services to individuals in the EEA and/or the monitoring of their behavior in the EEA. Further, notwithstanding the United Kingdom's withdrawal from the EU, by operation of the so called 'UK GDPR' (i.e., the GDPR as it continues to form part of the law of the United Kingdom by virtue of section 3 of the EU (Withdrawal) Act 2018 and as subsequently amended), the GDPR continues to apply in substantially equivalent form to processing operations carried out in the context of the activities of an establishment in the United Kingdom and any processing relating to the offering of goods or services to individuals in the United Kingdom and/or monitoring of their behavior in the United Kingdom. Therefore, reference to the GDPR herein also refers to the UK GDPR in the context of the United Kingdom, unless the context requires otherwise. The GDPR provides that EEA member states may make their own further laws and regulations to introduce specific requirements related to the processing of 'special categories of personal data,' including the personal data related to health and genetic information, which we may process in connection with clinical trials or otherwise; as well as personal data related to criminal offenses or convictions – in the United Kingdom, the United Kingdom Data Protection Act 2018 complements the UK GDPR in this regard. Such laws may introduce further conditions, including limitations which could limit our ability to collect, use and share personal data, and/or otherwise lead to greater divergence on the law that applies to the processing of such data types across the EEA and/or United Kingdom, compliance with which, as and where applicable, may increase our costs and could increase our overall compliance risk.

The GDPR has imposed stringent requirements for controllers and processors of personal data, including, for example, by extending the rights available to affected data subjects, materially expanding the definition of what is expressly noted to constitute personal data (including expanding the relevant definition to capture expressly the 'pseudonymized' or key-coded data that is commonly processed in a clinical trial-related context), introducing mandatory personal data breach notifications to Supervisory Authorities and affected individuals (in certain circumstances), setting limitations on retention of information, increasing requirements pertaining to special categories of personal data (such as health data, biometric data, genetic information), and requiring that prescriptive obligations must be met when we engage third-party processors to process of personal data on our behalf. A particular issue presented by certain European data protection laws, including the GDPR, is that they generally restrict transfers of personal data from Europe, including the EEA, United Kingdom and Switzerland, to the United States and most other countries unless the parties to the transfer have implemented specific safeguards to protect the transferred personal data. Recent legal developments in the EU have created complexity and uncertainty regarding such transfers of personal data from the EEA to the United States, e.g. on July 16, 2020 in a case known colloquially as "Schrems II," the Court of Justice of the European Union, or the CJEU, invalidated the EU-US Privacy Shield Framework, or the Privacy Shield, under which personal data could be transferred from the EEA to U.S. entities who had self-certified under the Privacy Shield scheme. Following this decision, the United Kingdom government has similarly invalidated use of the EU-U.S. Privacy Shield as a mechanism for lawful personal data transfers from the United Kingdom to the United States under the so-called UK GDPR, and the Swiss Federal Data Protection and Information Commissioner announced that the Swiss-U.S. Privacy Shield does not provide adequate safeguards for the purposes of personal data transfers from Switzerland to the United States. The CJEU's decision in Schrems II also raised questions about whether one of the primary alternatives to the EU-U.S. Privacy Shield, namely, the European Commission's Standard Contractual Clauses, can lawfully be used for personal data transfers from Europe to the United States or other third countries that are not the subject of an adequacy decision of the European Commission. While the CJEU upheld the adequacy of the Standard Contractual Clauses in principle in Schrems II, it made clear that reliance on those Clauses alone may not necessarily be sufficient in all circumstances. Use of the Standard Contractual Clauses must now be assessed on a case-by-case basis taking into account the legal regime applicable in the destination country, in particular regarding applicable surveillance laws and relevant rights of individuals with respect to the transferred data. In the context of any given transfer, where the legal regime applicable in the destination country may or does conflict with the intended operation of the Standard Contractual Clauses and/or applicable European law, the decision in Schrems II and subsequent draft guidance from the European Data Protection Board, or EDPB, would require the parties to that transfer to implement certain supplementary technical, organizational and/or contractual measures to rely on the Standard Contractual Clauses as a compliant 'transfer mechanism.' However, the aforementioned draft guidance from the EDPB on such supplementary technical, organizational and/or contractual measures appears to conclude that no combination of such measures could be sufficient to allow effective reliance on the Standard Contractual Clauses in the context of transfers of personal data 'in the clear' to recipients in countries where the power granted to public authorities to access the transferred data goes beyond that which is 'necessary and proportionate in a democratic society' – which may, following the CJEU's conclusions in Schrems II on relevant powers of United States public authorities and commentary in that draft EDPB guidance, include the United States in certain circumstances (e.g., where Section 702 of the US Foreign Intelligence Surveillance Act applies). At present, there are few, if any, viable alternatives to the EU-U.S. Privacy Shield and the Standard Contractual Clauses. As such, if we are unable to implement a valid solution for personal data transfers from Europe, including, for example, obtaining individuals' explicit consent to transfer their personal data from Europe to the United States or other countries, we will face increased exposure to regulatory actions, substantial fines and injunctions against processing personal data from Europe. Inability to import personal data from Europe, including the EEA, United Kingdom or Switzerland, may also: restrict our activities in Europe; limit our ability to collaborate with partners as well as other service providers, contractors and other companies subject to European data protection laws; and/or require us to increase our data processing capabilities in Europe at significant expense or otherwise cause us to change the geographical location or segregation of our relevant systems and operations – any or all of which could adversely affect our financial results. Additionally, other countries outside of Europe have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business. The type of challenges we face in Europe will likely also arise in other jurisdictions that adopt laws similar in construction to the GDPR or regulatory frameworks of equivalent complexity.

