# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

# Form 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2014

OR

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

**Commission File Number 001-33038** 

# **ZIOPHARM Oncology, Inc.**

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization) 84-1475642 (I.R.S. Employer Identification No.)

One First Avenue, Parris Building 34, Navy Yard Plaza Boston, Massachusetts 02129 (617) 259-1970 (Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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:  $\square$  No:  $\square$ 

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 during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes: 🛛 No: 🗆

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

 Large accelerated filer
 Accelerated filer
 Accelerated filer
 Image: Comparison of the comparison of

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# NOTE REGARDING FORWARD-LOOKING STATEMENTS

This report contains forward-looking statements that are based on our current beliefs and expectations. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "estimate," "forecast," "intend," "may," "plan," "project," "target," "will" and other words and terms of similar meaning. Reference is made in particular to forward-looking statements regarding:

- the anticipated amount, timing and accounting of deferred revenues, milestone and other payments under licensing, collaboration or acquisition agreements, research and development costs and other expenses;
- the protection afforded by our patent rights;
- our assessment of the potential impact on our future revenues of healthcare reform legislation in the United States;
- the timing and impact of measures worldwide designed to reduce healthcare costs;
- the impact of the deterioration of the credit and economic conditions in certain countries in Europe;
- our ability to finance our operations and business initiatives and obtain funding for such activities;
- the sufficiency of our cash, investments and cash flows from operations and our expected uses of cash;
- the costs and timing of the development and commercialization of our pipeline products and services;
- additional planned regulatory filings for and commercialization of our synthetic biology product candidates and Palifosfamide; and
- contract manufacturing activity.

These forward-looking statements involve risks and uncertainties, including those that are described in the "*Risk Factors*" section of this report and elsewhere within this report that could cause actual results to differ materially from those reflected in such statements. You should not place undue reliance on these statements. Forward-looking statements speak only as of the date of this report. We do not undertake any obligation to publicly update any forward-looking statements.

# NOTE REGARDING COMPANY REFERENCES

Throughout this report, "ZIOPHARM," the "Company," "we," "us" and "our" refer to ZIOPHARM Oncology, Inc.

# NOTE REGARDING TRADEMARKS

All trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

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# Part I - Financial Information

Item 1. Consolidated Financial Statements

# ZIOPHARM Oncology, Inc.

# BALANCE SHEETS (unaudited)

# (in thousands, except share and per share data)

|   |           | December 31,<br>2013 |  |
|---|-----------|----------------------|--|
| ASSETS  |           |                      |  |
| Current assets:   |           |                      |  |
| Cash and cash equivalents   | \$ 53,001 | \$ 68,204            |  |
| Receivables   | 32        | 145                  |  |
| Prepaid expenses and other current assets   | 991       | 1,948                |  |
| Total current assets  | 54,024    | 70,297               |  |
| Property and equipment, net   | 683       | 801                  |  |
| Deposits  | 128       | 128                  |  |
| Other non-current assets  | 513       | 528                  |  |
| Total assets  | \$ 55,348 | \$ 71,754            |  |
| LIABILITIES AND STOCKHOLDERS' EQUITY  |           |                      |  |
| Current liabilities:  |           |                      |  |
| Accounts payable  | \$ 447    | \$ 422               |  |
| Accrued expenses  | 7,773     | 6,357                |  |
| Deferred revenue - current portion  | 2,333     | 800                  |  |
| Deferred rent - current portion   | 235       | 212                  |  |
| Total current liabilities   | 10,788    | 7,791                |  |
| Deferred revenue  | —         | 1,933                |  |
| Deferred rent   | 730       | 851                  |  |
| Warrant liabilities   | 6,094     | 11,776               |  |
| Other long-term liabilities   | 20        | 20                   |  |
| Total liabilities   | 17,632    | 22,371               |  |
| Commitments and contingencies (note 8)  |           |                      |  |
| Stockholders' equity:   |           |                      |  |
| Preferred stock, \$0.001 par value; 30,000,000 shares authorized and no shares issued and outstanding         |           |                      |  |
| Common stock, \$0.001 par value; 250,000,000 shares authorized; 100,725,066 and 100,159,618 shares issued and |           |                      |  |
| outstanding at June 30, 2014 and December 31, 2013, respectively  | 101       | 100                  |  |
| Additional paid-in capital - common stock   | 390,192   | 386,511              |  |
| Additional paid-in capital - warrants issued  | 3,541     | 3,603                |  |
| Accumulated deficit   | (356,118) | (340,831)            |  |
| Total stockholders' equity  | 37,716    | 49,383               |  |
| Total liabilities and stockholders' equity  | \$ 55,348 | \$ 71,754            |  |

The accompanying notes are an integral part of the unaudited interim financial statements.

# STATEMENTS OF OPERATIONS (unaudited)

# (in thousands, except share and per share data)

|  | For the Three Months Ended<br>June 30, |           |    |           | For the Six Months Ended<br>June 30, |           |    |           |
|--|--|-----------|----|-----------|--------------------------------------|-----------|----|-----------|
|  |  | 2014      |    | 2013      | 2014                                 |           |    | 2013      |
| Research contract revenue  | \$                                     | 200       | \$ | 200       | \$                                   | 400       | \$ | 400       |
| Operating expenses:  |  |           |    |           |                                      |           |    |           |
| Research and development, including costs of research contracts                              |  | 8,346     |    | 14,775    |                                      | 14,888    |    | 33,887    |
| General and administrative   |  | 3,031     |    | 3,721     |                                      | 6,473     |    | 8,392     |
| Total operating expenses   |  | 11,377    |    | 18,496    |                                      | 21,361    |    | 42,279    |
| Loss from operations   |  | (11,177)  |    | (18,296)  |                                      | (20,961)  |    | (41,879)  |
| Other income (expense), net  |  | 1         |    | 7         |                                      | (8)       |    | 3         |
| Change in fair value of warrants   |  | 5,600     |    | (403)     |                                      | 5,682     |    | 10,385    |
| Net loss   | \$                                     | (5,576)   | \$ | (18,692)  | \$                                   | (15,287)  | \$ | (31,491)  |
| Net loss per share - basic and diluted   | \$                                     | (0.06)    | \$ | (0.22)    | \$                                   | (0.15)    | \$ | (0.38)    |
| Weighted average common shares outstanding to compute net loss per share - basic and diluted | 10                                     | 0,422,564 | 8  | 3,082,633 | 10                                   | 0,326,416 | 8  | 2,994,845 |

The accompanying notes are an integral part of the unaudited interim financial statements.

