

**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: 001-35403**

**Verastem, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)  
**117 Kendrick Street, Suite 500**  
**Needham, MA**  
(Address of principal executive offices)

**27-3269467**  
(I.R.S. Employer  
Identification Number)

**02494**  
(Zip Code)

**(781) 292-4200**

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Securities Exchange Act of 1934:

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 10, 2021, there were 171,919,345 shares of Common Stock outstanding.

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### FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements related to present facts or current conditions or historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. Such statements relate to, among other things, the development and activity of our programs and product candidates, VS-6766 (rapidly accelerated fibrosarcoma (RAF)/ mitogen-activated protein kinase kinase (MEK) program) and defactinib (focal adhesion kinase (FAK) program), the structure of our planned and pending clinical trials, and the timeline and indications for clinical development, regulatory submissions and commercialization of activities. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the results discussed in the forward-looking statements we make. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the uncertainties inherent in research and development of VS-6766 and defactinib, such as negative or unexpected results of clinical trials; whether and when any applications for VS-6766 and defactinib may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for VS-6766 and defactinib, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether VS-6766 or defactinib will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for VS-6766 and defactinib; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of VS-6766 and defactinib; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that VS-6766 or defactinib will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels of efficacy; that we face substantial competition, which may result in others developing or commercializing products before or more successfully than we do which could result in reduced market share or market potential for VS-6766 or defactinib; that we will be unable to in-license additional compounds or successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we may not realize the operational efficiencies and cost savings from restructuring, that we or Chugai Pharmaceutical, Co. Ltd., will fail to fully perform under the license agreement; that we or Secura Bio, Inc. will fail to fully perform under the asset purchase agreement; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients; and that the duration and impact of COVID-19 may affect, precipitate or exacerbate one or more of the foregoing risks and uncertainties. Other risks and uncertainties include those identified in our Annual Report on Form 10-K for the year ended December 31, 2020 as filed with Securities and Exchange Commission (SEC) on March 18, 2021, and in any subsequent filings with the SEC.

As a result of these and other factors, we may not achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. The forward-looking statements contained in this Quarterly Report on Form 10-Q reflect our views as of the date hereof. We do not assume and specifically disclaim any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

**PART I—FINANCIAL INFORMATION****Item 1. Condensed Consolidated Financial Statements (unaudited).**

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
**(in thousands, except per share amounts)**

	<u>March 31,</u> <u>2021</u>	<u>December 31,</u> <u>2020</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 35,514	\$ 67,782
Short-term investments	85,583	73,444
Accounts receivable, net	1,125	239
Prepaid expenses and other current assets	5,456	3,473
Total current assets	127,678	144,938
Property and equipment, net	335	416
Right-of-use asset, net	2,628	2,726
Restricted cash	241	241
Long-term investments	5,998	5,995
Other assets	21	33
Total assets	<u>\$ 136,901</u>	<u>\$ 154,349</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 2,453	\$ 1,875
Accrued expenses	8,808	14,660
Lease liability, short-term	583	558
Total current liabilities	11,844	17,093
Non-current liabilities:		
Convertible senior notes	19,672	19,051
Lease liability, long-term	2,776	2,931
Total liabilities	34,292	39,075
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	—	—
Common stock, \$0.0001 par value; 300,000 shares authorized, 171,731 and 170,456 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	17	17
Additional paid-in capital	710,100	707,715
Accumulated other comprehensive income	34	53
Accumulated deficit	(607,542)	(592,511)
Total stockholders' equity	102,609	115,274
Total liabilities and stockholders' equity	<u>\$ 136,901</u>	<u>\$ 154,349</u>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(unaudited)**  
**(in thousands, except per share amounts)**

	<u>Three months ended March 31,</u>	
	<u>2021</u>	<u>2020</u>
Revenue:		
Product revenue, net	\$ —	\$ 5,034
License and collaboration revenue	—	22
Sale of COPIKTRA license and related assets	850	—
Transition services revenue	156	—
Total revenue	<u>1,006</u>	<u>5,056</u>
Operating expenses:		
Cost of sales - product	—	495
Cost of sales - intangible amortization	—	392
Research and development	8,896	10,924
Selling, general and administrative	6,218	19,604
Total operating expenses	<u>15,114</u>	<u>31,415</u>
Loss from operations	(14,108)	(26,359)
Other expense	—	(1,313)
Interest income	52	356
Interest expense	(975)	(10,674)
Net loss	<u>\$ (15,031)</u>	<u>\$ (37,990)</u>
Net loss per share—basic and diluted	<u>\$ (0.09)</u>	<u>\$ (0.35)</u>
Weighted average common shares outstanding used in computing net loss per share - basic and diluted	171,586	108,153
Net loss	\$ (15,031)	\$ (37,990)
Unrealized loss on available-for-sale securities	(19)	(5)
Comprehensive loss	<u>\$ (15,050)</u>	<u>\$ (37,995)</u>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**(unaudited)**  
**(in thousands, except share data)**

	Common stock		Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
<b>Balance at December 31, 2020</b>	<b>170,456,179</b>	<b>\$ 17</b>	<b>\$ 707,715</b>	<b>\$ 53</b>	<b>\$ (592,511)</b>	<b>\$ 115,274</b>
Net loss	—	—	—	—	(15,031)	(15,031)
Unrealized loss on available-for-sale marketable securities	—	—	—	(19)	—	(19)
Issuance of common stock resulting from exercise of stock options	173,890	—	381	—	—	381
Issuance of common stock resulting from vesting of restricted stock units	1,047,271	—	(52)	—	—	(52)
Stock-based compensation expense	—	—	1,980	—	—	1,980
Issuance of common stock under Employee Stock Purchase Plan	53,372	—	76	—	—	76
<b>Balance at March 31, 2021</b>	<b>171,730,712</b>	<b>\$ 17</b>	<b>\$ 710,100</b>	<b>\$ 34</b>	<b>\$ (607,542)</b>	<b>\$ 102,609</b>
<b>Balance at December 31, 2019</b>	<b>80,117,531</b>	<b>\$ 8</b>	<b>\$ 531,937</b>	<b>\$ 14</b>	<b>\$ (524,785)</b>	<b>\$ 7,174</b>
Net loss	—	—	—	—	(37,990)	(37,990)
Unrealized loss on available-for-sale marketable securities	—	—	—	(5)	—	(5)
Issuance of common stock resulting from exercise of stock options	645,628	—	983	—	—	983
Issuance of common stock resulting from vesting of restricted stock units	58,166	—	(51)	—	—	(51)
Stock-based compensation expense	—	—	1,370	—	—	1,370
Issuance of common stock resulting from private investment in public equity offering, net of issuance costs of \$6,171	46,511,628	5	93,824	—	—	93,829
Issuance of common stock under Employee Stock Purchase Plan	227,141	—	259	—	—	259
Conversion of 2019 Notes into common stock	34,796,350	3	57,411	—	—	57,414
<b>Balance at March 31, 2020</b>	<b>162,356,444</b>	<b>\$ 16</b>	<b>\$ 685,733</b>	<b>\$ 9</b>	<b>\$ (562,775)</b>	<b>\$ 122,983</b>

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(unaudited)**  
**(in thousands)**

	<b>Three months ended March 31,</b>	
	<b>2021</b>	<b>2020</b>
<b>Operating activities</b>		
Net loss	\$ (15,031)	\$ (37,990)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	81	94
Amortization of acquired intangible asset	—	392
Amortization of right-of-use asset and lease liability	(32)	17
Stock-based compensation expense	1,980	1,370
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	636	8,779
Change in fair value of interest make whole provision for 2019 Notes	—	1,313
Changes in operating assets and liabilities:		
Accounts receivable, net	(886)	(802)
Inventory	—	(1,276)
Prepaid expenses, other current assets and other assets	(1,814)	(2,109)
Accounts payable	774	(2,535)
Accrued expenses and other liabilities	(5,026)	(1,256)
Net cash used in operating activities	(19,318)	(34,003)
<b>Investing activities</b>		
Purchases of property and equipment	(196)	—
Purchases of investments	(17,551)	—
Maturities of investments	5,375	32,050
Net cash (used in) provided by investing activities	(12,372)	32,050
<b>Financing activities</b>		
Proceeds from the exercise of stock options and employee stock purchase program	300	1,242
Interest make-whole payments on the 2019 Notes	—	(1,763)
Settlement of restricted stock for tax withholdings	(878)	—
Proceeds from the issuance of common stock, net	—	93,942
Net cash (used in) provided by financing activities	(578)	93,421
(Decrease) increase in cash, cash equivalents and restricted cash	(32,268)	91,468
Cash, cash equivalents and restricted cash at beginning of period	68,023	79,262
Cash, cash equivalents and restricted cash at end of period	\$ 35,755	\$ 170,730
<b>Supplemental disclosure of non-cash investing and financing activities</b>		
Common stock issuance costs included in accounts payable and accrued expenses	\$ 15	\$ 128
Conversion of 2019 Notes into common stock	\$ —	\$ 57,414
Purchases of property and equipment included in accounts payable and accrued expenses	\$ —	\$ 13
Settlement of restricted stock units for tax withholdings included in accrued expenses	\$ 9	\$ 51
Receivables related to stock option exercises in prepaid expenses and other current assets	\$ 157	\$ —

See accompanying notes to the condensed consolidated financial statements.

**Verastem, Inc.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**  
**(unaudited)**

**1. Nature of business**

Verastem, Inc. (the Company) is a development-stage biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. The Company's pipeline is focused on novel anticancer agents that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, particularly RAF/MEK inhibition and FAK inhibition.

The Company's most advanced product candidates, VS-6766 and defactinib, are being investigated in both preclinical and clinical studies for treatment of various solid tumors, including, low-grade serous ovarian cancer, non-small cell lung cancer, colorectal cancer, pancreatic cancer, uveal melanoma, and endometrial cancer. The Company believes that these compounds may be beneficial as therapeutics either as single agents or when used together in combination with other agents, other pathway inhibitors, or other current and emerging standard of care treatments in cancers that do not adequately respond to currently available therapies.

On September 24, 2018, the Company's first commercial product, COPIKTRA® (duvelisib), was approved by the U.S. Food and Drug Administration (the FDA) for the treatment of adult patients with certain hematologic cancers including relapsed or refractory chronic lymphocytic leukemia/ small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. On August 10, 2020, the Company and Secura Bio, Inc. (Secura) entered into an asset purchase agreement (Secura APA). Pursuant to the Secura APA, the Company sold to Secura its exclusive worldwide license, including certain related assets for the research, development, commercialization, and manufacture in oncology indications of products containing COPIKTRA (duvelisib). The transaction closed on September 30, 2020. Refer to *Note 13. License, collaboration, and commercial agreements* for a detailed discussion of the Secura APA.

The condensed consolidated financial statements include the accounts of Verastem Securities Company and Verastem Europe GmbH, wholly-owned subsidiaries of the Company. All financial information presented has been consolidated and includes the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

The Company is subject to the risks associated with other life science companies, including, but not limited to, possible failure of preclinical testing or clinical trials, competitors developing new technological innovations, inability to obtain marketing approval of the Company's product candidates, VS-6766 and defactinib, market acceptance and commercial success of the Company's product candidates, VS-6766 and defactinib, following receipt of regulatory approval, and, protection of proprietary technology and the continued ability to obtain adequate financing to fund the Company's future operations. If the Company does not obtain marketing approval and successfully commercialize its product candidates, VS-6766 and defactinib, following regulatory approval, it will be unable to generate product revenue or achieve profitability and may need to raise additional capital.

The Company has historical losses from operations and anticipates that it will continue to incur losses as it continues the research and development of its product candidates. As of March 31, 2021, the Company had cash, cash equivalents, and investments of \$127.1 million, and accumulated deficit of \$607.5 million. The Company expects its existing cash resources will be sufficient to fund its planned operations through at least 12 months from the date of issuance of these condensed consolidated financial statements.

The Company expects to finance the future development costs of its clinical product portfolio with its existing cash, cash equivalents, and investments, through future milestones and royalties received through the Secura APA or through strategic financing opportunities that could include, but are not limited to collaboration agreements, future offerings of its equity, or the incurrence of debt. However, there is no guarantee that any of these strategic or financing opportunities will be executed or executed on favorable terms, and some could be dilutive to existing stockholders. If the



Company fails to obtain additional future capital, it may be unable to complete its planned preclinical studies and clinical trials and obtain approval of certain investigational product candidates from the FDA or foreign regulatory authorities.