Following the United Kingdom's withdrawal from the EU on January 31, 2020 and end of the transition period on December 31, 2020, as noted above, the United Kingdom has introduced the UK GDPR which currently makes the privacy regimes of the EEA and United Kingdom similar, though it is possible that either the EU, and consequently those further states that make up the remainder of the EEA, or United Kingdom could elect to change their approach and create differences in legal requirements and regulation in this area. This could expose us to two parallel regimes, each of which potentially authorizes similar fines (see below) and other potentially divergent enforcement actions for certain violations. Furthermore, there will now be increasing scope for divergence in application, interpretation and enforcement of the data protection law as between the United Kingdom and EEA. For example, it is unclear whether transfers of personal data from the EEA to the United Kingdom will be permitted to take place on the basis of a future adequacy decision of the European Commission, or whether a "transfer mechanism," such as the Standard Contractual Clauses, will be required. For the meantime, under the post-Brexit Trade and Cooperation Agreement between the European Union and the United Kingdom, it has been agreed that transfers of personal data to the United Kingdom from EEA Member States will not be treated as "restricted transfers" to a non-EEA country for a period of up to four months from January 1, 2021, plus a potential further two-month extension,

or the extended adequacy assessment period. If the European Commission does not adopt an 'adequacy decision' in respect of the United Kingdom prior to the expiry of the extended adequacy assessment period, from that point onwards the United Kingdom will be an "inadequate third country" under the GDPR and transfers of data from the EEA to the United Kingdom will require a "transfer mechanism," such as the Standard Contractual Clauses.

Under the GDPR, fines of up to €20 million or up to 4% of an undertaking's total worldwide annual turnover of the preceding financial year, whichever is higher, may be imposed. Further, following the withdrawal of the UK from the EU and the end of the transitional period, we will have to comply with the GDPR and separately the UK GDPR, each regime having the ability to fine up to the greater of €20 million/ £17.5 million or 4% of global turnover. In addition to administrative fines, a wide variety of other potential enforcement powers are available to competent authorities in respect of potential and suspected violations of the GDPR, including extensive audit and inspection rights, and powers to order temporary or permanent bans on all or some processing of personal data carried out by noncompliant actors. Implementing mechanisms to endeavor to ensure compliance with the GDPR and relevant local legislation in EEA Member States and the UK may be onerous and may interrupt or delay our development activities, and adversely affect our business, financial condition, results of operations, and prospects. In addition to the foregoing, a breach of the GDPR or other applicable privacy and data protection laws and regulations could result in regulatory investigations, reputational damage, orders to cease/change our use of data, enforcement notices, or potential civil claims including class action-type litigation. While we have taken steps to comply with the GDPR where applicable, including by reviewing our security procedures, engaging data protection personnel, and entering into data processing agreements with relevant contractors, our efforts to achieve and remain in compliance may not be fully successful.

Compliance with U.S. and foreign privacy and security laws, rules and regulations could require us to take on more onerous obligations in our contracts, require us to engage in costly compliance exercises, restrict our ability to collect, use and disclose data, or in some cases, impact our or our partners' or suppliers' ability to operate in certain jurisdictions. Each of these constantly evolving laws can be subject to varying interpretations. Failure to comply with U.S. and foreign data protection laws and regulations could result in government investigations and enforcement actions (which could include civil or criminal penalties), fines, private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

The withdrawal of the UK from the EU may adversely impact our ability to obtain regulatory approvals of our product candidates in the EU, result in restrictions or imposition of taxes and duties for importing our product candidates into the EU, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the EU.

Following the result of a referendum in 2016, the UK left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the UK and the EU, the UK was subject to a transition period until December 31, 2020, or the Transition Period, during which EU rules continued to apply. A trade and cooperation agreement, or the Trade and Cooperation Agreement, that outlines the future trading relationship between the United Kingdom and the European Union was agreed in December 2020.

Since a significant proportion of the regulatory framework in the UK applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and may continue to have, a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the UK or the EU. For example, Great Britain is no longer be covered by the centralized procedures for obtaining EU-wide marketing authorization from the EMA and, unless a specific agreement is entered into, a separate process for authorization of drug products, including tildacerfont and any future product candidates, will be required in Great Britain. It is currently unclear whether the Medicines & Healthcare products Regulatory Agency, or MHRA, in the UK is sufficiently prepared to handle the increased volume of marketing authorization applications that it is likely to receive. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our tildacerfont in the UK or the EU and restrict our ability to generate revenue and achieve and sustain profitability.