# STATEMENT OF STOCKHOLDERS' EQUITY For the Six Months Ended June 30, 2014 (unaudited)

# (in thousands, except share and per share data)

|   | Stockholders' Equity Additional Deficit |                     |               |                |                                       |  |   |                                  |
|---|---|---------------------|---------------|----------------|---------------------------------------|--|---|----------------------------------|
|   | Preferi<br>Shares                       | red Stock<br>Amount | Common Stores | tock<br>Amount | Paid-in<br>Capital<br>Common<br>Stock | Additional<br>Paid-in<br>Capital<br>Warrants | Accumulated<br>During the<br>Development<br>Stage | Total<br>Stockholders'<br>Equity |
| Balance at December 31, 2013                    |   | \$ —                | 100,159,618   | \$ 100         | \$386,511                             | \$ 3,603                                     | \$ (340,831)                                      | \$ 49,383                        |
| Warrant exercise                                |   |                     | 21,977        |                | 62                                    | (62)   |   | _                                |
| Stock-based compensation                        | —                                       | —                   | —             | _              | 2,435                                 |  |   | 2,435                            |
| Exercise of employee stock options              | _                                       | —                   | 583,038       | 1              | 1,316                                 | —  | _   | 1,317                            |
| Cancelled restricted stock                      | _                                       | —                   | (8,936)       | —              |                                       | _  | _   |                                  |
| Repurchase of shares of restricted common stock | _                                       | _                   | (30,631)      | _              | (132)                                 |  | _   | (132)                            |
| Net loss  |   | —                   | —             |                | —                                     | —  | (15,287)  | (15,287)                         |
| Balance at March 31, 2014                       |   | \$ —                | 100,725,066   | \$ 101         | \$390,192                             | \$ 3,541                                     | \$ (356,118)                                      | \$ 37,716                        |

The accompanying notes are an integral part of the unaudited interim financial statements.

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# STATEMENTS OF CASH FLOWS (unaudited)

# (in thousands)

|   | For the Si<br>Ended J | June 30,          |
|---|-----------------------|-------------------|
| Cash flows from operating activities:                                       | 2014                  | 2013              |
| Net loss  | \$(15,287)            | \$(31,491)        |
| Adjustments to reconcile net loss to net cash used in operating activities: | ¢(10, <b>1</b> 07)    | ¢(01,101)         |
| Depreciation and amortization   | 217                   | 396               |
| Stock-based compensation  | 2,435                 | 1,673             |
| Change in fair value of warrants  | (5,682)               | (10,385)          |
| Change in operating assets and liabilities:                                 |                       |                   |
| (Increase) decrease in:   |                       |                   |
| Receivables   | 113                   | 27                |
| Prepaid expenses and other current assets                                   | 957                   | 2,845             |
| Other noncurrent assets   | 15                    | 269               |
| Deposits  | —                     | 4                 |
| Increase (decrease) in:   |                       |                   |
| Accounts payable  | 25                    | (344)             |
| Accrued expenses  | 1,416                 | 2,453             |
| Deferred revenue  | (400)                 | (400)             |
| Deferred rent   | (98)                  | (11)              |
| Net cash used in operating activities                                       | (16,289)              | (34,964)          |
| Cash flows from investing activities:                                       |                       |                   |
| Purchases of property and equipment   | (99)                  | (115)             |
| Net cash used in investing activities                                       | (99)                  | (115)             |
| Cash flows from financing activities:                                       |                       |                   |
| Proceeds from exercise of stock options                                     | 1,317                 | 854               |
| Payments to employees for repurchase of common stock                        | (132)                 | (250)             |
| Proceeds from exercise of warrants  | —                     | 101               |
| Net cash provided by financing activities                                   | 1,185                 | 705               |
| Net increase (decrease) in cash and cash equivalents                        | (15,203)              | (34,374)          |
| Cash and cash equivalents, beginning of period                              | 68,204                | 73,306            |
| Cash and cash equivalents, end of period                                    | \$ 53,001             | \$ 38,932         |
|   | \$ 55,001             | \$ 00,00 <u>2</u> |
| Supplementary disclosure of cash flow information:                          |                       |                   |
| Cash paid for interest  | \$ —                  | \$ —              |
| Cash paid for income taxes  | \$                    | \$                |
| Supplementary disclosure of noncash investing and financing activities:     |                       |                   |
| Exercise of equity-classified warrants to common shares                     | \$ 62                 | <u>\$ 72</u>      |

The accompanying notes are an integral part of the unaudited interim financial statements.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

#### 1. Business

#### Overview

ZIOPHARM Oncology, Inc., which we refer to as "ZIOPHARM" or the "Company", is a biopharmaceutical company that seeks to acquire, develop and commercialize, on its own or with commercial partners, a diverse portfolio of cancer therapies that can address unmet medical needs.

The Company's operations to date have consisted primarily of raising capital and conducting research and development. The Company's fiscal year ends on December 31.

The Company has operated at a loss since its inception in 2003 and has minimal revenues. The Company anticipates that losses will continue for the foreseeable future. At June 30, 2014, the Company's accumulated deficit was approximately \$356.1 million. Based upon our current plans, we anticipate that our cash resources will be sufficient to fund our operations into the third quarter of 2015. The Company's ability to continue operations after its current cash resources are exhausted depends on its ability to obtain additional financing or to achieve profitable operations, as to which no assurances can be given. Cash requirements may vary materially from those now planned because of changes in the Company's focus and direction of its research and development programs, competitive and technical advances, patent developments, regulatory changes or other developments. Additional financing will be required to continue operations after the Company exhausts its current cash resources and to continue its long-term plans for clinical trials and new product development. There can be no assurance that any such financing can be obtained by the Company, or if obtained, what the terms thereof may be, or that any amount that the Company is able to raise will be adequate to support the Company's working capital requirements until it achieves profitable operations.

#### Basis of Presentation

The accompanying unaudited interim financial statements have been prepared in accordance with the instructions to Form 10-Q pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and note disclosures required by generally accepted accounting principles in the United States have been condensed or omitted pursuant to such rules and regulations.

It is management's opinion that the accompanying unaudited interim financial statements reflect all adjustments (which are normal and recurring) that are necessary for a fair statement of the results for the interim periods. The unaudited interim financial statements should be read in conjunction with the audited financial statements and the notes thereto for the year ended December 31, 2013, included in the Company's Form 10-K, for such fiscal year.

The year-end balance sheet data was derived from the audited financial statements but does not include all disclosures required by generally accepted accounting principles in the United States.

The results disclosed in the Statements of Operations for the three and six months ended June 30, 2014 are not necessarily indicative of the results to be expected for the full fiscal year.

#### Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Although the Company regularly assesses these estimates, actual results could differ from those estimates. Changes in estimates are recorded in the period in which they become known.