## 2. Summary of significant accounting policies

### Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of the Company have been prepared in accordance with generally accepted accounting principles in the United States (GAAP) for interim financial reporting and as required by Regulation S-X, Rule 10-01 under the assumption that the Company will continue as a going concern for the next twelve months. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements, or any adjustments that might result from the uncertainty related to the Company's ability to continue as a going concern. In the opinion of management, all adjustments (including those which are normal and recurring) considered necessary for a fair presentation of the interim financial information have been included. When preparing financial statements in conformity with GAAP, the Company must make estimates and assumptions that affect the reported amounts and related disclosures at the date of the financial statements. Actual results could differ from those estimates. Additionally, operating results for the three months ended March 31, 2021 are not necessarily indicative of the results that may be expected for any other interim period or for the year ending December 31, 2021. For further information, refer to the financial statements and footnotes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2020, as filed with the Securities and Exchange Commission (SEC) on March 18, 2021.

### Significant Accounting Policies

The significant accounting policies are described in *Note 2. Significant accounting policies* in the Company's Annual Report on Form 10-K for the year ended December 31, 2020. During the three months ended March 31, 2021, the Company did not adopt any additional significant accounting policies except as outlined within "*Recently Adopted Accounting Standards Updates*" section immediately below.

#### *Recently Adopted Accounting Standards Updates*

In December 2019, the FASB issued Accounting Standard Update (ASU) No 2019-12, Simplifying Accounting for Income Taxes (ASU 2019-12). ASU 2019-12 removes certain exceptions for recognizing deferred taxes for investments, performing intraperiod allocations, calculating income taxes in interim periods, and adds certain guidance to remove complexity in certain areas. ASU 2019-12 is effective for all entities for annual and interim periods beginning after December 15, 2020. In the first quarter of 2021, the Company adopted ASU 2019-12. The provisions related to intraperiod tax allocation and interim recognition of enactment of tax laws are being adopted on a prospective basis. The adoption of ASU 2019-12 did not have an effect on the Company's condensed financial statements or disclosures.

#### *Recently Issued Accounting Standards Updates*

In June 2016, the FASB issued ASU No. 2016-13, Measurement of Credit Losses on Financial Instruments (ASU 2016-13). ASU 2016-13 will replace the incurred loss impairment methodology under current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. In November 2019, the FASB issued ASU 2019-10, Financial Instruments – Credit Losses (Topic 326), Derivatives (Topic 815), and Leases (Topic 842). This ASU delayed the required adoption for SEC filers that are smaller reporting companies as of their determination on November 15, 2019, until annual and interim periods beginning after December 15, 2022, with early adoption permitted. The Company has determined that as of November 15, 2019, it is a smaller reporting company and has not elected to early adopt this standard. The Company is currently evaluating the impact the adoption of the standard will have on its condensed consolidated financial statements and related disclosures.

In August 2020, the FASB issued No. ASU 2020-06, Debt – Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging – Contracts in Entity's Own Equity (Subtopic 815 – 40) (ASU 2020-06). ASU 2020-06 simplifies the complexity associated with applying U.S. GAAP for certain financial instruments with

characteristics of liabilities and equity. More specifically, the amendments focus on the guidance for convertible instruments and derivative scope exception for contracts in an entity's own equity. The ASU also simplifies the diluted earnings per share (EPS) calculation in certain areas. For smaller reporting companies, ASU 2020-06 is effective for fiscal years beginning after December 15, 2023, and interim periods within those fiscal years. The Company is currently evaluating the impact ASU 2020-06 will have on its condensed consolidated financial statements and related disclosures.

#### Concentrations of credit risk and off-balance sheet risk

Cash, cash equivalents, investments and trade accounts receivable are financial instruments that potentially subject the Company to concentrations of credit risk. The Company mitigates this risk by maintaining its cash and cash equivalents and investments with high quality, accredited financial institutions. The management of the Company's investments is not discretionary on the part of these financial institutions. As of March 31, 2021, the Company's cash, cash equivalents and investments were deposited at three financial institutions and it has no significant off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

As of March 31, 2021 there was one customer, Secura, that made up more than 60% of the Company's accounts receivable balance. The Company assesses the creditworthiness of all its customers and sets and reassesses customer credit limits to ensure collectability of any accounts receivable balances are assured.

For the three months ended March 31, 2021, there was one customer, Secura, who individually accounted for all of the Company's revenue. Refer to *Note 13. License, collaboration, and commercial agreements* for a detailed discussion of the Secura APA.

#### 3. Cash, cash equivalents and restricted cash

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the condensed consolidated balance sheets that sum to the total of the same such amounts shown in the condensed consolidated statements of cash flows (in thousands):

	March 31, 2021	December 31, 2020
Cash and cash equivalents	\$ 35,514	\$ 67,782
Restricted cash	241	241
<b>Total cash, cash equivalents and restricted cash</b>	<b>\$ 35,755</b>	<b>\$ 68,023</b>

Amounts included in restricted cash as of March 31, 2021 and December 31, 2020 represent cash held to collateralize outstanding letters of credit provided as a security deposit for the Company's office space located in Needham, Massachusetts in the amount of approximately \$0.2 million. The letters of credit are included in non-current restricted cash on the condensed consolidated balance sheets at March 31, 2021 and December 31, 2020.

#### 4. Fair value of financial instruments

The Company determines the fair value of its financial instruments based upon the fair value hierarchy, which prioritizes valuation inputs based on the observable nature of those inputs. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The hierarchy defines three levels of valuation inputs:

Level 1 inputs	Quoted prices in active markets for identical assets or liabilities that the Company can access at the measurement date.
Level 2 inputs	Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly.

Level 3 inputs Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability.

**Items Measured at Fair Value on a Recurring Basis**

The following table presents information about the Company's financial instruments that are measured at fair value on a recurring basis (in thousands):

Description	March 31, 2021			
	Total	Level 1	Level 2	Level 3
<b>Financial assets</b>				
Cash equivalents	\$ 33,820	\$ 33,820	\$ —	\$ —
Short-term investments	\$ 85,583	\$ —	\$ 85,583	\$ —
Long-term investments	\$ 5,998	\$ —	\$ 5,998	\$ —
<b>Total financial assets</b>	<b>\$ 125,401</b>	<b>\$ 33,820</b>	<b>\$ 91,581</b>	<b>\$ —</b>

Description	December 31, 2020			
	Total	Level 1	Level 2	Level 3
<b>Financial assets</b>				
Cash equivalents	\$ 65,610	\$ 60,611	\$ 4,999	\$ —
Short-term investments	73,444	—	73,444	—
Long-term investments	5,995	—	5,995	—
<b>Total financial assets</b>	<b>\$ 145,049</b>	<b>\$ 60,611</b>	<b>\$ 84,438</b>	<b>\$ —</b>

The Company's cash equivalents and short-term investments consist of U.S. Government money market funds, corporate bonds, agency bonds and commercial paper of publicly traded companies. The investments and cash equivalents have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third party pricing services or other market observable data. The pricing services utilize industry standard valuation models, including both income and market-based approaches and observable market inputs to determine value. These observable market inputs include reportable trades, benchmark yields, credit spreads, broker/dealer quotes, bids, offers, current spot rates and other industry and economic events. The Company validates the prices provided by third party pricing services by reviewing their pricing methods and matrices, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming that the relevant markets are active. After completing its validation procedures, the Company did not adjust or override any fair value measurements provided by the pricing services as of March 31, 2021 and December 31, 2020.

**Fair Value of Financial Instruments**

The fair value of the Company's 2018 issued 5.00% Convertible Senior Notes due 2048 (the 2018 Notes) and 2020 issued 5.00% Convertible Senior Notes due 2048 (the 2020 Notes, together with the 2018 Notes referred to as the Notes) was approximately \$0.3 million and \$31.4 million, respectively, as of March 31, 2021, which differs from the aggregate carrying value of the Notes of \$19.7 million as of March 31, 2021. The fair value of the 2018 Notes and 2020 Notes was approximately \$0.3 million and \$30.0 million, respectively, as of December 31, 2020, which differs from the aggregate carrying value of the Notes of \$19.1 million as of December 31, 2020. The fair value of the Notes is influenced by the Company's stock price, stock price volatility, and current market yields and was determined using Level 3 inputs.

**5. Investments**

Cash, cash equivalents, restricted cash and investments consist of the following (in thousands):

	March 31, 2021			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash, cash equivalents & restricted cash:				
Cash and money market accounts	\$ 35,755	\$ —	\$ —	\$ 35,755
Total cash, cash equivalents & restricted cash:	<u>\$ 35,755</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 35,755</u>
Investments:				
Corporate bonds, agency bonds and commercial paper (due within 1 year)	\$ 85,550	\$ 50	\$ (17)	\$ 85,583
Corporate bonds, agency bonds and commercial paper (due between 1 and 5 years)	5,998	—	—	5,998
Total investments	<u>\$ 91,548</u>	<u>\$ 50</u>	<u>\$ (17)</u>	<u>\$ 91,581</u>
<b>Total cash, cash equivalents, restricted cash and investments</b>	<b><u>\$ 127,303</u></b>	<b><u>\$ 50</u></b>	<b><u>\$ (17)</u></b>	<b><u>\$ 127,336</u></b>

	December 31, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash, cash equivalents & restricted cash:				
Cash and money market accounts	\$ 63,024	\$ —	\$ —	\$ 63,024
Corporate bonds, agency bonds and commercial paper (due within 90 days)	4,998	\$ 1	\$ —	\$ 4,999
Total cash, cash equivalents & restricted cash:	<u>\$ 68,022</u>	<u>\$ 1</u>	<u>\$ —</u>	<u>\$ 68,023</u>
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 73,389	\$ 55	\$ —	\$ 73,444
Corporate bonds and commercial paper (due between 1 and 5 years)	5,998	—	(3)	5,995
Total investments	<u>\$ 79,387</u>	<u>\$ 55</u>	<u>\$ (3)</u>	<u>\$ 79,439</u>
<b>Total cash, cash equivalents, restricted cash and investments</b>	<b><u>\$ 147,409</u></b>	<b><u>\$ 56</u></b>	<b><u>\$ (3)</u></b>	<b><u>\$ 147,462</u></b>

There were no realized gains or losses on investments for the three months ended March 31, 2021 or 2020, respectively. There were three investments and one investment in an unrealized loss position as of March 31, 2021 and December 31, 2020, respectively. None of these investments had been in an unrealized loss position for more than 12 months. The fair value of these securities as of March 31, 2021 and December 31, 2020 was \$13.4 million and \$6.0 million, respectively, and the aggregate unrealized loss was immaterial. The Company considered the decline in the market value for these securities to be primarily attributable to current economic conditions. As it was not more likely than not that the Company would be required to sell these securities before the recovery of their amortized cost basis, which may be at maturity, the Company did not consider these investments to be other-than-temporarily impaired as of March 31, 2021 and December 31, 2020, respectively.

## 6. Accrued expenses

Accrued expenses consist of the following (in thousands):

	<u>March 31, 2021</u>	<u>December 31, 2020</u>
Research and development expenses	4,554	5,176
Compensation and related benefits	1,310	5,930
Interest	589	236
Consulting fees	543	1,091
Professional fees	503	615
Commercialization costs	87	330
Other	1,222	1,282
<b>Total accrued expenses</b>	<b>\$ 8,808</b>	<b>\$ 14,660</b>

## 7. Product revenue reserves and allowances

From September 24, 2018 (the date of the Company's U.S. commercial launch of COPIKTRA) through September 30, 2020 (the date the Company sold COPIKTRA to Secura), the Company's sole source of product revenue was from the gross sales of COPIKTRA in the United States less provisions for product sales allowances and accruals. The following table summarizes activity in each of the product revenue allowance and reserve categories for the three months ended March 31, 2021 (in thousands):

	<u>Trade discounts and allowances</u>	<u>Government rebates and other incentives</u>	<u>Returns</u>	<u>Total</u>
<b>Balance at December 31, 2020</b>	<b>\$ 23</b>	<b>\$ 67</b>	<b>\$ 31</b>	<b>\$ 121</b>
Provision related to sales in the current year	—	—	—	—
Adjustments related to prior period sales	—	—	—	—
Credits and payments made	(19)	(67)	(3)	(89)
<b>Ending balance at March 31, 2021</b>	<b>\$ 4</b>	<b>\$ —</b>	<b>\$ 28</b>	<b>\$ 32</b>

Trade discounts are recorded as a reduction to accounts receivable, net on the condensed consolidated balance sheets. Trade allowances, government rebates, other incentives and returns are recorded as a component of accrued expenses on the condensed consolidated balance sheets.

## 8. Leases

On April 15, 2014, the Company entered into a lease agreement for approximately 15,197 square feet of office and laboratory space in Needham, Massachusetts. Effective February 15, 2018, the Company amended its lease agreement to relocate within the facility to another location consisting of 27,810 square feet of office space (the Amended Lease Agreement). The Amended Lease Agreement extends the expiration date of the lease from September 2019 through June 2025. Pursuant to the Amended Lease Agreement, the initial annual base rent amount is approximately \$0.7 million, which increases during the lease term to \$1.1 million for the last twelve-month period.