While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the UK and the EU there may be additional non-tariff costs to such trade which did not exist prior to the end of the Transition Period. Further, should the UK diverge from the EU from a regulatory perspective in relation to medicinal products, tariffs could be put into place in the future. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the UK or the EU for tildacerfont and any future product candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global

trade and, in particular, trade between the impacted nations and the UK. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of tildacerfont and any future product candidates.

We face an inherent risk of product liability as a result of the clinical testing of tildacerfont and any future product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if tildacerfont or any future product candidates causes or is perceived to cause injury or is found to be otherwise unsuitable during clinical 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, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of tildacerfont. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for tildacerfont and any future product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulatory authorities;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing, or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize tildacerfont and any future product candidates; or
- a decline in our share price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry an aggregate of up to $7.0 million of product liability insurance covering our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. If we determine that it is prudent to increase our product liability coverage due to the commercial launch of any approved product, we may be unable to obtain such increased coverage on acceptable terms, or at all. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, clinical research organizations, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products internationally once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, clinical research organizations, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in

substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

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

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. As of December 31, 2020, after reducing net operating losses, or NOLs, and tax credits for amounts not expected to be utilized, we had federal NOL carryforwards of approximately $58.6 million and state NOL carryforwards of approximately $58.4 million. The federal NOL carryforwards arising in taxable years beginning prior to 2018 will begin to expire in 2036 and state NOL carryforwards will begin to expire in 2036, unless previously utilized. We also have federal and state tax credit carryforwards totaling $3.0 million and $0.6 million, respectively. The federal tax credit carryforwards will begin to expire in 2036, unless previously utilized. The state tax credits will not expire.

Under the Tax Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, federal NOL carryforwards generated in tax years beginning after December 31, 2017 may be carried forward indefinitely but, in the case of tax years beginning after 2020, may only be used to offset 80% of our taxable income annually. Similar rules may apply under state tax laws. For example, on June 29, 2020, California enacted A.B. 85 which imposed limits on the usability of California state net operating losses and certain tax credits in tax years beginning after 2019 and before 2023. Such limitations could result in the expiration of our carryforwards before they can be utilized and, if we are profitable, our future cash flows could be adversely affected due to our increased taxable income or tax liability. Our NOLs and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual limitations under Section 382 and 383 of the Internal Revenue Code of 1986, as amended. Under Section 382, certain cumulative changes in the ownership interest of significant stockholders over a rolling three-year period in excess of 50 percentage points (by value), could result in an ownership change that may limit our ability to utilize our NOL carryforwards and other tax attributes to offset future taxable income or tax liabilities. An ownership change analysis covering periods through December 31, 2020 concluded that an ownership change occurred in May 2016 and in August 2020. As a result of the ownership change, we derecognized NOL-related deferred tax assets down to the amount expected to be realized. Our ability to use our remaining NOL carryforwards may be further limited if we experience a Section 382 ownership change as a result of future changes in our stock ownership. As of December 31, 2020, we recorded a full valuation allowance on our net deferred tax assets.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, the Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act, modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or future reform legislation could have a material impact on the value of our net deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Risks Related to Our Reliance on Third Parties

We depend on intellectual property licensed from Lilly, the termination of which could result in the loss of significant rights, which would harm our business.

We are dependent on technology, patents, know-how, and proprietary materials, both our own and licensed from others. We entered into a license agreement with Lilly in May 2016 pursuant to which we were granted an exclusive, worldwide, royalty bearing, sublicensable license under certain technology, patent rights, know-how, and proprietary materials relating to certain CRF1 receptor antagonist compounds. Any termination of this license will result in the loss of significant rights and will restrict our ability to develop and commercialize tildacerfont.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below under “Risks Related to Our Intellectual Property.” If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize tildacerfont.

We currently rely on, and intend to continue relying on, third-party CROs in connection with our clinical trials for tildacerfont. We control or will control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with applicable protocol, legal, regulatory, and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, such regulatory authorities may determine that our clinical trials do not comply with the GCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our failure or any failure by our CROs to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

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 preclinical, clinical and non-clinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed, or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize tildacerfont and any future product candidates. As a result, our financial results and the commercial prospects for tildacerfont and any future product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Although we carefully manage our relationships with our CROs, we may encounter challenges or delays in the future and these delays or challenges may have a material adverse impact on our business, prospects, financial condition, and results of operations.

In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition, and results of operations.

We rely completely on third parties to manufacture our preclinical and clinical drug supplies and we intend to rely on third parties to produce commercial supplies of tildacerfont and any future product candidates, if approved, and these third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug product or fail to do so at acceptable quality levels or prices.*

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture tildacerfont and any future product candidates on a clinical or commercial scale. Instead, we rely on contract manufacturers for such production. In particular, we currently rely on a single-source manufacturer for drug product, and a single-source manufacturer for drug substance.