# NOTES TO FINANCIAL STATEMENTS (unaudited)

### 1. Business - (continued)

The Company's most significant estimates and judgments used in the preparation of its financial statements are:

- Clinical trial expenses;
- Fair value measurements for stock based compensation and warrants; and
- Income taxes.

# Subsequent Events

The Company evaluated all events and transactions that occurred after the balance sheet date through the date of this filing. Except as disclosed in Note 8, the Company did not have any other material subsequent events that impacted its financial statements or disclosures.

# 2. Summary of Significant Accounting Policies

The Company's significant accounting policies were identified in the Company's Form 10-K for the fiscal year ended December 31, 2013. There have been no material changes in those policies since the filing of our Form 10-K.

# 3. Recently Issued Accounting Pronouncements

On June 10, 2014, the FASB issued ASU 2014-10, Development Stage Entities (Topic 915), which simplifies financial reporting for development stage entities by eliminating requirements specific to development stage entities. As a result, entities in a development stage will no longer need to present inception-to-date information about income statement line items, cash flows, and equity transactions. Instead, the new guidance clarifies how these entities should tailor existing disclosures to explain the risks and uncertainties related to their activities. This update is effective for annual periods beginning after December 15, 2014, and early application is permitted for any annual or interim period for which the entity's financial statements have not yet been issued. The Company adopted this guidance prior to issuing the financial statements in this Quarterly Report on Form 10-Q. The adoption of ASU 2014-10 impacted presentation and disclosure only and did not have any impact on financial position or results of operations.

# 4. Restructuring

On April 3, 2013, the Company completed a workforce reduction plan to reduce costs as part of the Company's decision to terminate development of palifosfamide in first-line metastatic soft tissue sarcoma and place exclusive strategic focus on its synthetic biology programs, which are being developed in partnership with Intrexon Corporation ("Intrexon") (see Notes 7 and 8). Pursuant to the workforce reduction plan, the Company eliminated a total of 65 positions, comprised of 40 filled positions and 25 unfilled positions across various functions and locations. Employees whose positions were eliminated as part of the plan were notified beginning on April 2, 2013. Affected employees were offered separation benefits, including severance payments, and temporary healthcare coverage assistance. In connection with the elimination of filled positions as part of the workforce reduction plan, the Company incurred charges of \$1.7 million during the second quarter of 2013, primarily for one-time contractual severance benefits.

On August 30, 2013, the Company entered into a sublease agreement to lease 5,249 square feet in the Company's Boston office to a subtenant. The company remains primarily liable to pay rent on the original lease. The Company recorded a loss on the sublease in the amount of \$42 thousand during the year ended December 31, 2013, representing the remaining contractual obligation of \$367 thousand, less \$325 thousand in future payments from the subtenant. The Company retired assets in this subleased area as a result of this sublease with a net book value of \$194 thousand, and recorded a loss on disposal of fixed assets for the same amount in the year ended December 31, 2013.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 4. Restructuring – (continued)

On October 17, 2013, the Company entered into a sublease agreement to lease 7,259 square feet in the Company's New York office to a subtenant. The Company remains primarily liable to pay rent on the original lease. The Company recorded a loss on the sublease in the amount of \$729 thousand for the year ended December 31, 2013, representing the remaining contractual obligation of \$2.3 million, less \$1.6 million in future payments from the subtenant. The Company retired assets in this subleased area as a result of this sublease with a net book value of \$392 thousand, and recorded a loss on disposal of fixed assets for the same amount for the year ended December 31, 2013. The Company continues to maintain a \$388 thousand letter of credit. The collateral for the letter of credit is recorded in other non-current assets on the balance sheet as of June 30, 2014.

# 5. Fair Value Measurements

The Company accounts for its financial assets and liabilities using fair value measurements. The accounting standard defines fair value, establishes a framework for measuring fair value under generally accepted accounting principles and enhances disclosures about fair value measurements. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The standard describes a fair value hierarchy based on three levels of inputs, of which the first two are considered observable and the last unobservable, that may be used to measure fair value which are the following:

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

Assets and liabilities measured at fair value on a recurring basis as of June 30, 2014 and December 31, 2013 are as follows:

| (\$ in thousands)                       |                                | Fair Value Measurements at Reporting Date Using |        |    |                          |    |                         |
|---|--------------------------------|---|--------|----|--------------------------|----|-------------------------|
|   |                                | Quoted P<br>Active Ma                           |        |    |                          |    |                         |
|   | Delener er ef                  | Ident   |        |    | icant Other              |    | nificant                |
| Description                             | Balance as of<br>June 30, 2014 | Assets/Li<br>(Leve                              |        |    | /able Inputs<br>.evel 2) |    | vable Inputs<br>evel 3) |
| Cash equivalents                        | \$ 51,689                      | \$  | 51,689 | \$ |                          | \$ |                         |
| Warrant liability                       | \$ 6,094                       | \$  |        | \$ | 6,094                    | \$ |                         |
| , i i i i i i i i i i i i i i i i i i i |                                |   |        |    |                          |    |                         |

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 5. Fair Value Measurements – (continued)

| (\$ in thousands) |                                 | Fair Value Measurements at Reporting Date Using<br>Quoted Prices in |   |   |  |  |  |
|-------------------|---------------------------------|---|---|---|--|--|--|
| Description       | Balance as of December 31, 2013 | Active Markets for<br>Identical<br>Assets/Liabilities<br>(Level 1)  | Significant Other<br>Observable Inputs<br>(Level 2) | Significant<br>Unobservable<br>Inputs (Level 3) |  |  |  |
| Cash equivalents  | \$ 66,974                       | \$ 66,974   | \$  | \$  |  |  |  |
| Warrant liability | <u>\$ 11,776</u>                | <u>\$                                    </u>                       | \$ 11,776   | <u>\$                                    </u>   |  |  |  |

The cash equivalents represent deposits in a short term United States treasury money market mutual fund quoted in an active market and classified as a Level 1 asset. The Company's Level 2 financial liabilities consist of long-term investor and placement agent warrants issued in connection with its December 2009 public offering. The warrants were valued using Binomial/Monte Carlo valuation models. See Note 9 for additional disclosures on the valuation methodology and significant assumptions.