The Company has accounted for its Needham, Massachusetts office space as an operating lease. The Company's lease contains an option to renew and extend the lease terms and an option to terminate the lease prior to the expiration date. The Company has not included the lease extension or the termination options within the right-of-use asset and lease liability on the condensed consolidated balance sheets as neither option is reasonably certain to be exercised. The Company's lease includes variable non-lease components (e.g., common area maintenance, maintenance, consumables, etc.) that are not included in the right-of-use asset and lease liability and are reflected as an expense in the period incurred. The Company does not have any other operating or finance leases.

As of March 31, 2021, a right-of-use asset of \$2.6 million and lease liability of \$3.4 million are reflected on the condensed consolidated balance sheets. The elements of lease expense were as follows (dollar amounts in thousands):

	Three months ended March 31,	
	2021	2020
<b>Lease Expense</b>		
Operating lease expense	\$ 221	\$ 221
<b>Total Lease Expense</b>	<b>\$ 221</b>	<b>\$ 221</b>
<b>Other Information - Operating Leases</b>		
Operating cash flows paid for amounts included in measurement of lease liabilities	\$ 252	\$ 204
<b>March 31, 2021</b>		
<b>Other Balance Sheet Information - Operating Leases</b>		
Weighted average remaining lease term (in years)		4.3
Weighted average discount rate		14.6%
<b>Maturity Analysis</b>		
2021		767
2022		1,039
2023		1,060
2024		1,081
2025		546
<b>Total</b>	<b>\$</b>	<b>4,493</b>
Less: Present value discount		(1,134)
<b>Lease Liability</b>	<b>\$</b>	<b>3,359</b>

## 9. Convertible Senior Notes

### 2018 Notes

On October 17, 2018, the Company closed a registered direct public offering of \$150.0 million aggregate principal amount of the Company's 5.00% Convertible Senior Notes due 2048 (the 2018 Notes). The 2018 Notes are governed by the terms of a base indenture for senior debt securities (the 2018 Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the 2018 Base Indenture, the 2018 Indenture), each dated October 17, 2018, by and between the Company and Wilmington Trust, National Association, as trustee. The 2018 Notes are senior unsecured obligations of the Company and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The 2018 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The 2018 Notes are convertible into shares of the Company's common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 139.5771 shares of common stock per \$1,000 principal amount of the 2018 Notes, which corresponds to an initial conversion price of approximately \$7.16 per share of common stock and represents a conversion premium of approximately 15.0% above the last reported sale price of the common stock of \$6.23 per share on October 11, 2018. Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted 2018 Notes. The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

The Company has the right, exercisable at its option, to cause all Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the 2018 Indenture) per share of the Company's common stock equals or exceeds 130% of the conversion price on each of at least 20 VWAP Trading Days (as defined in the 2018 Indenture),

whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the 2018 Notes.

The 2018 Indenture includes customary covenants and sets forth certain events of default after which the 2018 Notes may be declared immediately due and payable and sets forth certain types of bankruptcy or insolvency events of default involving the Company or certain of its subsidiaries after which the 2018 Notes become automatically due and payable.

The Company assessed all terms and features of the 2018 Notes in order to identify any potential embedded features that would require bifurcation. As part of this analysis, the Company assessed the economic characteristics and risks of the 2018 Notes, including the conversion, put and call features. The conversion feature was initially bifurcated as an embedded derivative but subsequently qualified for a scope exception to derivative accounting upon the Company's stockholders approving an increase in the number of authorized shares of Common Stock in December 2018. The Company determined that all other features of the 2018 Notes were clearly and closely associated with the debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's condensed consolidated financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through March 31, 2021.

#### **2019 Notes**

On November 14, 2019 and December 23, 2019, the Company entered into privately negotiated agreements to exchange approximately \$114.3 million and \$7.4 million, respectively, aggregate principal amount of the 2018 Notes for (i) approximately \$62.9 million and \$4.0 million, respectively, aggregate principal amount of 5.00% Convertible Senior Second Lien Notes due 2048 (the 2019 Notes), (ii) an aggregate of approximately \$11.4 million and \$0.7 million in 2018 Notes principal repayment and (iii) accrued interest on the 2018 Notes through November 14, 2019 and December 23, 2019, respectively. The 2019 Notes were governed by the terms of an indenture (the 2019 Indenture). The 2019 Notes were senior secured obligations of the Company and bear interest at 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year. The 2019 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with the terms thereof. During the three months ended March 31, 2020, 2019 Note holders converted \$57.4 million aggregate principal of 2019 Notes in exchange for 34,796,350 shares of common stock and \$1.8 million of cash for the 2019 Note Interest Make-Whole Provision. The Company recorded approximately \$1.3 million for the three months ended March 31, 2020 as other expense for the change in fair value of the 2019 Notes Interest Make-Whole Provision in the condensed consolidated statements of operations and comprehensive loss. The Company determined that all other features of the 2019 Notes were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's condensed consolidated financial statements. As of March 31, 2020, all 2019 Notes have converted into shares of common stock.

The 2019 Notes were convertible into shares of the Company's common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 606.0606 shares of common stock per \$1,000 principal amount of the 2019 Notes, which corresponds to an initial conversion price of approximately \$1.65 per share of common stock and represents a conversion premium of approximately 52.8% above the last reported sale price of the Company's common stock of \$1.08 per share on November 11, 2019.

The Company had the right, exercisable at the Company's option, to cause all 2019 Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the 2019 Indenture) per share of the Company's common stock equaled or exceeded 121% of the conversion price on each of at least 20 VWAP Trading Days, whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the 2019 Notes. (Company's Mandatory Conversion Option).

Upon conversion, converting noteholders were entitled to receive accrued interest on their converted 2019 Notes. In addition, if the 2019 Notes were converted with a conversion date that is on or prior to November 1, 2020, other than in connection with the Company's exercise of the Company's Mandatory Conversion Option then the

consideration due upon any such conversion will also include a cash interest make-whole payment for all future scheduled interest payments on the converted 2019 Notes through November 1, 2020 (2019 Notes Interest Make-Whole Provision).

The Company assessed all terms and features of the 2019 Notes in order to identify any potential embedded features that would require bifurcation. As part of this analysis, the Company assessed the economic characteristics and risks of the 2019 Notes, including the conversion, put and call features. In consideration of the 2019 Notes Interest Make-Whole Provision, the Company concluded the provision required bifurcation as a derivative. It was determined that the fair value of the derivative upon the November 14, 2019 and December 23, 2019 issuance of the 2019 Notes was \$0.2 million in the aggregate; and the Company recorded this amount as a derivative liability and the offsetting amount as a debt discount as a reduction to the carrying value of the 2019 Notes on the closing dates.

#### **2020 Notes**

On November 6, 2020, the Company entered into a privately negotiated agreement with an investor who is a holder of the Company's 2018 Notes to exchange approximately \$28.0 million aggregate principal amount of 2018 Notes for approximately \$28.0 million aggregate principal amount of newly issued 5.00% Convertible Senior Notes due 2048 (the 2020 Notes). The issuance of the 2020 Notes closed on November 13, 2020. The 2020 Notes are governed pursuant to the Base Indenture between the Company and Wilmington Trust, National Association, as trustee and collateral agent, dated as of October 17, 2018 as supplemented by the second supplemental indenture thereto, dated as of November 13, 2020, (the Supplemental Indenture and together with the Base Indenture, the 2020 Indenture).

The Company will have the right, exercisable at its option, to cause all 2020 Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the 2020 Indenture) per share of the Company's common stock equals or exceeds 123.08% of the conversion price on each of at least 20 "VWAP Trading Days" (as defined in the 2020 Indenture), whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the 2020 Notes.

The initial conversion rate for the 2020 Notes is 307.6923 shares of the Company's common stock per \$1,000 principal amount of the 2020 Notes, which is equivalent to an initial conversion price of approximately \$3.25 per share, representing an approximately 153.9% premium to the sale price of \$1.28 per share of the Company's common stock on November 5, 2020, as reported on the Nasdaq Global Market. The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

Prior to November 1, 2023, the Company will not have the right to redeem the 2020 Notes. On or after November 1, 2023, the Company may elect to redeem the 2020 Notes, in whole or in part, at a cash redemption price equal to the principal amount of the 2020 Notes to be redeemed, plus accrued and unpaid interest, if any.

Unless the Company has previously called all outstanding 2020 Notes for redemption, the 2020 Notes will be subject to repurchase by the Company at the holders' option on each of November 1, 2023, November 1, 2028, November 1, 2033, November 1, 2038 and November 1, 2043 (or, if any such date is not a business day, on the next business day) at a cash repurchase price equal to the principal amount of the 2020 Notes to be repurchased, plus accrued and unpaid interest, if any.

If a Fundamental Change (as defined in the 2020 Indenture) occurs at any time, subject to certain conditions, holders may require the Company to purchase all or any portion of their 2020 Notes at a purchase price equal to 100% of the principal amount of the 2020 Notes to be purchased, plus accrued and unpaid interest, if any, to, but excluding, the "Fundamental Change Repurchase Date" (as defined in the 2020 Indenture). If a "Make-Whole Fundamental Change" (as defined in the 2020 Indenture) occurs on or before November 1, 2022 and a holder elects to convert its 2020 Notes in connection with such Make-Whole Fundamental Change, such holder may be entitled to an increase in the conversion rate in certain circumstances as set forth in the 2020 Indenture.



Upon conversion of the 2020 Notes, holders will receive a cash payment equal to the accrued and unpaid interest on the converted 2020 Notes.

The 2020 Notes are the Company's senior unsecured obligations and will be senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the 2020 Notes, and equal in right of payment with the Company's existing and future indebtedness that is not so subordinated, and effectively subordinated to the Company's existing and future indebtedness, to the extent of the value of the collateral securing such indebtedness. The 2020 Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries.

The Company determined the 2020 Notes exchange met the definition of a debt modification under ASC 470-50, *Modifications and Extinguishments*. The Company reduced the carrying value of the 2020 Notes by the change in fair value of the conversion option driven by the reduction in conversion price. The change in fair value of the conversion option was determined to be \$2.3 million.

The Company determined that all features of the 2020 Notes were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's condensed consolidated financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through March 31, 2021.

## 10. Common stock

### *Private Investment in Public Equity (PIPE)*

On February 27, 2020, the Company entered into a Securities Purchase Agreement (Purchase Agreement) with certain institutional investors in which the Company agreed to sell 46,511,628 shares of common stock at a purchase price of \$2.15 per share, which represents 12.6% premium to the last reported sale price of the Company's common stock of \$1.91 per share on February 27, 2020. On March 3, 2020, the closing occurred. The aggregate proceeds net of underwriting discounts and offering costs, were approximately \$93.8 million.

## 11. Stock-based compensation

### *Stock options*

A summary of the Company's stock option activity and related information for the three months ended March 31, 2021 is as follows:

	Shares	Weighted-average exercise price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2020	12,690,745	\$ 3.90	6.5	\$ 3,390
Granted	801,659	2.21		
Exercised	(246,758)	1.54		
Forfeited/cancelled	(725,888)	3.76		
Outstanding at March 31, 2021	12,519,758	\$ 3.85	6.3	\$ 5,699
Vested at March 31, 2021	8,007,568	\$ 4.61	5.0	\$ 3,678

The fair value of each stock option granted during the three months ended March 31, 2021 and 2020 was estimated on the grant date using the Black-Scholes option-pricing model using the following weighted-average assumptions:

	March 31,	
	2021	2020
Risk-free interest rate	0.55 %	1.58 %
Volatility	97 %	88 %
Dividend yield	—	—
Expected term (years)	6.1	6.1

**Restricted stock units (RSUs)**

The Company awards RSUs to employees under its Amended and Restated 2012 Incentive Plan and its inducement award program. Each RSU entitles the holder to receive one share of the Company's common stock when the RSU vests. The RSUs generally vest in either (i) four substantially equal installments on each of the first four anniversaries of the vesting commencement date, (ii) 100 percent on the first anniversary of the vesting commencement date, (iii) thirty three and one-third percent (33 1/3%) on the first anniversary of the vesting commencement date and as to an additional eight and two-thirds percent (8.33%) at the end of each successive three-month period thereafter, (iv) 100 percent after approximately 21 months from the vesting commencement date, and (v) 50% after approximately four months from vesting commencement date and 50% after one year from vesting commencement date subject to the employee's continued employment with, or service to, the Company on such vesting date. Compensation expense is recognized on a straight-line basis.