We do not currently have any long-term agreement with a manufacturer to produce raw materials, APIs, and the finished products of tildacerfont or the associated packaging and administration syringes used in our current product format and we may rely on single source suppliers for clinical supply of API and drug product of tildacerfont. We will need to identify and qualify a third-party manufacturer prior to commercialization of tildacerfont, and we intend to enter into agreements for commercial production with third-party suppliers. As tildacerfont is intended to treat rare endocrine disorders, we will only require a low-volume of raw materials and APIs, and in some cases with single-source suppliers and manufacturers. Our reliance on third-party suppliers and manufacturers, including single-source suppliers, could harm our ability to develop tildacerfont and any future product candidates or to commercialize any product candidates that are approved. Further, any delay in identifying and qualifying a manufacturer for commercial production could delay the potential commercialization of tildacerfont and any future product candidates, and, in the event that we do not have sufficient product to complete our clinical trials, it could delay such trials.

The facilities used by our contract manufacturers to manufacture tildacerfont and any future product candidates must be approved by the applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after an NDA or comparable foreign regulatory marketing application is submitted. We currently do not control the manufacturing process of tildacerfont and are completely dependent on our contract manufacturing partners for compliance with the FDA's cGMP requirements for manufacture of both the active drug substances and finished drug product. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's strict regulatory requirements, they will not be able to secure or maintain FDA approval for the manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authority does not approve these facilities for the manufacture of tildacerfont or any future product candidates or if it withdraws any such approval in the future, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all, which would significantly impact our ability to develop, obtain regulatory approval for, or market tildacerfont and any future product candidates.

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. Furthermore, if contaminants are discovered in our supply of tildacerfont or any future product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. Any stability or other issues relating to the manufacture of tildacerfont may occur in the future. In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns, or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidate to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

If we or our third-party manufacturers use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials, by our third-party manufacturers. Our manufacturers are subject to federal, state, and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development, and production efforts, which could harm our business, prospects, financial condition, or results of operations.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for tildacerfont, any future product candidates, and other proprietary technologies we develop, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize tildacerfont, if approved, any future product candidates, and other proprietary technologies if approved, may be adversely affected.

Our commercial success will depend in part on our ability to obtain and maintain a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to tildacerfont, any future product candidates, and other proprietary technologies we develop. If we are unable to obtain or maintain patent protection with respect to tildacerfont, any future product candidates, and other proprietary technologies we may develop, our business, financial condition, results of operations, and prospects could be materially harmed.

The patent position of biotechnology and pharmaceutical companies is highly uncertain and involves complex legal, scientific, and factual questions and has been the subject of frequent litigation in recent years. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain. Our patent applications may not result in patents being issued which protect tildacerfont, any future product candidates, and other proprietary technologies we may develop or which effectively prevent others from commercializing competitive technologies and products. Further, no consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States or in many jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators will be successful in protecting tildacerfont, any future product candidates, and other proprietary technologies and their uses by obtaining, defending and enforcing patents. These risks and uncertainties include the following:

- the United States Patent and Trademark Office, or USPTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable, or may otherwise not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with, or eliminate our ability to make, use and sell our potential product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict with our patent applications, either by claiming the same composition of matter, methods or formulations or by claiming subject matter that could dominate our patent position;
- any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to tildacerfont, any future product candidates, and other proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our applications for any application with an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop, and market competing product candidates in those countries.

The patent prosecution process is expensive, time-consuming, and complex, and we may not be able to file, prosecute, or maintain all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection for such output. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the

actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use tildacerfont, any future product candidates, and other proprietary technologies and erode or negate any competitive advantage we may have, which could have a material adverse effect on our financial condition and results of operations. For example:

- others may be able to make compounds that are similar to tildacerfont and any future product candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- we may not develop additional proprietary technologies that are patentable;
- our competitors might conduct research and development activities in countries where we do not have patent rights or where patent protection is weak and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our products;
- we cannot ensure that we will be able to successfully commercialize our products on a substantial scale, if approved, before the relevant patents that we own or license expire; or
- the patents of others may have an adverse effect on our business.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in interference, opposition or invalidity proceedings before U.S. or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering tildacerfont or any future product candidates will be considered patentable by the USPTO, courts in the United States, or by patent offices and courts in foreign countries. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property internationally.

The strength of patents in the biotechnology and pharmaceutical fields involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover tildacerfont and any future product candidates in the United States or in foreign countries. Even if such patents do successfully issue, third parties may challenge the ownership, validity, enforceability, or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to our patents could deprive us of exclusive rights necessary for the successful commercialization of tildacerfont and any future product candidates. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for tildacerfont or any future product candidates or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents we hold with respect to tildacerfont or any future product candidates is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, tildacerfont or any future product candidates.

For U.S. patent applications in which claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management and other employees.

