INSMED Inc
Form 10-Q
August 06, 2014
Table of Contents

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 June 30, 2014

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)

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post 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, or a small reporting Company (See the definitions of large accelerated filer, accelerated filer, and small reporting Company in Rule 12b-2 of the Exchange Act).

Large accelerated filer o Accelerated filer x Non-accelerated filer o Small Reporting Company 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 July 31, 2014, there were 39,283,016 shares of the registrant s common stock, $0.01 par value, outstanding.

Table of Contents

INSMED INCORPORATED

FORM 10-Q

FOR THE QUARTER ENDED JUNE 30, 2014

INDEX

In this Form 10-Q, we use the words Insmed Incorporated to refer to Insmed Incorporated, a Virginia corporation, and we use the words Company, Insmed, Insmed Incorporated, we, us and our to refer to Insmed Incorporated and its consolidated subsidiaries. ARIKACE® and IPLEX® are registered trademarks of Insmed Incorporated. ARIKAYCETM and INSMEDTM 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 June 30, 2014 (unaudited)	As of December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 82,697	$ 113,894
Prepaid expenses and other current assets	4,015	2,269
Total current assets	86,712	116,163
In-process research and development	58,200	58,200
Fixed assets, net	4,778	1,812
Other assets	420	323
Total assets	$ 150,110	$ 176,498
Liabilities and shareholders equity		
Current liabilities:		
Accounts payable	$ 11,407	$ 5,929
Accrued expenses	4,546	3,905
Accrued compensation	2,071	2,839
Accrued lease expense, current	314	307
Deferred rent	167	129
Capital lease obligations, current	32	64
Current portion of long term debt	3,281	3,283
Total current liabilities	21,818	16,456
Accrued lease expense, long-term	250	380
Debt, long-term	16,494	16,338
Total liabilities	38,562	33,174
Shareholders equity:		
Common stock, $0.01 par value; 500,000,000 authorized shares, 39,276,389 and 39,137,679 issued and outstanding shares at June 30, 2014 and December 31, 2013, respectively	393	391
Additional paid-in capital	540,298	534,554
Accumulated deficit	(429,143)	(391,621)
Total shareholders equity	111,548	143,324
Total liabilities and shareholders equity	$ 150,110	$ 176,498

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 June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Other revenue	$	$ 11,500	$	$ 11,500
Operating expenses:				
Research and development	14,942	12,225	26,293	22,559
General and administrative	7,874	7,544	14,602	11,520
Total operating expenses	22,816	19,769	40,895	34,079
Operating loss	(22,816)	(8,269)	(40,895)	(22,579)
Investment income	12	50	29	101
Interest expense	(595)	(635)	(1,201)	(1,277)
Other income, net	175		156	2
Loss before income taxes	(23,224)	(8,854)	(41,911)	(23,753)
Benefit from income taxes			(4,389)	(1,221)
Net loss and comprehensive loss	$ (23,224)	$ (8,854)	$ (37,522)	$ (22,532)
Basic and diluted net loss per share	$ (0.59)	$ (0.28)	$ (0.96)	$ (0.71)
Weighted average basic and diluted common shares outstanding	39,273	31,754	39,256	31,654

See accompanying notes to consolidated financial statements

Table of Contents

INSMED INCORPORATED

Consolidated Statements of Cash Flows (Unaudited)

(in thousands)

	Six months ended June 30,	
	2014	**2013**
Operating activities		
Net loss	$ (37,522)	$ (22,532)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	342	304
Stock based compensation expense	5,336	4,953
Gain on sale of asset, net		(2)
Amortization of debt discount and debt issuance costs	192	236
Accrual of the end of term charge on the debt	62	97
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,843)	(1,186)
Accounts payable	3,642	805
Accrued expenses and deferred rent	103	1,449
Accrued lease expenses	(123)	(127)
Accrued compensation	(768)	(318)
Net cash used in operating activities	(30,579)	(16,321)
Investing activities		
Purchases of fixed assets	(895)	(493)
Proceeds from sale of asset		2
Net cash used in investing activities	(895)	(491)
Financing activities		
Payments on capital lease obligations	(32)	(59)
Proceeds from exercise of stock options	409	677
Payment of debt issuance costs	(100)	
Net cash provided by financing activities	277	618
Decrease in cash and cash equivalents	(31,197)	(16,194)
Cash and cash equivalents at beginning of period	113,894	90,782
Cash and cash equivalents at end of period	$ 82,697	$ 74,588
Supplemental disclosures of cash flow information:		
Cash paid for interest	$ 930	$ 944
Cash received for taxes	$ 4,389	$ 1,221
Supplemental disclosures of non-cash investing and financing activities:		
Value of warrant exercised by converting the warrant into shares of common stock (net issuance method)	$	$ 790

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 biopharmaceutical company currently focused on developing and commercializing inhaled therapies for patients battling serious lung diseases that are often life threatening. The Company s lead product candidate, ARIKAYCETM, or liposomal amikacin for inhalation, is an inhaled antibiotic treatment that delivers a proven and potent anti-infective directly to the site of serious lung infections. The Company was incorporated in the Commonwealth of Virginia on November 29, 1999. On December 1, 2010, the Company completed a business combination with Transave, Inc. (Transave), a privately held, New Jersey-based pharmaceutical company focused on the development of differentiated and innovative inhaled pharmaceuticals for the treatment of serious lung infections (the Merger). The Company s continuing operations are based on the technology and products historically developed by Transave. The Company s principal executive offices are located in Bridgewater, New Jersey.

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 United States for complete consolidated financial statements have been condensed or are not included herein. The interim statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in the Company s Form 10-K for the year ended December 31, 2013.

The results of operations of any interim period are not necessarily indicative of the results of operations for the full year. The unaudited interim condensed 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 is responsible for the unaudited interim consolidated financial statements included in this report.

The consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, Transave, LLC, Insmed Pharmaceuticals, Incorporated, Insmed Limited, and Celtrix Pharmaceuticals, Incorporated. All intercompany transactions and balances have been eliminated in consolidation.

2. Summary of Significant Accounting Policies

The following are interim updates to certain of the policies described in Note 2 to the Company s audited consolidated financial statements in the Company s Annual Report on Form 10-K for the year ended December 31, 2013:

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 judgments 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:

- 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.

Table of Contents

- 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 are categorized in the tables below 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 following table presents assets and liabilities measured at fair value as of June 30, 2014 and December 31, 2013 (in thousands):

			Fair Value Measurements at Reporting Date Using		
		Total	Quoted Prices in Active Markets for Identical Assets (Level 1)	Quoted Prices in Inactive Markets for Identical Assets (Level 2)	Significant Unobservable Inputs (Level 3)
As of June 30, 2014:					
Assets:					
Cash and cash equivalents	$	82,697	$ 82,697	$	$
	$	82,697	$ 82,697	$	$
As of December 31, 2013:					
Assets:					
Cash and cash equivalents	$	113,894	$ 113,894	$	$
	$	113,894	$ 113,894	$	$

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 a maturity of three months or less from the date of purchase.

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 three and six months ended June 30, 2014 and 2013, respectively.

As of June 30, 2014 and December 31, 2013, 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.

Revenue Recognition Other revenue during the three and six months ended June 30, 2013 solely consists of an $11.5 million payment the Company received from Premacure (now Shire plc) in exchange for the Company s right to receive future royalties under its license agreement with Premacure. The Company recorded this as Other revenue during the three months ended June 30, 2013, since all four revenue recognition

criteria were met at that time and the Company had no continuing performance obligations related to the payment received. Also see Note 11 of the Company s audited consolidated financial statements on Form 10-K for the year ended December 31, 2013 for more information.

Table of Contents

Net Loss Per Common Share - Basic net loss per common share is computed by dividing net loss by the weighted average number of common shares outstanding during the period. Diluted net loss per common 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 and warrants to purchase common stock would be antidilutive as the Company incurred a net loss. Potentially dilutive common shares resulting from the assumed exercise of outstanding stock options and warrants are determined based on the treasury stock method.

The following table sets forth the reconciliation of the weighted average number of shares used to compute basic and diluted net loss per share for the three and six months ended June 30, 2014 and 2013:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(In thousands, except per share amounts)			
Numerator:				
Net loss	$ (23,224)	$ (8,854)	$ (37,522)	$ (22,532)
Denominator:				
Weighted average common shares used in calculation of basic net loss per share:	39,273	31,754	39,256	31,654
Effect of dilutive securities:				
Common stock options				
Restricted stock and restricted stock units				
Common stock warrant				
Weighted average common shares outstanding used in calculation of diluted net loss per share	39,273	31,754	39,256	31,654
Net loss per share:				
Basic and Diluted	$ (0.59)	$ (0.28)	$ (0.96)	$ (0.71)

The following potentially dilutive securities have been excluded from the computations of diluted weighted-average common shares outstanding as of June 30, 2014 and 2013 as their effect would have been anti-dilutive (in thousands):

	2014	2013
Stock options to purchase common stock	5,058	3,275
Restricted stock and restricted stock units	21	111

3. Identifiable Intangible Assets and Goodwill

The Company believes there are no indicators of impairment relating to its in-process research and development intangible assets as of June 30, 2014.

Table of Contents

4. Accrued Expenses

Accrued expenses consist of the following:

	As of June 30, 2014	As of December 31, 2013
	(in thousands)	
Accrued clinical trial expenses	$ 2,882	$ 2,484
Accrued technical operation expenses	516	1,220
Accrued professional fees	669	24
Accrued interest payable	155	159
Other accrued expenses	324	18
	$ 4,546	$ 3,905

5. Debt

On June 29, 2012, the Company and its domestic subsidiaries, as co-borrowers, entered into a Loan and Security Agreement with Hercules Technology Growth Capital, Inc. (Hercules) that allowed the Company to borrow up $20.0 million in $10.0 million increments (Loan Agreement). The Company borrowed the first and second $10.0 million increments by signing two Secured Promissory Notes (Note A and Note B and collectively, the Notes) on June 29, 2012 and December 27, 2012, respectively. Notes A and B bear interest at 9.25%. Note A was originally scheduled to be repaid over a 42-month period with the first twelve monthly payments representing interest only followed by thirty monthly equal payments of principal and interest. Note B was originally scheduled to be repaid over a 36-month period with the first six monthly payments representing interest only followed by thirty monthly equal payments of principal and interest. The Loan Agreement provided that in certain circumstances the Company could delay the first principal payment by five months. In July 2013, subsequent to the completion of certain ARIKAYCE-related development milestones, the Company elected to extend the interest only period under the Notes from July 31, 2013 to December 31, 2013 and delay the first monthly principal repayments for Notes A and B from August 1, 2013 to January 1, 2014. On November 25, 2013, the Company and Hercules entered into an amendment (the Amendment) of the Loan Agreement. The Amendment initially extended the interest-only period through June 30, 2014 and called for the first monthly principal payment on July 1, 2014. The Amendment also allowed the Company to further extend the interest-only period through December 31, 2014 and delay the first payment of principal until January 1, 2015, so long as the Company paid a $100,000 fee and obtained positive data from its phase 2 clinical trial of ARIKAYCE in patients who have lung infections caused by nontuberculous mycobacteria (NTM). In June 2014, the Company paid the $100,000 fee and exercised its option to extend the interest-only period and delay the first payment of principal to January 1, 2015. The election and amendment did not change the maturity date for Notes A and B, which is January 1, 2016.

In connection with the Loan Agreement, the Company granted the lender a first position lien on all of the Company s assets, excluding intellectual property. Prepayment of the loans made pursuant to the Loan Agreement is subject to penalty and the Company is required to pay an end of term charge of $390,000, which is being charged to interest expense (and accreted to the debt) using the effective interest method over the life of the Loan Agreement. Debt issuance fees paid to the lender were recorded as a discount on the debt and are being amortized to interest expense using the effective interest method over the life of the Loan Agreement. Debt issuance fees paid to third parties were capitalized and are being amortized to interest expense using the effective interest method over the life of the Loan Agreement.

The Loan Agreement also contains representations and warranties by the Company and the lender and indemnification provisions in favor of the lender and customary covenants (including limitations on other indebtedness, liens, acquisitions, investments and dividends, but no financial covenants), and events of default (including payment defaults, breaches of covenants following any applicable cure period, a material impairment in

Table of Contents

the perfection or priority of the lender s security interest or in the collateral, and events relating to bankruptcy or insolvency). Upon the occurrence of an event of default, a default interest rate of an additional 5% may be applied to the outstanding loan balances, and the lender may terminate its lending commitment, declare all outstanding obligations immediately due and payable, and take such other actions as set forth in the Loan Agreement. In addition, pursuant to the Loan Agreement, the lender has the right to participate, in an amount of up to $1.0 million, in certain future private equity financing(s) by the Company.

In conjunction with entering into the Loan Agreement, the Company granted a warrant to the lender to purchase shares of the Company s common stock. Since the warrant was granted in conjunction with entering into the Loan Agreement, the relative fair value of the warrant was recorded as equity and debt discount. The debt discount is being amortized to interest expense over the term of the related debt using the effective interest method.

The following table presents the components of the Company s debt balance as of June 30, 2014 (in thousands):

Debt:		
Notes payable	$	20,000
Accretion of end of term charge		266
Issuance fees paid to lender		(247)
Discount from warrant		(244)
Current portion of long-term debt		(3,281)
Long-term debt	$	16,494

As of June 30, 2014, future principal repayments of the two Notes for the period July 1, 2014 to December 31, 2014 and in each of the years ending December 31, 2015 and 2016 were as follows (in thousands):

Year Ending in December 31:		
2014	$	
2015		7,430
2016		12,570
	$	20,000

The estimated fair value of the debt (categorized as a Level 2 liability for fair value measurement purposes) is determined using current market factors and the ability of the Company to obtain debt at comparable terms to those that are currently in place. The Company believes the estimated fair value at June 30, 2014 approximates the carrying amount.

6. Stockholders Equity

Common Stock As of June 30, 2014, the Company had 500,000,000 shares of common stock authorized with a par value of $0.01 and 39,276,389 shares of common stock issued and outstanding. In addition, as of June 30, 2014, the Company had reserved 5,058,459 shares of common stock for issuance upon the exercise of outstanding common stock options and 20,960 for issuance upon the vesting of restricted stock

units.

On July 22, 2013, the Company completed an underwritten public offering of 6,900,000 shares of the Company s common stock, which included the underwriter s exercise in full of its over-allotment option of 900,000 shares, at a price to the public of $10.40 per share. The Company s net proceeds from the sale of the shares, after deducting the underwriter s discount and offering expenses of $4.7 million, were $67.0 million.