A summary of RSU activity during the three months ended March 31, 2021 is as follows:

	Shares	Weighted-average grant date fair value per share
Outstanding at December 31, 2020	2,649,317	\$ 1.73
Granted	497,590	\$ 2.26
Vested	(72,390)	\$ 2.45
Forfeited/cancelled	(223,562)	\$ 2.01
Outstanding at March 31, 2021	2,850,955	\$ 1.78

**Employee stock purchase plan**

At the Special Meeting of Stockholders, held on December 18, 2018, the stockholders approved the 2018 Employee Stock Purchase Plan (2018 ESPP). On June 21, 2019, the board of directors of the Company amended and restated the 2018 ESPP, to account for certain non-material changes to the plan's administration (the Amended and Restated 2018 ESPP). The Amended and Restated 2018 ESPP provides eligible employees with the opportunity, through regular payroll deductions, to purchase shares of the Company's common stock at 85% of the lesser of the fair market value of the common stock (a) on the date the option is granted, which is the first day of the purchase period, and (b) on the exercise date, which is the last business day of the purchase period. The Amended and Restated 2018 ESPP generally allows for two six-month purchase periods per year beginning in January and July, or such other periods as determined by the compensation committee of the Company's board of directors. The Company has reserved 2,000,000 shares of common stock for the administration of the Amended and Restated 2018 ESPP. The fair value of shares expected to be purchased under the Amended and Restated 2018 ESPP was calculated using the following weighted-average assumptions:

	Three months ended March 31,	
	2021	2020
Risk-free interest rate	0.09 %	1.57 %
Volatility	65 %	78 %
Dividend yield	—	—
Expected term (years)	0.5	0.5

For the three months ended March 31, 2021 and 2020, the Company has recognized less than \$0.1 million in each period of stock-based compensation expense under the Amended and Restated 2018 ESPP. During the three months ended March 31, 2021 the Company issued 53,372 shares of common stock for proceeds of \$0.1 million under the Amended and Restated 2018 ESPP.

## 12. Net loss per share

Basic loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period. Diluted net loss per common share is calculated by increasing the denominator by the weighted-average number of additional shares that could have been outstanding from securities convertible into common stock, such as stock options, restricted stock units, and employee stock purchase plan shares (using the “treasury stock” method), and the Notes (using the “if-converted” method), unless their effect on net loss per share is anti-dilutive.

The following potentially dilutive securities were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Three months ended March 31,	
	2021	2020
Outstanding stock options	12,519,758	14,202,430
Outstanding restricted stock units	2,850,955	1,614,784
2018 Notes	41,873	3,950,032
2020 Notes	8,615,384	—
Employee stock purchase plan	33,034	128,545
<b>Total potentially dilutive securities</b>	<b>24,061,004</b>	<b>19,895,791</b>

## 13. License, collaboration and commercial agreements

### *Secura Bio, Inc. (Secura)*

On August 10, 2020, the Company and Secura signed the Secura APA and on September 30, 2020, the transaction closed.

Pursuant to the Secura APA, the Company sold to Secura its exclusive worldwide license, including related assets, for the research, development, commercialization, and manufacture in oncology indications of products containing duvelisib. The sale included certain intellectual property related to duvelisib in oncology indications, certain existing duvelisib inventory, claims and rights under certain contracts pertaining to duvelisib. Pursuant to the Secura APA, Secura assumed all operational and financial responsibility for activities that were part of the Company’s duvelisib oncology program, including all commercialization efforts related to duvelisib in the United States and Europe, as well as the Company’s ongoing duvelisib clinical trials. Further, Secura assumed all obligations with existing collaboration partners developing and commercializing duvelisib, which include Yakult Honsha Co., Ltd. (Yakult), CSPC Pharmaceutical Group Limited (CSPC), and Sanofi. Additionally, Secura assumed all royalty payment obligations due under the amended and restated license agreement with Infinity.

Pursuant to the terms of the Secura APA, Secura has paid the Company an up-front payment of \$70.0 million and agreed to pay the Company (i) regulatory milestone payments up to \$45.0 million, consisting of a payment of \$35.0 million upon receipt of regulatory approval of COPIKTRA in the United States for the treatment of peripheral T-

cell lymphoma and a payment of \$10.0 million upon receipt of the first regulatory approval for the commercial sale of COPIKTRA in the European Union for the treatment of peripheral T-cell lymphoma, (ii) sales milestone payments of up to \$50.0 million, consisting of \$10.0 million when total worldwide net sales of COPIKTRA exceed \$100.0 million, \$15.0 million when total worldwide net sales of COPIKTRA exceed \$200.0 million and \$25.0 million when total worldwide net sales of COPIKTRA exceed \$300.0 million, (iii) low double-digit royalties on the annual aggregate net sales above \$100.0 million in the United States, European Union, and the United Kingdom of Great Britain and Northern Ireland and (iv) 50% of all royalty, milestone and sublicense revenue payments payable to Secura under the Company's existing license agreements with Sanofi, Yakult, and CSPC, and 50% of all royalty and milestone payments payable to Secura under any license or sublicense agreement entered into by Secura in certain jurisdictions.

In connection with the Secura APA, the Company and Secura entered into a transition services agreement (Secura TSA). Under the terms of the Secura TSA, the Company will provide certain support functions at Secura's direction for a term of less than one year from the date of execution, unless earlier terminated or extended according to the terms of the Secura TSA (Secura TSA Services). Secura may cancel the Secura TSA at sole discretion for any or no reason with five days' notice. Services performed are paid at a mutually agreed upon rate.

The Company evaluated the Secura APA and Secura TSA in accordance with ASC 606 as the Company concluded that the counterparty, Secura, is a customer. The Company identified the following performance obligations under the Secura APA and Secura TSA:

- a bundled performance obligation consisting of delivery of the duvelisib global license and intellectual property, certain existing duvelisib inventory, certain duvelisib contracts and clinical trials, certain regulatory approvals, and certain regulatory documentation and books and records (the Bundled Secura Performance Obligation); and
- Secura TSA Services.

The Company concluded that the duvelisib global license and intellectual property were not distinct within the context of the contract (i.e. separately identifiable) because the other assets including certain existing duvelisib inventory, certain duvelisib contracts and clinical trials, certain regulatory approval, and certain regulatory documentation and books and records do not have stand-alone value from other duvelisib global license and intellectual property and Secura could not benefit from them without the duvelisib global license and intellectual property. Consistent with the guidance under ASC 606-10-25-16A, the Company disregarded immaterial promised goods and services when determining performance obligations.

The Company has determined that the upfront payment of \$70.0 million, future potential milestone payments and royalties including from Secura's sublicensees should be allocated to the delivery of the Bundled Secura Performance Obligation. The Company has the right to consideration for TSA services in an amount that corresponds directly with the value to Secura of the Company's performance to date. Consideration allocated to the Secura TSA Services will be recognized as such services are provided over the performance period using an output method based on the amount to which the Company has a right to invoice.

The Company determined \$0.1 million of future potential royalties we expect to receive pursuant to the Secura APA were not constrained as of March 31, 2021. When estimating the amount of royalties to be received that was not constrained, the Company used the expected value method as there are a range of possible outcomes. When estimating royalties to be received, the Company used a combination of internal projections and forecasts and data from external sources. The Company determined that all other future potential royalties were constrained under the guidance as of March 31, 2021. As part of the Company's evaluation of the constraint on future royalties, the Company considered a number of factors in determining whether there is significant uncertainty associated with the future events that would result in royalty payments. Those factors include: the likelihood and magnitude of revenue reversals related to future royalties, the amount of variable consideration is highly susceptible to factors outside of the Company's influence, the amount of time to resolve the uncertainty, and lack of significant history of selling COPIKTRA outside of the United States.

As the consideration for future royalties is conditional, the Company recorded a corresponding contract asset for the expected royalties. Portions of the contract asset will be reclassified to accounts receivable when the right to consideration becomes unconditional. As of March 31, 2021, the \$0.1 million contract asset has been recorded within prepaid and other current assets on the condensed consolidated balance sheet.

The following table presents changes in the Company's contract asset for the three months ended March 31, 2021 (in thousands):

<b>Contract Asset:</b>	<b>Balance at December 31, 2020</b>	<b>Additions</b>	<b>Reclassification to receivable</b>	<b>Balance at March 31, 2021</b>
Contract asset - Secura	—	100	—	100
Total	\$ —	\$ 100	\$ —	\$ 100

During the three months ended March 31, 2021, one regulatory milestone was achieved by Secura's sublicensee, Sanofi, of which 50% of the milestone or \$0.8 million is due to the Company pursuant to the Secura APA. The Company determined all other future potential milestones were excluded from the transaction price, as all other milestone amounts were fully constrained under the guidance as of March 31, 2021. As part of the Company's evaluation of the constraint, the Company considered a number of factors in determining whether there is significant uncertainty associated with the future events that would result in the milestone payments. Those factors include: the likelihood and magnitude of revenue reversals related to future milestones, the amount of variable consideration is highly susceptible to factors outside of the Company's influence and the uncertainty about the consideration is not expected to be resolved for a long period of time. All other future potential milestone payments were fully constrained as the risk of significant revenue reversal related to these amounts has not yet been resolved.

During the three months ended March 31, 2021, the Company recognized \$0.9 million of sale of COPIKTRA license and related assets revenue related to one regulatory milestone for \$0.8 million achieved by Secura's sublicensee and \$0.1 million related to future royalties expected to be received pursuant to the Secura APA. The Company also recognized \$0.2 million in transition services revenue within the statements of operations and comprehensive loss.

#### ***Chugai Pharmaceutical Co., Ltd (Chugai)***

On January 7, 2020, the Company entered into a license agreement with Chugai (the Chugai Agreement) whereby Chugai granted the Company an exclusive worldwide license for the development, commercialization and manufacture of products containing VS-6766, a dual RAF/MEK inhibitor.

Under the terms of the Chugai Agreement, the Company received an exclusive right to develop and commercialize products containing VS-6766 at the Company's own cost and expense. The Company is required to pay Chugai a non-refundable payment of \$3.0 million which was paid in February 2020. The Company is further obligated to pay Chugai double-digit royalties on net sales of products containing VS-6766, subject to reduction in certain circumstances. Chugai also obtained opt back rights to develop and commercialize VS-6766 (a) in the European Union, which option may be exercised through the date the Company submits a NDA to the FDA for a product which contains VS-6766 as the sole active pharmaceutical ingredient and (b) in Japan and Taiwan, which option may be exercised through the date the Company receives marketing authorization from the FDA for a product which contains VS-6766 as the sole active pharmaceutical ingredient. As consideration for executing either option, Chugai would have to make a payment to the Company calculated on the Company's development costs to date. Chugai and the Company have made customary representations and warranties and have agreed to certain customary covenants, including confidentiality and indemnification.

Unless earlier terminated, the Chugai Agreement will expire upon the fulfillment of the Company's royalty obligations to Chugai for the sale of any products containing the VS-6766, which royalty obligations expire on a product-by-product and country-by-country basis, upon the last to occur, in each specific country, of (a) expiration of valid patent claims covering such product or (b) 12 years from the first commercial sale of such product in such country.

The Company may terminate the Chugai Agreement upon 180 days' written notice. Subject to certain limitations, Chugai may terminate the Chugai Agreement upon written notice if the Company challenges any patent licensed by Chugai to the Company under the Chugai Agreement. Either party may terminate the license agreement in its entirety with 120 days' written notice for the other party's material breach if such party fails to cure the breach. Either party may also terminate the Chugai Agreement in its entirety upon certain insolvency events involving the other party.

The Company evaluated the license agreement with Chugai under ASC Topic 805, *Business Combinations* (ASC 805) and concluded that as the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar assets, the transaction did not meet the requirements to be accounted for as a business combination and therefore was accounted for as an asset acquisition. The Company recorded the up-front payment of \$3.0 million as research and development expense within the condensed consolidated statement of operations for the three months ended March 31, 2020.

#### **Sanofi**

On July 25, 2019, the Company entered into a license and collaboration agreement with Sanofi (the Sanofi Agreement), under which the Company granted exclusive rights to Sanofi to develop and commercialize products containing duvelisib in Russia, the Commonwealth of Independent States (CIS), Turkey, the Middle East and Africa (collectively the "Sanofi Territory") for the treatment, prevention, palliation or diagnosis of any oncology indication in humans or animals.

Sanofi paid the Company an upfront, non-refundable payment of \$5.0 million in August 2019. The Company is also entitled to receive aggregate payments of up to \$42.0 million if certain regulatory and commercial milestones are successfully achieved. Sanofi is obligated to pay the Company double-digit royalties on net sales of products containing duvelisib in the Sanofi Territory, subject to reduction in certain circumstances.

As discussed above as of September 30, 2020, Secura has assumed from the Company all responsibilities and obligations under the Sanofi Agreement. After September 30, 2020, the Company is entitled to 50% of future milestone payments and royalties pursuant to the Secura APA discussed under heading *Secura Bio, Inc. (Secura)* above. Future milestone and royalty payments pursuant to the Sanofi Agreement will be paid by Sanofi to Secura. The Company's portion of such milestone and royalty payments will be subsequently remitted to the Company by Secura.

