INSMED Inc
Form 10-Q
October 30, 2018
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

OR

o **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 0-30739

INSMED INCORPORATED

(Exact name of registrant as specified in its charter)

Virginia	**54-1972729**
(State or other jurisdiction of incorporation or organization)	(I.R.S. employer identification no.)
10 Finderne Avenue, Building 10	
Bridgewater, New Jersey	**08807**
(Address of principal executive offices)	(Zip Code)

(908) 977-9900

(Registrant's telephone number including area code)

Not Applicable

(Former name, former address and former fiscal year, if changed since last report)

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 x No o

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 x No o

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

Large accelerated filer x Accelerated filer o

Non-accelerated filer o Smaller reporting company o

Emerging growth company o

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. o

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

As of October 26, 2018, there were 77,090,229 shares of the registrant's common stock, $0.01 par value, outstanding.

Table of Contents

INSMED INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED SEPTEMBER 30, 2018

INDEX

Unless the context otherwise indicates, references in this Form 10-Q to "Insmed Incorporated" refers to Insmed Incorporated, a Virginia corporation, and "Company," "Insmed," "we," "us" and "our" refer to Insmed Incorporated together with its consolidated subsidiaries. INSMED, ARIKAYCE, and CONVERT are trademarks of Insmed Incorporated. This Form 10-Q also contains trademarks of third parties. Each trademark of another company appearing in this Form 10-Q is the property of its owner.

Table of Contents

PART I. FINANCIAL INFORMATION

ITEM 1. CONSOLIDATED FINANCIAL STATEMENTS

INSMED INCORPORATED
Consolidated Balance Sheets
(in thousands, except par value and share data)

	As of September 30, 2018 (unaudited)	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$567,574	$381,165
Prepaid expenses and other current assets	9,921	8,279
Total current assets	577,495	389,444
Intangible assets	59,941	58,200
Fixed assets, net	19,526	12,432
Other assets	4,551	1,971
Total assets	$661,513	$462,047
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	$16,579	$14,671
Accrued expenses	40,368	29,339
Other current liabilities	472	646
Total current liabilities	57,419	44,656
Long-term debt, net	311,861	55,567
Other long-term liabilities	826	765
Total liabilities	370,106	100,988
Shareholders' equity:		
Common stock, $0.01 par value; 500,000,000 authorized shares, 77,085,715 and 76,610,508 issued and outstanding shares at September 30, 2018 and December 31, 2017, respectively	771	766
Additional paid-in capital	1,481,205	1,318,181
Accumulated deficit	(1,190,589)	(957,885)
Accumulated other comprehensive income (loss)	20	(3)
Total shareholders' equity	291,407	361,059
Total liabilities and shareholders' equity	$661,513	$462,047

See accompanying notes to consolidated financial statements

Table of Contents

INSMED INCORPORATED
Consolidated Statements of Comprehensive Loss (unaudited)
(in thousands, except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues	$—	$—	$—	$—
Operating expenses:				
Research and development	39,538	26,675	105,358	75,800
General and administrative	44,445	17,408	114,258	47,767
Total operating expenses	83,983	44,083	219,616	123,567
Operating loss	(83,983)	(44,083)	(219,616)	(123,567)
Investment income	2,741	326	7,510	649
Interest expense	(6,675)	(1,496)	(18,805)	(4,459)
Loss on extinguishment of debt	—	—	(2,209)	—
Other income, net	220	101	550	206
Loss before income taxes	(87,697)	(45,152)	(232,570)	(127,171)
Provision for income taxes	46	27	134	94
Net loss	$(87,743)	$(45,179)	$(232,704)	$(127,265)
Basic and diluted net loss per share	$(1.14)	$(0.69)	$(3.03)	$(2.01)
Weighted average basic and diluted common shares outstanding	77,066	65,312	76,819	63,199
Net loss	$(87,743)	$(45,179)	$(232,704)	$(127,265)
Other comprehensive income:				
Foreign currency translation gains	2	76	23	99
Total comprehensive loss	$(87,741)	$(45,103)	$(232,681)	$(127,166)

See accompanying notes to consolidated financial statements

Table of Contents

INSMED INCORPORATED
Consolidated Statements of Cash Flows (unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2018	2017
Operating activities		
Net loss	$(232,704)	$(127,265)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	2,652	2,168
Stock-based compensation expense	20,205	13,332
Amortization of debt issuance costs	1,000	91
Accretion of debt discount	11,541	—
Accretion of back-end fee on debt	50	506
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(4,315)	(1,052)
Accounts payable	2,536	(921)
Accrued expenses and other	10,596	1,745
Net cash used in operating activities	(188,439)	(111,396)
Investing activities		
Purchase of fixed assets	(10,063)	(1,301)
Net cash used in investing activities	(10,063)	(1,301)
Financing activities		
Proceeds from exercise of stock options	6,390	2,953
Loss on extinguishment of debt	(2,209)	—
Payment of debt	(55,000)	—
Proceeds from issuance of 1.75% convertible senior notes due 2025	450,000	—
Payment of debt issuance costs	(14,235)	—
Proceeds from issuance of common stock, net	—	377,703
Net cash provided by financing activities	384,946	380,656
Effect of exchange rates on cash and cash equivalents	(35)	128
Net increase in cash and cash equivalents	186,409	268,087
Cash and cash equivalents at beginning of period	381,165	162,591
Cash and cash equivalents at end of period	$567,574	$430,678
Supplemental disclosures of cash flow information:		
Cash paid for interest	$4,975	$3,876
Cash paid for income taxes	$127	$62

See accompanying notes to consolidated financial statements

Table of Contents

INSMED INCORPORATED
NOTES TO UNAUDITED CONSOLIDATED FINANCIAL STATEMENTS

1. The Company and Basis of Presentation

Insmed is a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. The Company's first commercial product, ARIKAYCE (amikacin liposome inhalation suspension), received accelerated approval in the United States (US) on September 28, 2018 for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a rare and often chronic infection that can cause irreversible lung damage and can be fatal. The Company's clinical-stage pipeline includes INS1007 and INS1009. INS1007 is a novel oral, reversible inhibitor of dipeptidyl peptidase 1 (DPP1) with therapeutic potential in non-cystic fibrosis (non-CF) bronchiectasis and other inflammatory diseases. INS1009 is an inhaled formulation of a treprostinil prodrug that may offer a differentiated product profile for rare pulmonary disorders, including pulmonary arterial hypertension (PAH).

The Company was incorporated in the Commonwealth of Virginia on November 29, 1999 and its principal executive offices are in Bridgewater, New Jersey. The Company has legal entities in the US, Ireland, Germany, France, the United Kingdom (UK), the Netherlands, and Japan. All intercompany transactions and balances have been eliminated in consolidation.

The accompanying unaudited interim consolidated financial statements have been prepared pursuant to the rules and regulations for reporting on Form 10-Q. Accordingly, certain information and disclosures required by accounting principles generally accepted in the US for complete consolidated financial statements are not included herein. The unaudited interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2017.

The results of operations of any interim period are not necessarily indicative of the results of operations for the full year. The unaudited interim consolidated financial information presented herein reflects all normal adjustments that are, in the opinion of management, necessary for a fair statement of the financial position, results of operations and cash flows for the periods presented.

The Company had $567.6 million in cash and cash equivalents as of September 30, 2018 and reported a net loss of $232.7 million for the nine months ended September 30, 2018. Historically, the Company has funded its operations through public offerings of equity securities and debt financings. To date, the Company has not generated material revenue from ARIKAYCE. The Company commenced commercial shipments in October 2018. The Company expects to continue to incur operating losses while funding commercial launch efforts for ARIKAYCE, research and development (R&D) activities, regulatory submissions outside the US, and general and administrative expenses. The Company expects its future cash requirements to be substantial, and the Company may need to raise additional capital to fund operations, including the commercialization of ARIKAYCE and additional clinical trials related to ARIKAYCE, to develop INS1007 and INS1009 and to develop, acquire, in-license or co-promote other products that address orphan or rare diseases.