Warrant - In conjunction with entering into the Loan Agreement (See Note 5 Debt), the Company granted a warrant to the lender to purchase 329,932 shares of the Company s common stock at an exercise price of

Table of Contents

$2.94 per share. The fair value of the warrant of $0.8 million was calculated using the Black-Scholes warrant-pricing methodology at the date of issuance and was recorded as equity and as a discount to the debt and is being amortized to interest expense over the term of the related debt using the effective interest method. On April 30, 2013, the lender exercised the warrant in full via the net issuance method specified in the warrant agreement. In accordance with such provisions, the Company issued and delivered 223,431 shares of common shares to the lender on May 1, 2013. As a result of the exercise, the warrant is no longer outstanding and there are no additional shares issuable under this instrument.

7. Stock-Based Compensation

The Company currently has one equity compensation plan, the 2013 Incentive Plan, which was approved by shareholders at the Company s Annual Meeting of Shareholders on May 23, 2013 (the 2013 Incentive Plan). The 2013 Incentive Plan is administered by the Compensation Committee and the Board of Directors of the Company. Under the terms of the 2013 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), performance shares and other stock awards, as well as the payment of incentive bonuses to all employees and non-employee directors. As of June 30, 2014, 323,222 shares of the Company s common stock were reserved for future grants (or issuances) of restricted stock, restricted stock units, stock options, and stock warrants under the 2013 Incentive Plan. The 2013 Incentive Plan will terminate on April 16, 2023 unless it is extended or terminated earlier pursuant to its terms.

During the first quarter of 2013, the Company completed a review of equity compensation awards granted under its previous equity compensation plan and determined that it had inadvertently exceeded the annual per-person sub-limits involving certain awards previously made to certain of its current and past officers and directors (the excess awards). The aggregate amount of common stock represented by these excess awards, which consisted of Restricted Stock Units (RSUs) and stock options, was approximately 1.4 million shares. These awards were deemed to be granted outside of the plan and as such the Company applied liability accounting to these awards. On May 23, 2013 (the date of the Company s 2013 Annual Meeting of Stockholders), the Company s shareholders approved the grants associated with the excess awards, which as of that date, allowed the excess awards to be deemed granted under the previous equity compensation plan. As a result, the excess awards were remeasured at fair value on May 23, 2013 and the liability was reclassified to additional paid-in capital. The unrecognized fair value calculated for the excess awards as of May 23, 2013 is recognized as compensation expense ratably over the remaining requisite service period for each award.

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 stock options granted under its equity compensation plans:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	**2013**	**2014**	**2013**
Volatility	83.1%-85.0%	90.2%-96.0%	83.1%-85.5%	90.2%-96.0%
Risk-free interest rate	1.52%-1.76%	0.65%-1.13%	1.46%-1.76%	0.65%-1.13%
Dividend yield	0.0%	0.0%	0.0%	0.0%
Expected option term (in years)	6.25	6.25	6.25	6.25
Weighted-average fair value of stock options granted	$9.61	$8.10	$11.83	$7.14

11

Table of Contents

For all periods presented, the volatility factor was based on the Company s historical volatility since the closing of the Company s merger with Transave on December 1, 2010. The expected life was determined using the simplified method as described in ASC Topic 718, Accounting for Stock Compensation , which is the midpoint between the vesting date and the end of the contractual term. The risk-free interest rate is based on the US Treasury yield in effect at the date of grant. Forfeitures are based on actual percentage of option forfeitures since the closing of the Company s merger with Transave on December 1, 2010, and this is the basis for future forfeiture expectations.

The following table summarizes the Company s stock option activity for the six months ended June 30, 2014:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life in Years	Aggregate Intrinsic Value (in thousands)
Options outstanding at December 31, 2013	3,632,996	$ 7.94		
Granted	1,517,350	$ 16.22		
Exercised	(59,788)	$ 6.86		
Forfeited or expired	(32,099)	$ 11.27		
Options outstanding at June 30, 2014	5,058,459	$ 10.42	8.92	$ 48,601
Vested and expected to vest at June 30, 2014	4,732,947	$ 10.29	8.90	$ 46,057
Exercisable at June 30, 2014	827,113	$ 5.37	8.07	$ 12,083

The total intrinsic value of stock options exercised during the three months ended June 30, 2014 and 2013 was $0.0 million and $1.0 million and during the six months ended June 30, 2014 and 2013 was $0.6 million and $1.1 million, respectively.

As of June 30, 2014, there was $35.4 million of unrecognized compensation expense related to unvested stock options, which is expected to be recognized over a weighted average period of 2.8 years. The following table summarizes the range of exercise prices and the number of stock options outstanding and exercisable:

		Outstanding as of June 30, 2014			Exercisable as of June 30, 2014	
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	3.29	272,100	7.39	3.04	167,455	3.04
3.40	3.40	708,314	8.20	3.40	265,619	3.40
3.48	6.90	793,153	8.36	6.19	207,660	5.83
6.96	10.35	535,200	8.73	7.59	99,125	7.36
11.14	11.46	257,000	9.09	11.22		
12.44	12.44	543,642	8.87	12.44	78,024	12.44
12.58	12.58	626,050	9.92	12.58		
12.70	15.61	521,950	9.42	14.19	9,230	13.05
15.63	20.49	786,050	9.62	19.38		
21.54	21.54	15,000	9.56	21.54		

12

Table of Contents

Restricted Stock and Restricted Stock Units The Company may grant Restricted Stock (RS) and RSUs to eligible employees, including its executives, and non-employee directors. Each RS and 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 granted are generally valued at the market price of the Company s common stock on the date of grant. RSUs granted in excess of certain plan sub-limits were considered to be granted outside the previous equity compensation plan and were classified as a liability and remeasured at fair value at the end of each reporting period and changes in fair value are included in compensation expense in the Consolidated Statements of Comprehensive Loss (see additional disclosures related to certain RSUs granted outside the previous equity compensation plan at the end of this note). The Company recognizes noncash compensation expense for the fair values of these RS and RSUs on a straight-line basis over the requisite service period of these awards.

The following table summarizes the Company s RSU award activity during the six months ended June 30, 2014:

	Number of RSUs	Weighted Average Grant Price
Outstanding at December 31, 2013	92,641	$ 6.27
Granted	20,258	19.53
Released	(91,939)	6.21
Outstanding at June 30, 2014	20,960	$ 19.35
Expected to vest	20,960	$ 19.35

Awards Granted outside of the previous equity compensation plan As described above, during the first quarter of 2013, the Company completed a review of equity compensation awards granted under its previous equity compensation plan and determined that it had inadvertently exceeded the annual per-person sub-limits involving certain awards previously made to certain of its current and past officers and directors. The aggregate amount of common stock represented by these excess awards, which consisted of RSUs and stock options, was approximately 1.4 million shares. These awards were deemed to be granted outside of the plan and as such the Company applied liability accounting to these awards. On May 23, 2013 (the date of the Company s 2013 Annual Meeting of Stockholders), the Company s shareholders approved the grants associated with the excess awards, which as of that date, allowed the excess awards to be deemed granted under the previous equity compensation plan. As a result, the excess awards were remeasured at fair value on May 23, 2013 and the liability was reclassified to additional paid-in capital. The unrecognized fair value calculated for the excess awards as of May 23, 2013 is recognized as compensation expense ratably over the remaining requisite service period for each award.

The following table summarizes the aggregate stock-based compensation recorded in the Consolidated Statements of Comprehensive Loss related to stock options and RSUs during the three and six months ended June 30, 2014 and 2013:

	Three months ended June 30,		Six months ended June 30,	
	2014	2013	2014	2013
	(in millions)		(in millions)	
Research and development expenses	$ 1.4	$ 1.0	$ 2.3	$ 1.3
General and administrative expenses	1.6	3.0	3.1	3.7
Total	$ 3.0	$ 4.0	$ 5.4	$ 5.0

Table of Contents

8. Income Taxes

The benefit for income taxes was $4.4 million and $1.2 million for the six months ended June 30, 2014 and 2013, respectively. The benefit for income taxes recorded for the six months ended June 30, 2014 and 2013 solely reflect the reversal of a valuation allowance previously recorded against the Company s New Jersey State net operating losses (NOL) that resulted from the Company s sale of a portion of its New Jersey State NOLs under the State of New Jersey s Technology Business Tax Certificate Transfer Program (the Program) for cash of $4.4 million and $1.2 million, respectively and net of commissions. The Program allows qualified technology and biotechnology businesses in New Jersey to sell unused amounts of NOLs and defined research and development tax credits for cash.

The Company is subject to US federal and state income taxes. The Company has never been audited and the statute of limitations for tax audit is generally open for the years 2010 and later. The Company has incurred net operating losses since inception, except in 2009. Such loss carryforwards would be subject to audit in any tax year in which those losses are utilized, notwithstanding the year of origin. The Company s policy is to recognize interest accrued related to unrecognized tax benefits and penalties in income tax expense. The Company has recorded no such expense. As of June 30, 2014 and December 31, 2013, the Company has recorded no reserves for unrecognized income tax benefits, nor has 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 twelve months. Due to the Company s history of operating losses, the Company recorded a full valuation allowance on its net deferred tax assets as it is more likely than not that such tax benefits will not be realized.

At December 31, 2013, the Company had federal net operating loss carryforwards for income tax purposes of approximately $398.7 million available to offset future taxable income, if any. The NOL carryovers and general business tax credits expire in various years beginning in 2018.

Utilization of the Company s NOL and general business tax credit carryforwards generated in prior years through September 2012 (the September 2012 and prior NOLs) are likely subject to substantial limitations under Section 382 of the Internal Revenue Code (Section 382) due to ownership changes that occurred at various points during years prior to 2012 and during September 2012. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50 percentage points over a three-year period. Since the Company s formation, it has raised capital through the issuance of common stock on several occasions which, combined with the purchasing shareholders subsequent disposition of those shares, likely resulted in multiple changes in ownership, as defined by Section 382 since the Company s formation in 1999. The substantial limitations on the use of the September 2012 and prior NOLs are likely to result in expiration of a substantial portion of these NOL or general business tax credit carryforwards before utilization which would substantially reduce the Company s gross deferred tax assets. The Company is currently in the process of completing a Section 382 analysis regarding the limitation of its NOL and general business tax credit carryforwards and intends to disclose the results of this analysis when it is completed.

9. Collaboration Agreements

Therapure Biopharma Inc. In February 2014, the Company entered into a Contract Manufacturing Agreement (the Agreement) with Therapure Biopharma Inc. (Therapure) for the manufacture of the Company s product ARIKAYCE. Pursuant to the Agreement, the Company and Therapure are collaborating to construct a production area for the manufacture of ARIKAYCE in Therapure s existing manufacturing facility in Mississauga, Ontario, Canada. Therapure will manufacture ARIKAYCE for the Company on a non-exclusive basis. The Agreement has an initial term of five years from the first date on which Therapure delivers ARIKAYCE to Insmed after Insmed obtains permits related to the manufacture of ARIKAYCE, and will

Table of Contents

renew automatically for successive periods of two years each, unless terminated by either party by providing the required two years prior written notice to the other party. Notwithstanding the foregoing, the parties have rights and obligations under the Agreement prior to the commencement of the initial term. The Agreement allows for termination by either party upon the occurrence of certain events, including (i) the material breach by the other party of any provision of the Agreement or the quality agreement expected to be entered into between the parties, or (ii) the default or bankruptcy of the other party. In addition, the Company may terminate the Agreement for any reason upon no fewer than one hundred eighty days advance notice. Costs incurred under this agreement will be recorded as a component of research and development expense until such time as the Company receives US Food and Drug Administration approval for ARIKAYCE.

PARI Pharma GmbH In July 2014, the Company entered into a Commercialization Agreement (the PARI Agreement) with PARI Pharma GmbH (PARI) for the manufacture and supply of eFlow nebulizer systems and related accessories (the Device) as optimized for use with the Company s proprietary liposomal amikacin for inhalation. The PARI Agreement has an initial term of fifteen years from the first commercial sale of the Device (the Initial Term). The term of the PARI Agreement may be extended by the Company for an additional five years by providing written notice to PARI at the least one year prior to the expiration of the Initial Term. Notwithstanding the foregoing, the parties have certain rights and obligations under the PARI Agreement prior to the commencement of the Initial Term. The PARI Agreement allows for termination by either party upon the occurrence of certain events, including (i) the material breach by the other party of any provision of the PARI Agreement, (ii) the default or bankruptcy of the other party, or (iii) upon termination by the Company of the License Agreement between the parties.

10. Commitments and Contingencies

Commitments

The Company has two operating leases for office and laboratory space located in Monmouth Junction, New Jersey through December 31, 2014. Future minimum rental payments under these two leases as of June 30, 2014 total approximately $0.4 million. The Company also has an operating lease for office and laboratory space located in Bridgewater, New Jersey that terminates in November 2019. Future minimum rental payments under this lease as of June 30, 2014 total approximately $3.6 million. The Company also leases office space in Richmond, Virginia, where the Company s corporate headquarters were once located, through October 2016. Future minimum rental payments under this lease as of June 30, 2014 total approximately $1.2 million.

Rent expense charged to operations was $0.3 million and $0.2 million for the three months ended June 30, 2014 and 2013 and $0.6 million and $0.6 million for the six months ended June 30, 2014 and 2013, respectively. Future minimum rental payments required under the Company s operating leases for the period from July 1, 2014 to December 31, 2014 and for each of the next five years are as follows (in thousands):

2014 (remaining)	$	680
2015		1,106
2016		1,144
2017		741
2018		762
2019		718
	$	5,151

15

Table of Contents

Legal Proceedings

Pilkiewicz v. Transave LLC

On March 28, 2011, Frank G. Pilkiewicz and other former stockholders of Transave (collectively, the Petitioners) filed an appraisal action against the Company s subsidiary Transave, LLC in the Delaware Court of Chancery captioned *Frank G. Pilkiewicz, et al. v. Transave, LLC*, C.A. No. 6319-CS. On December 13, 2011, following the mailing of the revised notice of appraisal rights in accordance with the settlement terms of *Mackinson et al. v. Insmed*, an Amended Petition for Appraisal of Stock was filed by the Petitioners.

The Petitioners sought appraisal under Delaware law of their total combined common stock holdings representing total dissenting shares of approximately 7.77 million shares of Transave common stock (the Transave Stock). The Petitioners were challenging the value of the consideration that they would have been entitled to receive for their Transave Stock under the terms of the Company s merger with Transave.

Under the terms of the Merger Agreement, certain of the former stockholders of Transave (the Transave Stockholders) were obligated to indemnify the Company for certain liabilities in connection with the appraisal action. The Company notified the Transave Stockholders in May 2012 that the Company was seeking indemnification in accordance with the Merger Agreement and that it would continue to retain the aggregate amount of the holdback shares totaling 1,765,271 shares, as security for any indemnification payments due under the Merger Agreement. Discovery was completed and a trial was scheduled. Prior to commencement of the trial, in May 2014, the parties entered into a settlement, dismissal and release agreement. The Company was indemnified in full for the settlement and related costs incurred in defending the appraisal action.