#### **Yakult Honsha Co., Ltd. (Yakult)**

On June 5, 2018, the Company entered into a license and collaboration agreement with Yakult (the Yakult Agreement), under which the Company granted exclusive rights to Yakult to develop and commercialize products containing duvelisib in Japan for the treatment, prevention, palliation or diagnosis of all oncology indications in humans or animals.

Yakult paid the Company an upfront, non-refundable payment of \$10.0 million in June 2018. The Company is also entitled to receive aggregate payments of up to \$90.0 million if certain development, regulatory and commercial milestones are successfully achieved. Yakult is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in Japan, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which Yakult has opted to participate on a pro-rata basis.

As discussed above as of September 30, 2020, Secura has assumed from the Company all responsibilities and obligations under the Yakult Agreement. After September 30, 2020, the Company is entitled to 50% of future milestone payments and royalties pursuant to the Secura APA discussed under heading *Secura Bio, Inc. (Secura)* above. Payments pursuant to the Yakult Agreement will be paid by Yakult to Secura. The Company's portion of such milestone and royalty payments will be subsequently remitted to the Company by Secura.

#### **CSPC Pharmaceutical Group Limited (CSPC)**

On September 25, 2018, the Company entered into a license and collaboration agreement with CSPC (the CSPC Agreement), under which the Company granted exclusive rights to CSPC to develop and commercialize products containing duvelisib in the People's Republic of China (China), Hong Kong, Macau and Taiwan (each, a Region and collectively, the CSPC Territory) for the treatment, prevention, palliation or diagnosis of all oncology indications in humans.

CSPC paid the Company an aggregate upfront, non-refundable payment of \$15.0 million in 2018. The Company is also entitled to receive aggregate payments of up to \$160.0 million if certain development, regulatory and commercial milestones are successfully achieved. CSPC is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in the CSPC Territory, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which CSPC has opted to participate on a pro-rata basis.

As discussed above as of September 30, 2020, Secura has assumed from the Company all responsibilities and obligations under the CSPC Agreement. After September 30, 2020, the Company is entitled to 50% of future milestone payments and royalties pursuant to the Secura APA discussed under heading *Secura Bio, Inc. (Secura)* above. Payments pursuant to the CSPC Agreement will be paid by CSPC to Secura. The Company's portion of such milestone and royalty payments will be subsequently remitted to the Company by Secura.

#### **14. Income taxes**

The Company did not record a federal or state income tax provision or benefit for the three months ended March 31, 2021 and 2020, respectively, due to the expected loss before income taxes to be incurred for the years ended December 31, 2021 and 2020, as well as the Company's continued maintenance of a full valuation allowance against its net deferred tax assets.

#### **15. Commitments and contingencies**

The Company has no other commitments other than minimum lease payments as disclosed in Note 8. *Leases*.

#### **16. Restructurings**

On February 27, 2020, following analysis of the Company's strategy, the Company committed to an operational plan to reduce overall operating expenses, including the elimination of approximately 31 positions across the Company and other cost-saving measures (the February 2020 Restructuring). The February 2020 Restructuring was designed to streamline operations, speed execution of the Company's clinical development of defactinib and VS-6766, and reflect a focused, account-based approach in the field. During the three months ended March 31, 2020, the Company recorded an aggregate expense of \$1.8 million, which is reflected in the condensed consolidated statements of operations and comprehensive loss as selling general, and administrative expense and research and development expense for \$1.3 million and \$0.5 million, respectively, for one-time termination benefits for employee severance, benefits, and related costs.

In August 2020, in connection with the duvelisib sale to Secura pursuant to the Secura APA, the Company committed to a strategic restructuring (the August 2020 Restructuring). The restructuring included a workforce reduction of approximately 41 positions primarily in the Company's commercial operations department.

The following table summarizes the accrued liabilities activity recorded in connection with the restructurings for the three months ended March 31, 2021 (in thousands):

<b>Employee severance, benefits and related costs</b>	<b>Amounts accrued at December 31, 2020</b>	<b>Charges</b>	<b>Amount Paid</b>	<b>Adjustments</b>	<b>Amounts accrued at March 31, 2021</b>
August 2020 Restructuring	1,027	—	(907)	—	120
Total	<u>\$ 1,027</u>	<u>\$ —</u>	<u>\$ (907)</u>	<u>\$ —</u>	<u>\$ 120</u>

#### 17. Subsequent events

The Company reviews all activity subsequent to the end of the quarter but prior to issuance of the condensed consolidated financial statements for events that could require disclosure or that could impact the carrying value of assets or liabilities as of the balance sheet date. The Company is not aware of any material subsequent events.



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

*You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for our fiscal year ended December 31, 2020. Please also refer to the sections under headings "Forward-Looking Statements" and "Risk Factors" in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for our fiscal year ended December 31, 2020.*

### OVERVIEW

We are a development-stage biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel anticancer agents that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, particularly RAF/MEK inhibition and FAK inhibition.

Our most advanced product candidates, VS-6766 and defactinib, are being investigated in both preclinical and clinical studies for treatment of various solid tumors, including, low-grade serous ovarian cancer, non-small cell lung cancer, colorectal cancer, pancreatic cancer, uveal melanoma, and endometrial cancer. We believe that these compounds may be beneficial as therapeutics either as single agents or when used together in combination with other agents, other pathway inhibitors or other current and emerging standard of care treatments in cancers that do not adequately respond to currently available therapies.

VS-6766 is an orally available first-in-class unique small molecule RAF/MEK inhibitor. In contrast to other MEK inhibitors commercially available and in development, VS-6766 is a dual RAF/MEK inhibitor that blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. MEK-only inhibitors (e.g. PD0325901) paradoxically induce MEK phosphorylation (pMEK) by relieving extracellular-signal-regulated-kinase (ERK)-dependent feedback inhibition of RAF which may limit their efficacy. By inhibiting RAF-mediated phosphorylation of MEK, VS-6766 has the advantage of not inducing pMEK. This unique mechanism of VS-6766 enables more effective inhibition of ERK signaling and may confer enhanced therapeutic activity against ERK-dependent, RAS or BRAF mutant tumors.

VS-6766 has been shown to inhibit signaling and proliferation of tumor cell lines with a variety of KRAS, HRAS, or BRAF mutations. VS-6766 has also been shown to synergize with G12C inhibitors in KRAS mutant non-small cell lung cancer (NSCLC) and colorectal cancer (CRC) in preclinical models and enhances the anti-tumor effects of anti-PD-1 in KRAS mutant NSCLC mouse models. VS-6766 has shown compelling synergy with defactinib in preclinical trials.

Defactinib, is a targeted inhibitor of FAK. FAK is a non-receptor tyrosine kinase encoded by the protein tyrosine kinase-2 (PTK-2) gene that is involved in cellular adhesion and, in cancer, metastatic capability. Defactinib targets malignant cells both directly and through modulation of the tumor microenvironment. Defactinib has received orphan drug designation in ovarian cancer in the United States, European Union, and Australia. Preclinical research by our scientists and collaborators at world-renowned research institutions has described the effect of FAK inhibition to enhance immune response by decreasing immuno-suppressive cells, increasing cytotoxic T cells, and reducing stromal density, which allows tumor-killing immune cells to enter the tumor. Defactinib is an oral small molecule inhibitor of FAK and proline-rich tyrosine kinase (PYK2) that is currently being evaluated as a potential combination therapy for various solid tumors.

In an ongoing investigator-initiated Phase 1/2 study (The FRAME study), the combination of VS-6766 and defactinib is being evaluated in patients with recurrent LGSOC, KRAS mutant NSCLC and CRC. Updated data from this study presented at the 2nd Annual RAS-Targeted Drug Development Summit in September 2020 demonstrated a 56% overall response rate (ORR) and long duration of therapy among patients with KRAS-G12 mutant LGSOC. In an updated December 2020 data read-out of the FRAME study LGSOC cohort (n=24), the ORR is 52% (11 of 21 response

evaluable patients). Among the 21 response evaluable patients, the data demonstrated 70% ORR (7 of 10 response evaluable patients) in KRAS mutant LGSOC, 44% ORR (4 of 9 response evaluable patients) in KRAS wild type LGSOC and 0% ORR (0 of 2 response evaluable patients) in KRAS status undetermined LGSOC. Based on an observation of higher response rates seen in NSCLC patients with KRAS-G12V mutations in the study, we are also exploring the role of VS-6766 and defactinib in KRAS-G12V mutant NSCLC. The FRAME study was expanded in August 2020 to include new cohorts in pancreatic cancer, KRAS mutant endometrial cancer and KRAS-G12V mutant NSCLC.

We have met with regulatory authorities in third quarter of 2020, and have commenced registration-directed trials investigating VS-6766 as a monotherapy and in combination with defactinib in the fourth quarter of 2020. The registration-directed trials are called RAF and MEK Program (RAMP) 201 and 202 studies. RAMP 201 is an adaptive two-part multicenter, parallel cohort, randomized, open label trial to evaluate the efficacy and safety of VS-6766 alone and in combination with defactinib in patients with recurrent LGSOC. RAMP 202 study is a Phase 2, adaptive two-part multicenter, parallel cohort, randomized, open-label trial to evaluate the efficacy and safety of VS-6766 alone and in combination with defactinib in patients with KRAS mutant NSCLC, following treatment with a platinum-based regimen and immune checkpoint inhibitor

In addition, defactinib is currently being investigated in combination with immunotherapeutic and other agents through ISTs.

On August 10, 2020, we and Secura Bio, Inc. (Secura) signed an Asset Purchase Agreement (Secura APA) and on September 30, 2020, the transaction closed. Pursuant to the Secura APA, we sold our exclusive worldwide license for the research, development, commercialization, and manufacture in oncology indications of products containing duvelisib. With the transition of the duvelisib program to Secura, we are focusing our efforts on our lead product candidates, VS-6766 and defactinib.

Our operations to date have been organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates, undertaking preclinical studies and clinical trials for our product candidates and initiating U.S. commercial operations following the approval of COPIKTRA and through our ownership period ending in September 2020. We have financed our operations to date primarily through public offerings of our common stock, sales of common stock under our at-the-market equity offering programs, our loan and security agreement executed with Hercules Capital, Inc. (Hercules) in March 2017, as amended, the upfront payments under our license and collaboration agreements with Sanofi, Yakult and CSPC, the upfront payment under the Secura APA, the issuance of the 2018 Notes in October 2018 and the proceeds in connection with the PIPE. With our U.S. commercial launch of COPIKTRA on September 24, 2018 through our ownership period ending in September 2020, we financed a portion of our operations through product revenue.

As of March 31, 2021, we had an accumulated deficit of \$607.5 million. Our net loss was \$15.0 million and \$38.0 million for the three months ended March 31, 2021 and 2020, respectively. We expect to incur significant expenses and operating losses for the foreseeable future as a result of the continued research and development of VS-6766, and defactinib. As of March 31, 2021, we had cash, cash equivalents, and investments of \$127.1 million. We expect our existing cash resources will be sufficient to fund our planned operations through at least 12 months from the date of issuance of these condensed consolidated financial statements.

We expect to finance the future development costs of our clinical product portfolio with our existing cash, cash equivalents and investments, through future milestones and royalties received pursuant to the Secura APA or through strategic financing opportunities that could include, but are not limited to collaboration agreements, future offerings of our equity, or the incurrence of debt. However, there is no guarantee that any of these strategic or financing opportunities will be executed or executed on favorable terms, and some could be dilutive to existing stockholders. If we fail to obtain

additional future capital, we may be unable to complete our planned preclinical studies and clinical trials and obtain approval of certain investigational product candidates from the FDA or foreign regulatory authorities.

**COVID-19 pandemic**

The current COVID-19 pandemic has presented a substantial public health and economic challenge around the world and is affecting our employees, patients, communities and business operations, as well as the U.S. economy and financial markets. We have been carefully monitoring the COVID-19 pandemic and its impact on our operations. All employees who are able to work from home have been working from home since mid-March 2020. As countries have eased restrictions related to the pandemic, patient accruals within our clinical trials have increased. However, restrictions in many European countries remain, which continues to result in delays in startup activities related to our clinical trials operating in those jurisdictions. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning COVID-19, the actions taken to contain it or treat its impact and the economic impact on local, regional, national and international markets.

For additional information on the various risks posed by the COVID-19 pandemic, please read Item 1A. Risk Factors included in our Annual Report on Form 10-K for our fiscal year ended December 31, 2020.