The source, timing and availability of any future financing or other transaction will depend principally upon continued progress in the Company’s commercial, regulatory and development activities. Any equity or debt financing will also be contingent upon equity and debt market conditions and interest rates at the time. If the Company is unable to obtain sufficient additional funds when required, the Company may be forced to delay, restrict or eliminate all or a portion of its development programs or commercialization efforts.

2. Summary of Significant Accounting Policies

The following are the required interim disclosure updates to the Company's significant accounting policies described in Note 2 of the notes to the consolidated financial statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2017:

Fair Value Measurements - The Company categorizes its financial assets and liabilities measured and reported at fair value in the financial statements on a recurring basis based upon the level of judgment associated with the inputs used to measure their fair value. Hierarchical levels, which are directly related to the amount of subjectivity associated with the inputs used to determine the fair value of financial assets and liabilities, are as follows:

Table of Contents

- Level 1 — Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

- Level 2 — Inputs (other than quoted prices included in Level 1) are either directly or indirectly observable for the assets or liability through correlation with market data at the measurement date and for the duration of the instrument's anticipated life.

- Level 3 — Inputs reflect management's best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

Each major category of financial assets and liabilities measured at fair value on a recurring basis is categorized based upon the lowest level of significant input to the valuations. The fair value hierarchy also requires an entity to maximize the use of observable inputs and minimize the use of unobservable inputs when measuring fair value. Financial instruments in Level 1 generally include US treasuries and mutual funds listed in active markets.

The Company's only financial assets and liabilities which were measured at fair value as of September 30, 2018 and December 31, 2017 were Level 1 assets comprised of cash and cash equivalents of $567.6 million and $381.2 million, respectively. The estimated fair value of the liability component of the 1.75% convertible senior notes due 2025 (the Convertible Notes) (categorized as a Level 2 liability for fair value measurement purposes) was determined using current market factors and the ability of the Company to obtain debt on comparable terms to the Convertible Notes. The following table shows certain assets and liabilities and their carrying values and fair values:

	As of September 30, 2018	
	Carrying Value	Fair Value
	(in millions)	
Level 1		
Cash and cash equivalents	$567.6	$567.6
Level 2		
Convertible Notes ($450.0 face value)	$311.9*	$381.6

* The carrying value of the Convertible Notes excludes $129.4 million of the unamortized portion of the debt discount.

The Company's cash and cash equivalents permit daily redemption and the fair values of these investments are based upon the quoted prices in active markets provided by the holding financial institutions. Cash equivalents consist of liquid investments with an original maturity of three months or less from the date of purchase. As of September 30, 2018, the Company's cash and cash equivalents balance included US treasury bills of $399.8 million.

The Company recognizes transfers between levels within the fair value hierarchy, if any, at the end of each quarter. There were no transfers in or out of Level 1, Level 2 or Level 3 during the nine months ended September 30, 2018 and 2017, respectively.

As of September 30, 2018 and December 31, 2017, the Company held no securities that were in an unrealized gain or loss position. The Company reviews the status of each security quarterly to determine whether an other-than-temporary impairment has occurred. In making its determination, the Company considers a number of factors, including: (1) the significance of the decline; (2) whether the securities were rated below investment grade; (3) how long the securities have been in an unrealized loss position; and (4) the Company's ability and intent to retain the investment for a sufficient period of time for it to recover.

Net Loss Per Share - Basic net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding during the period. Diluted net loss per share is computed by dividing net loss by the weighted average number of common shares and other dilutive securities outstanding during the period. Potentially dilutive securities from stock options, restricted stock units (RSUs) and convertible debt securities would be anti-dilutive as the Company incurred a net loss. Potentially dilutive common shares resulting from the assumed exercise of outstanding stock options and from the assumed conversion of the Convertible Notes are determined based on the treasury stock method.

Table of Contents

The following table sets forth the reconciliation of the weighted average number of common shares used to compute basic and diluted net loss per share for the three and nine months ended September 30, 2018 and 2017:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
	(in thousands, except per share amounts)			
Numerator:				
Net loss	$(87,743)	$(45,179)	$(232,704)	$(127,265)
Denominator:				
Weighted average common shares used in calculation of basic net loss per share:	77,066	65,312	76,819	63,199
Effect of dilutive securities:				
Common stock options	—	—	—	
RSUs	—	—	—	
Convertible debt securities	—	—	—	—
Weighted average common shares outstanding used in calculation of diluted net loss per share	77,066	65,312	76,819	63,199
Net loss per share:				
Basic and diluted	$(1.14)	$(0.69)	$(3.03)	$(2.01)

The following potentially dilutive securities have been excluded from the computations of diluted weighted average common shares outstanding as of September 30, 2018 and 2017 as their effect would have been anti-dilutive (in thousands):

	As of September 30,	
	2018	2017
Stock options to purchase common stock	9,608	8,601
Unvested RSUs	245	47
Convertible debt securities	11,492	—

Inventory - Inventory is stated at the lower of cost and net realizable value with cost determined on a standard costing method. The Company began capitalizing inventory costs subsequent to US Food and Drug Administration (FDA) approval of ARIKAYCE on September 28, 2018, when it was determined that the inventory had a probable future economic benefit. Inventory will be sold beginning in the fourth quarter of 2018 based on first-in, first out basis. Manufacturing variances, such as material usage variance and yield variance, will be capitalized in inventory until the respective units are sold at which point the variances will be released in cost of goods sold. The Company will periodically review inventory for expiry and obsolescence and write down accordingly. The Company performs quality control procedures throughout the manufacturing processes of ARIKAYCE; however, certain batches or units of ARIKAYCE may not meet quality specifications and result in a charge to cost of goods sold.

Prior to FDA approval of ARIKAYCE, the Company expensed all inventory related costs in the period incurred; therefore, inventory is not included in the September 30, 2018 consolidated balance sheet. Inventory used for clinical development purposes is expensed to research and development (R&D) expense when consumed.

Recently Adopted Accounting Pronouncements - In August 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*, which addressed eight specific cash flow issues with the objective of reducing the

existing diversity in practice. Among the updates, the standard requires debt extinguishment costs to be classified as cash outflows for financing activities. This standard update is effective as of the first quarter of 2018. As a result of the adoption of the standard, in the first quarter of 2018, the Company reported a $2.2 million loss on extinguishment of debt in the financing activities section of its consolidated statement of cash flows. The Company had no material debt extinguishment costs prior to the first quarter of 2018. The impact of adopting this standard was not material to the Company.

Table of Contents

New Accounting Pronouncements (Not Yet Adopted)—In February 2016, the FASB issued ASU 2016-2, *Leases (Topic 842)* in order to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet for those leases classified as operating leases under previous generally accepted accounting principles. ASU 2016-2 requires a lessee to recognize a liability to make lease payments (the lease liability) and a right-of-use asset representing its right to use the underlying asset for the lease term on the balance sheet. ASU 2016-2 is effective for fiscal years beginning after December 15, 2018 (including interim periods within those periods) and early adoption is permitted. In August 2018, the FASB issued ASU 2018-11, *Targeted Improvements to ASC 842*, which provides a new transition option in which an entity initially applies ASU 2016-2 at the adoption date and recognizes a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption. The Company will use the new transition option and is also utilizing the package of practical expedients that allows it to not reassess: (1) whether any expired or existing contracts are or contain leases, (2) lease classification for any expired or existing leases, and (3) initial direct costs for any expired or existing leases. The Company additionally expects to use the practical expedient that allows it to treat the lease and non-lease components of its leases as a single component. The Company has identified approximately ten leasing arrangements and is currently assessing the financial impact on the consolidated balance sheet. The Company expects to adopt ASU 2016-2 in the first quarter of 2019 and is in the process of evaluating the impact of adoption on its consolidated financial statements.