From time to time, the Company is a party to various other lawsuits, claims and other legal proceedings that arise in the ordinary course of our 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.

11. Retirement Plan

The Company has a 401(k) defined contribution plan for the benefit for all employees and permits voluntary contributions by employees subject to IRS-imposed limitations. There were no employer contributions in the three and six months ended June 30, 2014 and 2013.

Table of Contents

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. 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 include, but are not limited to: failure or delay of European Medicines Agency, Health Canada, United States Food and Drug Administration and other regulatory reviews and approvals; competitive developments affecting the Company s product candidates; delays in product development or clinical trials or other studies; patent disputes and other intellectual property developments relating to the Company s product candidates; unexpected regulatory actions, delays or requests; the failure of clinical trials or other studies or results of clinical trials or other studies that do not meet expectations; the fact that subsequent analyses of clinical trial or study data may lead to different (including less favorable) interpretations of trial or study results or may identify important implications of a trial or study that are not reflected in Company s prior disclosures, and the fact that trial or study results or subsequent analyses may be subject to differing interpretations by regulatory agencies; the inability to successfully develop the Company s product candidates or receive necessary regulatory approvals; inability to make product candidates commercially successful; changes in anticipated expenses; changes in the Company s financing requirements or ability raise additional capital; our ability to complete development of, receive regulatory approval for, and successfully commercialize ARIKAYCE; our estimates of expenses and future revenues and profitability; our plans to develop and market new products and the timing of these development programs; our estimates of the size of the potential markets for our product candidates; our selection and licensing of product candidates; our ability to attract third parties with acceptable development, regulatory and commercialization expertise; the benefits to be derived from corporate license agreements and other third party efforts, including those relating to the development and commercialization of our product candidates; the degree of protection afforded to us by our intellectual portfolio; the safety and efficacy of our product candidates; sources of revenues and anticipated revenues, including contributions from license agreements and other third party efforts for the development and commercialization of products; our ability to create an effective direct sales and marketing infrastructure for products we elect to market and sell directly; the rate and degree of market acceptance of our product candidates; the timing and amount of reimbursement for our product candidates; the success of other competing therapies that may become available; and the availability of adequate supply and manufacturing capacity and quality for our product candidates.

Forward-looking statements are based upon 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 factors include, among others, the factors discussed in Item 1A Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2013 filed with the Securities and Exchange Commission (SEC) on March 6, 2014. We caution readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the Securities and Exchange Commission, 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, 2013.

17

Table of Contents

OVERVIEW

Insmed is a biopharmaceutical company currently focused on developing and commercializing inhaled therapies for patients battling serious lung diseases that are often life threatening. Our lead product candidate, ARIKAYCE, or liposomal amikacin for inhalation (LAI), is an inhaled antibiotic treatment that delivers a proven and potent anti-infective directly to the site of serious lung infections.

In March 2014, we reported top-line clinical results from the double-blind portion of our phase 2 clinical trial in the United States (US) and Canada of ARIKAYCE in patients who have lung infections caused by nontuberculous mycobacteria (NTM). The randomized, double-blind, placebo-controlled phase 2 clinical trial compared ARIKAYCE (590 mg delivered once daily), added to standard of care treatment, versus standard of care treatment plus placebo, in 90 adult patients with treatment resistant NTM lung disease. Eligibility for the study required patients to have been on the American Thoracic Society/Infectious Disease Society of America guideline therapy for at least six months prior to screening and to continue to have persistently positive mycobacterial cultures. The primary efficacy endpoint of the study was a semi-quantitative measurement of the change in mycobacterial density on a seven-point scale from baseline (day one) to the end of the randomized portion of the trial (day 84). ARIKAYCE did not meet the pre-specified level for statistical significance although there was a positive trend (p=0.148) in favor of ARIKAYCE. However, ARIKAYCE did achieve statistical significance with regard to the clinically relevant key secondary endpoint of culture conversion, with 11 out of 44 patients treated with ARIKAYCE (added to standard of care treatment) demonstrating clearance of the infecting mycobacterial organism (culture negative) by day 84 of the study as compared to 3 out of 45 patients treated with placebo (added to standard of care treatment) (p=0.01).

In May 2014, additional data were presented at the American Thoracic Society meeting. At the conclusion of the 84-day double blind phase of the trial, 78 of the 80 patients agreed to receive once-daily ARIKAYCE plus standard of care treatment for an additional 84 days. Data from 68 of these patients who completed the visits during the additional open label phase were available for inclusion in the poster. These results collected from the open label phase show that 21 of these patients were culture negative for NTM at Day 168. This data reflects 10 patients who were culture negative at Day 84 as well as 5 additional patients from the ARIKAYCE arm and 6 additional patients who were on placebo, switching to ARIKAYCE during the open-label phase.

In June 2014, the US Food and Drug Administration (FDA) granted ARIKAYCE Breakthrough Therapy Designation for the treatment of adult patients with NTM lung disease who are treatment refractory. This designation is based on findings from our U.S. phase 2 clinical trial of ARIKAYCE to treat NTM lung infections. ARIKAYCE has already received Orphan Drug, Qualified Infectious Disease Product (QIDP) and Fast Track designations from the FDA for the treatment of NTM lung infections and has also received Orphan Drug Designation from the European Medicines Agency (EMA).

In August 2014 we announced that, following discussions with European regulatory authorities, we intend to file by the end of 2014 a Marketing Authorization Application (MAA) with the EMA for ARIKAYCE for the treatment of NTM lung infections in treatment refractory patients as well as for *Pseudomonas aeruginosa (Pseudomonas)* lung infections in cystic fibrosis (CF) patients.

In August 2014 we also announced that, we will proceed with our previously planned Phase 3 study of the effectiveness of ARIKAYCE for the treatment of lung infections in the broad NTM population. This decision follows a meeting with the FDA in which the FDA acknowledged that exploration of the effectiveness of ARIKAYCE in a broader population is appropriate, based on the results of the recently conducted Phase 2 trial, which showed statistically significant negative culture conversion in patients refractory to standard therapy. We also plan to initiate a second Phase 3 study which will be designed to confirm, in as short a timeframe as possible, the positive culture conversion results seen in the Phase 2 study. This confirmatory study will primarily investigate ARIKAYCE for use in the treatment refractory population with

mycobacterium avium complex (MAC) NTM lung infections. This subgroup of the Phase 2 trial s patients responded particularly strongly to the treatment.

We believe this two-trial approach will enable both the rapid confirmation of the previous study results to provide the quickest path to filing, as well as expansion of the potential overall label for approval. Following discussions with the FDA, both trials will focus on culture conversion as the primary measure of efficacy with additional goals of demonstrating sustainability and safety. We expect results from the smaller confirmatory study in the first half of 2016 and results for the larger trial in 2017.

The CF and NTM target indications address orphan patient populations. Our strategy includes plans to continue to develop ARIKAYCE in the NTM patient population and for additional indications beyond *Pseudomonas* in CF and NTM. We also plan to develop, acquire, in-license or co-promote other products that address orphan or rare diseases in the fields of pulmonology and infectious disease. Our current primary development focus is to obtain regulatory approval for ARIKAYCE in these two initial indications and to prepare for commercialization in the US, Europe, Canada and Japan. We anticipate that if approved, ARIKAYCE would be the first once-a-day inhaled antibiotic treatment option available for the CF indication and the NTM indication. The following table summarizes the current status of ARIKAYCE development.

Table of Contents

Product Candidate/Target Indications	Status	Next Expected Milestones
ARIKAYCE Non-tuberculous mycobacteria (NTM) lung infections	• We reported top-line clinical results from our phase 2 clinical trial which stated that ARIKAYCE did not meet the pre-specified level for statistical significance with respect to the primary endpoint, but did achieve statistical significance with regard to the clinically relevant key secondary endpoint of culture conversion. • Results collected from the open label phase show that 21 of these patients were culture negative for NTM at Day 168. • Granted Orphan Drug designation in Europe and the US. • Granted QIDP designation, which includes Priority Review, by the FDA. • Granted Fast Track designation by the FDA which permits a rolling submission of an NDA. • Granted Breakthrough Therapy designation for ARIKAYCE in the US in June 2014 based upon the culture conversion results in the phase 2 clinical trial.	• We intend to file by the end of 2014 a MAA with the EMA for the treatment of NTM lung infections in treatment refractory patients as well as for *Pseudomonas* lung infections in CF patients. • We will proceed with our previously planned Phase 3 study of the effectiveness of ARIKAYCE for the treatment of lung infections in the broad NTM population. • We also plan to initiate a second Phase 3 study which will be designed to confirm, in as short a timeframe as possible, the positive culture conversion results seen in the Phase 2 study. This confirmatory study will primarily investigate ARIKAYCE for use in the treatment refractory population with MAC NTM lung infections. • If approved, we expect ARIKAYCE would be the first approved inhaled antibiotic treatment for NTM lung infections. • We are developing plans to commercialize ARIKAYCE, if approved, in certain countries in Europe and in the US, and eventually Canada, Japan and certain other countries including Korea, Taiwan and China.
ARIKAYCE *Pseudomonas aeruginosa* lung infections in CF patients	• Reported top-line results from our phase 3 clinical trial conducted in Europe and Canada, in which once-daily ARIKAYCE achieved its primary endpoint of non-inferiority when compared to twice-daily tobramycin inhaled solution. • Conducting a two-year, open-label safety study in patients who completed the phase 3 clinical trial. We expect to complete this study in mid-2015. • Reported top-line results from the first group of patients who completed the first year of the two-year open label extension study. • Granted orphan drug designation in Europe and the US.	• We intend to file a MAA with the EMA by the end of 2014 and in Canada during the first half of 2015. • We are developing plans to commercialize ARIKAYCE, if approved, in certain countries in Europe and in Canada where we expect it would be the only once-a-day treatment for *Pseudomonas* lung infections in CF patients. • We currently do not plan to initiate any further studies in *Pseudomonas* lung infections.

Table of Contents

ARIKAYCE *Pseudomonas aeruginosa* and other susceptible organisms causing lung infections in non-CF bronchiectasis patients	• Completed phase 2 study in the US. • Granted orphan drug designation in the US.	• We currently do not intend to initiate further clinical studies with respect to a non-CF bronchiectasis indication.

Amikacin sulfate is an FDA-approved antibiotic with proven efficacy in the treatment of a broad range of gram-negative infections, including *Pseudomonas* and NTM. ARIKAYCE is in the aminoglycoside class of antibiotics. We believe there currently is no drug approved in Europe or the US for treatment of NTM lung infections, and as a result all current drug treatments for NTM are used off-label. If approved for NTM patients, we believe ARIKAYCE would be the first and only approved inhaled antibiotic for the treatment of NTM lung infections. If approved for CF patients with *Pseudomonas* lung infections, we believe ARIKAYCE would be the first inhaled antibiotic to be approved for once-daily administration in this indication. ARIKAYCE has been granted orphan drug designations in the following indications:

- US: NTM lung infections, *Pseudomonas* lung infections in CF patients, and lung infections in non-CF bronchiectasis patients; and
- European Union (EU): NTM lung infections and *Pseudomonas* lung infections in CF patients.

Corporate History

We were incorporated in the Commonwealth of Virginia on November 29, 1999. On December 1, 2010, we completed a business combination with Transave, Inc. (Transave), a privately held, New Jersey-based pharmaceutical company focused on the development of differentiated and innovative inhaled pharmaceuticals for the site-specific treatment of serious lung infections.

Our Strategy

Our strategy is to focus on the development and commercialization of innovative inhaled therapies for patients with serious lung diseases in orphan indications. While we believe that ARIKAYCE has the potential to treat many different diseases, our attention is initially focused on regulatory approval and commercialization preparation for our two initial indications: (1) NTM lung infections and (2) *Pseudomonas* lung infections in CF patients. Our current priorities are as follows:

- Continue generating additional clinical data from studies showing the effects of ARIKAYCE to treat NTM lung infections and *Pseudomonas* lung infections in CF patients;
- Actively pursue new drug filings to secure approval for ARIKAYCE to treat NTM lung infections in the US, Europe, Canada and Japan;
- Actively pursue new drug filings to secure approval for ARIKAYCE to treat *Pseudomonas* lung infections in CF patients in Europe and Canada;

- Expand our product supply chain in support of clinical development and if approved, commercialization;

- Prepare for commercial launch in the NTM indication in the US, Europe, Canada and eventually Japan and certain other countries including Korea, Taiwan and China;

- Prepare for commercial launch in *Pseudomonas* in CF patients indication in Europe and Canada;

- Attempt to develop, acquire, in-license or co-promote promising late stage or commercial products that we believe are complementary to ARIKAYCE and our core competencies; and

- Continue to develop novel formulations of existing therapies, where such reformulation could materially improve the treatment paradigm for the underlying disease or to enable pursuit of a new indication.

Table of Contents

In support of these priorities, we completed our registrational phase 3 clinical study of ARIKAYCE in CF patients with *Pseudomonas* lung infections in Europe and Canada. We plan to submit regulatory marketing applications for the CF and NTM indications in Europe by the end of 2014 and in Canada in the first half of 2015. In the first half of 2014, we completed our US and Canadian phase 2 clinical study of ARIKAYCE for the treatment of NTM lung infections in treatment refractory patients. We plan to initiate two global Phase 3 clinical trials of ARIKAYCE in NTM; one for the broad NTM patient population and one confirmatory study for treatment refractory patients with NTM lung infections. We plan to scale up manufacturing, we are identifying second source suppliers, and we plan to implement supply and quality agreements in preparation for commercialization of ARIKAYCE. In February 2014, we entered into a contract manufacturing agreement with Therapure Biopharma Inc. (Therapure) for the manufacture of ARIKAYCE at the larger scales necessary to support commercialization. In July 2014, we entered into a commercialization agreement with PARI Pharma GmbH (PARI), the manufacturer of our drug delivery nebulizer, to address our commercial supply needs. We have commenced the build-out of our commercial infrastructure in preparation for potential commercial launches in Europe, Canada and the US. We will continue to evaluate opportunities for additional products through various business development channels.

Product Candidates

Our lead product candidate, ARIKAYCE, or LAI, is a once-a-day inhaled antibiotic treatment engineered to deliver an anti-infective directly to the site of serious lung infections. There are two key components of ARIKAYCE: the liposomal formulation of the drug and the nebulizer device through which ARIKAYCE is inhaled via the mouth and into the lung. The nebulizer technology is owned by PARI, but we have exclusive access to this technology, which is specifically developed for the delivery of our liposomal encapsulation of amikacin, through our licensing agreement with PARI. Our proprietary liposomal technology and the nebulizer are designed specifically for delivery of pharmaceuticals to the lung and provides for potential improvements to existing treatments. We believe that ARIKAYCE has potential usage for at least two orphan patient populations with high unmet need: patients who have NTM lung infections and CF patients who have *Pseudomonas* lung infections. We estimate the combined global market potential for these two orphan indications to be approximately $1 billion.