**CRITICAL ACCOUNTING POLICIES AND SIGNIFICANT JUDGMENTS AND ESTIMATES**

Our discussion and analysis of our financial condition and results of operations is based upon our condensed consolidated financial statements prepared in accordance with U.S. generally accepted accounting principles. The preparation of these condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of certain assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our financial statements, and the amounts of revenues and expenses during the reported periods.

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as “critical” because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used, which would have resulted in different financial results.

The critical accounting policies we identified in our most recent Annual Report on Form 10-K for the fiscal year ended December 31, 2020, related to revenue recognition including product revenue, net and licenses and sale of intellectual property, accrued research and development expenses, stock-based compensation, intangible assets, and leases. During the three months ended March 31, 2021, there were no material changes to our critical accounting policies.

**RESULTS OF OPERATIONS**

**Comparison of the three months ended March 31, 2021 and 2020**

	Three months ended March 31,			
	2021	2020	Change	% Change
Revenue:				
Product revenue, net	\$ —	\$ 5,034	\$ (5,034)	(100)%
License and collaboration revenue	—	22	(22)	(100)%
Sale of COPIKTRA license and related assets	850	—	850	100%
Transition services revenue	156	—	156	100%
<b>Total revenue</b>	<b>1,006</b>	<b>5,056</b>	<b>(4,050)</b>	<b>(80)%</b>
Operating expenses:				
Cost of sales - product	—	495	(495)	(100)%
Cost of sales - intangible amortization	—	392	(392)	(100)%
Research and development	8,896	10,924	(2,028)	(19)%
Selling, general and administrative	6,218	19,604	(13,386)	(68)%
<b>Total operating expenses</b>	<b>15,114</b>	<b>31,415</b>	<b>(16,301)</b>	<b>(52)%</b>
Loss from operations	(14,108)	(26,359)	12,251	(46)%
Other expense	—	(1,313)	1,313	(100)%
Interest income	52	356	(304)	(85)%
Interest expense	(975)	(10,674)	9,699	(91)%
<b>Net loss</b>	<b>\$ (15,031)</b>	<b>\$ (37,990)</b>	<b>\$ 22,959</b>	<b>(60)%</b>

*Product revenue, net.* Product revenue, net for the three months ended March 31, 2021 (2021 Quarter) was \$0.0 million compared to \$5.0 million for the three months ended March 31, 2020 (2020 Quarter). Product revenue, net consisted of net product sales of COPIKTRA in the United States. Pursuant to the Secura APA discussed below under the heading *License and collaboration arrangements*, we have sold our COPIKTRA license and as of September 30, 2020, we no longer sell COPIKTRA in the United States.

*License and collaboration revenue.* License and collaboration revenue for the 2021 Quarter was \$0.0 million compared to less than \$0.1 million for the 2020 Quarter. 2020 Quarter license and collaboration revenue was comprised of duvelisib shipments to CSPC Pharmaceutical Group Limited.

*Sale of COPIKTRA license and related assets revenue.* Sale of COPIKTRA license and related assets revenue for the 2021 Quarter of \$0.9 million was comprised of \$0.8 million for a regulatory milestone achieved by Sanofi, a sub-licensee of Secura and \$0.1 million for royalties we expect to receive pursuant to the Secura APA from COPIKTRA sales by Secura's sublicensees that are not constrained.

*Transition services revenue.* Transition services revenue for the 2021 Quarter of \$0.2 million consisted of us providing certain support functions pursuant to the Secura transition service agreement, which was entered into in connection with the Secura APA. The services were provided at a mutually agreed upon rate. There was no transition services revenue in the 2020 Quarter.

*Costs of sales - product.* Costs of sales - product for the 2021 Quarter was \$0.0 million compared to \$0.5 million for the 2020 Quarter. Pursuant to the Secura APA discussed below under the heading *License and collaboration arrangements*, we have sold our COPIKTRA license and as of September 30, 2020, we no longer sell COPIKTRA in the United States. Therefore, there was no cost of sales-product in the 2021 Quarter.

*Cost of sales - intangible amortization.* Cost of sales - intangible amortization for the 2021 Quarter was \$0.0 million compared to \$0.4 million for the 2020 Quarter. Pursuant to the Secura APA discussed below under the heading *License and collaboration arrangements*, as of September 30, 2020, we have sold our COPIKTRA license, to which our intangible asset related.

*Research and development expense.* Research and development expense for the 2021 Quarter was \$8.9 million compared to \$10.9 million for the 2020 Quarter. The \$2.0 million decrease was primarily driven by a decrease of \$3.0 million in license fees related to a non-refundable payment of \$3.0 million made to Chugai in the 2020 Quarter for the VS-6766 license described further below under the heading *License and collaboration agreements* and a \$0.7 million decrease in contract research organization (CRO) costs. The decrease is partially offset by an increase of \$0.8 million in drug substance and drug product costs for VS-6766 and defactinib, increase of \$0.6 million in investigator sponsored trial (IST) expenses, and an increase of \$0.3 million in pre-clinical expenses. In future periods, we continue to expect expenses for VS-6766 and defactinib to increase as we have commenced our registration directed trials.

*Selling, general and administrative expense.* Selling, general and administrative expense for the 2021 Quarter was \$6.2 million compared to \$19.6 million for the 2020 Quarter. The decrease of \$13.4 million from the 2020 Quarter to the 2021 Quarter primarily resulted from a decrease of \$7.4 million in personnel related costs, including non-cash stock-based compensation, as a result of reduced headcount, \$5.0 million in consulting and professional fees, primarily related to the support of commercial activities in 2020 Quarter, \$0.5 million in reduced commercial activities costs and \$0.5 million in reduced travel and other costs.

*Other expense.* Other expense for the 2020 Quarter of \$1.3 million was for the mark-to-market adjustment related to the bifurcated make-whole interest provision derivative liability related to our 5.00% Convertible Senior Second Lien Notes due 2048 (the 2019 Notes). All 2019 Notes have converted to common stock as of March 31, 2020 and the derivative liability will no longer be remeasured. There was no other expense in the 2021 Quarter.

*Interest income.* Interest income for the 2021 Quarter was \$0.1 million compared to \$0.4 million for the 2020 Quarter. The decrease of \$0.3 million was primarily due to lower interest rates on investments.

*Interest expense.* Interest expense for the 2021 Quarter was \$1.0 million compared to \$10.7 million for the 2020 Quarter. The decrease of \$9.7 million was primarily due to non-cash interest expense recorded upon conversion of the 2019 Notes into common stock and decreased interest as a result of repayment of our term loan facility in 2020.

*Restructuring.* On February 27, 2020, we committed to an operational plan to reduce overall operating expenses, including the elimination of approximately 31 positions and other cost-saving measures (the "February 2020 Restructuring"). During the 2020 Quarter, we recorded an aggregate expense of \$1.8 million, which is reflected in the condensed consolidated statements of operation and comprehensive loss as selling general, and administrative expense and research and development expense for \$1.3 million and \$0.5 million, respectively, for one-time termination benefits for employee severance, benefits, and related costs. There were no restructuring charges in the 2021 Quarter.

## **LIQUIDITY AND CAPITAL RESOURCES**

### **Sources of liquidity**

We have financed our operations to date primarily through public and private offerings of our common stock, sales of common stock under our at-the-market equity offering programs, our loan and security agreement executed with Hercules in March 2017, as amended, the upfront payments under our license and collaboration agreements with Sanofi, Yakult, and CSPC, the upfront payment under the Secura APA, the issuance of 2018 Notes in October 2018, and the proceeds in connection with the PIPE. With the commercial launch of COPIKTRA in the United States in September 2018 through our ownership period ending in September 2020, we had recently begun financing a portion of our operations through product revenue. As of September 30, 2020, we have sold our COPIKTRA license and no longer sell COPIKTRA in the United States. We expect to finance a portion of our business through future milestones and royalties received pursuant to the Secura APA.

As of March 31, 2021, we had \$127.1 million in cash, cash equivalents, and investments. We primarily invest our cash, cash equivalents and investments in U.S. Government money market funds, agency bonds, corporate bonds and commercial paper of publicly traded companies.

Risks and uncertainties include those identified under Item 1A. Risk Factors, in our Annual Report on Form 10-K for the year ended December 31, 2020 as filed with the Securities and Exchange Commission (SEC) on March 18, 2021 and in any subsequent filings with the SEC.

#### Cash flows

The following table sets forth the primary sources and uses of cash for the 2021 Quarter and the 2020 Quarter (in thousands):

	Three months ended March 31,	
	2021	2020
Net cash (used in) provided by:		
Operating activities	\$ (19,318)	\$ (34,003)
Investing activities	(12,372)	32,050
Financing activities	(578)	93,421
<b>(Decrease) increase in cash, cash equivalents and restricted cash</b>	<b>\$ (32,268)</b>	<b>\$ 91,468</b>

*Operating activities.* The use of cash in both periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. Our cash outflow from net losses adjusted for non-cash charges was \$12.3 million and \$26.0 million for the 2020 Quarter and 2021 Quarter, respectively. Our cash outflow from operating activities due to changes in operating assets and liabilities was \$7.0 million and \$8.0 million for the 2021 Quarter and 2020 Quarter, respectively. The \$14.7 million decrease in cash used in operating activities for the 2021 Quarter compared to the 2020 Quarter was primarily due to decreased net loss, and a net decrease in components of working capital.

*Investing activities.* The cash used by investing activities for the 2021 Quarter primarily relates to the net purchases of investments of \$12.2 million and purchases of fixed assets of \$0.2 million. The cash provided by investing activities for the 2020 Quarter primarily reflects the net maturities of investments of \$32.1 million.

*Financing activities.* The cash used by financing activities for the 2021 Quarter primarily represents \$0.9 million in payments for settlement of restricted stock for tax withholdings, partially offset by \$0.3 million of proceeds received related to exercise of stock options and employee stock purchase plan. The cash provided by financing activities for the 2020 Quarter primarily represents \$93.9 million in net proceeds from sales of our common stock under the Purchase Agreement described below, and \$1.2 million of proceeds received related to exercise of stock options and employee stock purchase plan. This is partially offset by \$1.8 million of interest-make whole payments on the 2019 Notes.

On February 27, 2020, we entered into a Securities Purchase Agreement (Purchase Agreement) with certain institutional investors in which we agreed to sell 46,511,628 shares of common stock at a purchase price of \$2.15 per share, which represents 12.6% premium to the last reported sale price of our common stock of \$1.91 per share on February 27, 2020. On March 3, 2020, the transaction closed. The aggregate proceeds net of underwriting discounts and offering costs, were approximately \$93.8 million.

In March 2017, we established an at-the-market equity offering program (ATM) pursuant to which we were able to offer and sell up to \$35.0 million of our common stock at then current market prices from time to time through Cantor Fitzgerald & Co. (Cantor) as sales agent. In August 2017, we amended our sales agreement with Cantor to increase the maximum aggregate offering price of shares of common stock that can be sold under the at-the-market equity offering program to \$75.0 million.

During the 2021 Quarter, we sold 0 shares under the ATM. Through March 31, 2021, we have sold a total of 18,287,913 shares under this program for net proceeds of approximately \$59.6 million (after deducting commissions and other offering expenses). As of March 31, 2021, we can issue an aggregate amount of \$14.1 million of common stock under this program.

On October 17, 2018, we closed a registered direct public offering of \$150.0 million aggregate principal amount of our 2018 issued 5.00% Convertible Senior Notes due 2048 (the 2018 Notes), for net proceeds of approximately \$145.3 million. The 2018 Notes are governed by the terms of a base indenture for senior debt securities (the Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the Base Indenture, the 2018 Indenture), each dated October 17, 2018, by and between us and Wilmington Trust, National Association, as trustee. The 2018 Notes are senior unsecured obligations of us and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The 2018 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The 2018 Notes are convertible into shares of our common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 139.5771 shares of common stock per \$1,000 principal amount of the 2018 Notes, which corresponds to an initial conversion price of approximately \$7.16 per share of common stock and represents a conversion premium of approximately 15.0% above the last reported sale price of our common stock of \$6.23 per share on October 11, 2018. Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted Notes.

We will have the right, exercisable at our option, to cause all 2018 Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the 2018 Indenture) per share of our common stock equals or exceeds 130% of the conversion price, which equates to approximately \$9.31 per share, on each of at least 20 VWAP Trading Days, whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date we first issued the 2018 Notes.

On November 6, 2020, we entered into a privately negotiated agreement with an investor who is a holder of our 2018 Notes to exchange approximately \$28.0 million aggregate principal amount of 2018 Notes for approximately \$28.0 million aggregate principal amount of newly issued 5.00% Convertible Senior Notes due 2048 (the 2020 Notes). The issuance of the 2020 Notes closed on November 13, 2020. The 2020 Notes are governed pursuant to the Base Indenture between us and Wilmington dated as of October 17, 2018 as supplemented by the second supplemental indenture thereto dated as of November 13, 2020 (the Supplement Indenture and together with the Base Indenture, the 2020 Indenture).