3. Intangible Assets

As of September 30, 2018, the Company's identifiable intangible assets consisted of acquired ARIKAYCE R&D, formerly referred to as in-process research and development, and a milestone payable to PARI Pharma GmbH (PARI) for the license to use PARI's Lamira™ Nebulizer System for the delivery of ARIKAYCE to patients. The total carrying value of the acquired ARIKAYCE R&D was $58.2 million as of September 30, 2018 and December 31, 2017, resulting from the initial amount recorded at the time of the Company's merger with Transave, Inc. (Transave) in 2010 and subsequent adjustments. On September 28, 2018, as a result of the FDA approval for ARIKAYCE, the Company recorded a milestone payment of $1.7 million due to PARI.

Intangible assets consist of the following:

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Acquired ARIKAYCE R&D	$58,200	$58,200
PARI milestone upon FDA approval	1,741	—
Intangible assets	$59,941	$58,200

Intangible assets are measured at their respective fair values on the date they were recorded and, with respect to the Acquired ARIKAYCE milestone, at the date of subsequent adjustments of fair value. Intangible assets will be amortized beginning October 1, 2018 over the initial regulatory exclusivity period for ARIKAYCE (12 years). As of September 30, 2018, the Company performed a qualitative assessment on the assets. This assessment did not identify any indicators of impairment of the intangible assets and indicated that the implied value of the assets was more than 100% greater than the book value as of that date.

4. Accrued Expenses

Accrued expenses consist of the following:

9

Table of Contents

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Accrued clinical trial expenses	$6,576	$ 7,837
Accrued compensation	11,387	12,197
Accrued professional fees	10,746	4,500
Accrued technical operation expenses	5,137	2,182
Accrued milestone payment	1,741	—
Accrued interest payable	1,663	423
Accrued construction costs	2,069	1,719
Other accrued expenses	1,049	481
	$40,368	$ 29,339

5. Debt

In January 2018, the Company completed an underwritten public offering of the Convertible Notes, in which the Company sold $450.0 million aggregate principal amount of Convertible Notes, including the exercise in full of the underwriters' option to purchase additional Convertible Notes of $50.0 million. The Company's net proceeds from the offering, after deducting underwriting discounts and commissions and other offering expenses of $14.2 million, were approximately $435.8 million. The Convertible Notes bear interest payable semiannually in arrears on January 15 and July 15 of each year, beginning on July 15, 2018. The Convertible Notes mature on January 15, 2025, unless earlier converted, redeemed, or repurchased.

On or after October 15, 2024, until the close of business on the second scheduled trading day immediately preceding January 15, 2025, holders may convert their Convertible Notes at any time. Upon conversion, holders may receive cash, shares of the Company's common stock or a combination of cash and shares of the Company's common stock, at the Company's option. The initial conversion rate is 25.5384 shares of common stock per $1,000 principal amount of Convertible Notes (equivalent to an initial conversion price of approximately $39.16 per share of common stock). The conversion rate will be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

Holders may convert their Convertible Notes prior to October 15, 2024, only under the following circumstances, subject to the conditions set forth in an indenture, dated as of January 26, 2018, between the Company and Wells Fargo Bank, National Association (Wells Fargo), as trustee, as supplemented by the first supplemental indenture, dated January 26, 2018, between the Company and Wells Fargo (as supplemented, the Indenture): (i) during the five business day period immediately after any five consecutive trading day period (the measurement period) in which the trading price per $1,000 principal amount of convertible notes, as determined following a request by a holder of the convertible notes, for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the common stock and the conversion rate on such trading day, (ii) the Company elects to distribute to all or substantially all holders of the common stock (a) any rights, options or warrants (other than in connection with a stockholder rights plan for so long as the rights issued under such plan have not detached from the associated shares of common stock) entitling them, for a period of not more than 45 days from the declaration date for such distribution, to subscribe for or purchase shares of common stock at a price per share that is less than the average of the last reported sale prices of the common stock for the 10 consecutive trading day period ending on, and including, the trading day immediately preceding the declaration date for such distribution, or (b) the Company’s assets, debt securities or rights to purchase securities of the Company, which distribution has a per share value, as reasonably determined by the board of directors, exceeding 10% of the last reported sale price of the common stock on the trading day immediately preceding the declaration date for such distribution, (iii) if a transaction or event that

constitutes a fundamental change or a make-whole fundamental change occurs, or if the Company is a party to (a) a consolidation, merger, combination, statutory or binding share exchange or similar transaction, pursuant to which the common stock would be converted into, or exchanged for, cash, securities or other property or assets, or (b) any sale, conveyance, lease or other transfer or similar transaction in one transaction or a series of transactions of all or substantially all of the consolidated assets of the Company and its subsidiaries, taken as a whole, all or any portion of the Convertible Notes may be surrendered by a holder for conversion at any time from or after the date that is 30 scheduled trading days prior to the anticipated effective date of the transaction, (iv) if during any calendar quarter commencing after the calendar quarter ending on March 31, 2018 (and only during such calendar quarter), the last reported sale price of the common stock for at least 20 trading days (whether or not consecutive) during the period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day, or, (v) if the Company sends a notice of redemption, a holder may

Table of Contents

surrender all or any portion of its Convertible Notes, to which the notice of redemption relates, for conversion at any time on or after the date the applicable notice of redemption was sent until the close of business on (a) the second business day immediately preceding the related redemption date or (b) if the Company fails to pay the redemption price on the redemption date as specified in such notice of redemption, such later date on which the redemption price is paid.

The Convertible Notes can be settled in cash, common stock, or a combination of cash and common stock at the Company's option, and thus, the Company determined the embedded conversion options in the convertible notes are not required to be separately accounted for as a derivative. However, since the Convertible Notes are within the scope of the accounting guidance for cash convertible instruments, the Company is required to separate the Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated equity component. The fair value was based on data from readily available pricing sources which utilize market observable inputs and other characteristics for similar types of instruments. The carrying amount of the equity component representing the embedded conversion option was determined by deducting the fair value of the liability component from the gross proceeds of the Convertible Notes. The excess of the principal amount of the liability component over its carrying amount is amortized to interest expense over the expected life of a similar liability that does not have an associated equity component using the effective interest method. The equity component is not remeasured as long as it continues to meet the conditions for equity classification in the accounting guidance for contracts in an entity’s own equity. The fair value of the liability component of the Convertible Notes on the date of issuance was estimated at $309.1 million using an effective interest rate of 7.6%, and accordingly, the residual equity component on the date of issuance was $140.9 million. The discount is being amortized to interest expense over the term of the Convertible Notes and has a remaining period of approximately 6.28 years.

For the three and nine months ended September 30, 2018, total interest expense related to the Convertible Notes was $6.7 million and $17.9 million, respectively, which includes the contractual interest coupon payable semi-annually in cash, the amortization of the issuance costs, and accretion of debt discount, as described in the table below. The following table presents the carrying value of the Company’s debt balance as of September 30, 2018 (in thousands):

1.75% convertible senior notes due 2025	$450,000
Debt issuance costs, unamortized	(8,789)
Discount on debt	(129,350)
Long-term debt, net	$311,861

As of September 30, 2018, future principal repayments of the debt for each of the fiscal years through maturity were as follows (in thousands):

Year Ending December 31:	
2018	$—
2019	—
2020	—
2021	—
2022	—
2023 and thereafter	450,000
	$450,000

In February 2018, the Company used part of the net proceeds from the issuance of the Convertible Notes to pay off its outstanding debt to Hercules Capital (Hercules). The payments to Hercules consisted of $55.0 million for the principal amount and an additional $3.2 million in back-end fees, outstanding interest, and prepayment penalty fees, which resulted in a $2.2 million loss on extinguishment of debt in the quarter ended March 31, 2018.