For U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is greater level of uncertainty in the patent law. In September 2011, the Leahy-Smith America Invents Act, or America Invents Act, was signed into law. The America Invents Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO is developing regulations and procedures to govern the administration of the America Invents Act, and many of the substantive changes to patent law associated with the America Invents Act, and in particular, the "first to file" provisions, were enacted on March 16, 2013. This will require us to be cognizant going forward of the time from invention to filing of a patent application and be diligent in filing patent applications, but circumstances could prevent us from promptly filing patent applications on our inventions. It remains unclear what impact the America Invents Act will have on the operation of our business. As such, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Extensions may be available under certain circumstances, but the life of a patent and, correspondingly, the protection it affords is limited. Even if we or our licensors obtain patents covering our product candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive products, including generic products. Given the amount of time required for the development, testing, and regulatory review and approval of new product candidates, patents protecting such candidates may expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Depending upon the timing, duration, and specifics of any FDA marketing approval of tildacerfont, or any future product candidate we may develop, one or more of patents issuing from our U.S. patent applications may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984, or Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term, or PTE, of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Similar patent term restoration provisions to compensate for commercialization delay caused by regulatory review are also available in certain foreign jurisdictions, such as in Europe under Supplemental Protection Certificate, or SPC. If we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market tildacerfont and any future product candidates under patent protection would be reduced. Additionally, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents, or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced.

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

We are a party to a license agreement with Lilly under which we are granted intellectual property rights that are important to our business and our only product candidate, tildacerfont. If we fail to comply with our obligations under the license agreement, or we are subject to a bankruptcy, the license agreement may be terminated, in which event we would not be able to develop, commercialize or market tildacerfont.

Licensing of intellectual property rights is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property rights subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property rights of the licensor that are not subject to the license agreement;
- our right to sublicense intellectual property rights to third parties under collaborative development relationships;

- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of tildacerfont, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

Further, our current licensor or any future licensor may not always act in our best interest. If disputes over intellectual property rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, our business, results of operations, financial condition, and prospects may be adversely affected. We may enter into additional licenses in the future and if we fail to comply with obligations under those agreements, we could suffer adverse consequences.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described below. If we, our current licensor, or any future licensor fail to adequately protect this intellectual property, our ability to commercialize tildacerfont and any future product could be impeded.

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 USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment, and other similar provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patents and/or applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to foreign patent agencies. These systems and processes may be negatively impacted by the COVID-19 pandemic in various aspects. While an inadvertent lapse may sometimes be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market with similar or identical products or technology earlier than should otherwise have been the case, which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly on obtaining and enforcing patents. Our patent rights may be affected by developments or uncertainty in U.S. or foreign patent statutes, patent case law, USPTO rules and regulations or the rules and regulations of foreign patent offices. Obtaining and enforcing patents in the biotechnology and pharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States may, at any time, enact changes to U.S. patent law and regulations, including by legislation, by regulatory rule-making, or by judicial precedent, that adversely affect the scope of patent protection available and weaken the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. For example, the scope of patentable subject matter under 35 U.S.C. 101 has evolved significantly over the past several years as the Court of Appeals for the Federal Circuit and the Supreme Court issued various opinions, and the USPTO modified its guidance for practitioners on multiple occasions. Other countries may likewise enact changes to their patent laws in ways that adversely diminish the scope of patent protection and weaken the rights of patent owners to obtain patents, enforce patent infringement, and obtain injunctions and/or damages.

Further, the United States and other governments may, at any time, enact changes to law and regulation that create new avenues for challenging the validity of issued patents. For example, the America Invents Act created new administrative post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings that allow third parties to challenge the validity of issued patents. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

Patents are of national or regional effect. Filing, prosecuting, and defending patents on tildacerfont, any future product candidates, and other proprietary technologies we develop in all countries throughout the world would be prohibitively expensive. In addition, the laws of some foreign countries do not protect intellectual property rights in the same manner and to the same extent as 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. 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 of such patent protection 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.

The requirements for patentability may differ in certain countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval for a drug and its patent status. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors.

In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology or pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, and 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.

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

We may be subject to claims that former employees (including former employees of our licensors), collaborators or other third parties have an interest in our patents rights, trade secrets, or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. For example, we may have inventorship disputes arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing tildacerfont or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. 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, results of operations and prospects. 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.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have intellectual property rights, through licenses from third parties including Lilly, related to tildacerfont. Because our program may require the use of additional proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, tildacerfont may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license, on reasonable terms, proprietary rights related to any compositions, formulations, methods of use, processes or other intellectual property rights from third parties that we identify as being necessary for tildacerfont. Even if we are able to obtain a license to such proprietary rights, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Where we obtain licenses from or collaborate with third parties, we may not have the right to control the preparation, filing, and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business, or in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such application. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, including making royalty and milestone payments, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could have a material adverse impact on our business. Our business would suffer if any such licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical or similar to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

The licensing and acquisition of third-party proprietary rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party proprietary rights that we may consider necessary or attractive in order to commercialize tildacerfont. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we may collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate an exclusive license to any of the institution's proprietary rights in technology resulting from the collaboration. Regardless of such option to negotiate a license, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer, on an exclusive basis, their proprietary rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights on commercially reasonable terms, our ability to commercialize our products, and our business, financial condition, and prospects for growth, could suffer.

Third-party claims alleging intellectual property infringement may prevent or delay our drug discovery and development efforts.

Our success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including *inter partes* review, interference and reexamination proceedings before the USPTO, or oppositions and other comparable proceedings in foreign jurisdictions. The America Invents Act introduced new procedures including *inter partes* review and post grant review. The implementation of these procedures brings uncertainty to the possibility of challenges to our patents in the future and the outcome of such challenges. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing tildacerfont. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our activities related to tildacerfont may give rise to claims of infringement of the patent rights of others.