# 6. Net Loss per Share

Basic net loss per share is computed by dividing net loss by the weighted average number of shares of common stock outstanding for the period. The Company's potential dilutive shares, which include outstanding common stock options, unvested restricted stock and warrants, have not been included in the computation of diluted net loss per share for any of the periods presented as the result would be anti-dilutive. Such potential shares of common stock at June 30, 2014 and 2013 consist of the following:

|                                  | June       | 30,        |
|----------------------------------|------------|------------|
|                                  | 2014       | 2013       |
| Stock options                    | 6,041,162  | 6,538,419  |
| Unvested restricted common stock | 295,496    | 454,325    |
| Warrants                         | 10,496,467 | 10,441,431 |
|                                  | 16,833,125 | 17,434,175 |

# 7. Related Party Transactions

On January 6, 2011, the Company entered into an Exclusive Channel Partner Agreement, or Channel Agreement, with Intrexon (see Note 7 to the financial statements, Commitments and Contingencies, for additional disclosure relating to the Channel Agreement). Our director, Randall J. Kirk, is the CEO, a director, and the largest stockholder of Intrexon. During the year ended December 31, 2012, the Company paid Intrexon approximately \$11.4 million, of which \$6.6 million was for services already incurred and the remaining \$4.8 million was for services expected to be incurred within a year. This amount was included as part of prepaid expenses and other current assets on the balance sheet as of December 31, 2012. During the year ended December 31, 2013, the Company expensed \$7.8 million for services performed by Intrexon, of which \$4.8 million was applied to the prepaid balance in other current assets, \$2.4 million was paid to Intrexon and \$0.6 million was recorded in accrued expenses. As of December 31, 2013, the prepaid balance in other current assets on the accompanying balance sheet has been reduced to \$0. During the six months ended June 30, 2014, the Company expensed \$5.6 million for services performed by Intrexon, of which \$4.2 million \$3, 2014, the Company expensed \$5.6 million for services performed by Intrexon, of which \$2.2 million is included in accrued expenses at June 30, 2014.

On January 25, 2012, Intrexon purchased 1,923,075 shares of common stock in the Company's public offering.

On November 7, 2012, the Company issued 3,636,926 shares of common stock to Intrexon under the terms of its Stock Purchase Agreement with Intrexon dated January 6, 2011.

On October 13, 2013, Intrexon purchased 2,857,143 shares of common stock in the Company's public offering.



# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies

#### **Operating** Leases

Prior to December 31, 2012, the Company entered into an operating lease in New York, NY, consisting of 6,251 square feet of office space. In accordance with this agreement, the Company entered into a letter of credit in the amount of \$388 thousand, naming the Company's landlord as beneficiary. In January 2012, the Company amended the lease agreement, adding 1,008 square feet of office space. As of December 31, 2012, the Company occupied 7,259 square feet of space in New York, NY, and maintained a \$388 thousand letter of credit. The collateral for the letter of credit is recorded in other non-current assets on the balance sheet as of December 31, 2012. The lease for office space in New York, NY expires in October 2018.

On October 17, 2013, the Company entered into a sublease agreement to lease 7,259 square feet in the Company's New York office to a subtenant. The Company remains primarily liable to pay rent on the original lease. The Company recorded a loss on the sublease in the amount of \$729 thousand for the year ended December 31, 2013, representing the remaining contractual obligation of \$2.3 million, less \$1.6 million in payments from the subtenant. The Company retired assets in this subleased area as a result of this sublease with a net book value of \$392 thousand, and recorded a loss on disposal of fixed assets for the same amount for the year ended December 31, 2013. The Company continues to maintain a \$388 thousand letter of credit. The collateral for the letter of credit is recorded in other non-current assets on the balance sheet as of June 30, 2014. The lease and sublease for office space in New York, NY expires in October 2018.

Prior to December 31, 2012, the Company entered into separate operating lease agreements for various spaces in a building in Boston, MA. That space consisted of 5,249 square feet on the first floor, 8,538 square feet on the second floor, and 6,959 square feet on the third floor. In June 2012, the Company renegotiated a master lease for the entire Boston office space, added 9,800 square feet of office space on the fourth floor, surrendered 4,113 square feet from the second floor, and incorporated all floors' lease agreements under the same master agreement expiring in August 2016. The Company provided an additional \$41 thousand security deposit for the additional space on the fourth floor. As of December 31, 2012, a total security deposit of \$127 thousand was paid to its landlord for security deposits for these agreements.

On August 30, 2013, the Company entered into a sublease agreement to lease 5,249 square feet in the Company's Boston office to a subtenant. The Company remains primarily liable to pay rent on the original lease. The Company recorded a loss on the sublease in the amount of \$42 thousand for the year ended December 31, 2013, representing the remaining contractual obligation of \$367 thousand, less \$325 thousand in payments from the subtenant. The Company retired assets in this subleased area as a result of this sublease with a net book value of \$194 thousand, and recorded a loss on disposal of fixed assets. In accordance with the subtenant, the subtenant provided the Company with a security deposit of \$20 thousand, which is recorded in other non-current assets and other liabilities on the balance sheet on June 30, 2014. The sublease expires in August 2016.

As of June 30, 2014, the Company occupies 21,184 square feet of space in its Boston, MA office and has paid a total of \$127 thousand for security deposits, which are recorded in other non-current assets on the balance sheet.



# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies - (continued)

Total rent expense was approximately \$305 thousand and \$619 thousand for the six months ended June 30, 2014 and 2013, respectively.

The Company records rent expense on a straight-line basis over the term of the lease. Accordingly, the Company has recorded a liability for deferred rent at June 30, 2014 and December 31, 2013 of \$965 thousand (\$235 thousand current and \$730 thousand long-term) and \$1.1 million (\$212 thousand current and \$851 thousand long-term), respectively, which is recorded in deferred rent on the balance sheet.

License Agreements Patent and Technology License Agreement—The University of Texas M. D. Anderson Cancer Center and the Texas A&M University System.

On August 24, 2004, the Company entered into a patent and technology license agreement with The Board of Regents of the University of Texas System, acting on behalf of The University of Texas M. D. Anderson Cancer Center and the Texas A&M University System, which the Company refers to, collectively, as the Licensors. Under this agreement, the Company was granted an exclusive, worldwide license to rights (including rights to U.S. and foreign patent and patent applications and related improvements and know-how) for the manufacture and commercialization of two classes of organic arsenicals (water- and lipid-based) for human and animal use. The class of water-based organic arsenicals includes darinaparsin.