Interest Expense

The following table sets forth the total interest expense recognized in the periods presented (in thousands):

Table of Contents

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Contractual interest expense	$1,971	$1,302	$6,214	$3,862
Amortization of debt issuance costs	415	30	1,000	91
Accretion of back-end fee on debt	—	164	50	506
Accretion of debt discount	4,289	—	11,541	—
Total interest expense	$6,675	$1,496	$18,805	$4,459

6. Shareholders' Equity

Common Stock — As of September 30, 2018, the Company had 500,000,000 shares of common stock authorized with a par value of $0.01 per share and 77,085,715 shares of common stock issued and outstanding. In addition, as of September 30, 2018, the Company had reserved 9,608,344 shares of common stock for issuance upon the exercise of outstanding stock options and 244,801 shares of common stock for issuance upon the vesting of RSUs. The Company has also reserved shares of common stock for issuance upon conversion of the Convertible Notes, subject to adjustment in accordance with the Indenture.

In January 2018, the Company completed an underwritten public offering of $450.0 million aggregate principal amount of Convertible Notes, including the exercise in full of the underwriter's option to purchase additional Convertible Notes. The fair value of the liability component of the Convertible Notes on the date of issuance was estimated at $309.1 million, and accordingly, the equity component (included in additional paid-in capital) on the date of issuance was calculated as $140.9 million using the residual method, as further described in *Note 5 Debt*.

In September 2017, the Company completed an underwritten public offering of 14,123,150 shares of the Company's common stock, which included the underwriter's exercise in full of its over-allotment option of 1,842,150 shares, at a price to the public of $28.50 per share. The Company's net proceeds from the sale of the shares, after deducting the underwriter's discount and offering expenses of $24.8 million, were $377.7 million.

Preferred Stock — As of September 30, 2018, the Company had 200,000,000 shares of preferred stock authorized with a par value of $0.01 per share and no shares of preferred stock were issued and outstanding.

The following table summarizes the changes in total shareholders' equity for the nine months ended September 30, 2018 (in thousands):

Balance at December 31, 2017	$361,059
Net loss for the period	(232,704)
Proceeds from exercise of stock options	6,390
Equity component of Convertible Notes	136,434
Stock-based compensation expense	20,205
Change in cumulative translation adjustment	23
Balance at September 30, 2018	$291,407

7. Stock-Based Compensation

The Company's current equity compensation plan, the 2017 Incentive Plan, was approved by shareholders at the Company's Annual Meeting of Shareholders on May 18, 2017. The 2017 Incentive Plan is administered by the Compensation Committee and the Board of Directors of the Company. Under the terms of the 2017 Incentive Plan, the Company is authorized to grant a variety of incentive awards based on its common stock, including stock options (both incentive stock options and non-qualified stock options), RSUs, performance options/shares and other stock awards, as well as pay incentive bonuses to eligible employees and non-employee directors. On May 18, 2017, upon the approval of the 2017 Incentive Plan by shareholders, 5,000,000 shares were authorized for issuance thereunder, plus any shares subject to then-outstanding awards under the 2015 Incentive Plan and the 2013 Incentive Plan that subsequently were canceled, terminated unearned, expired, were forfeited, lapsed for any reason or were settled in cash without the delivery of shares. As of September 30, 2018,

Table of Contents

3,408,995 shares remained for future issuance under the 2017 Incentive Plan. The 2017 Incentive Plan will terminate on April 3, 2027 unless it is extended or terminated earlier pursuant to its terms. In addition, from time to time, the Company makes inducement grants of stock options to new hires, which awards are made pursuant to the NASDAQ inducement grant exception. During the nine months ended September 30, 2018, the Company granted inducement stock options covering 236,730 shares of the Company's common stock to new employees.

On May 15, 2018, the 2018 Employee Stock Purchase Plan (2018 ESPP) was approved by shareholders at the Company's Annual Meeting of Shareholders. The Company has reserved the following for issuance under the 2018 ESPP: (i) 1,000,000 shares of common stock, plus (ii) commencing on January 1, 2019 and ending on December 31, 2023, an additional number of shares to be added on the first day of each calendar year equal to the lesser of (A) 1,200,000 shares of common stock, (B) 2% of the number of outstanding shares of common stock on such date and (C) an amount determined by the administrator.

Stock Options - The Company calculates the fair value of stock options granted using the Black-Scholes valuation model. The following table summarizes the Company's grant date fair value and assumptions used in determining the fair value of all stock options granted during the periods presented:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Volatility	66%-67%	72%-73%	66%-68%	72%-74%
Risk-free interest rate	2.75%-2.87%	1.73%-1.93%	2.25%-2.87%	1.73%-1.99%
Dividend yield	0.0%	0.0%	0.0%	0.0%
Expected option term (in years)	5.14	6.25	5.09	6.25
Weighted average fair value of stock options granted	$13.62	$9.59	$16.56	$10.18

For each period presented, the volatility factor was based on the Company's historical volatility during the expected option term. Estimated forfeitures are based on the actual percentage of option forfeitures since the closing of the Company's merger with Transave in December 2010. The expected option term for these grants was determined using the Company's historical exercise behavior of grantees.

From time to time, the Company has granted performance-condition options to certain of its employees. Vesting of these options was subject to the Company achieving certain performance criteria established at the date of grant and the grantees fulfilling a service condition (continued employment). During the quarter ended September 30, 2018, performance-condition options totaling $1.1 million, or 133,334 shares, met their recognition criteria as a result of the FDA approval of ARIKAYCE and vested in full. As of September 30, 2018, there were no performance-condition options outstanding.

The following table summarizes the Company's aggregate stock option activity for the nine months ended September 30, 2018:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life in Years	Aggregate Intrinsic Value (in thousands)
Options outstanding at December 31, 2017	8,608,921	$14.08		
Granted	1,610,010	$28.57		
Exercised	(427,729)	$14.94		
Forfeited or expired	(182,858)	$19.15		
Options outstanding at September 30, 2018	9,608,344	$16.37	7.08	$52,765

Vested and expected to vest at September 30, 2018	8,760,828	$15.90	6.92	$50,354
Exercisable at September 30, 2018	5,317,732	$13.23	5.96	$38,723

The total intrinsic value of stock options exercised during the three months ended September 30, 2018 and 2017 was $0.6 million and $1.4 million, respectively, and during the nine months ended September 30, 2018 and 2017 was $5.5 million and $3.5 million, respectively.

Table of Contents

As of September 30, 2018, there was $34.7 million of unrecognized compensation expense related to unvested stock options, which is expected to be recognized over a weighted average period of 2.5 years. The following table summarizes the range of exercise prices and the number of stock options outstanding and exercisable as of September 30, 2018:

Outstanding as of September 30, 2018					Exercisable as of September 30, 2018	
Range of Exercise Prices ($)		Number of Options	Weighted Average Remaining Contractual Term (in years)	Weighted Average Exercise Price ($)	Number of Options	Weighted Average Exercise Price ($)
$3.03	$6.90	1,087,941	3.94	$ 3.99	1,087,941	$ 3.99
$6.96	$10.85	997,221	7.53	$ 10.77	481,574	$ 10.68
$11.14	$12.58	984,936	5.49	$ 12.17	893,989	$ 12.20
$12.66	$13.67	984,161	7.97	$ 13.60	428,577	$ 13.56
$13.94	$16.07	1,139,533	6.80	$ 15.26	765,384	$ 15.17
$16.09	$17.16	1,438,502	7.95	$ 16.69	602,949	$ 16.48
$17.24	$22.76	1,366,680	6.56	$ 21.20	1,017,263	$ 21.26
$22.84	$30.46	1,439,910	9.21	$ 28.61	40,055	$ 24.26
$30.86	$31.78	155,960	9.25	$ 31.10	—	$ —
$32.46	$32.46	13,500	9.26	$ 32.46	—	$ —

Restricted Stock and Restricted Stock Units — Under the 2017 Incentive Plan, the Company may grant restricted stock (RS) and RSUs to eligible participants, including its executives, non-employee directors, and other service providers. Each share of RS vests, and each RSU represents a right to receive one share of the Company's common stock, upon the completion of a specific period of continued service or achievement of a certain milestone. RS and RSU awards are valued at the market price of the Company's common stock on the date of grant. The Company recognizes noncash compensation expense for the fair values of these RS and RSU awards on a straight-line basis over the requisite service period of these awards. As of September 30, 2018, there was $4.2 million of unrecognized compensation expense related to unvested RSU awards which is expected to be recognized over a weighted average period of 2.8 years. The following table summarizes the Company's RSU award activity during the nine months ended September 30, 2018:

	Number of RSUs	Weighted Average Grant Price ($)
Outstanding at December 31, 2017	46,914	$ 17.16
Granted	248,468	$ 29.41
Released	(47,478)	$ 17.32
Forfeited	(3,103)	$ 30.46
Outstanding at September 30, 2018	244,801	$ 29.40

The following table summarizes the aggregate stock-based compensation expense recorded in the consolidated statements of comprehensive loss related to stock options and RSUs during the three and nine months ended September 30, 2018 and 2017, respectively:

Three Months Ended September 30,	Nine Months Ended September 30,

	2018	2017	2018	2017
	(in millions)			
Research and development expenses	$2.8	$1.8	$7.4	$4.8
General and administrative expenses	5.1	2.9	12.8	8.5
Total	$7.9	$4.7	$20.2	$13.3

8. Income Taxes

Table of Contents

The Company's provision for income taxes was $46,000 and $134,000 for the three and nine months ended September 30, 2018, respectively, and $27,000 and $94,000 for the three and nine months ended September 30, 2017, respectively. The provision for income taxes in all periods was a result of certain of the Company's subsidiaries in Europe, which had taxable income during the three and nine months ended September 30, 2018 and 2017. In jurisdictions where the Company has net losses, there was a full valuation allowance recorded against the Company's deferred tax assets and therefore no tax benefit was recorded.

Following adoption of the Tax Cuts and Jobs Act during the fourth quarter of 2017, the Company remeasured certain deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21%. The Company recorded a provisional amount of $94.0 million as of December 31, 2017 related to the re-measurement of certain deferred tax balances, which was completely offset by a full valuation allowance. Upon further analyses, the Company determined in the second quarter of 2018 that the provisional amount would not need to be adjusted.

In addition to local taxes in foreign jurisdictions, the Company is subject to US federal, US state, and US tax on foreign earnings. In regard to the US tax on foreign earnings, the Company was subject to a one-time transition tax based on its total earnings and profits, which were generally deferred from US income taxes under previous US law. Due to the aggregate loss position of the Company's foreign subsidiaries, the Company did not record any provisional amount for the one-time transition tax liability at December 31, 2017. Additionally, the global intangible low-taxed income tax and base erosion provisions in the Tax Cuts and Jobs Act are effective for taxable years beginning after December 31, 2017. The Company does not currently expect these provisions to have a material impact (i) due to the aggregate loss position of its foreign subsidiaries and (ii) because the Company currently expects to be below the gross receipts threshold for purposes of the base erosion provisions.

The Company is subject to US federal and state income taxes and the statute of limitations for tax audit is open for the Company's federal tax returns for the years ended 2014 and later, and is generally open for certain states for the years 2013 and later. The Company has incurred net operating losses since inception, except for the year ended December 31, 2009. Such loss carryforwards would be subject to audit in any tax year in which those losses are utilized, notwithstanding the year of origin. As of September 30, 2018 and December 31, 2017, the Company had recorded no reserves for unrecognized income tax benefits, nor had it recorded any accrued interest or penalties related to uncertain tax positions. The Company does not anticipate any material changes in the amount of unrecognized tax positions over the next 12 months.

9. Commitments and Contingencies

The Company has an operating lease for office and laboratory space located in Bridgewater, NJ, its corporate headquarters, for which the initial lease term expires in November 2019. Future minimum rental payments under this lease are $1.2 million. In July 2016, the Company signed an operating lease for laboratory space located in Bridgewater, NJ for which the initial lease term expires in December 2021. Future minimum rental payments under this lease are $1.5 million.

In September 2018, the Company entered into a lease agreement for office space in Bridgewater, NJ for its future corporate headquarters. The lease provides for a commencement date of the earlier of (1) September 1, 2019, subject to completion of certain improvements by specified dates, and (2) the date on which the Company takes possession of the premises to commence its business operations therein (the Commencement Date). The initial lease term runs 130 months from the Commencement Date (plus any partial month from the Commencement Date until the first day of the next full calendar month during the term), and the Company has the option to extend that term for up to three additional five-year periods. Future minimum rental payments under this lease are $32.3 million.

Rent expense charged to operations was $0.4 million and $0.4 million for the three months ended September 30, 2018 and 2017, respectively, and $1.2 million and $1.1 million for the nine months ended September 30, 2018 and 2017, respectively. Future minimum rental payments required under the Company's leases for the period from October 1, 2018 to December 31, 2018 and for each of the five years thereafter are as follows (in thousands):

Table of Contents

Year Ending December 31:	
2018 (remaining)	$490
2019	3,863
2020	4,185
2021	3,958
2022	1,281
2023 and thereafter	23,261
	$37,038

Legal Proceedings

From time to time, the Company is a party to various lawsuits, claims and other legal proceedings that arise in the ordinary course of business. While the outcomes of these matters are uncertain, management does not expect that the ultimate costs to resolve these matters will have a material adverse effect on the Company's consolidated financial position, results of operations or cash flows.

ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Cautionary Note Regarding Forward Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. "Forward-looking statements," as that term is defined in the Private Securities Litigation Reform Act of 1995, are statements that are not historical facts and involve a number of risks and uncertainties. Words herein such as "may," "will," "should," "could," "would," "expects," "plans," "anticipates," "believes," "estimates," "projects," "predicts," "intends," "potential," "continues," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) identify forward-looking statements.

Forward-looking statements are based on our current expectations and beliefs, and involve known and unknown risks, uncertainties and other factors, which may cause our actual results, performance and achievements and the timing of certain events to differ materially from the results, performance, achievements or timing discussed, projected, anticipated or indicated in any forward-looking statements. Such risks, uncertainties and other factors include, among others, the following:

- *failure to successfully commercialize or maintain US approval for ARIKAYCE, our only approved product;*
- *uncertainties in the degree of market acceptance of ARIKAYCE by physicians, patients, third-party payers and others in the health-care community;*
- *our inability to obtain full approval of ARIKAYCE from the US Food and Drug Administration (FDA), including the risk that we will not successfully complete the confirmatory post-marketing study required for full approval;*
- *inability of us, PARI Pharma GmbH (PARI) or our third party manufacturers to comply with regulatory requirements related to ARIKAYCE or the Lamira Nebulizer System;*
- *our inability to obtain adequate reimbursement from government or third-party payers for ARIKAYCE or acceptable prices for ARIKAYCE;*
- *development of unexpected safety or efficacy concerns related to ARIKAYCE;*
- *inaccuracies in our estimates of the size of the potential markets for ARIKAYCE;*
- *our inability to create an effective direct sales and marketing infrastructure or to partner with third parties that offer such an infrastructure for distribution of ARIKAYCE;*
- *failure to obtain regulatory approval to expand ARIKAYCE's indication to a broader patient population;*
- *failure to successfully conduct future clinical trials for ARIKAYCE and our product candidates, including due to our limited experience in conducting preclinical development activities and clinical trials necessary for regulatory*

approval and our inability to enroll or retain sufficient patients to complete the trials or generate data necessary for regulatory approval;

- *risks that our clinical studies will be delayed or that serious side effects will be identified during drug development;*
- *failure to obtain regulatory approvals for ARIKAYCE outside the US or for our product candidates in the US, Europe, Japan or other markets;*