ARIKAYCE has the potential to be differentiated from amikacin and certain marketed drugs for the treatment of chronic lung infections if it can be demonstrated to provide improved efficacy, safety and patient convenience. We believe ARIKAYCE s ability to deliver high, sustained levels of amikacin directly to the lung and to the specific site of the underlying infection could distinguish it from other alternatives. We are also investigating ARIKAYCE s potential for durability of effect, benefiting patients when off treatment or for an extended period of treatment. In addition, the inhalation delivery of ARIKAYCE may reduce the potential for adverse events such as ototoxicity (hearing loss, ringing in the ears and/or loss of balance) and nephrotoxicity (toxicity to the kidneys), as compared with intravenous (IV) administration of amikacin. If approved, we expect that ARIKAYCE will be administered once-daily for approximately 13 minutes via inhalation using the eFlow® Nebulizer System, which has been optimized specifically for ARIKAYCE by PARI. We believe that this nebulizer system will reduce treatment time or dosing frequency, as compared with the currently marketed inhaled antibiotics, which require dosing two to three times daily with treatment times ranging from approximately 10 to 40 minutes per day. By easing the patients treatment burden we believe that ARIKAYCE can potentially improve patient compliance, which we believe may in turn lead to a reduction in the development of antibiotic resistance and, ultimately, lead to clinical benefit.

We believe that ARIKAYCE may provide: (i) improved efficacy resulting from sustained deposition of drug in the lung and improved ability to reach the site of infection (for CF *Pseudomonas* infections, this means penetration of biofilm and facilitated drug release by factors that are secreted by the bacteria, and for NTM, this means enhanced uptake into macrophages, where NTM often grows); (ii) decreased adverse events and improved tolerability as compared with amikacin delivered intravenously, and (iii) reduced dosing frequency or treatment time as compared to existing products. In the future we may conduct head-to-head comparative studies that would be necessary to make comparative statements against other products.

21

Table of Contents

ARIKAYCE for Patients with NTM Lung Infections

Overview of NTM Lung Infections

Nontuberculous mycobacteria, or NTM, are organisms common in soil and water that have been associated with lung disease in select patient groups. NTM have characteristics that are similar to tuberculosis, or TB, but NTM are not believed to be contagious. Many people have NTM in their bodies, but NTM do not normally lead to an infection, perhaps because the body s immune system successfully overcomes the threat of infection. It is not completely understood why certain individuals are susceptible to NTM infections. However, the patients who become infected by NTM often are immune-compromised, due to comorbidities such as HIV or rheumatoid arthritis, or have structural damage in their lungs, due to smoking, chronic obstructive pulmonary disease or CF, at the time of the infection.

NTM are organisms that invade and multiply chiefly within macrophages. They are characteristically resistant to most antibiotics. NTM lung infections are chronic, debilitating and progressive and often require lengthy, repeat hospitalizations. Signs and symptoms of NTM pulmonary disease are variable and nonspecific. They include chronic cough, sputum production and fatigue. Less commonly, malaise, dyspnea, fever, hemoptysis, and weight loss also can occur, usually with advanced NTM disease. Evaluation is often complicated by the symptoms caused by co-existing lung diseases. According to a study published in the *American Journal of Respiratory and Critical Care Medicine*, these conditions include chronic obstructive airway disease associated with smoking, bronchiectasis, previous mycobacterial diseases, CF and pneumoconiosis (Olivier et al. 2003).

Current Treatment Options and Limitations

We believe there currently is no drug approved in Europe or the US for treatment of NTM lung infections, and as a result all current drug treatments for NTM are used off-label. Patients are often treated with the same antibiotics that are used to treat TB. Such treatments usually consist of lengthy multi-drug antibiotic regimens, which are often poorly tolerated and not very effective, especially in patients with severe disease and patients who have failed prior treatments. NTM patients average 7.6 antibiotic courses per year (SDI Healthcare Database, July 2009). Treatment guidelines published in 2007 in the *American Journal of Respiratory and Critical Care Medicine* reported that few clinical trials were under way to identify treatment recommendations, and no new antibiotics had been studied for the treatment of NTM lung infections in multi-center, randomized clinical trials since the late 1990s.

Although approved for other indications, amikacin sulfate is not approved by the FDA for NTM lung infections. In practice, however, it is often recommended by physicians as part of the standard treatment regimen for some NTM patients. It is delivered most commonly by intravenous administration and, far less often, by inhalation. Because the drug is delivered for months at a time, resulting in high systemic (blood) levels of the drug, there can be considerable toxicity, including ototoxicity and nephrotoxicity, associated with intravenous treatment. There are very few prior studies to support what doses should be administered to effectively treat NTM patients even with these existing medications and they are often titrated on a patient by patient basis.

Market

The prevalence of human disease attributable to NTM has increased over the past two decades. In 2012, in collaboration with the NIH, we funded a study performed by Clarity Pharma Research that showed there were an estimated 50,000 cases of pulmonary disease attributable to NTM in the US in 2011 and that such cases were estimated to be growing at a rate of 10% per year. NTM is four to five times more prevalent than TB in the US (Incidence of TB from Center for Disease Control and Prevention Morbidity and Mortality Weekly Report, March 2012). In a decade-long study, researchers found that the diagnosis of NTM in the US is increasing at approximately 8% per year and that those NTM patients over the age of 65 are 40% more likely to die than those

Table of Contents

who do not have the disease (Adjemian et al, Prevalence of Pulmonary Nontuberculous Mycobacterial Disease among Medicare Beneficiaries, USA, 1997-2007, American Journal of Respiratory and Critical Care Medicine, April 2012).

In 2013, we engaged Clarity Pharma Research to perform a similar chart audit study of NTM in Europe and Japan. Based on results of this study, researchers estimated that there are approximately 20,000 cases of pulmonary disease attributable to NTM within the European nations of France, Germany, the United Kingdom, Italy and Spain combined and approximately 30,000 in the 28 countries comprising the EU. In addition, there are nearly 32,000 cases in Japan. Although population-based data on the epidemiology of NTM infections in Europe are limited, consistent with US prevalence trends, recent published studies concur that prevalence in Europe is increasing and, according to a study published in the Japanese journal Kekkaku in 2011, Japan has one of the world s highest NTM disease rates.

Although there are many species of NTM that have been reported to cause lung infections, ARIKAYCE is intended to treat two of the most common, *Mycobacterium Avium* Complex (MAC) and *Mycobacterium abscessus* (*M. abscessus*). MAC accounts for the vast majority of NTM lung infections with prevalence rates from 72% to more than 85% in the US. The reported prevalence rates for *M. abscessus* range from 3% to 11% in the US. The diagnosed prevalence of NTM species causing lung infections varies geographically with MAC rates of 25% to 55% reported in Europe. MAC is also the most common NTM pathogen in Japan.

ARIKAYCE for NTM Lung Infections: Potential Advantages and Distinguishing Features

If approved, we believe ARIKAYCE would be the first and only approved treatment for patients battling NTM lung infections.

Liposomal Design and Formulation

We believe that ARIKAYCE may be effective in treating patients with NTM lung infections due to the apparent ability of the ARIKAYCE liposomes to be taken up inside lung macrophages that harbor NTM. Macrophages are immune cells whose primary function includes removing foreign particles and bacteria from the lungs. NTM are taken up by and multiply inside these macrophages. Many antibiotics cannot efficiently gain access to the macrophage interior. ARIKAYCE liposomes, however, are designed to be internalized by lung macrophages and thereby deliver high levels of drug inside the macrophages where the NTM bacteria are located.

Route of Administration

We believe ARIKAYCE has the potential to offer a safety profile different from that of intravenous delivery of amikacin. For example, unlike the intravenous administration of amikacin, ARIKAYCE would deliver the drug more directly to the site of disease. We anticipate this will result in less exposure of non-disease sites to amikacin. We believe this may reduce the potential for the occurrence of any drug-related systemic toxicity, such as nephrotoxicity, which is especially important with diseases like NTM that require long-term drug administration.

Anticipated Dosage Regimen

We believe ARIKAYCE, if approved, could improve patient convenience by providing once-a-day dosing. According to *SDI Healthcare Database* NTM patients average 7.6 antibiotic courses and 10.2 hospital days per year. We anticipate that ARIKAYCE will be administered once daily outside of the hospital for approximately 13 minutes per day for a period of 84 days for this indication. We believe that an effective inhaled treatment that improves the outcomes for an NTM patient would represent a significant benefit in the patient s quality of life.

Table of Contents

Current Clinical Program

In March 2014, we reported top-line clinical results from the double-blind portion of our phase 2 clinical trial in the US and Canada of ARIKAYCE in patients who have lung infections caused by NTM. The randomized, double-blind, placebo-controlled phase 2 clinical trial compared ARIKAYCE (590 mg delivered once daily), added to standard of care treatment, versus standard of care treatment plus placebo, in 90 adult patients with treatment resistant NTM lung disease. Eligibility for the study required patients to have been on the American Thoracic Society/Infectious Disease Society of America guideline therapy for at least six months prior to screening and to continue to have persistently positive mycobacterial cultures. The primary efficacy endpoint of the study was a semi-quantitative measurement of the change in mycobacterial density on a seven-point scale from baseline (day one) to the end of the randomized portion of the trial (day 84). ARIKAYCE did not meet the pre-specified level for statistical significance although there was a positive trend in favor of ARIKAYCE. However, ARIKAYCE did achieve statistical significance with regard to the clinically relevant key secondary endpoint of culture conversion, with 11 out of 44 patients treated with ARIKAYCE (added to standard of care treatment) demonstrating negative cultures by day 84 of the study as compared to 3 out of 45 patients treated with placebo (added to standard of care treatment).

In May 2014, additional data were presented at the American Thoracic Society meeting. At the conclusion of the 84-day double blind phase of the trial, 78 of the 80 patients agreed to receive once-daily ARIKAYCE plus standard of care treatment for an additional 84 days. Data from 68 of these patients who completed the visits during the additional open label phase were available for inclusion in the poster. These results collected from the open label phase show that 21 of these patients were culture negative for NTM at Day 168. This data reflects 10 patients who were culture negative at Day 84 as well as 5 additional patients from the ARIKAYCE arm and 6 additional patients who were on placebo, switching to ARIKAYCE during the open-label phase.

In June 2014, the FDA granted ARIKAYCE, Breakthrough Therapy Designation for the treatment of adult patients with NTM lung disease who are treatment refractory. This designation is based on findings from our U.S. phase 2 clinical trial of ARIKAYCE to treat NTM lung infections. ARIKAYCE has already received Orphan Drug, Qualified Infectious Disease Product (QIDP) and Fast Track designations from the FDA for the treatment of NTM lung infections and recently received Orphan Drug Designation from the EMA.

In August 2014 we announced that, following discussions with European regulatory authorities, we intend to file by the end of 2014 a MAA with the EMA for ARIKAYCE for the treatment of NTM lung infections in treatment refractory patients as well as for *Pseudomonas* lung infections in CF patients.

In August 2014 we also announced that, we will proceed with our previously planned Phase 3 study of the effectiveness of ARIKAYCE for the treatment of lung infections in the broad NTM population. This decision follows a meeting with the FDA in which the FDA acknowledged that exploration of the effectiveness of ARIKAYCE in a broader population is appropriate, based on the results of the recently conducted Phase 2 trial, which showed statistically significant negative culture conversion in patients refractory to standard therapy. We also plan to initiate a second Phase 3 study which will be designed to confirm, in as short a timeframe as possible, the positive culture conversion results seen in the Phase 2 study. This confirmatory study will primarily investigate ARIKAYCE for use in the treatment refractory population with MAC NTM lung infections. This subgroup of the Phase 2 trial s patients responded particularly strongly to the treatment.

We believe this two-trial approach will enable both the rapid confirmation of the previous study results to provide the quickest path to filing, as well as expansion of the potential overall label for approval. Following discussions with the FDA, both trials will focus on culture conversion as the primary measure of efficacy with additional goals of demonstrating sustainability and safety. We expect results from the smaller confirmatory study in the first half of 2016 and results for the larger trial in 2017.

Additionally, we have initiated a scintigraphy sub-study to examine drug deposition and distribution of ARIKAYCE in the lung.

ARIKAYCE received orphan drug status in the US and Europe for the treatment of NTM.

Development History

Nonclinical evaluations of ARIKAYCE in relation to NTM infections indicate: (1) high concentrations of drug are deposited in the lung, and high levels are sustained for prolonged periods, with low serum concentrations, and (2) ARIKAYCE has *in vitro* activity that is superior to amikacin solution against different strains of NTM.

Data obtained from *in vitro* testing of ARIKAYCE with respect to four different strains of MAC and *M. abscessus* indicate dose response with ARIKAYCE and superior activity to free amikacin. We believe that the safety and efficacy data obtained from the phase 3, phase 2 and open label studies of ARIKAYCE in CF and non-CF patients with chronic lung disease and pulmonary infections and the non-clinical data collected to date serve as the basis for further development of ARIKAYCE in patients with NTM lung infections.

Table of Contents

We submitted an IND to launch a phase 3 study of ARIKAYCE in CF and non-CF patients for the treatment of NTM lung infections in treatment refractory patients. In August 2011, prior to starting the NTM study, we announced that the FDA placed a clinical hold on our phase 3 trial. The clinical hold for the NTM study was lifted in January 2012. The FDA based its clinical hold decision on an initial review of the results of a long-term rat inhalation carcinogenicity study with ARIKAYCE. When rats were given ARIKAYCE daily by inhalation for two years, 2 of the 120 rats receiving the highest dose developed lung tumors. These rats received ARIKAYCE doses that were within two-fold of those in clinical studies (normalized on a body surface area basis or a lung weight basis). ARIKAYCE was not associated with changes that may lead to tumors in shorter-term studies in animals. Additionally, ARIKAYCE was not shown to be genotoxic in our standard series of tests. The relevance of the observed rat tumors to the use of ARIKAYCE in humans is not known. The FDA requested we conduct a phase 2 clinical trial, instead of our previously agreed upon phase 3 clinical trial in adult NTM patients, to provide proof-of-concept efficacy and safety data for ARIKAYCE in NTM patients. Despite the change in status from phase 3 to phase 2, the study design and target enrollment did not change. In connection with the FDA s decision to lift the clinical hold for all disease indications, we agreed to conduct a dog inhalation toxicity study of ARIKAYCE. In 2013, we concluded the dog inhalation toxicity study. In summary, the final report from the study stated that the lung macrophage response in dogs was similar to that seen in our previous 3 month dosing dog study, and there was no evidence of neoplasia, squamous metaplasia or proliferative changes.