As partial consideration for the license rights obtained, the Company made an upfront payment in 2004 of \$125 thousand and granted the Licensors 250,487 shares of the Company's common stock. In addition, the Company issued options to purchase an additional 50,222 shares outside the 2003 Stock Option Plan for \$0.002 per share following the successful completion of certain clinical milestones, which vested with respect to 12,555 shares upon the filing of an Investigation New Drug application, or IND, for darinaparsin in 2005 and vested with respect to another 25,111 shares upon the completion of dosing of the last patient for both Phase 1 clinical trials in 2007. The Company recorded \$120 thousand of stock based compensation expense related to the vesting in 2007. The remaining 12,556 shares will vest upon enrollment of the first patient in a multi-center pivotal clinical trial i.e. a human clinical trial intended to provide the substantial evidence of efficacy necessary to support the filing of an approvable New Drug Application, or NDA. In addition, the Licensors are entitled to receive certain milestone payments, including \$100 thousand that was paid in 2005 upon the commencement of Phase 1 clinical trial and \$250 thousand that was paid in 2006 upon the dosing of the first patient in the Registrant-sponsored Phase 2 clinical trial for darinaparsin. The Company may be required to make additional payments upon achievement of certain other milestones in varying amounts which on a cumulative basis could total up to an additional \$4.5 million. In addition, the Licensors are entitled to receive single digit percentage royalty payments on sales from a licensed product and will also be entitled to receive a portion of any fees that the Company may receive from a possible sublicense under certain circumstances. In addition, the Company also paid the Licensors \$100 thousand in 2006 and 2007 to conduct scientific research with the Company obtaining exclusive right to all resulting intellectual property rights. The sponso

The license agreement also contains other provisions customary and common in similar agreements within the industry, such as the right to sublicense the Company rights under the agreement. On July 31, 2014, the Company amended and restated the License and Collaboration Agreement between the Company and Solasia Pharma K.K. or Solasia, granting to Solasia an exclusive worldwide license to develop and commercialize darinaparsin, and related organoarsenic molecules, in both intravenous and oral forms in all indications for human use. In exchange, the Company will be eligible to receive from Solasia development-and sales-based milestones, a royalty on net sales of darinaparsin, once commercialized, and a percentage of any sublicense revenues generated by Solasia. Solasia will be responsible for all costs related to the development, manufacturing and commercialization of darinaparsin. The Licensors will receive a portion of all milestone and royalty payments made by Solasia to the Company in accordance with the terms of the Company's license agreement with the Licensors.

The term of the license agreement between the Company and the Licensors extends until the expiration of all claims under patents and patent applications associated with the licensed technology, subject to earlier termination in the event of defaults by the Company or the Licensors under the license agreement, or if the Company becomes bankrupt or insolvent. No milestones under the license agreement were reached or expensed during the six months ended June 30, 2014 or during the years ended December 31, 2013, 2012 or 2011.



### NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies - (continued)

#### License Agreement with DEKK-Tec, Inc.

On October 15, 2004, the Company entered into a license agreement with DEKK-Tec, Inc., pursuant to which it was granted an exclusive, worldwide license for palifosfamide. As part of the signing of license agreement with DEKK-Tec, the Company expensed an upfront \$50 thousand payment to DEKK-Tec in 2004.

In consideration for the license rights, DEKK-Tec is entitled to receive payments upon achieving certain milestones in varying amounts which on a cumulative basis may total \$4.0 million. Of the aggregate milestone payments, most will be creditable against future royalty payments as referenced below. The Company expensed a \$100 thousand milestone payment upon achieving Phase 2 milestones during the year ended December 31, 2006. Additionally, in 2004 the Company issued DEKK-Tec an option to purchase 27,616 shares of the Company's common stock for \$0.02 per share. Upon the execution of the license agreement, 6,904 shares vested and were subsequently exercised in 2005 and the remaining options will vest upon certain milestone events, culminating with final FDA approval of the first NDA submitted by the Company (or by its sublicensee) for palifosfamide. DEKK-Tec is entitled to receive single digit percentage royalty payments on the sales of palifosfamide should it be approved for commercial sale. On March 16, 2010, the Company expensed a \$100 thousand milestone payment and vested 6,904 stock options upon achieving Phase 3 milestones. These options were subsequently exercised in 2011. The Company's obligation to pay royalties will terminate on a country-by-country basis upon the expiration of all valid claims of patents in such country covering licensed product, subject to earlier termination in the event of defaults by the parties under the license agreement. No milestones under the license agreement have been reached or expensed since 2010.

# Option Agreement with Southern Research Institute ("SRI")

On December 22, 2004, the Company entered into an Option Agreement with the Southern Research Institute, or SRI, pursuant to which the Company was granted an exclusive option to obtain an exclusive license to SRI's interest in certain intellectual property, including exclusive rights related to certain isophosphoramide mustard analogs.

Also on December 22, 2004, the Company entered into a Research Agreement with SRI pursuant to which the Company agreed to spend a sum not to exceed \$200 thousand between the execution of the agreement and December 21, 2006, including a \$25 thousand payment that was made simultaneously with the execution of the agreement, to fund research and development work by SRI in the field of isophosphoramide mustard analogs. The option agreement was exercised on February 13, 2007. Under the license agreement entered into upon exercise of the option, the Company is required to remit minimum annual royalty payments of \$25 thousand until the first commercial sale of a licensed product. These payments were made for the years ended December 31, 2013, 2012, 2011, 2010, 2009 and 2008. Additionally, no payments have been made for the six months ended June 30, 2014. The Company may be required to make payments upon achievement of certain milestones in varying amounts which on a cumulative basis could total up to \$775,000. In addition, SRI will be entitled to receive single digit percentage royalty payments on the sales of a licensed product in any country until all licensed patents rights in that country which are utilized in the product have expired. No milestones under the license agreement have been reached or expensed since the agreement's inception.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

#### 8. Commitments and Contingencies - (continued)

#### License Agreement with Baxter Healthcare Corporation

On November 3, 2006, the Company entered into a definitive Asset Purchase Agreement for indibulin and a License Agreement to proprietary nanosuspension technology with affiliates of Baxter Healthcare S.A. The purchase included the entire indibulin intellectual property portfolio as well as existing drug substance and capsule inventories. The terms of the Asset Purchase Agreement included an upfront cash payment of approximately \$1.1 million and an additional \$100 thousand payment for existing inventory, both of which were expensed in 2006. In addition to the upfront costs, the Asset Purchase Agreement includes additional diligence and milestone payments that could amount to approximately \$8 million in the aggregate and royalties on net sales of products covered by a valid claim of a patent for the life of the patent on a country-by-country basis. The Company expensed a \$625 thousand milestone payment upon the successful U.S. IND application for indibulin in 2007. The term of the license agreement extends until the expiration of the last to expire of the patents covering the licensed products, subject to earlier termination in the event of defaults by the parties under the license agreement. No milestones under the license agreement were reached or expensed during the years ended December 31, 2011 or 2010. During each of the years ended December 31, 2013 and 2012, milestones of \$250 thousand were reached and expensed. No milestones have been met during the six months ended June 30, 2014. In connection with the Company's decision to focus on its synthetic biology programs, the Company will no longer pursue development of indibulin.