Table of Contents

- *failure of third parties on which we are dependent to manufacture sufficient quantities of ARIKAYCE or our product candidates for commercial or clinical needs, to conduct our clinical trials, or to comply with our agreements or laws and regulations that impact our business;*
- *our inability to attract and retain key personnel or to effectively manage our growth;*
- *our inability to adapt to our highly competitive and changing environment;*
- *our inability to adequately protect our intellectual property rights or prevent disclosure of our trade secrets and other proprietary information and costs associated with litigation or other proceedings related to such matters;*
- *restrictions imposed on us by material license agreements, including our license agreements with PARI and AstraZeneca AB (AstraZeneca), and failure to comply with our obligations under such agreements;*
- *the cost and potential reputational damage resulting from litigation to which we are or may become a party, including product liability claims;*
- *limited experience operating internationally;*
- *changes in laws and regulations applicable to our business and failure to comply with such laws and regulations; and*
- *inability to repay our existing indebtedness and uncertainties with respect to our ability to access future capital.*

We caution readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they are made. Any forward-looking statement is based on information current as of the date of this Quarterly Report on Form 10-Q and speaks only as of the date on which such statement is made. Actual events or results may differ materially from the results, plans, intentions or expectations anticipated in these forward-looking statements as a result of a variety of factors, many of which are beyond our control. More information on factors that could cause actual results to differ materially from those anticipated is included from time to time in our reports filed with the Securities and Exchange Commission (SEC), including, but not limited to, those described in the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the fiscal year ended December 31, 2017. We disclaim any obligation, except as specifically required by law, and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

The following discussion should be read in conjunction with our consolidated financial statements and related notes thereto included elsewhere in this Quarterly Report on Form 10-Q and the consolidated financial statements and related notes thereto in our Annual Report on Form 10-K for the year ended December 31, 2017.

OVERVIEW

We are a global biopharmaceutical company on a mission to transform the lives of patients with serious and rare diseases. Our first commercial product, ARIKAYCE (amikacin liposome inhalation suspension), received accelerated approval in the United States (US) on September 28, 2018 for the treatment of *Mycobacterium avium* complex (MAC) lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a rare and often chronic infection that can cause irreversible lung damage and can be fatal. Our clinical-stage pipeline includes INS1007 and INS1009. INS1007 is a novel oral, reversible inhibitor of dipeptidyl peptidase 1 (DPP1) with therapeutic potential in non-cystic fibrosis (non-CF) bronchiectasis and other inflammatory diseases. INS1009 is an inhaled formulation of a treprostinil prodrug that may offer a differentiated product profile for rare pulmonary disorders, including pulmonary arterial hypertension (PAH).
The table below summarizes the current status and anticipated milestones for ARIKAYCE and our product candidates INS1007 and INS1009.

Table of Contents

Principal Product/Product Candidate	Status	Next Expected Milestones
ARIKAYCE for MAC lung disease	•We are focused on having a successful commercial launch of ARIKAYCE in the US for appropriate patients. We began commercial shipments of ARIKAYCE in October 2018. • In September 2018, the FDA granted accelerated approval of ARIKAYCE for the treatment of MAC lung disease as part of a combination antibacterial drug regimen for adult patients who have limited or no alternative treatment options. • We announced interim data from the CONVERT study and the 312 extension study in January 2018, which we view as consistent with the six-month results of the CONVERT study. The data included interim long-term durability data for the CONVERT study and interim efficacy data for the 312 study. These studies are ongoing. • We announced top-line data for the CONVERT study in September 2017. The CONVERT study met its primary endpoint of culture conversion, which we defined as three consecutive negative monthly sputum cultures by month six with statistical and clinical significance, with 29% of patients in the ARIKAYCE plus current guideline-based therapy (GBT) arm achieving culture conversion, compared to 9% of patients in the GBT-only arm ($p<0.0001$). • The FDA has designated ARIKAYCE as an orphan drug and a qualified infectious disease product (QIDP) for nontuberculous mycobacterial (NTM) lung disease, and the European Commission has granted an orphan designation for ARIKAYCE for the treatment of NTM lung disease.	• We intend to seek regulatory approvals for ARIKAYCE outside the US, such as in Europe and Japan, when sufficient data are available. If approved, we expect ARIKAYCE would be the first inhaled therapy specifically indicated for the treatment of MAC lung disease in Europe and Japan • We intend to collaborate with the FDA on, and invest in, the post-approval confirmatory clinical trial required by the FDA to support full approval and lifecycle management programs. • If approved, we plan to commercialize ARIKAYCE in certain countries in Europe, Japan, and certain other countries.
INS1007 (oral reversible inhibitor of DPP1) for non-CF bronchiectasis and other rare diseases	• We are enrolling patients in the WILLOW study, a global phase 2, randomized, double-blind, placebo-controlled, parallel-group, multi-center clinical study to assess the efficacy, safety and tolerability, and pharmacokinetics of INS1007 administered once daily for 24 weeks in subjects with non-CF bronchiectasis. • We are currently assessing regulatory strategies which could expedite the development and regulatory reviews of INS1007 in the US and the European Union (EU).	• We expect to continue to advance enrollment in the WILLOW clinical study of INS1007 during 2018. • We are exploring the potential of INS1007 in various neutrophil-driven inflammatory conditions.
INS1009 (inhaled formulation of a treprostinil prodrug) for rare pulmonary disorders	• The results of our phase 1 study of INS1009 were presented at the European Respiratory Society international congress in September 2016.	• We believe INS1009 may offer a differentiated product profile for rare pulmonary disorders, including PAH, and we are currently evaluating our options to advance its development including exploring its use as an inhaled dry powder formulation.

Our earlier-stage pipeline includes preclinical compounds that we are evaluating in multiple rare diseases of unmet medical need, including gram positive pulmonary infections and NTM lung disease. To complement our internal research and development, we actively evaluate in-licensing and acquisition opportunities for a broad range of rare diseases.

Table of Contents

Our Strategy

Our strategy focuses on the needs of patients with rare diseases. We are currently primarily focused on the US commercial launch of ARIKAYCE. We are not aware of any other approved inhaled therapies specifically indicated to treat MAC lung disease in North America, Europe, or Japan. We secured US regulatory approval of ARIKAYCE for the treatment of MAC lung disease in patients with limited or no alternative treatment options. We also believe that ARIKAYCE has the potential to treat a number of different bacterial infections. We are also advancing earlier-stage programs in other rare pulmonary disorders.

Our current priorities are as follows:

- Launching ARIKAYCE in the US for appropriate patients;
- Establishing patient access and providing appropriate support for patients being prescribed ARIKAYCE;
- Ensuring our product supply chain will support the commercialization and potential future lifecycle management programs of ARIKAYCE;
- Designing and conducting the required confirmatory clinical trial to support full US approval of ARIKAYCE;
- Developing the core value dossier to support the reimbursement for ARIKAYCE in the US, Europe and Japan;
- Obtaining determinations of coverage and reimbursement in the US for ARIKAYCE from governmental and other third-party payers;
- Supporting further research and lifecycle management strategies for ARIKAYCE, including exploring the potential use of ARIKAYCE as part of a front-line, multi-drug regimen and as maintenance monotherapy to prevent recurrence (defined as true relapse or reinfection) of MAC lung disease;
- Continuing expansion efforts in certain countries in Europe and Japan to prepare for regulatory filings for ARIKAYCE;
- Enrolling patients in the WILLOW phase 2 study of INS1007 in non-CF bronchiectasis;
- Exploring INS1009 for use as an inhaled dry powder formulation and generating preclinical findings from our earlier-stage programs; and
- Expanding our rare disease pipeline through corporate development.

ARIKAYCE for Patients with MAC Lung Disease

ARIKAYCE (amikacin liposome inhalation suspension) is our first approved product. ARIKAYCE received accelerated approval in the US on September 28, 2018 for the treatment of MAC lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options. MAC lung disease is a rare and often chronic infection that can cause irreversible lung damage and can be fatal. Amikacin solution for parenteral administration is an established drug that has activity against a variety of NTM; however, its use is limited by the need to administer it intravenously and by toxicity to hearing, balance, and kidney function (Peloquin et al., 2004). Unlike amikacin solution for intravenous administration, our advanced liposome technology uses charge-neutral liposomes to deliver amikacin directly to the lungs where it is taken up by the lung macrophages where the MAC infection resides. This technology prolongs the release of amikacin in the lungs, while minimizing systemic exposure, thereby offering the potential for decreased systemic toxicities. ARIKAYCE's ability to deliver high levels of amikacin directly to the lung distinguishes it from intravenous amikacin. ARIKAYCE is administered once-daily, using the Lamira Nebulizer System, an inhalation device developed and manufactured by PARI. Lamira is a portable nebulizer that enables aerosolization of liquid medications, including liposomal formulations such as ARIKAYCE, via a vibrating, perforated membrane.