We will have the right, exercisable at our option, to cause all 2020 Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the 2020 Indenture) per share of our common stock equals or exceeds 123.08% of the conversion price on each of at least 20 “VWAP Trading Days” (as defined in the 2020 Indenture), whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date we first issued the 2020 Notes.

The initial conversion rate for the 2020 Notes is 307.6923 shares of our common stock per \$1,000 principal amount of the 2020 Notes, which is equivalent to an initial conversion price of approximately \$3.25 per share, representing an approximately 153.9% premium to the sale price of \$1.28 per share of our common stock on November 5, 2020, as reported on the Nasdaq Global Market. The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

Prior to November 1, 2023, we will not have the right to redeem the 2020 Notes. On or after November 1, 2023, we may elect to redeem the 2020 Notes, in whole or in part, at a cash redemption price equal to the principal amount of the 2020 Notes to be redeemed, plus accrued and unpaid interest, if any.

Unless we have previously called all outstanding 2020 Notes for redemption, the 2020 Notes will be subject to repurchase by us at the holders’ option on each of November 1, 2023, November 1, 2028, November 1, 2033, November 1, 2038 and November 1, 2043 (or, if any such date is not a business day, on the next business day) at a cash repurchase price equal to the principal amount of the 2020 Notes to be repurchased, plus accrued and unpaid interest, if any.

Upon conversion of the 2020 Notes, holders will receive a cash payment equal to the accrued and unpaid interest on the converted 2020 Notes.

The 2020 Notes are our senior unsecured obligations and will be senior in right of payment to our future indebtedness that is expressly subordinated in right of payment to the 2020 Notes, and equal in right of payment with our existing and future indebtedness that is not so subordinated, and effectively subordinated to our existing and future indebtedness, to the extent of the value of the collateral securing such indebtedness. The 2020 Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent we are not a holder thereof) preferred equity, if any, of our subsidiaries.

As of March 31, 2021 and December 31, 2020, there was \$0.3 million and \$28.0 million aggregate principal amount outstanding of 2018 Notes and 2020 Notes, respectively, for a total of \$28.3 million aggregate principal amount outstanding.

#### **License and collaboration agreements**

##### **Secura**

On August 10, 2020, we and Secura signed an Asset Purchase Agreement (Secura APA) and on September 30, 2020, the transaction closed.

Pursuant to the Secura APA, we sold to Secura our exclusive worldwide license for the research, development, commercialization, and manufacture in oncology indications of products containing duvelisib. The sale included certain intellectual property related to duvelisib in oncology indications, certain existing duvelisib inventory, claims and rights under certain contracts pertaining to duvelisib. Pursuant to the Secura APA, Secura assumed all operational and financial responsibility for activities that were part of the duvelisib oncology program, including all commercialization efforts related to duvelisib in the United States and Europe, as well as our ongoing duvelisib clinical trials. Further, Secura assumed all obligations with existing collaboration partners developing and commercializing duvelisib, which include Yakult Honsha Co., Ltd. (Yakult), CSPC Pharmaceutical Group Limited (CSPC), and Sanofi. Additionally, Secura assumed all royalty payment obligations due under the amended and restated license agreement with Infinity.

Pursuant to the terms of the Secura APA, Secura has paid us an up-front payment of \$70.0 million and has agreed to pay us (i) regulatory milestone payments up to \$45.0 million, consisting of a payment of \$35.0 million upon receipt of regulatory approval of COPIKTRA in the United States for the treatment of peripheral T-cell lymphoma and a payment of \$10.0 million upon receipt of the first regulatory approval for the commercial sale of COPIKTRA in the European Union for the treatment of peripheral T-cell lymphoma, (ii) sales milestone payments of up to \$50.0 million, consisting of \$10.0 million when total worldwide net sales of COPIKTRA exceed \$100.0 million, \$15.0 million when total worldwide net sales of COPIKTRA exceed \$200.0 million and \$25.0 million when total worldwide net sales of COPIKTRA exceed \$300.0 million, (iii) low double-digit royalties on the annual aggregate net sales above \$100.0 million in the United States, European Union, and the United Kingdom of Great Britain and Northern Ireland and (iv) 50% of all royalty, milestone and sublicense revenue payments payable to Secura under our existing license agreements with Sanofi, Yakult, and CSPC, and 50% of all royalty and milestone payments payable to Secura under any license or sublicense agreement entered into by Secura in certain jurisdictions.

Secura's royalty obligations remain in effect on a country-by-country basis upon the last to occur (a) 10 years from the first commercial sale of product containing duvelisib in such country or (b) the expiration of all valid patent claims covering products containing duvelisib in such country.

In connection with the Secura APA, we and Secura entered into a transition services agreement (Secura TSA). Under the terms of the Secura TSA, we will provide certain support functions at Secura's directions for a term of less than one year from the date of execution, unless earlier terminated or extended according to the terms of the Secura TSA. Services performed are paid at a mutually agreed upon rate.



During the 2021 Quarter, we recognized \$0.9 million of sale of COPIKTRA license and related assets revenue related to one regulatory milestone achieved by Secura's sublicensee, Sanofi, of which we recognized \$0.8 million and \$0.1 million related to future royalties expected to be received pursuant to the Secura APA. We also recognized \$0.2 million in transition services revenue.

### **Chugai**

On January 7, 2020, we entered into a license agreement with Chugai (the Chugai Agreement) whereby Chugai granted us an exclusive worldwide license for the development, commercialization and manufacture of products containing VS-6766, a dual RAF/MEK inhibitor.

Under the terms of the Chugai Agreement, we received an exclusive right to develop and commercialize products containing VS-6766 at our own cost and expense. We are required to pay Chugai a non-refundable payment of \$3.0 million which was paid in February 2020. We are further obligated to pay Chugai double-digit royalties on net sales of products containing VS-6766, subject to reduction in certain circumstances. Chugai also obtained opt back rights to develop and commercialize VS-6766 (a) in the European Union, which option may be exercised through the date we submits a NDA to the FDA for a product which contains VS-6766 as the sole active pharmaceutical ingredient and (b) in Japan and Taiwan, which option may be exercised through the date we receive marketing authorization from the FDA for a product which contains VS-6766 as the sole active pharmaceutical ingredient. As consideration for executing either option, Chugai would have to make a payment to us calculated on our development costs to date. Chugai and we have made customary representations and warranties and have agreed to certain customary covenants, including confidentiality and indemnification.

Unless earlier terminated, the Chugai Agreement will expire upon the fulfillment of the royalty obligations to Chugai for the sale of any products containing the VS-6766, which royalty obligations expire on a product-by-product and country-by-country basis, upon the last to occur, in each specific country, of (a) expiration of valid patent claims covering such product or (b) 12 years from the first commercial sale of such product in such country.

We may terminate the Chugai Agreement upon 180 days' written notice. Subject to certain limitations, Chugai may terminate the Chugai Agreement upon written notice if we challenge any patent licensed by Chugai to us under the Chugai Agreement. Either party may terminate the license agreement in its entirety with 120 days' written notice for the other party's material breach if such party fails to cure the breach. Either party may also terminate the Chugai Agreement in its entirety upon certain insolvency events involving the other party.

We evaluated the license agreement with Chugai under ASC 805 and concluded that as the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar assets, the transaction did not meet the requirements to be accounted for as a business combination and therefore was accounted for as an asset acquisition. We recorded the up-front payment of \$3.0 million as research and development expense within the condensed consolidated statement of operations for the three months ended March 31, 2020.

### **Funding requirements**

We expect to continue to incur significant expenses and operating losses. We anticipate that our expenses and operating losses will continue as we:

- continue our ongoing clinical trials, including with VS-6766 and defactinib;
- initiate additional clinical trials for our product candidates;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other products and technologies;
- hire additional clinical, development and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts; and

- establish and maintain a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval.

We expect our existing cash resources will be sufficient to fund our obligations for at least the next twelve months from the date of filing of this Quarterly Report on Form 10-Q. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, and the extent to which we may enter into collaborations with third parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development of our current product candidates. Our future capital requirements will depend on many factors, including:

- the costs and timing of commercialization activities for our product candidates for which we expect to receive marketing approval;
- the scope, progress and results of our ongoing and potential future clinical trials;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs, timing and outcome of regulatory review of our product candidates (including our efforts to seek approval and fund the preparation and filing of regulatory submissions);
- revenue received from commercial sales our product candidates, should any of our other product candidates also receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property related claims;
- our ability to establish collaborations or partnerships on favorable terms, if at all; and
- Receipt of milestone payments and royalties pursuant to the Secura APA including timing of such receipt.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

#### **CONTRACTUAL OBLIGATIONS AND COMMITMENTS**

The disclosure of our contractual obligations and commitments was reported in our Annual Report on Form 10-K for the year ended December 31, 2020. There have not been any material changes from the contractual obligations and commitments previously disclosed in such report.

#### **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 under SEC rules.

#### **Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents and investments of \$127.1 million as of March 31, 2021 consisting of cash, U.S. Government money market funds, agency bonds, corporate bonds and commercial paper of publicly traded companies. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because most

of our investments are interest bearing. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration most of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and contract manufacturers globally which may be denominated in foreign currencies. We may be subject to fluctuations in foreign currency rates in connection with these agreements. Transactions denominated in currencies other than the functional currency are recorded based on exchange rates at the time such transactions arise. As of March 31, 2021, an immaterial amount of our total liabilities were denominated in currencies other than the functional currency.

The 2018 Notes and 2020 Notes bear interest at a fixed rate and therefore have minimal exposure to changes in interest rates; however, because the interest rates are fixed, we may be paying a higher interest rate, relative to market, in the future if our credit rating improves or other circumstances change.

#### **Item 4. Controls and Procedures.**

##### **Evaluation of disclosure controls and procedures**

Our management, with the participation of our Chief Executive Officer and our Chief Business and Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2021. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act of 1934 (Exchange Act), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2021, our Chief Executive Officer and our Chief Business and Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

##### **Changes in internal control over financial reporting**

There have been no changes in our internal control over financial reporting during the three months ended March 31, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**PART II—OTHER INFORMATION**

**Item 1. Legal Proceedings.**

None.

**Item 1A. Risk Factors.**

You should carefully review and consider the information regarding certain factors that could materially affect our business, financial condition or future results set forth under Item 1A. (Risk Factors) in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020 as filed with the SEC on March 18, 2021. There have been no material changes from the risk factors disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020, although we may disclose changes to such risk factors or disclose additional risk factors from time to time in our future filings with the SEC.

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

**RECENT SALES OF UNREGISTERED SECURITIES**

None.

**PURCHASE OF EQUITY SECURITIES**

We did not purchase any of our equity securities during the period covered by this Quarterly Report on Form 10-Q.

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

None.

**Item 5. Other Information.**

None.

**Item 6. Exhibits.**

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

**EXHIBIT INDEX**

31.1*	<a href="#">Certification of Principal Executive Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>
31.2*	<a href="#">Certification of Chief Financial Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>
32.1*	<a href="#">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.</a>
32.2*	<a href="#">Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</a>
99.1*	<a href="#">Press Release issued by Verastem, Inc. on May 11, 2021.</a>
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page from this Current Report on form 10-Q, formatted in Inline XBRL

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\* Filed or furnished herewith.

**SIGNATURES**

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

**VERASTEM, INC.**

Date: May 11, 2021

By: \_\_\_\_\_ /s/ BRIAN M. STUGLIK

Brian M. Stuglik  
*Chief Executive Officer*  
*(Principal executive officer)*

Date: May 11, 2021

By: \_\_\_\_\_ /s/ ROBERT GAGNON

Robert Gagnon  
*Chief Business and Financial Officer*  
*(Principal financial and accounting officer)*

## CERTIFICATIONS

I, Brian M. Stuglik, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Verastem, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ BRIAN M. STUGLIK

Brian M. Stuglik  
Chief Executive Officer  
(Principal executive officer)

Date: May 11, 2021

## CERTIFICATIONS

I, Robert Gagnon, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Verastem, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ ROBERT GAGNON

Robert Gagnon  
Chief Business and Financial Officer  
(Principal financial and accounting officer)

Date: May 11, 2021



**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 on Form 10-Q of Verastem, Inc. (the "Company") for the period ended March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Brian M. Stuglik, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ BRIAN M. STUGLIK

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Brian M. Stuglik  
*Chief Executive Officer*  
*(Principal executive officer)*

Date: May 11, 2021

**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 on Form 10-Q of Verastem, Inc. (the "Company") for the period ended March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Robert Gagnon, Chief Business and Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ ROBERT GAGNON

Robert Gagnon  
*Chief Business and Financial Officer*  
*(Principal financial and accounting officer)*

Date: May 11, 2021

## Verastem Oncology Reports First Quarter 2021 Financial Results and Highlights Recent Company Progress

*Updated Phase 1/2 Data in Recurrent Low-Grade Serous Ovarian Cancer (LGSOC) Continues to Demonstrate Activity, Durability and a Favorable Tolerability Profile for VS-6766 + Defactinib Combination*

*Two Registration-Directed Phase 2 Trials Underway in LGSOC and KRAS G12V Non-Small Cell Lung Cancer with Multiple Key Catalysts Expected in 1H 2022*

**BOSTON, MA – May 11, 2021** – Verastem, Inc. (Nasdaq: VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to advancing new medicines for patients battling cancer, today reported financial results for the three months ended March 31, 2021, highlighted recent progress and outlined key corporate objectives.