# Exclusive Channel Partner Agreement with Intrexon Corporation

On January 6, 2011, the Company entered into an Exclusive Channel Partner Agreement, or the Channel Agreement, with Intrexon that governs a "channel partnering" arrangement in which the Company uses Intrexon's technology directed towards *in vivo* expression of effectors in connection with the development of Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex and generally to research, develop and commercialize products, in each case in which DNA is administered to humans for expression of anti-cancer effectors for the purpose of treatment or prophylaxis of cancer, which the Company collectively refers to as the Cancer Program. The Channel Agreement establishes committees comprised of representatives of ZIOPHARM and Intrexon that govern activities related to the Cancer Program in the areas of project establishment, chemistry, manufacturing and controls, clinical and regulatory matters, commercialization efforts and intellectual property.

The Channel Agreement grants the Company a worldwide license to use patents and other intellectual property of Intrexon in connection with the research, development, use, importing, manufacture, sale, and offer for sale of products involving DNA administered to humans for expression of anti-cancer effectors for the purpose of treatment or prophylaxis of cancer, which the Company collectively refers to as the ZIOPHARM Products. Such license is exclusive with respect to any clinical development, selling, offering for sale or other commercialization of ZIOPHARM Products, and otherwise is non-exclusive. Subject to limited exceptions, the Company may not sublicense the rights described without Intrexon's written consent.

Under the Channel Agreement, and subject to certain exceptions, the Company is responsible for, among other things, the performance of the Cancer Program, including development, commercialization and certain aspects of manufacturing of ZIOPHARM Products. Intrexon is responsible for the costs of establishing manufacturing capabilities and facilities for the bulk manufacture of products developed under the Cancer Program, certain other aspects of manufacturing and costs of discovery-stage research with respect to platform improvements and costs of filing, prosecution and maintenance of Intrexon's patents.

Subject to certain expense allocations and other offsets provided in the Channel Agreement, the Company will pay Intrexon on a quarterly basis 50% of net profits derived in that quarter from the sale of ZIOPHARM Products, calculated on a ZIOPHARM Product-by- ZIOPHARM Product basis. The Company has likewise agreed to pay Intrexon on a quarterly basis 50% of revenue obtained in that quarter from a sublicensor in the event of a sublicensing arrangement. In addition, in partial consideration for each party's execution and delivery of the Channel Agreement, the Company entered into a Stock Purchase Agreement with Intrexon.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies - (continued)

Following the first 24 months of the agreement, Intrexon had the option to terminate the Channel Agreement, if the Company failed to use diligent efforts to develop and commercialize ZIOPHARM Products or if the Company elected not to pursue the development of a Cancer Program identified by Intrexon that is a "Superior Therapy" as defined in the Channel Agreement. Also following the first 24 months of the agreement, the Company had the option to voluntarily terminate the Channel Agreement, upon 90 days written notice to Intrexon. The 24 month termination period expired during the year ended December 31, 2013.

Upon termination of the Channel Agreement, the Company may continue to develop and commercialize any ZIOPHARM Product that, at the time of termination:

- Is being commercialized by the Company;
- Has received regulatory approval;
- Is a subject of an application for regulatory approval that is pending before the applicable regulatory authority; or
- Is the subject of at least an ongoing Phase 2 clinical trial (in the case of a termination by Intrexon due to an uncured breach or a voluntary termination by the Company), or an ongoing Phase 1 clinical trial in the field (in the case of a termination by the Company due to an uncured breach or a termination by Intrexon following an unconsented assignment by the Company or the Company's election not to pursue development of a Superior Therapy).

The Company's obligation to pay 50% of net profits or revenue described above with respect to these "retained" products will survive termination of the Channel Agreement.

# Collaboration Agreement with Harmon Hill, LLC

On April 8, 2008, the Company signed a collaboration agreement for Harmon Hill, LLC, or Harmon Hill, to provide consulting and other services for the development and commercialization of oncology therapeutics by ZIOPHARM. Under the agreement the Company has agreed to pay Harmon Hill \$20 thousand per month for the consulting services and has further agreed to pay Harmon Hill (a) \$500 thousand upon the first patient dosing of the Specified Drug, as defined in the collaboration agreement, in a pivotal trial, which trial uses a dosing Regime introduced by Harmon Hill; and (b) provided that the Specified Drug receives regulatory approval from the FDA, the European Medicines Agency or another regulatory agency for the marketing of the Specified Drug, a 1% royalty of the Company's net sales will be awarded to Harmon Hill.

If the Specified Drug is sublicensed to a third party, the agreement entitles Harmon Hill to 1% award of royalties or other payments received from a sublicense. Subject to renewal or extension by the parties, the term of the agreement was for a one year period that expired April 8, 2009. Following such expiration, the parties continued to operate under the terms of the agreement and during 2010, the agreement was formally extended through April 8, 2011 and again through April 8, 2012. The agreement was extended through November 8, 2012 and has now expired. The Company expensed \$240 thousand during the years ended December 31, 2011 and 2010 and expensed \$200 thousand during the year ended December 31, 2012 for consulting services per the aforementioned agreement. No milestones under the collaboration agreement were reached or expensed during the years ended December 31, 2013, 2012, 2011 or 2010.

On June 27, 2013, the Company signed a new collaboration agreement with Harmon Hill to provide consulting and other services for the development and commercialization of oncology therapeutics by ZIOPHARM, effective April 1, 2013. Under the agreement the Company has agreed to pay Harmon Hill \$15 thousand per month for the consulting services. Subject to renewal or extension by the parties, the term of the agreement is for a one year period. The Company expensed \$135 thousand for the year ended December 31, 2013. The Company expensed \$90 thousand for the six months ended June 30, 2014.

# Collaboration Agreement with Solasia Pharma K.K.

On March 7, 2011, the Company entered into a License and Collaboration Agreement with Solasia Pharma K.K., or Solasia.

Pursuant to the License and Collaboration Agreement, the Company granted Solasia an exclusive license to develop and commercialize darinaparsin in both IV and oral forms and related organic arsenic molecules, in all indications for human use in a pan- Asian/Pacific territory comprised of Japan, China, Hong Kong, Macau, Republic of Korea, Taiwan, Singapore, Australia, New Zealand, Malaysia, Indonesia, Philippines and Thailand.



# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies - (continued)

As consideration for the license, the Company received an upfront payment of \$5.0 million to be used exclusively for further clinical development of darinaparsin outside of the pan-Asian/Pacific territory, and will be entitled to receive additional payments of up to \$32.5 million in development-based milestones and up to \$53.5 million in sales-based milestones. The Company will also be entitled to receive double digit royalty payments from Solasia based upon net sales of licensed products in the applicable territories, once commercialized, and a percentage of sublicense revenues generated by Solasia. Under the License and Collaboration Agreement, the Company provides Solasia with drug product to conduct clinical trials. These transfers are accounted for as a reduction of research and development costs and an increase in collaboration receivables. The agreement provides that Solasia will be responsible for the development and commercialization of darinaparsin in the pan-Asian/Pacific territory.