Strategy for Commercialization

We currently plan to retain marketing rights for ARIKAYCE for the NTM indication. Given the current lack of approved treatments for NTM lung infections, we believe we will immediately have a strong market position if ARIKAYCE is approved for commercialization in the NTM indication. We believe ARIKAYCE will require a limited commercial infrastructure because of the small focused nature of the potential physician prescribing population for NTM patients. In 2013, we commenced preparations for the potential commercialization of ARIKAYCE and we have filled several new positions to support our future sales and marketing efforts. We may also seek to out-license ARIKAYCE in certain countries in Europe, as well as outside of Europe, Canada and the US.

ARIKAYCE for CF Patients with *Pseudomonas* Lung Infections

Overview of CF and Pseudomonas Lung Infections

CF is an inherited chronic disease that is often diagnosed before the age of two. CF occurs primarily in individuals of central and western European origin. CF affects roughly 70,000 children and adults worldwide, including 30,000 children and adults in the US (Cystic Fibrosis Foundation Patient Registry, 2011) and 35,000 patients in Europe (Hoiby, BMC Medicine, 2011, 9:32). There is no cure for CF.

Despite extensive treatment with multiple antibiotics, improved nutrition, and other treatments, life expectancy of a CF patient is only 38-40 years (Cystic Fibrosis Foundation Patient Registry, 2012). Median predicted age of survival is calculated using life table analysis (as calculated by actuaries) given the ages of the patients in the registry and the distribution of deaths. Using this calculation, half of the people in the patient registry are expected to live beyond the median predicted survival age, and half are expected to live less than the median predicted survival age.

Among other issues, CF causes thick, sticky mucus to develop in and clog the lungs. This creates an ideal environment for various pathogens, such as *Pseudomonas*, to colonize and lead to chronic infection of the lung, inflammation and progressive loss of lung function. In fact, chronic

bronchial infections with *Pseudomonas* are a major cause of morbidity and mortality among patients with CF. Once a CF patient acquires a *Pseudomonas* infection, it is difficult to eradicate. The current, best available treatment is chronic administration of antibiotics to suppress the bacteria, reduce inflammation and preserve lung function for as long as possible. The rate of infection

Table of Contents

with *Pseudomonas* in CF patients increases with age. It is estimated that 80% of adult CF patients have chronic infection due to *Pseudomonas* (CFF Patient Registry, 2012). A study reported in the *Journal of Cystic Fibrosis* (Liou, 2010) found that deterioration in lung function of CF patients is the main cause of death and that, despite best efforts, lung function declines by 1% to 3% annually.

Current Treatment Options and Limitations

CF therapy significantly impacts patients quality of life. Patients generally receive extensive antibiotic treatments, which can be delivered via the oral, intravenous and inhaled routes. Some CF patients spend up to three hours per day taking medications and other treatments, including inhaled antibiotics, and often face the burden of taking in excess of 20 pills per day. All currently approved inhalation treatments for *Pseudomonas* lung infections require two- to three-times a day dosing.

Antibiotics delivered via inhalation are part of the standard treatment for CF patients with *Pseudomonas* lung infections and are generally thought to be a way to deliver more active drug directly to the site of infection compared with other routes of administration. The most used treatment in the US for the management of chronic *Pseudomonas* infection in subjects with CF is suppressive therapy with tobramycin. One example is twice daily Tobi inhaled solution, which is approved by the FDA for CF patients ages six years and above with a FEV1 (forced expiratory volume in 1 second) of 25%-75%, has been sold in the US since January 1998. A 1999 study reported that Tobi, 300 mg, administered twice a day for cycles of 28 days followed by 28-days-off treatment was shown to reduce *Pseudomonas* colony counts, increase FEV1 percent predicted, reduce hospitalizations and decrease additional antibiotic use (Ramsey et al., 1999, New England Journal of Medicine). High levels of tobramycin can be attained in the lung with relatively low systemic exposure with inhaled drug compared to intravenous tobramycin. However, patients using Tobi must be dosed twice a day for approximately 15 to 20 minutes of inhalation session per dose for a total of approximately 30 to 40 minutes per day. Recent data show that the effect of Tobi on pulmonary function in CF patients has lessened since its introduction into the marketplace more than a decade ago (Konstan et al., Journal of Cystic Fibrosis, January 2011, and Assael et al., 34th European Cystic Fibrosis Society Conference, Poster 86, June 2011). In addition, according to information presented at a FDA advisory panel, resistance to Tobi has increased 85% in the ten-year period from 1999 to 2009 (FDA advisory panel US-FDA-AIDAC for Tobi-Podhaler, September 2012).

Market

We estimate that the global market for the treatment of *Pseudomonas* lung infections in CF patients is approximately $500 million. We believe this market is being driven by physicians desire to maintain the lung function of CF patients, which continues to decline in many patients despite extensive treatment with current therapies including currently approved inhaled antibiotics. We believe that the following additional factors may lead to further market growth:

- Better patient adherence to physician prescribed regimens resulting from more convenient (less frequent and less time consuming) treatments;
- Physicians initiating treatment with inhaled antibiotics earlier for patients with *Pseudomonas* in their lungs;
- CF patients living longer;

- Physicians moving to a different antibiotic every other month as opposed to giving patients off-treatment holidays on alternate months; and

- The standard of care in the rest of the world continuing to advance closer to that in the EU and the US.

Table of Contents

ARIKAYCE for CF Patients with Pseudomonas Lung Infections: Potential Advantages and Distinguishing Features

Patient Compliance Considerations

We believe ARIKAYCE may facilitate better patient compliance with prescribed treatment regimens; patient compliance with or adherence to prescribed treatment is generally expected to impact the effectiveness of treatment. If a product can improve adherence, it may be able to differentiate itself from other marketed drugs. In the case of treatment and management of chronic *Pseudomonas* lung infections in CF patients, currently the most used treatment in the US is suppressive therapy with 300 mg twice daily of Tobi inhaled solution and tobramycin inhaled powder. Tobi is administered twice daily for 28 days followed by a 28-day-off period. This cycle of on and off treatment is repeated in a chronic pattern. We anticipate that ARIKAYCE would be administered once daily for approximately 13 minutes per day for 28 days followed by a 28-day off-drug period. We believe that any inhaled treatment that reduces the treatment burden on a CF patient could represent a significant improvement in the patient s quality of life and result in improved compliance, as well as reduce the development of antibiotic resistance.

Liposomal Design and Formulation

We believe ARIKAYCE has the potential to deliver high levels of amikacin directly to the site of bacteria in the lung for a sustained period of time, which we expect would differentiate it from other marketed drugs for the treatment of chronic *Pseudomonas* lung infections in CF patients. Current inhaled antibiotics are commonly used as standard treatments for CF patients with *Pseudomonas* lung infections and generally are thought to be a way to deliver more drug directly to the site of infection as compared with other methods of delivery. However, CF patients seldom clear the *Pseudomonas* permanently from their lungs, in part because of the thick sticky mucus these patients produce in their lungs, and often become chronically infected despite existing antibiotic treatments. All existing aminoglycoside antibiotics, including tobramycin and amikacin, are positively charged and tend to bind to the negative surfaces of mucus and the biofilm. In contrast, we have designed ARIKAYCE to be a neutrally charged liposome, which has been shown in laboratory studies, to penetrate both CF mucus and a *Pseudomonas* biofilm. This means that ARIKAYCE may reach the site of the *Pseudomonas* infection in CF patients lungs more efficiently than the other currently available aminoglycoside antibiotics, including currently available inhaled antibiotics.

In addition, ARIKAYCE has demonstrated a prolonged half-life in animals lungs. We believe this effect is due to our proprietary liposomal technology. One important measure of the effectiveness of antibiotics is the maintenance of anti-bacterial drug levels in the lung above the minimum inhibitory concentration. We anticipate that ARIKAYCE will be maintained in the human lung in a manner similar to what was demonstrated in animal studies.

We believe ARIKAYCE may be further differentiated from other marketed drugs for the treatment of chronic *Pseudomonas* lung infections in CF patients due to improved lung function during both on-treatment and off-treatment cycles. Typically an inhaled antibiotic is given to CF patients with chronic *Pseudomonas* lung infections for 28 days followed by a 28-day off-treatment cycle, which is often repeated chronically or for the rest of a patient s life. In February 2014, we reported interim data from our two-year open label extension study which showed a mean increase in relative change in FEV1 which was sustained during both on-treatment and off-treatment months. In addition, during phase 2 studies ARIKAYCE demonstrated statistically significant and clinically meaningful improvement in pulmonary function throughout the 28-day treatment period, and such improvement was sustained during the 28-days off treatment period.

We have also reported data showing durability of effect for longer off-treatment periods. In an open-label phase 2 extension trial (TR02-105), CF patients using ARIKAYCE demonstrated sustained efficacy in lung function improvement during a 28-day treatment period and 56-day off-treatment period across multiple cycles of therapy as compared to baseline. In this clinical study, ARIKAYCE produced an improvement in lung function that was sustained over six cycles totaling approximately 17 months. During the off-treatment periods for this study,

Table of Contents

approximately 50% to 70% of the benefit achieved during the on-treatment periods was sustained at the end of the off-treatment periods. To our knowledge, no other inhaled antibiotic has shown sustained improvement in lung function at the end of a 56-day off-treatment period.

Route of Administration

We believe ARIKAYCE has the potential to offer a safety profile different from that of intravenous delivery of aminoglycosides. *Pseudomonas* is susceptible to several broad spectrum antibiotics, notably aminoglycosides. Some examples of aminoglycoside antibiotics include tobramycin and amikacin. Studies found that aminoglycosides are an important class of antibiotics for the treatment of *Pseudomonas* lung infections in CF patients because of their broad antimicrobial activity and concentration dependent bactericidal activity (Lacy et al., 1998; Lortholary et al., 1995; Zembower et al., 1998). Intravenous antibiotics were originally used for treatment of chronic infections associated with CF and are still used for pulmonary exacerbations. Studies report that ototoxicity and nephrotoxicity are common adverse events associated with the use of intravenous aminoglycosides and these effects are related to plasma drug levels (Mingeot-Leclercq and Tulkens, 1999).

There are two main obstacles to effective and safe treatment of CF:

- Drug Resistance. High-level multi-drug resistance complicates eradication of such strains from the bronchial secretions of CF patients. *Pseudomonas* lung infections are commonly treated using aminoglycoside antimicrobial agents, such as amikacin and tobramycin. However, due to drug resistance, significantly higher concentrations of these drugs above the minimum inhibitory concentration are required at the site of infection. The intravenous dosage levels required to achieve such exposures can be nephrotoxic and ototoxic.

- Limited Penetration. There is limited penetration into and through the sputum/biofilm matrix by aminoglycoside antibiotics. The antibiotics are positively charged and the biofilm is negatively charged. As a result, the antibiotics bind to the biofilm and the availability of the drug at the location of the microorganism is suboptimal. We believe that our proprietary liposomal technology will result in localized targeting of drugs, leading to increased availability of the drug at the location of the microorganism, while significantly reducing drug exposure at non-disease sites throughout the body and reducing the occurrence of systemic drug-related toxicity.

Current Clinical Program

We completed a registrational phase 3 clinical trial of ARIKAYCE for CF patients with *Pseudomonas* lung infections in Europe and Canada during the second quarter of 2013. The phase 3 trial was a randomized, open label, multi-center study designed to assess the comparative safety and efficacy of once-daily ARIKAYCE administered for approximately 13 minutes via the eFlow Nebulizer System and twice-daily Tobi (tobramycin inhalation solution) administered for approximately 15 minutes per treatment via the PARI LC Plus Nebulizer System for a daily total of approximately 30 minutes per day in CF patients with *Pseudomonas*. A total of 302 adult and pediatric CF patients with chronic *Pseudomonas* were randomized to receive 28-days of ARIKAYCE treatment or Tobi delivered twice-daily via the PARI LC Plus® Nebulizer System over a 24-week treatment period. The primary endpoint of the study was relative change in forced expiratory volume in one second (FEV1) measured after three treatment cycles, with each cycle consisting of 28 days on treatment and 28 days off treatment. The study was designed to demonstrate non-inferiority to Tobi at a 5% non-inferiority margin with 80% power agreed upon by us and the EMA. Secondary endpoints measured were relative changes in FEV1 at other time points, time to and number of pulmonary exacerbations, time to antibiotic rescue treatment, change in density of *Pseudomonas* in sputum, respiratory hospitalizations and changes in Patient Reported Outcomes assessing Quality of Life. Top-line results from this study indicated:

- ARIKAYCE achieved its primary endpoint of non-inferiority to Tobi for relative change in FEV1 from baseline to the end of the study;

Table of Contents

- Overall, secondary endpoints, as summarized above, showed comparability of once-daily ARIKAYCE compared with twice-daily Tobi; and

- The safety profile of ARIKAYCE was comparable to Tobi during all three treatment cycles, with adverse events consistent with those seen in similar studies and expected in a population of CF patients receiving inhaled antibiotics. There was no difference between arms in the reporting of serious adverse events and there were no unexpected adverse events.

We are conducting a two-year, open label safety study in patients that also completed our registrational phase 3 clinical study of ARIKAYCE for CF patients with *Pseudomonas* lung infections in Europe and Canada. Approximately 75% of the eligible patients that completed our registrational phase 3 clinical study consented to participate in the safety study. The patients in this study will receive ARIKAYCE for up to an additional two year period, using the same cycles of a 28 day on-treatment period and a 28 day off-treatment period. In February 2014, we reported interim data from our two-year open label extension study which showed a mean increase in relative change in FEV1 which was sustained during both on-treatment and off-treatment months. We expect to use this interim data from this study as part of our regulatory filings with the EMA, which we expect to submit by the end of 2014 and Health Canada, which we expect to submit in the first half of 2015. We expect to complete this study in mid-2015.

ARIKAYCE has been granted orphan drug status in the US and Europe for the treatment of *Pseudomonas* lung infections in CF patients.

Development History

Nonclinical evaluations of ARIKAYCE in relation to *Pseudomonas* lung infections indicate:

- High concentrations of drug are deposited in the lung, and high levels are maintained for prolonged periods, with low serum concentrations;

- ARIKAYCE penetrates CF sputum and *Pseudomonas* biofilm;

- ARIKAYCE exhibits antipseudomonal activity in *in vitro* and *in vivo* models, including against resistant isolates; and

- Virulence factors secreted by *Pseudomonas* facilitate the release of amikacin from ARIKAYCE.