The FDA has designated ARIKAYCE as an orphan drug and a QIDP for NTM lung disease. Orphan designated drugs are eligible for seven years of exclusivity for the orphan indication. QIDP designation features an additional five years

of exclusivity for the designated indication. The FDA granted a total of 12 years exclusivity in the indication for which ARIKAYCE was approved.

Accelerated Approval

Table of Contents

In March 2018, we submitted a new drug application for ARIKAYCE to the FDA pursuant to Section 506(c) of the Federal Food Drug and Cosmetic Act and 21 C.F.R. Part 314 Subpart H (Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses) (Subpart H). Accelerated approval under Subpart H allows drugs that (i) are being developed to treat a serious or life-threatening disease or condition and (ii) provide a meaningful therapeutic benefit over existing treatments to be approved substantially based on an intermediate endpoint or a surrogate endpoint that is reasonably likely to predict clinical benefit, rather than a clinical endpoint such as survival or irreversible morbidity. The FDA granted our request for a priority review and set a PDUFA action date of September 28, 2018. On August 7, 2018, the FDA advisory committee voted 12-2 in favor of the safety and effectiveness of ARIKAYCE for adults with MAC lung disease who have limited or no treatment options. The committee also voted in favor of the surrogate endpoint of sputum culture conversion used in the Phase 3 CONVERT study being reasonably likely to predict clinical benefit. In a separate vote, the committee voted against the safety and effectiveness of ARIKAYCE in the broadest population of adult patients with MAC lung disease. On September 28, 2018, the FDA granted approval for ARIKAYCE under the Limited Population Pathway for Antibacterial and Antifungal Drugs (LPAD) for the treatment of MAC lung disease as part of a combination antibacterial drug regimen for adult patients with limited or no alternative treatment options and the accelerated approval pathway. LPAD, which was enacted as part of the 21st Century Cures Act, serves to advance the development of new antibacterial drugs to treat serious or life-threatening infections in limited populations of patients with unmet needs. As required for drugs approved under the LPAD pathway, labeling for ARIKAYCE includes certain statements to convey that the drug has been shown to be safe and effective only for use in a limited population. Approval under the LPAD pathway may be supported by a streamlined clinical development program.

As a condition of accelerated approval, we must conduct a post-approval confirmatory clinical trial. The required confirmatory trial, which is currently under discussion with FDA, is proposed to be a randomized, double-blind, placebo-controlled clinical trial to assess and describe the clinical benefit of ARIKAYCE in patients with MAC lung disease. The trial will evaluate the effect of ARIKAYCE on a clinically meaningful endpoint, as compared to an appropriate control, in the intended patient population of patients with MAC lung disease. Pursuant to the timetable agreed upon with the FDA, the study protocol is scheduled to be finalized in 2019, with trial results to be reported by 2024. Continued approval of ARIKAYCE will be contingent upon verification and description of clinical benefit in this study.

Further Research and Lifecycle Management for ARIKAYCE

Along with the post-approval confirmatory clinical study described above, we are currently exploring and supporting research and lifecycle management programs for ARIKAYCE beyond treatment of MAC lung disease as part of a combination antibacterial regimen for adult patients who have limited or no treatment options. Specifically, we are evaluating future study designs focusing on the MAC lung disease treatment pathway, including front-line treatment and monotherapy maintenance to prevent recurrence (defined as true relapse or reinfection) of MAC lung disease.

If the data from the CONVERT study are sufficient to support our marketing authorization applications (MAAs) in Europe and Japan and those regulatory bodies approve ARIKAYCE, lifecycle management studies could potentially enable us to reach more patients worldwide. In addition, we are evaluating the use of ARIKAYCE to treat infections caused by non-MAC NTM species, such as *M. abscessus*. These initiatives include investigator-initiated studies, which are clinical studies initiated and sponsored by physicians or research institutions with funding from us, and may also include new clinical studies sponsored by us.

Market Opportunity for ARIKAYCE in MAC Lung Disease in 2018

NTM lung disease is associated with increased rates of morbidity and mortality, and MAC is the predominant pathogenic species in NTM lung disease in the US, Europe and Japan. The prevalence of NTM lung disease has

increased over the past two decades, and we believe it is an emerging public health concern worldwide. Based on currently available information from external sources, including market research funded by us and third parties, and internal analyses and calculations, we estimate potential patient populations in the US, Japan and the EU5 (comprised of France, Germany, Italy, Spain and the United Kingdom) for 2018 as follows:

Table of Contents

Potential Market	Estimated Number of Patients with Diagnosed NTM Lung Disease	Estimated Number of Patients Treated for MAC Lung Disease	Estimated Number of MAC lung disease Patients Refractory to Treatment**
United States	75,000-105,000	40,000-50,000	10,000-15,000
Japan	125,000-145,000	60,000-70,000	15,000-18,000
EU5	14,000	4,400	1,400

*** ARIKAYCE received accelerated approval for this population in the US in September 2018.*

We are not aware of any other approved inhaled therapies specifically indicated for NTM lung disease in North America, Europe or Japan. Current guideline-based approaches for NTM lung disease, including those from the American Thoracic Society and Infectious Diseases Society of America, involve multi-drug regimens not approved for the treatment of NTM lung disease and treatment that could last two years or more. Based on a burden of illness study that we conducted in the US with a major medical benefits provider, we previously concluded that patients with NTM lung disease are costly to healthcare plans, while a recent claims-based study in the US has shown that patients with NTM lung disease have higher resource utilization and costs than their age and gender-matched controls. Accordingly, we believe that a significant market opportunity for ARIKAYCE in NTM lung disease exists in the US and internationally.

We are currently exploring the MAC lung disease market opportunity for ARIKAYCE in Japan. The CONVERT study included a comprehensive pharmacokinetic sub-study in Japanese subjects in lieu of a separate local pharmacokinetic study in Japan, as agreed with the PDMA. If the data from the CONVERT study are sufficient to support our MAAs, we expect to submit regulatory filings in Europe and Japan. We established a Japanese subsidiary and, in 2018, began hiring local employees, including a general manager, to closely manage our regulatory and pre-commercial activities.

Product Pipeline

INS1007

INS1007 is a small molecule, oral, reversible inhibitor of DPP1, which we licensed from AstraZeneca in October 2016. DPP1 is an enzyme responsible for activating neutrophil serine proteases in neutrophils when they are formed in the bone marrow. Neutrophils are the most common type of white blood cell and play an essential role in pathogen destruction and inflammatory mediation. Neutrophils contain the neutrophil serine proteases (including neutrophil elastase, proteinase 3, and cathepsin G) that have been implicated in a variety of inflammatory diseases. In chronic inflammatory lung diseases, neutrophils accumulate in the airways and release active neutrophil serine proteases in excess that cause lung destruction and inflammation. INS1007 may decrease the damaging effects of inflammatory diseases, such as non-CF bronchiectasis, by inhibiting DPP1 and its activation of neutrophil serine proteases. Non-CF bronchiectasis is a progressive pulmonary disorder in which the bronchi become permanently dilated due to chronic inflammation and infection. Currently, there is no cure, and we are not aware of any FDA-approved therapies specifically indicated for non-CF bronchiectasis.

The WILLOW Study

The WILLOW study is a global phase 2, randomized, double-blind, placebo-controlled, parallel group, multi-center clinical study to assess the efficacy, safety and tolerability, and pharmacokinetics of INS1007 administered once daily for 24 weeks in subjects with non-CF bronchiectasis. We commenced enrollment in the WILLOW study in December 2017. In addition, we are exploring the potential of INS1007 in various neutrophil-driven inflammatory conditions.