On July 31, 2014, the Company entered into an amendment and restatement of the License and Collaboration Agreement granting Solasia an exclusive worldwide license to develop and commercialize darinaparsin, and related organoarsenic molecules, in both intravenous and oral forms in all indications for human use. In exchange, the Company will be eligible to receive from Solasia development-and sales-based milestones, a royalty on net sales of darinaparsin, once commercialized, and a percentage of any sublicense revenues generated by Solasia. Solasia will be responsible for all costs related to the development, manufacturing and commercialization of darinaparsin. The Company's Licensors will receive a portion of all milestone and royalty payments made by Solasia to the Company in accordance with the terms of the Company's license agreement with the Licensors.

The upfront payment for research and development funding is earned over the period of effort. The Company originally estimated this period to be 75 months. In accordance with the amended and restated License and Collaboration Agreement with Solasia, the Company is no longer obligated to continue their research and development efforts in connection with the upfront payment. Accordingly, the remaining deferred revenue of \$2.3 million at June 30, 2014 has been classified as current.

# CRO Services Agreement with PPD Development, L. P.

The Company is party to a Master Clinical Research Organization Services Agreement with PPD Development, L. P., or PPD, dated January 29, 2010, a related work order dated June 25, 2010 and a related work order dated April 8, 2011 under which PPD provides clinical research organization, or CRO, services in support of the Company's clinical trials. PPD is entitled to cumulative payments of up to \$20.0 million under these arrangements, which is payable by the Company in varying amounts upon PPD achieving specified milestones. During the year ended December 31, 2010, the Company expensed \$1.8 million upon contract execution and \$1.1 million upon a clinical study commencement of enrollment in North America. During the year ended December 31, 2011, additional milestones related to commencing enrollment in Europe, Latin America and Asia along with enrollment based milestones were met and the Company recorded an aggregate \$4.0 million expense. During the year ended December 31, 2012, additional enrollment-based and contract modification milestones were met and expensed totaling \$3.8 million. During the year ended December 31, 2013, patient progression and data based milestones totaling \$9.2 million were met and expensed. There are no remaining milestones on this agreement.

# CRO Services Agreement with Pharmaceutical Research Associates, Inc.

On December 13, 2011, the Company entered into a Master Clinical Research Organization Services Agreement with Pharmaceutical Research Associates, Inc., or PRA, under which PRA provides CRO services in support of our clinical trials. PRA is entitled to cumulative payments of up to \$9.5 million under these arrangements, which is payable by the Company in varying amounts upon PRA achieving specified milestones. During the year ended December 31, 2012, the Company expensed \$7.3 million upon the achievement of various letter of intent and enrollment-based milestones. During the year ended December 31, 2013, contract modification and patient enrollment based milestones totaling \$2.2 million were met and expensed. There are no remaining milestones on this agreement.

# **ZIOPHARM Oncology, Inc.**

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 8. Commitments and Contingencies - (continued)

#### CRO Services Agreement with Novella Clinical, Inc.

On December 4, 2008, the Company entered into a Master Clinical Research Organization Services Agreement with Novella Clinical, Inc., or Novella, under which PRA provides CRO services in support of our clinical trials. The work order for the newest trial being conducted by Novella was signed on November 2, 2012. Novella is entitled to cumulative payments of up to \$790 thousand under these arrangements, which is payable in varying amounts upon Novella achieving specified milestones. During the year ended December 31, 2012, the Company expensed \$256 thousand upon the achievement of various milestones. During the year ended December 31, 2014, one clinical milestones and one site activation related milestone were met and expensed totaling \$136 thousand. During the six months ended June 30, 2014, one clinical milestone was met and expensed totaling \$10 thousand.

#### 9. Warrants

The Company has issued both warrants that are accounted for as liabilities and warrants that are accounted for as equity instruments.

The Company follows accounting standards that provide guidance in assessing whether an equity-issued financial instrument is indexed to an entity's own stock for purposes of determining whether a financial instrument should be treated as a derivative and classified as a liability. Accounting standards require that liability classified warrants be recorded at their fair value at each financial reporting period and the resulting gain or loss be recorded as other income (expense) in the Statements of Operations. Fair value is measured using the binomial valuation model.

The number of warrants outstanding at June 30, 2014 and December 31, 2013 were as follows:

|                               | June 30,<br>2014 | December 31,<br>2013 |
|-------------------------------|------------------|----------------------|
| Liability-classified warrants | 8,235,076        | 8,235,076            |
| Equity-classified warrants    | 2,261,391        | 2,304,691            |
| Total warrants                | 10,496,467       | 10,539,767           |
|                               |                  |                      |

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 9. Warrants – (continued)

#### Liability-Classified Warrants

In connection with its December 2009 public securities offering, the Company issued warrants to purchase an aggregate of 8,206,520 shares of common stock (including the investor warrants and 464,520 warrants issued to the underwriters for the offering) (the "2009 Warrants"). The 2009 Warrants issued to investors were exercisable immediately and the warrants issued to underwriters became exercisable six months after the date of issuance. The 2009 Warrants have an exercise price of \$4.02 per share and have a five-year term. The fair value of the 2009 Warrants was estimated at \$22.9 million using a Black-Scholes model with the following assumptions: expected volatility of 105%, risk free interest rate of 2.14%, expected life of five years and no dividends.

Subject to certain exceptions, these warrants provide for anti-dilution protection should common stock or common stock equivalents be subsequently issued at a price less than the exercise price of the warrants then in effect, which was initially \$4.02 per share. This provision was triggered in 2013 when stock was sold at \$3.50 per share in our 2013 public offering. Accordingly, the shares of the Company's common stock underlying the outstanding 2009 Warrants were increased by 184,367 shares to 8,235,076 shares, and the exercise price of the 2009 Warrants was decreased to \$3.93.

The Company assessed whether the 2009 Warrants require accounting as derivatives. The Company determined that these warrants were not indexed to the Company's own stock in accordance with accounting standards codification Topic 815, *Derivatives and Hedging*. As such, the Company has concluded these warrants did not meet the scope exception for determining whether the instruments require accounting as derivatives and were classified as liabilities.