The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. We cannot assure you that any of our current or future product candidates will not infringe existing or future patents. We may not be aware of patents that have already issued that a third party might assert are infringed by one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing tildacerfont.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of tildacerfont. Because patent applications can take many years to issue and may be confidential for 18 months or more after filing, there may be currently pending third-party patent applications which may later result in issued patents that tildacerfont, any future product candidates, and other proprietary technologies may infringe, or which such third parties claim are infringed by the use of our technologies. Parties making claims against us for infringement or misappropriation of their intellectual property rights may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize tildacerfont or future product candidates. Defense of these claims, regardless of their merit, could involve substantial expenses and could be a substantial diversion of management and other employee resources from our business.

If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. In the future, we may agree to indemnify our commercial collaborators against certain intellectual property infringement claims brought by third parties.

Any claims of patent infringement asserted by third parties would be time-consuming and could:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing tildacerfont or any future product candidates until the asserted patent expires or is finally held invalid, unenforceable, or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be willfully infringing; and/or
- require us to enter into royalty or license agreements, which may not be available on commercially reasonable terms, or at all.

If we are sued for patent infringement, we would need to demonstrate that our products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do either. Proving invalidity or unenforceability is difficult. For example, in the United States, proving invalidity before federal courts requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's 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, which may not be available, defend an infringement action or challenge the validity or enforceability of the patents in court. Patent litigation is costly and time-consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid or unenforceable, we may incur substantial monetary damages, encounter significant delays in bringing tildacerfont to market and be precluded from developing, manufacturing or selling tildacerfont.

We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. We cannot be certain that any of our or our licensors' patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, Europe and elsewhere that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction, because:

- some patent applications in the United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived;

- pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, tildacerfont, and any future product candidates or the use of tildacerfont and any future product candidates;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and the difficulty in assessing the meaning of patent claims;
- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. Further, we may incorrectly determine that our technologies, products, or product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or internationally that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our products or product candidates.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours, and others may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import tildacerfont and future approved products or impair our competitive position. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of tildacerfont. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. Other countries have similar laws that permit secrecy of patent applications and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If a third party prevails in a patent infringement lawsuit against us, we may have to stop making and selling the infringing product, pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing a third party's patents, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of tildacerfont. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize tildacerfont, which could harm our business significantly. Even if we were able to obtain a license, the rights may be nonexclusive, which may give our competitors access to the same intellectual property.

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

As is common in the biotechnology and pharmaceutical industries, in addition to our employees, we engage the services of consultants to assist us in the development of tildacerfont, any future product candidates, and other proprietary technologies. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

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. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

Third parties including competitors may infringe, misappropriate or otherwise violate our patents, patents that may issue to us in the future, or the patents of our licensors that are licensed to us. To counter infringement or unauthorized use, we may need to or choose to file infringement claims, which can be expensive and time-consuming. We may not be able to prevent, alone or with our licensors, infringement, misappropriation, or other violation of our intellectual property, particularly in countries where the laws may not protect those rights as fully as in the United States.

If we choose to go to court to stop another party from using the inventions claimed in our patents, that individual or company has the right to ask the court to rule that such patents are invalid, unenforceable, or should not be enforced against that third party for any number of reasons. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements for patentability, including lack of novelty, obviousness, lack of written description, indefiniteness, or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution, i.e., committed inequitable conduct. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign patent offices and courts and may result in the revocation, cancellation, or amendment of any foreign patents we or our licensors hold now or in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. 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 such product candidate. Such a loss of patent protection would have a material adverse impact on our business.

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

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

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. 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.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers, and other advisors, and inventions agreements with employees, consultants, and advisors, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer, or third party with authorized access. Our security measures may not prevent an employee, consultant or customer from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our products that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets will over time be disseminated within the industry through independent development, the publication of journal articles, and the movement of personnel skilled in the art from company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors, and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our products and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our future trademarks or trade names may be unable to be obtained, challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with

our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights, or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

Moreover, any name we have proposed to use with tildacerfont 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 (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, it 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. Similar requirements exist in Europe. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

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. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our products or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with tildacerfont and any future product candidates;
- a collaborator with marketing, manufacturing, and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development, or commercialization of our current or future products or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future products;
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property;
- we may not be able to obtain intellectual property rights in technologies or products resulting from the collaboration; under certain situations, the collaborators may provide us with an option to negotiate a license to such developed technologies or products, however, we may not be able to negotiate such license; and
- a collaborator’s sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Risks Related to Ownership of Our Common Stock

An active, liquid and orderly trading market for our common stock may not be sustained.