INS1009

INS1009 is an investigational sustained-release inhaled treprostinil prodrug formulation that has the potential to address certain of the current limitations of existing prostanoid therapies. We believe that INS1009 prolongs duration of effect and may provide PAH patients with greater consistency in pulmonary arterial pressure reduction over time. Current inhaled prostanoid therapies must be dosed four to nine times per day for the treatment of PAH. Reducing dose frequency has the potential to ease patient burden and improve compliance. Additionally, we believe that INS1009 may be associated with fewer side effects, including elevated heart rate, low blood pressure, and severity and/or frequency of cough, associated with high initial drug levels and local upper airway exposure when using current inhaled prostanoid therapies. We believe INS1009 may offer a differentiated product profile for rare pulmonary disorders, including PAH, and we are currently evaluating our options to advance its development, including exploring its use as an inhaled dry powder formulation.

Table of Contents

KEY COMPONENTS OF OUR STATEMENT OF OPERATIONS

Research and Development (R&D) Expenses

R&D expenses consist of salaries, benefits and other related costs, including stock-based compensation, for personnel serving in our research and development functions, including medical affairs. Expenses also include other internal operating expenses, the cost of manufacturing ARIKAYCE and our product candidates for clinical study, the cost of conducting clinical studies, and the cost of conducting preclinical and research activities. In addition, our R&D expenses include payments to third parties for the license rights to products or components thereof in development prior to regulatory approval, such as the Lamira Nebulizer System and INS1007. Our expenses related to manufacturing ARIKAYCE and our product candidates for clinical studies and commercial inventory, if any, prior to regulatory approvals are primarily related to activities at contract manufacturing organizations (CMOs) that manufacture ARIKAYCE and our product candidates for our use, including purchases of active pharmaceutical ingredients. R&D expenses also include spending to build-out the CMO facilities to support commercialization of ARIKAYCE in the US and potential future global production requirements. Our expenses related to clinical trials are primarily related to activities at CROs that conduct and manage clinical trials on our behalf.

Since 2011, we have focused our development activities principally on our proprietary, advanced liposomal technology designed specifically for inhaled therapies. Our development efforts since 2015 have principally related to the development of ARIKAYCE in NTM lung disease.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, benefits and other related costs, including stock-based compensation, for our non-employee directors and personnel serving in our executive, finance and accounting, legal and compliance, pre-commercial, corporate development, information technology, program management and human resource functions. General and administrative expenses also include professional fees for legal services and patent-related expenses, consulting services including for pre-commercial planning activities such as non-branded disease awareness, insurance, board of director fees, tax and accounting services.

Investment Income and Interest Expense

Investment income consists of interest income earned on our cash and cash equivalents. Interest expense consists primarily of contractual interest expense, amortization of debt issuance costs and accretion of debt discount. Debt issuance costs are amortized, and the debt discount is accreted to interest expense using the effective interest rate method over the term of the debt. Unamortized debt issuance costs associated with extinguished debt are expensed in the period of the extinguishment.

RESULTS OF OPERATIONS

Comparison of the Three Months Ended September 30, 2018 and 2017

Net Loss

Net loss for the quarter ended September 30, 2018 was $87.7 million, or $1.14 per share—basic and diluted, compared with a net loss of $45.2 million, or $0.69 per share—basic and diluted, for the quarter ended September 30, 2017. The $42.6 million increase in our net loss for the quarter ended September 30, 2018 as compared to the same period in 2017 was primarily due to:

- Increased R&D expenses of $12.9 million, primarily resulting from an increase in external manufacturing expenses and higher compensation and related expenses due to an increase in headcount; and
- Increased general and administrative expenses of $27.0 million, resulting from higher compensation and related expenses due to an increase in headcount, and an increase in consulting fees relating to pre-commercial planning

activities in preparation for the launch of ARIKAYCE.

In addition, there was a $5.2 million increase in interest expense resulting from the issuance of $450.0 million aggregate principal amount of 1.75% convertible senior notes due 2025 (the Convertible Notes) in January 2018.

R&D Expenses

Table of Contents

R&D expenses for the quarters ended September 30, 2018 and 2017 were comprised of the following components (in thousands):

	Quarters Ended September 30,		Increase (decrease)	
	2018	2017	$	%
External Expenses				
Clinical development & research	$ 8,047	$ 12,307	$(4,260)	(34.6)%
Manufacturing	13,130	2,884	10,246	355.3 %
Regulatory and quality assurance	2,969	1,370	1,599	116.7 %
Subtotal—external expenses	$ 24,146	$ 16,561	$7,585	45.8 %
Internal Expenses				
Compensation and related expenses	$ 12,863	$ 8,373	$4,490	53.6 %
Other internal operating expenses	2,529	1,741	788	45.3 %
Subtotal—internal expenses	$ 15,392	$ 10,114	$5,278	52.2 %
Total	$ 39,538	$ 26,675	$12,863	48.2 %

R&D expenses increased to $39.5 million during the quarter ended September 30, 2018 from $26.7 million in the same period in 2017. The $12.9 million increase was primarily due to an increase of $10.2 million in external manufacturing expenses, specifically related to purchases of ARIKAYCE raw materials, CMO expenses related to ARIKAYCE commercial inventory production, and construction costs relating to the build-out of a third party CMO production facility. In addition, there was a $4.5 million increase in compensation and related expenses due to an increase in headcount in the quarter ended September 30, 2018 as compared to the prior year period.

General and Administrative Expenses

General and administrative expenses for the quarters ended September 30, 2018 and 2017 were comprised of the following (in thousands):

	Quarters Ended September 30,		Increase (decrease)	
	2018	2017	$	%
General & administrative	$ 17,290	$ 9,355	$7,935	84.8 %
Pre-commercial expenses	27,155	8,053	19,102	237.2%
Total general & administrative expenses	$ 44,445	$ 17,408	$27,037	155.3%

General and administrative expenses increased to $44.4 million during the quarter ended September 30, 2018 from $17.4 million in the same period in 2017. The $27.0 million increase was primarily due to $11.7 million in higher compensation and related expenses due to an increase in headcount, including the hiring of our field force, and $13.2 million in consulting fees relating to pre-commercial planning activities in preparation for the launch of ARIKAYCE, including non-branded disease awareness, patient support planning, field operations and other professional fees.

Interest Expense

Interest expense was $6.7 million for the quarter ended September 30, 2018 as compared to $1.5 million in the same period in 2017. The $5.2 million increase in interest expense in the quarter ended September 30, 2018 as compared to the prior year period relates to the issuance of $450.0 million aggregate principal amount of the Convertible Notes in January 2018. The interest expense on the Convertible Notes is based on an effective interest rate of 7.6%.

Comparison of the Nine Months Ended September 30, 2018 and 2017

Net Loss

Net loss for the nine months ended September 30, 2018 was $232.7 million, or $3.03 per share—basic and diluted, compared with a net loss of $127.3 million, or $2.01 per share—basic and diluted, for the nine months ended September 30,

Table of Contents

2017. The $105.4 million increase in our net loss for the quarter ended September 30, 2018 as compared to the same period in 2017 was primarily due to:

- Increased R&D expenses of $29.6 million, primarily resulting from an increase in external manufacturing expenses and higher compensation and related expenses due to an increase in headcount; and
- Increased general and administrative expenses of $66.5 million, resulting from higher compensation and related expenses due to an increase in headcount, and an increase in consulting fees relating to pre-commercial planning activities in the preparation for the launch of ARIKAYCE.

In addition, there was a $14.3 million increase in interest expense resulting from the issuance of $450.0 million aggregate principal amount of the Convertible Notes in January 2018.

R&D Expenses

R&D expenses for the nine months ended September 30, 2018 and 2017 were comprised of the following components (in thousands):

	Nine Months Ended September 30,		Increase (decrease)	
	2018	2017	$	%
External Expenses				
Clinical development & research	$ 22,785	$		