Our predecessor liposomal amikacin formulations for inhalation were evaluated in a series of phase 1 clinical studies involving healthy volunteers and CF patients with *Pseudomonas* lung infections. The current formulation of ARIKAYCE was evaluated in phase 2 clinical studies in CF patients with *Pseudomonas* lung infections. We completed two randomized, placebo-controlled phase 2 studies with ARIKAYCE in 105 CF patients with chronic *Pseudomonas* lung infections in Europe and the US. In these studies, patients in the ARIKAYCE 560 mg cohort demonstrated statistically significant and clinically meaningful improvement in lung function throughout the 28-day on-treatment period compared with placebo. In addition, the improvement in lung function that was achieved at the end of the 28-day on-treatment period was sustained during the 28-day off-treatment period and was statistically significantly better than placebo.

In a separate follow-on open-label, multi-cycle clinical trial conducted in Europe, ARIKAYCE was given at a dose of 560 mg once daily via an eFlow Nebulizer System for six cycles which consisted of a 28-day on-treatment and 56-day off-treatment period, which is double the standard 28-day off-treatment period. In this clinical study, ARIKAYCE produced a statistically significant improvement in lung function that was sustained over the six cycles (approximately 17 months). In addition, approximately 50% to 70% of the benefit achieved during the 28-day on-treatment periods was sustained at the end of the 56-day off-treatment periods. In other words, ARIKAYCE demonstrated sustained efficacy in lung function improvement during the treatment and off-treatment periods across multiple cycles of therapy. To our knowledge, no other inhaled antibiotic has shown sustained improvement in lung

Table of Contents

function at the end of a 56-day off-treatment period. In addition, ARIKAYCE was well tolerated with overall adverse events reported as consistent with those expected in a population of CF patients receiving other inhaled medicines.

In August 2011, we announced that the FDA placed a clinical hold on our phase 3 trial for ARIKAYCE in CF patients with *Pseudomonas* lung infections, which was lifted in May 2012. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical trial or suspend an ongoing clinical trial. The FDA based its clinical hold decision on an initial review of the results of a long-term rat inhalation carcinogenicity study with ARIKAYCE. When rats were given ARIKAYCE daily by inhalation for two years, 2 of the 120 rats receiving the highest dose developed lung tumors. These rats received ARIKAYCE doses that were within two-fold of those in clinical studies (normalized on a body surface area basis or a lung weight basis). ARIKAYCE was not associated with changes that may lead to tumors in shorter-term studies in animals. Additionally, ARIKAYCE was not shown to be genotoxic in our standard series of tests. The relevance of the observed rat tumors to the use of ARIKAYCE in humans is not known.

In connection with the FDA s decision to lift the clinical hold for the CF *Pseudomonas aeruginosa* lung infection indication, we agreed to conduct a 9 month dog inhalation toxicity study of ARIKAYCE. In 2013, we concluded the 9 month dog inhalation toxicity study. In summary, the final report from the study stated that the lung macrophage response in dogs was similar to that seen in our previous 3 month dosing dog study, and there was no evidence of neoplasia, squamous metaplasia or proliferative changes.

We currently do not plan to initiate any further studies in *Pseudomonas* lung infections.

Strategy for Commercialization

We currently plan to retain marketing rights for ARIKAYCE for CF patients with *Pseudomonas* lung infections. We believe ARIKAYCE will require a limited commercial infrastructure because of the small focused nature of the potential physician prescribing population for CF patients. We may seek to out-license ARIKAYCE in certain countries in Europe, as well as outside of Europe, Canada and the US.

ARIKAYCE for Non-CF Bronchiectasis Patients with *Pseudomonas* Lung Infections

Overview of Non-CF Bronchiectasis and Pseudomonas Lung Infections

We believe ARIKAYCE has the potential to be used to treat non-CF bronchiectasis characterized by *Pseudomonas* lung infections. However, we are currently concentrating our development efforts on the treatment of *Pseudomonas* lung infections in CF patients and patients with NTM lung infections.

Non-CF bronchiectasis is a serious pulmonary condition characterized by localized, irreversible enlargement of the bronchial tubes. Accumulation of mucus in the bronchi leads to frequent infections, which causes inflammation and further reduces lung function. Patients evolve to a chronic inflammation-infection cycle. Disease burden has primarily been linked to productive cough and high levels of sputum production.

Market

It is estimated that there are more than 250,000 non-CF bronchiectasis patients in the US (SDI Innovations in Healthcare Analytics, 2008), of which approximately 30% of non-CF bronchiectasis patients are infected with *Pseudomonas* (Wilson, C.B., et al., Eur Respir, 1997, 10(8):1754-1760); Nicotra, M.B., et al., Chest, 1995 108(4):955-961). Currently there are no approved antibiotics for this indication. When bronchiectasis patients become infected with *Pseudomonas*, they tend to have more frequent exacerbations and hospitalizations and are more frequent users of antibiotics.

Table of Contents

Development Program

In May 2009 we completed our randomized, placebo controlled US phase 2 study (TR02-107) of ARIKAYCE in the treatment of chronic *Pseudomonas* infection in non-CF patients with bronchiectasis. In the study, 64 study subjects were randomized (1:1:1) to receive ARIKAYCE 280 mg, ARIKAYCE 560 mg or a placebo on a daily basis during a 28-day on-treatment period. The subjects completed follow-up assessments at the end of a 28-day off-treatment period. This study provided initial evidence of safety, tolerability and clinically meaningful improvement in pulmonary function throughout the on-treatment period in the treatment of chronic *Pseudomonas* infection in non-CF patients with bronchiectasis.

In the study both ARIKAYCE 280 mg and ARIKAYCE 560 mg were well tolerated. The adverse events experienced by patients during the study were consistent with underlying chronic lung disease in bronchiectasis patients. There was no evidence of renal toxicity or ototoxicity. Patients in the 560-mg cohort had a slightly higher frequency of dry cough post administration than patients in the 280 mg cohort. Cough was of short duration and self-limiting. One patient discontinued treatment due to dysphonia (hoarseness or difficulty speaking) and cough.

There was a statistically significant reduction in *Pseudomonas* density observed in the 560 mg ARIKAYCE cohort relative to the placebo cohort. Patients receiving ARIKAYCE experienced fewer pulmonary exacerbations at a rate of 4.7%, as compared to 10.5% in those receiving placebo. No patients in the ARIKAYCE cohorts required anti-*Pseudomonas* rescue treatment, whereas 15% of patients in the placebo cohort required treatment. Hospitalization from any cause occurred at a 5.3% rate for patients in the placebo cohort, as compared to a 2.3% rate for patients in the ARIKAYCE cohort. Patients receiving ARIKAYCE achieved improvements in patient respiratory symptoms and quality of life assessments compared with patients receiving placebo.

Although we believe there is an opportunity to develop ARIKAYCE for non-CF bronchiectasis, we currently do not intend to initiate further clinical studies with respect to a non-CF bronchiectasis indication.

ARIKAYCE has been granted orphan drug status in the US for the treatment of bronchiectasis in patients with *Pseudomonas* aeruginosa and other susceptible microbial pathogens.

Optimized eFlow Nebulizer System

If approved for commercialization, we expect that ARIKAYCE will be administered once daily via inhalation using an eFlow Nebulizer System optimized specifically for ARIKAYCE by PARI, a third-party vendor.

The optimized eFlow Nebulizer System is a medical device that uses PARI s patented eFlow technology to enable highly efficient delivery of inhaled medication, also called aerosolization, including liposomal formulations via a vibrating, perforated membrane that includes thousands of specially designed laser-drilled holes, which aids the delivery of ARIKAYCE to the lung. We believe the optimized eFlow Nebulizer System is state of the art and highly efficient. The eFlow Nebulizer System delivers a very high density of active drug, in a precisely defined and

controlled droplet size, with a high proportion of respirable droplets delivered in a relatively short period of time. In addition, the eFlow Nebulizer System has a quiet mode of operation, is small in size, light weight and provides for optional battery-powered operation. We believe that using the eFlow Nebulizer System to deliver ARIKAYCE will reduce treatment time and ease the patient s treatment burden and thereby potentially improve patient compliance. We believe that improved compliance with the prescribed treatment regimen may lead to a reduction in the development of antibiotic resistance by increasing the exposure of the infection to the minimum inhibitory concentration of antibiotic and therefore may ultimately lead to clinical benefit.

MANUFACTURING OF ARIKAYCE

The ARIKAYCE used in our clinical studies is manufactured for us by Ajinomoto Althea, Inc. (Althea), a third-party contract manufacturing organization in the US. We are working with Althea to develop commercial

Table of Contents

production capabilities for ARIKAYCE. Our agreement with Althea provides for a term expiring in July 2014, subject to an earlier termination upon the provision of 180 days notice by either party, or in the event of an uncured material breach, certain bankruptcy or liquidation events, or upon the occurrence of certain other specified termination events. We are negotiating with Althea to extend the manufacture of ARIKAYCE at Althea beyond July 2014. There can be no assurance that we will enter into an agreement to extend the manufacture or that we will enter into an agreement on terms favorable to us.

In February 2014, we entered into a contract manufacturing agreement with Therapure for the manufacture of ARIKAYCE at the larger scales necessary to support commercialization. Pursuant to the agreement, the Company and Therapure are collaborating to construct a production area for the manufacture of ARIKAYCE in Therapure s existing manufacturing facility in Mississauga, Ontario, Canada. Therapure will manufacture ARIKAYCE for us on a non-exclusive basis. The agreement has an initial term of five years from the first date on which Therapure delivers ARIKAYCE to us after we obtain permits related to the manufacture of ARIKAYCE.

We are also exploring the possibility of establishing our own manufacturing facilities in order to support clinical studies and commercial supply of ARIKAYCE.

All sites of manufacture of ARIKAYCE use the technology developed and optimized by us. We and all our manufacturing partners must comply with applicable regulations relating to the current good manufacturing practices (cGMP) regulations of regulatory agencies. The cGMP regulations include requirements relating to the organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports, and returned or salvaged products. We believe that all facilities will meet cGMP requirements for the sterile manufacturing of finished ARIKAYCE product.

The eFlow nebulizer system is manufactured by PARI under the names PARI Pharma GmbH in Europe and PARI Respiratory Equipment, Inc., in the US. PARI manufactures eFlow nebulizer systems utilizing technology licensed, developed and optimized within its company and produces several commercially available eFlow technology based products for use in Europe, North America and other countries. PARI maintains facilities and equipment necessary to support manufacture of eFlow nebulizers for use with ARIKAYCE. PARI must comply with applicable governmental regulations relating to medical device production in each country of manufacture. We will continue to work with PARI to address our manufacturing needs for our clinical program. In July 2014, we entered into a commercialization agreement with PARI, the manufacturer of our drug delivery nebulizer, to address our commercial supply needs.

We seek to maintain the quality of our suppliers through quality agreements and our vendor audit program.

IPLEX

In addition to the ARIKAYCE development program, we have a second proprietary compound, IPLEX®, which is IGF-1, with its natural binding protein, IGFBP-3. IPLEX is no longer a development priority for us. We no longer have protein development capability or the in-house capability to manufacture IPLEX. Previously, under the proprietary IPLEX protein platform, we maintained an expanded access program for amyotrophic lateral sclerosis (also known as ALS or Lou Gehrig s disease) until drug supplies were exhausted at the end of 2011. It is our intention to seek licensing partners for the IPLEX development programs. In 2012, we out-licensed the IPLEX technology to Premacure

Holdings AB and Premacure AB of Sweden (collectively, Premacure) for retinopathy of prematurity indication. In March 2013, we amended the Premacure License Agreement to provide Premacure with the option to pay us $11.5 million and assume any of our royalty obligations to other parties in exchange for a fully paid license. In March 2013, Shire plc announced that they acquired Premacure. In April 2013 Shire exercised this option and paid us $11.5 million, and as a result we are not entitled to future royalties from Shire.

Table of Contents

KEY COMPONENTS OF OUR STATEMENT OF OPERATIONS

Revenues

We currently do not recognize any revenue from product sales or other sources.

Research and Development Expenses

Research and development expenses consist primarily of salaries, benefits and other related costs, including stock-based compensation, for personnel serving in our research and development functions, and other internal operating expenses, the cost of manufacturing our drug candidate for clinical study, the cost of conducting clinical studies, and the cost of conducting preclinical and research activities. Our expenses related to manufacturing our drug candidate for clinical study are primarily related to activities at contract manufacturing organizations that manufacture ARIKAYCE for our use. Our expenses related to clinical trials are primarily related to activities at contract research organizations that conduct and manage clinical trials on our behalf. These contracts set forth the scope of work to be completed at a fixed fee or amount per patient enrolled. Payments under these contracts primarily depend mainly on performance criteria such as the successful enrollment of patients or the completion of clinical trial milestones as well as time-based fees. Expenses are accrued based on contracted amounts applied to the level of patient enrollment and to activity according to the clinical trial protocol. Nonrefundable advance payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are then recognized as an expense as the related goods are delivered or the services are performed, or when the goods or services are no longer expected to be provided.

Since 2011, we have focused our development activities principally on our proprietary, advanced liposomal technology designed specifically for inhalation lung delivery. In the first half of 2014, we reported top-line clinical results from our phase 2 clinical trial in the US and Canada of ARIKAYCE in patients who have lung infections caused by NTM. In 2013, we completed a phase 3 trial in Europe and Canada in which we evaluated ARIKAYCE in CF patients with *Pseudomonas* lung infections. We are currently conducting two clinical trials: (i) the completion of the open-label portion of a phase 2 trial in the US in which we are evaluating ARIKAYCE for NTM infections and (ii) an open label extension study in which CF patients that completed our phase 3 trial receive ARIKAYCE for a period of two years. Since our business combination with Transave, the majority of our research and development expenses have been for our ARIKAYCE program. We expect that our development efforts in 2014 will principally relate to the development of ARIKAYCE in the CF and NTM indications.

Our clinical trials with ARIKAYCE are subject to numerous risks and uncertainties that are outside of our control, including the possibility that necessary regulatory approvals may not be obtained. In addition, the duration and the cost of clinical trials may vary significantly from trial to trial over the life of a project as a result of differences in the study protocol for each trial as well as differences arising during the clinical trial, including, among others, the following:

- The number of patients that ultimately participate in the trial;
- The duration of patient follow-up that is determined to be appropriate in view of results;

- The number of clinical sites included in the trials;
- The length of time required to enroll suitable patient subjects; and
- The efficacy and safety profile of the product candidate.