Prior to the closing of our IPO in October 2020, there was no public market for shares of our common stock. An active trading market for our shares may not be sustained. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. As a result of these and other factors, you may be unable to resell your shares of our common stock. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The trading price of our common stock may be volatile, and you could lose all or part of your investment.*

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. For example, the closing price of our common stock since its trading began on October 9, 2020 to May 11, 2021 has ranged from a low of $14.63 to a high of $32.42. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this Quarterly Report on Form 10-Q, these factors include:

- the commencement, enrollment or results of our ongoing and planned clinical trials of tildacerfont or any future clinical trials we may conduct of tildacerfont and any future product candidates, or changes in the development status of tildacerfont and any future product candidates;
- acceptance by the FDA and EMA of data from our two Phase 2b clinical trials or any future clinical trials we conduct;
- any delay in our regulatory filings for tildacerfont and any future product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results or delays in clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial, or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for tildacerfont and any future product candidates;
- changes in laws or regulations applicable to tildacerfont and any future product candidates, including but not limited to clinical trial requirements for approvals;
- the failure to obtain coverage and adequate reimbursement of tildacerfont and any future product candidates, if approved;
- changes on the structure of healthcare payment systems;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our inability to establish collaborations if needed;
- our failure to commercialize tildacerfont and any future product candidates;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of tildacerfont and any future product candidates;
- introduction of new products or services offered by us or our competitors, or the release or publication of clinical trial results from competing product candidates;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth, if any, of the markets for classic CAH in adult and pediatric patients and a subpopulation of females with a rare form of PCOS, and other rare endocrine disorders that we may target;
- actual or anticipated variations in quarterly or annual operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;

- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- general political, health, and economic conditions, including the COVID-19 pandemic; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

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

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation, and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, pursuant to the Loan Agreement, we are prohibited from paying cash dividends without the prior written consent of Silicon Valley Bank, and future debt instruments may materially restrict our ability to pay dividends on our common stock. Any return to stockholders would therefore be limited to the appreciation, if any, of their stock.

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

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding capital stock, beneficially own shares representing a significant percentage of our common stock. Therefore, these stockholders have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

We are an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.*

We are an "emerging growth company" as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of certain exemptions from various public company reporting requirements, including being permitted to provide only two years of audited financial statements, in addition to any required financial statements with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this Quarterly Report on Form 10-Q, not being required to have our internal control over financial reporting audited by our independent registered public accounting firm under Section 404 of the Sarbanes-Oxley Act or 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We may take advantage of these exemptions until December 31, 2025 or until we are no longer an emerging growth company, whichever is earlier. We will cease to be an emerging growth company prior to the end of such five-year period if certain earlier events occur, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act, our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to use this extended transition period under the JOBS Act until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company, which would allow us to take advantage of many of the same exemptions available to emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation. We will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter. Investors may find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We previously identified and remediated a material weakness in our internal control over financial reporting and may identify material weaknesses in the future or otherwise fail to maintain proper and effective internal controls, which may impair our ability to produce accurate financial statements on a timely basis.

During the preparation of our financial statements for the years ended December 31, 2018 and 2019, we identified a material weakness in internal control over financial reporting primarily related to a lack of timely review over the financial statement close process. During these periods, we did not have a sufficient complement of qualified personnel within the accounting function and had a lack of segregation of duties to adequately conduct review and analysis of certain routine transactions.

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis. This material weakness could result in a misstatement of account balances or disclosures that would result in a material misstatement to the annual or interim financial statements that would not be prevented or detected. To address our material weakness, we added a chief financial officer and controller, and we implemented new processes and controls, formalized documentation of policies and procedures, and recruited additional accounting personnel. We have fully remediated the material weakness as of the filing date of our Annual Report for the fiscal year ending December 31, 2020. Completion of remediation does not provide assurance that our remediation or other controls will continue operating effectively. Remediation costs consisted primarily of additional personnel expenses, which did not have a material impact to our financial statements.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Commencing with our fiscal year ending December 31, 2021, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our IPO, we have never been required to test our internal controls within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

The measures we have taken to date, and actions we may take in the future, may not be sufficient to remediate the control deficiencies that led to our material weakness in our internal control over financial reporting or to prevent or avoid potential future material weaknesses. We may not have identified all material weaknesses. Moreover, our current controls and any new controls that we develop may become inadequate because of changes in conditions in our business. Further, weaknesses in our disclosure controls and internal control over financial reporting may be discovered in the future. Any failure to develop or maintain effective controls or any difficulties encountered in their implementation or improvement could harm our operating results or cause us to fail to meet our reporting obligations and may result in a restatement of our financial statements for prior periods, which could cause the price of our common stock to decline. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on Nasdaq.