“During the first quarter of 2021, we expanded the selection portion of the Phase 2 RAMP 201 study to include all recurrent low-grade serous ovarian cancer types. This decision was based on the positive updated data from the LGSOC cohort of the Phase 1/2 FRAME which continues to show strong response rates across both KRAS mutant and wild-type recurrent LGSOC, along with robust durability and a favorable tolerability profile,” said Brian Stuglik, Chief Executive Officer of Verastem Oncology. “We closed the first quarter with just over \$127 million in cash, cash equivalent and investments, leaving us well positioned to execute on our two ongoing Phase 2 studies evaluating VS-6766 and defactinib in LGSOC and KRAS G12V non-small cell lung cancer (NSCLC), as well as our other key corporate objectives.”

### Recent Corporate Highlights

#### LGSOC

- Reported updated data from the LGSOC cohort of the ongoing, investigator-sponsored Phase 1/2 FRAME study evaluating VS-6766 in combination with defactinib in patients with recurrent LGSOC. Combination continues to demonstrate activity, durability and a favorable tolerability profile, including in patients who have progressed following treatment with a MEK inhibitor.
  - Overall response rate (ORR) across all patients was 52% (11 of 21 patients).
  - ORR for patients with KRAS mutant LGSOC was 70% (7 of 10 patients).
  - ORR for patients with wild type LGSOC was 44% (4 of 9 patients).
  - The most common side effects were Grade 1/2 rash, creatine kinase elevation, nausea, hyperbilirubinemia and diarrhea, which were reversible.

- Company-sponsored, registration-directed Phase 2 study (RAMP 201) underway investigating VS-6766 alone and in combination with defactinib for the treatment of recurrent LGSOC. Study recently expanded to include both KRAS mutant and KRAS wild-type patients in the selection phase to determine the optimal go-forward regimen for both types of LGSOC.

#### *KRAS G12V Mutant NSCLC*

- Company-sponsored, registration-directed Phase 2 study (RAMP 202) underway investigating VS-6766 alone and in combination with defactinib for the treatment of patients with KRAS G12V mutant NSCLC.

#### **Upcoming Milestones and Key Priorities for 2021-2022**

##### **LGSOC**

- Updated data from Phase 1/2 FRAME study to be submitted for presentation at a major medical meeting during the second half of 2021.
- Complete selection portion of RAMP 201 during first half of 2022; commence expansion portion.

##### **G12V NSCLC**

- Complete selection portion of RAMP 202 during first half of 2022; commence expansion portion.

#### **First Quarter 2021 Financial Results**

Verastem Oncology ended the first quarter 2021 with cash, cash equivalents and investments of \$127.1 million.

Total revenue for the three months ended March 31, 2021 (2021 Quarter) was \$1.0 million, compared to \$5.1 million for the three months ended March 31, 2020 (2020 Quarter).

Total operating expenses for the 2021 Quarter were \$15.1 million, compared to \$31.4 million for the 2020 Quarter.

Selling, general and administrative expenses for the 2021 Quarter were \$6.2 million, compared to \$19.6 million for the 2020 Quarter. The decrease of \$13.4 million, or 68.4%, primarily resulted from the Company's shift in strategic direction and COPIKTRA sale to Secura Bio, Inc., which led to lower employee related expenses and consulting and professional fees.

Research and development expense for the 2021 Quarter was \$8.9 million, compared to \$10.9 million for the 2020 Quarter. The decrease of \$2.0 million, or 18.3%, was primarily related to the upfront non-refundable payment of \$3.0 million to Chugai Pharmaceutical Co., Ltd for the VS-6766 license in the 2020 Quarter and decreased contract research organization costs, partially offset by increased drug substance and drug product costs and increased investigator sponsored trial expenses.

Net loss for the 2021 Quarter was \$15.0 million, or \$0.09 per share (basic and diluted), compared to \$38.0 million, or \$0.35 per share (basic and diluted), for the 2020 Quarter.

For the 2021 Quarter, non-GAAP adjusted net loss was \$12.4 million, or \$0.07 per share (diluted), compared to non-GAAP adjusted net loss of \$21.3 million, or \$0.20 per share (diluted), for the 2020 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

### **Financial Guidance and Outlook**

With the proceeds from the sale of COPIKTRA, Verastem Oncology expects that it will have a cash runway until at least 2024 to deliver on the current programs for VS-6766 and defactinib, including clinical and regulatory milestones and development in LGSOC and KRAS mutant NSCLC. Verastem Oncology expects its 2021 annual operating expenses to be approximately \$50 million.

### **Use of Non-GAAP Financial Measures**

To supplement Verastem Oncology's condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company's GAAP financial statements, because it provides greater transparency and period-over-period comparability with respect to the Company's operating performance and can enhance investors' ability to identify operating trends in the Company's business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company's operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three months ended March 31, 2021 and 2020 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

### **About VS-6766**

VS-6766 (formerly known as CH5126766 and RO5126766) is a unique inhibitor of the RAF/MEK signaling pathway. In contrast to other MEK inhibitors in development, VS-6766 blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows VS-6766 to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors.

### **About Defactinib**

Defactinib (VS-6063) is an oral small molecule inhibitor of FAK and PYK2 that is currently being evaluated as a potential combination therapy for various solid tumors. The Company has received Orphan Drug designation for defactinib in ovarian cancer and mesothelioma in the US, EU and Australia. Preclinical research by Verastem Oncology scientists and collaborators at world-renowned research institutions has described the effect of FAK inhibition to enhance immune response by decreasing immuno-suppressive

cells, increasing cytotoxic T cells, and reducing stromal density, which allows tumor-killing immune cells to enter the tumor.<sup>1,2</sup>

#### **About the VS-6766/Defactinib Combination**

RAS mutant tumors are present in ~30% of all human cancers, have historically presented a difficult treatment challenge and are often associated with significantly worse prognosis. Challenges associated with identifying new treatment options for these types of cancers include resistance to single agents, identifying tolerable combination regimens with MEK inhibitors and new RAS inhibitors in development addressing only a minority of all RAS mutated cancers.

The combination of VS-6766 and defactinib has been found to be clinically active in patients with KRAS mt tumors. In an ongoing investigator-initiated Phase 1/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRAS mt NSCLC and colorectal cancer (CRC). The FRAME study was expanded to include new cohorts in pancreatic cancer, KRASmt endometrioid cancer and KRAS-G12V NSCLC. Verastem Oncology is also supporting an investigator-initiated Phase 2 trial evaluating VS-6766 with defactinib in patients with metastatic uveal melanoma.

Verastem Oncology has initiated Phase 2 registration-directed trials of VS-6766 with defactinib in patients with recurrent LGSOC and in patients with recurrent KRAS-G12V NSCLC as part of its RAMP (**Raf And Mek Program**).

#### **About Verastem Oncology**

Verastem Oncology (Nasdaq: VSTM) is a development-stage biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including RAF/MEK inhibition and focal adhesion kinase (FAK) inhibition. For more information, please visit [www.verastem.com](http://www.verastem.com).

#### **Forward-Looking Statements Notice**

This press release includes forward-looking statements about Verastem Oncology's strategy and future plans and prospects, including statements related to the expected use of the Company's cash and cash equivalents, the potential clinical value of the RAF/MEK/FAK combination and the timing of commencing and completing registration-directed trials for the RAF/MEK/FAK combination. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "can," "promising" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement.

Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in the development and potential commercialization of our product candidates, including defactinib in combination with VS-6766; the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis or result in unmanageable safety profiles as compared to their levels of efficacy; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the scope, timing, and outcome of any legal

proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that our product candidates will experience manufacturing or supply interruptions or failures; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the VS-6766 license agreement; that we may not have sufficient cash to fund our contemplated operations; that we may be unable to obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will be unable to execute on our partnering strategies for defactinib in combination with VS-6766; that we will not pursue or submit regulatory filings for our product candidates; and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients.

Other risks and uncertainties include those identified under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2020 as filed with the Securities and Exchange Commission (SEC) on March 18, 2021 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology's views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

#### **References**

<sup>1</sup> Gerber D. et al. Phase 2 study of the focal adhesion kinase inhibitor defactinib (VS-6063) in previously treated advanced KRAS mutant non-small cell lung cancer. *Lung Cancer* 2020: 139:60-67.

<sup>2</sup> Chénard-Poirier, M. et al. Results from the biomarker-driven basket trial of RO5126766 (CH5127566), a potent RAF/MEK inhibitor, in RAS- or RAF-mutated malignancies including multiple myeloma. *Journal of Clinical Oncology* 2017: 35. 10.1200/JCO.2017.35.15\_suppl.2506.

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**Verastem, Inc.**  
**Condensed Consolidated Balance Sheets**  
(in thousands)  
(unaudited)

	<b>March 31, 2021</b>	<b>December 31, 2020</b>
Cash, cash equivalents, & investments	\$ 127,095	\$ 147,221
Accounts receivable, net	1,125	239
Prepaid expenses and other current assets	5,456	3,473
Property and equipment, net	335	416
Right-of-use asset, net	2,628	2,726
Restricted cash and other assets	262	274
<b>Total assets</b>	<b>\$ 136,901</b>	<b>\$ 154,349</b>
<b>Current Liabilities</b>	<b>\$ 11,844</b>	<b>\$ 17,093</b>
Convertible senior notes	19,672	19,051
Lease Liability, long-term	2,776	2,931
Stockholders' equity	102,609	115,274
<b>Total liabilities and stockholders' equity</b>	<b>\$ 136,901</b>	<b>\$ 154,349</b>

**Verastem, Inc.**  
**Condensed Consolidated Statements of Operations**  
(in thousands, except per share amounts)  
(unaudited)

	<b>Three months ended March 31,</b>	
	<b>2021</b>	<b>2020</b>
Revenue:		
Product revenue, net	\$ —	\$ 5,034
License and collaboration revenue	—	22
Sale of COPIKTRA license and related assets revenue	850	—
Transition services revenue	156	—
<b>Total revenue</b>	<b>1,006</b>	<b>5,056</b>
Operating expenses:		
Cost of sales - product	—	495
Cost of sales - intangible amortization	—	392
Research and development	8,896	10,924
Selling, general and administrative	6,218	19,604
<b>Total operating expenses</b>	<b>15,114</b>	<b>31,415</b>
Loss from operations	(14,108)	(26,359)
Other expense	—	(1,313)
Interest income	52	356
Interest expense	(975)	(10,674)
<b>Net loss</b>	<b>(15,031)</b>	<b>(37,990)</b>
Net loss per share—basic and diluted	\$ (0.09)	\$ (0.35)
Weighted average common shares outstanding used in computing:		
Net loss per share – basic and diluted	171,586	108,153

**Verastem, Inc.**  
**Reconciliation of GAAP to Non-GAAP Financial Information**  
(in thousands, except per share amounts)  
(unaudited)

	Three months ended March 31,	
	2021	2020
<b>Net loss reconciliation</b>		
Net loss (GAAP basis)	\$ (15,031)	\$ (37,990)
<b>Adjust:</b>		
Amortization of acquired intangible asset	—	392
Stock-based compensation expense	1,980	1,370
Non-cash interest, net	636	8,779
Severance and Other	—	1,788
Change in fair value of derivative	—	1,313
Chugai license payment	—	3,000
<b>Adjusted net loss (non-GAAP basis)</b>	<b>\$ (12,415)</b>	<b>\$ (21,348)</b>
<b>Reconciliation of Net loss Per Share</b>		
Net loss per share – diluted (GAAP Basis)	\$ (0.09)	\$ (0.35)
<b>Adjust per diluted share</b>		
Amortization of acquired intangible asset	—	—
Stock-based compensation expense	0.01	0.01
Non-cash interest, net	0.01	0.088
Severance and Other	—	0.02
Change in fair value of derivative	—	0.01
Chugai license payment	—	0.03
<b>Adjusted net loss per share – diluted (non-GAAP Basis)</b>	<b>\$ (0.07)</b>	<b>\$ (0.20)</b>
Weighted average common shares outstanding used in computing net loss per share—diluted	171,586	108,153