Our clinical trials may be subject to delays, particularly if we are unable to produce clinical trial material in sufficient quantities and of sufficient quality to meet the schedule for our clinical trials. Moreover, all of our product candidates must overcome significant regulatory, technological, manufacturing and marketing challenges before they can be successfully commercialized. Any significant delays that occur or additional expenses that we incur may have a material adverse effect on our financial position and may require us to raise additional capital sooner or

Table of Contents

in larger amounts than is presently expected. In addition, as a result of the risks and uncertainties related to the development and approval of our product candidates and the additional uncertainties related to our ability to market and sell these products once approved for commercial sale, we are unable to provide a meaningful prediction regarding when, if at all, we will generate positive cash inflow from these projects.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries, benefits and other related costs, including stock-based compensation, for personnel serving in our executive, finance and accounting, legal, pre-commercial, corporate development, information technology, program management and human resource functions. General and administrative expenses also include professional fees for legal, including patent-related expenses, consulting, insurance, board of director fees, tax and accounting services. We expect that our general and administrative expenses will increase in order to support increased levels of development activities and commencement of commercialization activities for our product candidates.

Debt Issuance Costs

Debt issuance costs are amortized to interest expense using the effective interest rate method over the term of the debt. Our balance sheet reflects debt net of debt issuance costs paid to the lender and reflects debt issuance costs paid to other third parties as other assets.

Investment Income and Interest Expense

Investment income consists of interest and dividend income earned on our cash, cash equivalents and short-term investments, along with realized gains (losses) on the sale of investments. Interest expense consists primarily of interest costs related to our debt and capital lease obligations.

RESULTS OF OPERATIONS

Comparison of the Three Months Ended June 30, 2014 and 2013

Net Loss

Net loss for the three months ended June 30, 2014 was $23.2 million, or ($0.59) per common share basic and diluted, compared with a net loss of $8.9 million, or ($0.28) per common share basic and diluted for the three months ended June 30, 2013. The $14.3 million increase in our net

loss in the second quarter of 2014 as compared to 2013 was primarily due to $11.5 million in Other revenue received during the three months ended June 30, 2013 related to a one-time payment for the sale of the Company s right to receive future royalties under its license agreement with Premacure (now Shire plc). An increase in expenses also contributed to the increase in net loss for the period and included:

- A $2.7 million increase in our research and development expenses that primarily resulted from an increase in internal expenses, specifically compensation and personnel related expenses, including non-cash stock compensation expense, and an increase in manufacturing expenses as a result of the build-out of a production and quality control area at Therapure s facility. These increases were offset, in part, by a decrease in external clinical expenses which was primarily related to the fact that our phase 3 pivotal study in CF patients was completed in 2013; and

- A $0.4 million increase in our general and administrative expenses primarily resulted from an increase in pre-commercial activities and an increase in personnel costs due to an increase in headcount, offset in part by a decrease in non-cash stock compensation expense.

Table of Contents

Other Revenue

Other revenue during the three months ended June 30, 2013 consists solely of a one-time $11.5 million payment we received from Premacure (now Shire plc) in exchange for the Company s right to receive future royalties under its license agreement with Premacure (see Note 11. License and Collaboration Agreements to the consolidated financial statements on Form 10-K for the year ended December 31, 2013 for additional information regarding our agreement with Premacure). We recorded this as Other revenue during the three months ended June 30, 2013, since all revenue recognition criteria were met and we had no continuing performance obligations related to the payment received.

Research and Development Expenses

Research and development expenses for the three months ended June 30, 2014 and 2013 comprised the following:

	Three Months Ended June 30,		Increase (Decrease)	
	2014	2013	$	%
External Expenses				
Clinical Development	$ 2,395	$ 5,102	$ (2,707)	-53.1%
Manufacturing	4,687	2,594	2,093	80.7%
Regulatory and Quality Assurance	2,025	684	1,341	196.1%
Subtotal - external expenses	$ 9,107	$ 8,380	$ 727	8.7%
Internal Expenses				
Compensation and related expenses	$ 4,432	$ 2,835	$ 1,597	56.3%
Other internal operating expenses	1,403	1,010	393	38.9%
Subtotal - internal expenses	$ 5,835	$ 3,845	$ 1,990	51.8%
Total	$ 14,942	$ 12,225	$ 2,717	22.2%

Research and development expenses increased to $14.9 million during the three months ended June 30, 2014 from $12.2 million in the same period in 2013. The $2.7 million increase was primarily due to a $2.0 million increase in internal expenses, specifically a $1.6 million increase in compensation and related expenses, which included additional expenses related to the transition and consulting agreement with our former chief medical officer. In addition, there was a $2.1 million increase in manufacturing expenses as a result of the build-out of a production area at Therapure s facility. These increases were offset, in part, by a decrease of $2.7 million in external clinical expenses which was primarily related to the fact that our phase 3 pivotal study in CF patients was completed in 2013.

General and Administrative Expenses

General and administrative expenses increased to $7.9 million during the three months ended June 30, 2014 from $7.5 million in the same period in 2013. The $0.4 million increase was primarily due to an increase in pre-commercial expenses, as there were minimal pre-commercial activities in the second quarter of 2013, and an increase in personnel costs due to an increase in headcount. These increases were almost completely offset by a $1.4 million decrease in non-cash stock compensation expense in the three months ended June 30, 2014 as compared to the same period in 2013.

Investment Income and Interest Expense

Investment income decreased to $0.0 million during the three months ended June 30, 2014 from $0.1 million in the same period in 2013. Interest expense was $0.6 million during the three months ended June 30, 2014 and 2013 and represents interest expense under our Loan Agreement.

Table of Contents

Comparison of the Six Months Ended June 30, 2014 and 2013

Net Loss

Net loss for the six months ended June 30, 2014 was $37.5 million, or ($0.96) per common share basic and diluted, compared with a net loss of $22.5 million, or ($0.71) per common share basic and diluted for the six months ended June 30, 2013. The $15.0 million increase in our net loss in the six months ended June 30, 2014 as compared to 2013 was primarily due to $11.5 million in Other revenue received during the three months ended June 30, 2013 related to a one-time payment for the sale of the Company s right to receive future royalties under its license agreement with Premacure (now Shire plc). An increase in expenses also contributed to the increase in net loss for the period and included:

- A $3.7 million increase in our research and development expenses that primarily resulted from an increase in internal expenses, specifically compensation and personnel related expenses, including non-cash stock compensation expense. In addition, there was an increase in manufacturing expenses as a result of the build-out of a production area at Therapure s facility and an increase as a result of the completion of certain process improvement projects at our third party manufacturing partner and the manufacture of ARIKAYCE for clinical supply. These increases were offset, in part, by a decrease in external clinical expenses which was primarily related to the fact that our phase 3 pivotal study in CF patients was completed in 2013; and

- A $3.1 million increase in our general and administrative expenses primarily resulted from an increase in pre-commercial expenses and an increase in personnel costs due to an increase in headcount, offset in part by a decrease in non-cash stock compensation expense.

Offsetting these expenses was a $3.2 million increase in the benefit from income taxes resulting from the sale of a portion of our New Jersey State NOLs under the State of New Jersey s Technology Business Tax Certificate Transfer Program for cash of $4.4 million and $1.2 million in 2014 and 2013, respectively, and net of commissions.

Other Revenue

Other revenue during the six months ended June 30, 2013 solely consists of a one-time $11.5 million payment we received from Premacure (now Shire plc) in exchange for the Company s right to receive future royalties under its license agreement with Premacure (see Note 11. License and Collaboration Agreements to the consolidated financial statements on Form 10-K for the year ended December 31, 2013 for additional information regarding our agreement with Premacure). We recorded this as Other revenue during the three months ended June 30, 2013, since all revenue recognition criteria were met and we had no continuing performance obligations related to the payment received.

Research and Development Expenses

Research and development expenses for the six months ended June 30, 2014 and 2013 comprised the following:

Table of Contents

	Six Months Ended June 30,		Increase (Decrease)	
	2014	2013	$	%
External Expenses				
Clinical Development	$ 5,479	$ 11,954	$ (6,475)	-54.2%
Manufacturing	7,548	3,156	4,392	139.2%
Regulatory and Quality Assurance	2,518	807	1,711	212.0%
Subtotal - external expenses	$ 15,545	$ 15,917	$ (372)	-2.3%
Internal Expenses				
Compensation and related expenses	$ 8,444	$ 4,642	$ 3,802	81.9%
Other internal operating expenses	2,304	2,000	304	15.2%
Subtotal - internal expenses	$ 10,748	$ 6,642	$ 4,106	61.8%
Total	$ 26,293	$ 22,559	$ 3,734	16.6%

Research and development expenses increased to $26.3 million during the six months ended June 30, 2014 from $22.6 million in the same period in 2013. The $3.7 million increase was primarily due to a $4.1 million increase in internal expenses, specifically a $3.8 million increase in compensation and related expenses, including a $1.0 million increase in non-cash stock compensation expense. In addition, there was a $4.4 million increase in manufacturing expenses as a result of the build-out of a production area at Therapure s facility and an increase in manufacturing expenses as a result of the completion of certain process improvement projects at our third party manufacturing partner and the manufacture of ARIKAYCE for clinical supply. These increases were offset, in part, by a decrease of $6.5 million in external clinical expenses which was primarily related to the fact that our phase 3 pivotal study in CF patients was completed in 2013.

General and Administrative Expenses

General and administrative expenses increased to $14.6 million during the six months ended June 30, 2014 from $11.5 million in the same period in 2013. The $3.1 million increase was primarily due to an increase in pre-commercial expenses, as there were minimal pre-commercial activities in the first half of 2013, and an increase in personnel costs due to an increase in headcount. These increases were offset, in part, by a $0.6 million decrease in non-cash stock compensation expense in the six months ended June 30, 2014 as compared to the same period in 2013.

Investment Income and Interest Expense

Investment income decreased to $0.0 million during the six months ended June 30, 2014 from $0.1 million in the same period in 2013. Interest expense under our Loan Agreement of $1.2 million for the six months ended June 30, 2014 was relatively consistent compared to the same period in 2013.

Benefit from Income Taxes

The benefit for income taxes was $4.4 million and $1.2 million for the six months ended June 30, 2014 and 2013, respectively. The benefit for income taxes recorded for the six months ended June 30, 2014 and 2013 solely reflect the reversal of a valuation allowance previously recorded against our New Jersey State net operating losses (NOLs) that resulted from the sale of a portion of our New Jersey State NOLs under the State of New Jersey s Technology Business Tax Certificate Transfer Program (the Program) for cash of $4.4 million and $1.2 million, respectively and net of commissions. The Program allows qualified technology and biotechnology businesses in New Jersey to sell unused amounts of NOLs and defined research and development tax credits for cash.

Table of Contents

LIQUIDITY AND CAPITAL RESOURCES

Overview

There is considerable time and cost associated with developing a potential drug or pharmaceutical product to the point of regulatory approval and commercialization. Historically, we have funded our operations through public and private placements of equity securities, through debt financing, from the proceeds from the sale of our follow-on biologics platform to Merck in 2009, from revenues related to sales of product and secondary revenue streams from our IPLEX expanded access program in Europe, which was discontinued in 2011 and revenue related to a one-time payment received in exchange for our right to receive royalties under our license agreement with Premacure (now Shire plc) in 2013. We expect to continue to incur losses because we plan to fund research and development activities and commercial launch activities, and we do not expect material revenues for at least the next few years.

We will need to raise capital during the next twelve months and may do so through equity or debt financing(s), strategic transactions or otherwise. Such additional funding may be necessary to continue to develop our potential product candidates, to pursue the license or purchase of other technologies, to commercialize our product candidates or to purchase other products. We cannot assure you that adequate capital will be available on favorable terms, or at all, when needed. If we are unable to obtain sufficient additional funds when required, we may be forced to delay, restrict or eliminate all or a portion of our research or development programs, dispose of assets or technology or cease operations. During 2014 we plan to continue to fund further clinical development of ARIKAYCE, increase our investment in third-party manufacturing capacity, support efforts to obtain regulatory approvals and prepare for commercialization. We estimate that our cash requirements to fund operations during the second half of 2014 will be in the range of $42 million to $47 million, which includes additional expenditures of $6 million to $8 million for third party manufacturing capacity. In addition, in the second half of 2014 we expect to invest an additional $2.5 million to $3.5 million in capital expenditures and pay current liabilities of approximately $2.5 million related to the build out of our new headquarters and laboratory facilities in Bridgewater, New Jersey. We relocated the majority our operations to the Bridgewater facility in June 2014 and the lease for our Monmouth Junction operating facility expires in December 2014.

Cash Flows

As of June 30, 2014, we had total cash and cash equivalents $82.7 million, as compared with $113.9 million as of December 31, 2013. Our working capital was $64.9 million as of June 30, 2014.

Net cash used in operating activities was $30.6 million and $16.3 million for the six months ended June 30, 2014 and 2013, respectively. Excluding the (i) $11.5 million one-time payment from Premacure in the six months ended June 30, 2013 and (ii) proceeds from the sale of a portion of our New Jersey State NOLs under the State of New Jersey s Technology Business Tax Certificate Transfer Program of $4.4 million and $1.2 million in 2014 and 2013, respectively, net cash used in operating activities for the first half of 2014 and 2013 would have been $35.0 million and $29.0 million, respectively. The net cash used in operating activities during the six months ended June 30, 2014 and 2013 was primarily for the clinical development of ARIKAYCE.

Net cash used in investing activities was $0.9 million and $0.5 million for the six months ended June 30, 2014 and 2013, respectively. The net cash used in investing activities in 2014 included $0.6 million for the build out of our new headquarters in Bridgewater, New Jersey and other

fixed asset purchases of $0.3 million for computers and lab equipment. The net cash used in investing activities in 2013 was for fixed asset purchases for lab and computer equipment.

Net cash provided by financing activities was $0.3 million and $0.6 million for the six months ended June 30, 2014 and 2013, respectively. Net cash provided by financing activities in both periods were primarily proceeds from stock option exercises.