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

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

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

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of March 31, 2021, there were 23,301,872 shares of our common stock outstanding.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, Rule 144 and Rule 701 under the Securities Act of 1933, as amended, or the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Further, the holders of 15,492,019 shares of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

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

We expect that we will need significant additional capital in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities, and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

Pursuant to our 2020 Plan, our management is authorized to grant stock options and other stock awards to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under our 2020 Plan will automatically increase on January 1 of each year continuing through and including January 1, 2030, by 5% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. In addition, pursuant to our ESPP, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year continuing through January 1, 2030, by the lesser of (i) 1% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of the automatic increase, and (ii) 441,280 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, the president, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer, or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware (and any appellate court therefrom) is the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative claim or cause of action brought on our behalf; (ii) any claim or cause of action for breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to us or our stockholders; (iii) any claim or cause of action against us or any of our current or former directors, officers, or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any claim or cause of action seeking to interpret, apply, enforce, or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws (in each case as may be amended from time to time); (v) any claim or cause of action as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any claim or cause of action against us or any of our current or former directors, officers, or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants. These provisions would not apply to claims or causes of action brought to enforce a duty or liability created by the Securities Act and the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation and our amended and restated bylaws further provide that, to the fullest extent permitted by law, the federal district courts of the United States of America is the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation and our amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and the provisions may not be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees and may discourage these types of lawsuits. Furthermore, the enforceability of similar choice of forum provisions in other companies' certificates of incorporation or bylaws has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. If a court were to find either exclusive forum provision contained in our amended and restated certificate of incorporation or amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving such action in other jurisdictions, all of which could seriously harm our business.

General Risk Factors

Our failure to meet Nasdaq's continued listing requirements could result in a delisting of our common stock.

If we fail to satisfy the continued listing requirements of 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 our 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 our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the Nasdaq minimum bid price requirement or prevent future non-compliance with the listing requirements of Nasdaq.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations, and those of our CROs and other contractors and consultants, could be subject to 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, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce tildacerfont. Our ability to obtain clinical supplies of tildacerfont and any future product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Our corporate headquarters is located in California near major earthquake faults and fire zones. The ultimate impact on us, our suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

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

Unregistered Sales of Equity Securities

None.

Use of Proceeds

We commenced our IPO pursuant to the registration statement on Form S-1 (File Nos. 333-248924 and 333-249397) that was declared effective on October 8, 2020 and registered an aggregate of 6,900,000 shares of our common stock. On October 8, 2020, we sold 6,900,000 shares of our common stock at a public offering price of $15.00 per share for aggregate gross proceeds of $103.5 million. In October 2020, we completed our IPO. Cowen and Company, LLC, SVB Leerink LLC, Credit Suisse Securities (USA) LLC, and RBC Capital Markets, LLC served as joint book-running managers.

The net proceeds of the IPO were approximately $93.4 million, after deducting underwriting discounts, commissions and offering expenses. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

Upon receipt, the net proceeds from our IPO were held in cash and cash equivalents. Through March 31, 2021, we have not used any of the net proceeds from our IPO. We are investing these funds in accordance with our investment policy. We expect to use the net proceeds from our IPO as described under "Use of Proceeds" in the final prospectus filed with the SEC on October 9, 2020 pursuant to rule 424(b)(4) of the Securities Act. We cannot predict with certainty all of the particular uses for the net proceeds from our IPO, or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of the net proceeds will vary depending on numerous factors, including our ability to access additional financing, the relative success and cost of our clinical trials. As a result, our management will have broad discretion in the application of the net proceeds, and investors will be relying on our judgment regarding the application of the net proceeds from our IPO.

Item 3. Defaults Upon Senior Securities

Not Applicable.

Item 4. Mine Safety Disclosures

Not Applicable.

Item 5. Other Information

None.

Item 6. Exhibits

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on October 14, 2020).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on October 14, 2020).
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-248924), filed with the SEC on October 5, 2020).
4.2	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated November 5, 2018 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-248924), filed with the SEC on September 18. 2020).
10.1†	First Amendment to Loan and Security Agreement, by and between the Registrant and Silicon Valley Bank dated March 19, 2021 (incorporated by reference to Exhibit 10.19 to the Registrant's Annual Report on Form 10-K (File No. 001-39594), filed with the SEC on March 22, 2021.
10.2†	Letter Agreement, by and between the Registrant and Daniel Spiegelman, dated August 31, 2020 (incorporated by reference to Exhibit 10.20 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-248924), filed with the SEC on September 18. 2020).
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as amended, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*#	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*#	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

* Filed herewith.
# The information in Exhibits 32.1 and 32.2 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (including this Quarterly Report on Form 10-Q), unless the Registrant specifically incorporates the foregoing information into those documents by reference.

SIGNATURES

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

SPRUCE BIOSCIENCES, INC.

Date: May 12, 2021

By: /s/ Richard King
Richard King
Chief Executive Officer
(Principal Executive Officer)

Date: May 12, 2021

By: /s/ Samir Gharib
Samir Gharib
Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Richard King, certify that:

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

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

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

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

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

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

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

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

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

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

Date: May 12, 2021

By: /s/ **Richard King**

Richard King
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Samir Gharib, certify that:

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

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

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

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

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

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

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

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

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

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

Date: May 12, 2021

By: /s/ Samir Gharib

Samir Gharib
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

In connection with the Quarterly Report of Spruce Biosciences, Inc. (the "Company") on Form 10-Q for the period ended March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: May 12, 2021

By: /s/ Richard King

Richard King
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

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

In connection with the Quarterly Report of Spruce Biosciences, Inc. (the "Company") on Form 10-Q for the period ended March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: May 12, 2021

By: /s/ Samir Gharib

Samir Gharib
Chief Financial Officer
(Principal Financial Officer)