Table of Contents

Contractual Obligations

On June 29, 2012, we and our domestic subsidiaries, as co-borrowers, entered into a Loan and Security Agreement with Hercules Technology Growth Capital, Inc. (Hercules) that allowed us to borrow up to $20.0 million in $10.0 million increments (Loan Agreement). We borrowed the first and second $10.0 million increments by signing two Secured Promissory Notes (Note A and Note B and collectively, the Notes) on June 29, 2012 and December 27, 2012, respectively. Notes A and B bear interest at 9.25%. Note A was originally scheduled to be repaid over a 42-month period with the first twelve monthly payments representing interest only followed by thirty monthly equal payments of principal and interest. Note B was originally scheduled to be repaid over a 36-month period with the first six monthly payments representing interest only followed by thirty monthly equal payments of principal and interest. The Loan Agreement provided that in certain circumstances we could delay the first principal payment by five months. In July 2013, subsequent to the completion of certain ARIKAYCE-related development milestones, we elected to extend the interest only period under the Notes from July 31, 2013 to December 31, 2013 and delay the first monthly principal repayments for the Notes from August 1, 2013 to January 1, 2014. On November 25, 2013, the Company and Hercules entered into an amendment (the Amendment) to the Loan Agreement. The Amendment initially extended the interest-only period through June 30, 2014 and called for the first monthly principal payment on July 1, 2014. The Amendment also allowed us to further extend the interest-only period through December 31, 2014 and delay the first payment of principal until January 1, 2015, so long as we paid a $100,000 fee and obtained positive data from our phase 2 clinical trial of ARIKAYCE in patients who have lung infections caused by NTM. In June 2014, we paid the $100,000 fee and exercised our option to extend the interest-only period and delay the first payment of principal to January 1, 2015. The election and amendment did not change the maturity date for Notes A and B, which is January 1, 2016. In connection with the Loan Agreement, we granted the lender a first position lien on all of our assets, excluding intellectual property. Prepayment of the loans made pursuant to the Loan Agreement is subject to penalty and we are required to pay an end of term charge of $390,000.

We have two operating leases for office and laboratory space located in Monmouth Junction, New Jersey that expire on December 31, 2014. Future minimum rental payments under these two leases total approximately $0.4 million. We also have an operating lease for office and laboratory space located in Bridgewater, NJ that expires in November 2019. Future minimum rental payments under this lease total approximately $3.6 million. We continue to lease office space in Richmond, Virginia where our corporate headquarters was once located. Future minimum rental payments under this lease total approximately $1.2 million.

As of June 30, 2014, future payments under the two promissory notes, the capital leases and minimum future payments under non-cancellable operating leases are as follows:

Table of Contents

	As of June 30, 2014 Payments Due By Period				
	Total	Less than 1 year	1-3 Years	4-5 Years	After 5 Years
			(In thousands)		
Debt obligations:					
Debt maturities	$ 20,000	$ 3,630	$ 16,370	$	$
Contractual interest	2,602	1,806	796		
Capital lease obligations:					
Debt maturities	32	32			
Contractual interest					
Operating leases	5,151	1,182	2,114	1,525	330
Purchase obligations					
Total contractual obligations	$ 27,785	$ 6,650	$ 19,280	$ 1,525	$ 330

This table does not include (i) any milestone payments which may become payable to third parties under our license and collaboration agreements as the timing and likelihood of such payments are not known, (ii) any royalty payments to third parties as the amounts of such payments, timing and/or the likelihood of such payments are not known, (iii) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above, or (iv) any payments related to the agreements mentioned below.

We currently have a licensing agreement with PARI for use of the optimized eFlow Nebulizer System for delivery of ARIKAYCE in treating patients with CF, bronchiectasis and NTM infections. We have rights to several US and foreign issued patents, and patent applications involving improvements to the optimized eFlow Nebulizer System. Under the licensing agreement, PARI is entitled to receive payments either in cash, qualified stock or a combination of both, at PARI s discretion, based on achievement of certain milestone events including phase 3 trial initiation (which occurred in 2012), first acceptance of MAA submission (or equivalent) in the US of ARIKAYCE and the device, first receipt of marketing approval in the US for ARIKAYCE and the device, and first receipt of marketing approval in a major EU country for ARIKAYCE and the device, and NDA acceptance and regulatory approval of ARIKAYCE. In addition, PARI is entitled to receive royalty payments on commercial sales of ARIKAYCE pursuant to the licensing agreement. In July 2014, we entered into a commercialization agreement with PARI, the manufacturer of our drug delivery nebulizer, to address our commercial supply needs.

In 2004 and 2009, we entered into research funding agreements with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) whereby we received $1.7 million and $2.2 million for each respective agreement in research funding for the development of ARIKAYCE. If ARIKAYCE becomes an approved product for CF patients in the US, we will owe a payment to CFFT of up to $13.4 million that is payable over a three-year period after approval as a commercialized drug in the US. Furthermore, if certain sales milestones are met within 5 years of the drug commercialization approval in the US, we would owe an additional $3.9 million in additional payments. Since there is significant development risk associated with ARIKAYCE, we have not accrued these obligations.

In 2009 and 2012, we entered into a cooperative research and development agreement (CRADA) with the National Institute of Allergy and Infectious Diseases (NIAID) to design and conduct our phase 2 study of ARIKAYCE in patients with NTM. NIAID has also agreed to provide biostatistical advisory input in connection with the phase 2 NTM study. If we decide not to continue with the commercialization of ARIKAYCE in NTM, NIAID will have the right to complete the clinical trial. Further NIAID may elect to pursue its rights to obtain license rights to certain inventions made under the CRADA.

Table of Contents

In February 2014, we entered into a contract manufacturing agreement with Therapure for the manufacture of ARIKAYCE at the larger scales necessary to support commercialization. Pursuant to the agreement, the Company and Therapure are collaborating to construct a production area for the manufacture of ARIKAYCE in Therapure s existing manufacturing facility in Mississauga, Ontario, Canada. We expect to pay Therapure approximately $12 million for the build out of the construction area and related manufacturing costs, of which approximately $5 million has been paid as of June 30, 2014. Therapure will manufacture ARIKAYCE for us on a non-exclusive basis. The agreement has an initial term of five years from the first date on which Therapure delivers ARIKAYCE to us after we obtain permits related to the manufacture of ARIKAYCE.

Future Funding Requirements

We will need to raise capital within the next twelve months to fund our operations, to develop and commercialize ARIKAYCE and to develop, acquire, in-license or co-promote other products that address orphan or rare diseases in the fields of pulmonology or infectious disease. Our future capital requirements may be substantial and will depend on many factors, including:

- The decisions of the FDA and EMA with respect to our applications for marketing approval of ARIKAYCE in the US and Europe; the costs of activities related to the regulatory approval process; and the timing of approvals, if received;

- The timing and cost of our anticipated clinical trials of ARIKAYCE for the treatment of adult patients with CF or for the treatment of patients with NTM lung infections;

- The cost of putting in place the sales and marketing capabilities necessary to be prepared for a potential commercial launch of ARIKAYCE, if approved;

- The cost of filing, prosecuting and enforcing patent claims;

- The costs of our manufacturing-related activities;

- The costs associated with commercializing ARIKAYCE if we receive marketing approval; and

- Subject to receipt of marketing approval, the levels, timing and collection of revenue received from sales of approved products, if any, in the future.

In May 2014, we filed a shelf registration statement on Form S-3 with the Securities and Exchange Commission. This shelf registration statement permits us to offer, from time to time, shares of common stock. Our business strategy may require us to, or we may otherwise determine to, raise capital at any time through equity or debt financing(s), strategic transactions or otherwise. Such additional funding may be necessary to continue to develop our potential product candidates, to pursue the license or purchase of complementary technologies, to commercialize our product candidates or to purchase other products. If we are unable to obtain additional financing, we may be required to reduce the scope of our planned product development and commercialization or our plans to establish a sales and marketing force, any of which could harm our business, financial condition and results of operations. The source, timing and availability of any future financing will depend principally upon equity and debt market conditions, interest rates and, more specifically, our continued progress in our regulatory, development and commercial activities. We cannot assure you that such capital funding will be available on favorable terms or at all. If we are unable to obtain sufficient additional funds when required, we may be forced to delay, restrict or eliminate all or a portion of our research or development programs, dispose of assets or technology or cease operations.

To date, we have not generated any revenue from ARIKAYCE. We do not know when or if we will generate any revenue. We do not expect to generate significant revenue unless or until we obtain marketing approval of, and commercialize, ARIKAYCE.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements, other than operating leases, that have or are reasonably likely to have a current or future material effect on our financial condition, revenues or expenses, results

Table of Contents

of operations, liquidity, capital expenditures or capital resources. We do not have any interest in special purpose entities, structured finance entities or other variable interest entities.

CRITICAL ACCOUNTING POLICIES

Preparation of financial statements in accordance with generally accepted accounting principles in the US requires us to make estimates and assumptions affecting the reported amounts of assets, liabilities, revenues and expenses and the disclosures of contingent assets and liabilities. We use our historical experience and other relevant factors when developing our estimates and assumptions. We continually evaluate these estimates and assumptions. The amounts of assets and liabilities reported in our consolidated balance sheets and the amounts of revenue reported in our consolidated statements of comprehensive loss are effected by estimates and assumptions, which are used for, but not limited to, the accounting for research and development, revenue recognition, beneficial conversion charge, stock-based compensation, identifiable intangible assets and goodwill, and accrued expenses. The accounting policies discussed below are considered critical to an understanding of our consolidated financial statements because their application places the most significant demands on our judgment. Actual results could differ from our estimates. There have been no material changes to our critical accounting policies as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2013. For the required interim updates of our accounting policies see Note 2 to our Consolidated Financial Statements Summary of Significant Accounting Policies in this Quarterly Report on Form 10-Q.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As of June 30, 2014, our cash and cash equivalents were in cash accounts or were invested in money market funds. Such accounts or investments are not insured by the federal government.

As of June 30, 2014, we had $20.0 million of fixed rate borrowings in the form of two secured promissory notes that bear interest at 9.25% outstanding under a Loan and Security Agreement we entered into in June 2012. A hypothetical 10% change in interest rates occurring on June 30, 2014 would not have had a material effect on the fair value of our debt as of that date, nor would it have had a material effect on our future earnings or cash flows.

The majority of our business is conducted in US dollars. However, we do conduct certain transactions in other currencies, including Euros or British Pounds. Historically, fluctuations in foreign currency exchange rates have not materially affected our results of operation and during the three and six months ended June 30, 2014 and 2013, our results of operation were not materially affected by fluctuations in foreign currency exchange rates.

ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2014. The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934, as amended (the Exchange Act), means controls and other procedures that are designed to provide reasonable assurance that information required to be disclosed by us in the periodic reports that we file or submit with the SEC is recorded, processed, summarized and reported, within the time periods specified in the SEC s rules and forms, and to ensure that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate to allow timely decisions regarding required disclosure. Based on that evaluation as of June 30, 2014, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures are effective at the reasonable assurance level.

Table of Contents

Changes in Internal Control Over Financial Reporting

There were no changes in the Company s internal control over financial reporting (as defined in Rule 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended June 30, 2014 that have materially affected, or are reasonably likely to materially affect, the Company s internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

From time to time, we are a party to various other lawsuits, claims and other legal proceedings that arise in the ordinary course of our business. Management does not expect that the ultimate costs to resolve these matters will materially adversely affect our business, financial position, or results of operations.

See Note 10 to the consolidated financial statements for the three months ended June 30, 2014 included in this Quarterly Report on Form 10-Q, and Note 12 to the consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2013 for a description of our significant legal proceedings, which are incorporated by reference herein.

ITEM 1A. RISK FACTORS

Except for the historical information in this report on Form 10-Q, the matters contained in this report include forward-looking statements that involve risks and uncertainties. Our operating results and financial condition have varied in the past and may in the future vary significantly depending on a number of factors. These factors, among others, could cause actual results to differ materially from those contained in forward-looking statements made in this report and presented elsewhere by management from time to time. Such factors may have a material adverse effect upon our business, results of operations and financial condition.

You should consider carefully the risk factors, together with all of the other information included in our Annual Report on Form 10-K for the year ended December 31, 2013. Each of these risk factors could adversely affect our business, results of operations and financial condition, as well as adversely affect the value of an investment in our common stock. There have been no material changes to our risk factors as previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2013, except for the following update:

Risks Related to Development and Commercialization of our Product Candidates

Our near term prospects are highly dependent on the success of our most advanced product candidate, ARIKAYCE. If we are unable to successfully complete the development of, obtain regulatory approval for, and successfully commercialize ARIKAYCE, our business and the value of our common stock may be materially adversely affected.

We are investing substantially all of our efforts and financial resources in the development of ARIKAYCE, our most advanced product candidate. Our ability to generate product revenue from ARIKAYCE, which may not occur for at least the next year or two, if ever, will depend heavily on the successful completion of development of, receipt of regulatory approval for, and commercialization of, ARIKAYCE.

Positive results from preclinical studies of a drug candidate may not be predictive of similar results in human clinical trials, and promising results from earlier clinical trials of a drug candidate may not be replicated in later clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in earlier stages of development.

Table of Contents

Accordingly, the results of the completed clinical trials for ARIKAYCE may not be predictive of the results we may obtain in future or ongoing clinical trials. On March 26, 2014, we reported top-line clinical results from a phase 2 clinical trial in the US and Canada of ARIKAYCE in patients who have lung infections caused by NTM. ARIKAYCE did not meet the pre-specified level for statistical significance with respect to the primary endpoint although there was a positive trend (p=0.148) in favor of ARIKAYCE.

On August 4, 2014 we announced that we intend to file by the end of 2014 a MAA with the EMA for ARIKAYCE for the treatment of NTM lung infections in treatment refractory patients as well as for *Pseudomonas* lung infections in CF patients.

In addition we also announced that we plan to initiate two Phase 3 studies of the effectiveness of ARIKAYCE for the treatment of lung infections in the treatment refractory and broad NTM populations, respectively.

We do not expect ARIKAYCE or any other drug candidates we may develop to be commercially available for at least a year, if at all.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

There were no unregistered sales of the Company s equity securities during the quarter ended June 30, 2014.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

On August 1, 2014, Matthew Pauls, the Company s Chief Commercial Officer (CCO), notified the Company of his resignation. The Company plans to initiate a search to identify a new CCO and in the interim all CCO responsibilities will be shared by the Company s management team.

ITEM 6. EXHIBITS

A list of exhibits filed herewith is included on the Exhibit Index, which immediately precedes such exhibits and is incorporated herein by reference.

Table of Contents

SIGNATURE

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.

INSMED INCORPORATED

Date: August 6, 2014

By /s/ Andrew T. Drechsler
Andrew T. Drechsler
Chief Financial Officer

Table of Contents

EXHIBIT INDEX

31.1 Certification of William H. Lewis, Chief Executive Officer of Insmed Incorporated, pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes Oxley Act of 2002.

31.2 Certification of Andrew T. Drechsler, Chief Financial Officer of Insmed Incorporated, pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes Oxley Act of 2002.

32.1 Certification of William H. Lewis, Chief Executive Officer of Insmed Incorporated, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes Oxley Act of 2002.

32.2 Certification of Andrew T. Drechsler, Chief Financial Officer of Insmed Incorporated, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes Oxley Act of 2002.

101.INS XBRL Instance Document

101.SCH XBRL Taxonomy Extension Schema Document

101.CAL XBRL Taxonomy Extension Calculation Linkbase Document

101.DEF XBRL Taxonomy Extension Definition Linkbase Document

101.LAB XBRL Taxonomy Extension Label Linkbase Document

101.PRE XBRL Taxonomy Extension Presentation Linkbase Document