The Company uses the Binomial/Monte Carlo pricing model to estimate the value of the liability-classified warrants. The following assumptions were used in the Binomial/Monte Carlo valuation model at June 30, 2014 and 2013:

|                         | June 30, 2014 | June 30, 2013 |
|-------------------------|---------------|---------------|
| Risk-free interest rate | 0.06%         | 0.26%         |
| Expected life in years  | 0.44          | 1.44          |
| Expected volatility     | 70%           | 70%           |
| Expected dividend yield | 0             | 0             |
| Steps per year          | 54            | 12            |

The change in the fair value of the warrant liability resulted in a gain of \$5.6 million and \$5.7 million for the three and six months ended June 30, 2014, respectively. The change in the fair value of the warrant liability resulted in a loss of \$0.4 million and a gain of \$10.4 million for the three and six months ended June 30, 2013. The change in the fair value of the warrant liability was charged to other income (expense) in the Statements of Operations.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 9. Warrants – (continued)

During the six months ended June 30, 2014, warrant exercises were as follows:

| (in thousands, except share data) | Equity<br>Warrants | Liability<br>Warrants | Common<br>Stock<br>Issued | Liability<br>Reclassed<br>to Equity | Cash<br><u>Received</u> |
|-----------------------------------|--------------------|-----------------------|---------------------------|-------------------------------------|-------------------------|
| Cash exercises                    |                    |                       |                           | \$ —                                | \$ —                    |
| Cashless exercises                | 43,300             | —                     | 21,977                    |                                     | _                       |
|                                   | 43,300             | _                     | 21,977                    | \$ —                                | \$ —                    |

During the six months ended June 30, 2013, warrant exercises were as follows:

| (in thousands, except share data) | Equity<br>Warrants | Liability<br>Warrants | Common<br>Stock<br>Issued | Liability<br>Reclassed<br>to Equity | Cash<br><u>Received</u> |
|-----------------------------------|--------------------|-----------------------|---------------------------|-------------------------------------|-------------------------|
| Cash exercises                    | 49,315             |                       | 49,315                    | \$ —                                | \$ 101                  |
| Cashless exercises                | —                  | —                     | —                         | —                                   | —                       |
|                                   | 49,315             |                       | 49,315                    | \$ —                                | \$ 101                  |

# 10. Common Stock

On October 23, 2013, the Company entered into an underwriting agreement with J. P. Morgan Securities LLC, as representative of the several underwriters named therein, relating to the issuance and sale of 14,300,000 shares of our common stock. The price to the public in the offering was \$3.50 per share, and the underwriters agreed to purchase the shares from the Company pursuant to the underwriting agreement at a purchase price of \$3.29 per share. Under the terms of the underwriting agreement, the Company also granted the underwriters an option, exercisable for 30 days, to purchase up to an additional 2,145,000 shares of common stock at a purchase price of \$3.29 per share, and the underwriters elected to exercise such option in full. The offering was made pursuant to the Company's effective registration statement on Form S-3 (Registration Statement No. 333-177793) previously filed with the SEC, and a prospectus supplement thereunder. The underwriters purchased the 14,300,000 shares and the additional 2,145,000 shares on October 29, 2013. The net proceeds from the offering were approximately \$53.9 million after deducting underwriting discounts and estimated offering expenses payable by the Company.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 11. Stock-Based Compensation

The Company recognized stock-based compensation expense on all employee and non-employee awards as follows:

|   |        | he three months<br>Ided June 30, | For the six months<br>ended June 30, |         |  |
|---|--------|----------------------------------|--------------------------------------|---------|--|
| (in thousands)                            | 2014   | 2013                             | 2014                                 | 2013    |  |
| Research and development                  | \$ 4   | 10 \$ (145)                      | \$ 786                               | \$ 392  |  |
| General and administrative                | 8      | 02 277                           | 1,649                                | 1,281   |  |
| Stock-based employee compensation expense | \$ 1,2 | 12 \$ 132                        | \$2,435                              | \$1,673 |  |

The Company granted 23,800 and 104,300 stock options during the three and six months ended June 30, 2014 that had a weighted-average grant date fair value of \$2.84 and \$3.23 per share, respectively. The Company granted 1,315,500 and 1,426,400 stock options during the three and six months ended June 30, 2013 that had a weighted-average grant date fair value of \$1.77 and \$1.89 per share, respectively.

At June 30, 2014, total unrecognized compensation costs related to unvested stock options outstanding amounted to \$6.2 million. The cost is expected to be recognized over a weighted-average period of 1.59 years.

On May 31, 2013, the Company extended the contractual life of 66,667 fully vested stock options held by one employee from 3 to 12 months.

On June 14, 2013, the Company extended the contractual life of 71,167 fully vested stock options held by one employee from 3 to 12 months.

On March 31, 2014, the Company extended the contractual life of 71,167 fully vested stock options held by one employee by an additional 9 months.

# NOTES TO FINANCIAL STATEMENTS (unaudited)

# 11. Stock-Based Compensation – (continued)

For the three months ended June 30, 2014 and 2013, the fair value of stock options was estimated on the date of grant using a Black-Scholes option valuation model with the following assumptions:

|                         | For the three months ended June 30, |        |  |
|-------------------------|-------------------------------------|--------|--|
|                         | 2014                                | 2013   |  |
| Risk-free interest rate | 1.98%                               | 1.65%  |  |
| Expected life in years  | 6                                   | 6      |  |
| Expected volatility     | 94.07%                              | 95.45% |  |
| Expected dividend yield | 0                                   | 0      |  |

Stock option activity under the Company's stock option plan for the six months ended June 30, 2014 is as follows:

| (in thousands, except share and per share data)       | Number of<br>Shares | Weighted-<br>Average Exercise<br>Price | Weighted-<br>Average<br>Contractual<br>Term (Years) | Aggregate<br>Intrinsic Value |
|---|---------------------|--|---|------------------------------|
| Outstanding, December 31, 2013                        | 6,747,302           | \$ 3.81                                |   |                              |
| Granted   | 104,300             | 3.54                                   |   |                              |
| Exercised   | (583,038)           | 2.26                                   |   |                              |
| Cancelled   | (227,402)           | 5.05                                   |   |                              |
| Outstanding, June 30, 2014                            | 6,041,162           | \$ 3.91                                | 7.24  | \$ 4,456                     |
| Vested and unvested expected to vest at June 30, 2014 | 5,988,240           | \$ 4.11                                | 5.61  | \$ 4,417                     |
| Options exercisable, June 30, 2014                    | 3,176,594           | \$ 4.11                                | 5.61  | \$ 1,846                     |
| Options exercisable, December 31, 2013                | 3,471,935           | \$ 4.01                                | 5.03  | \$ 2,654                     |
| Options available for future grant                    | 5,317,831           |  |   |                              |

A summary of the status of unvested restricted stock for the six months ended June 30, 2014 is as follows:

|                               | Number of Shares | ed-Average<br>ite Fair Value |
|-------------------------------|------------------|------------------------------|
| Non-vested, December 31, 2013 | 352,865          | \$<br>4.38                   |
| Granted                       | —                | —                            |
| Vested                        | (48,433)         | 4.51                         |
| Cancelled                     | (8,936)          | <br>4.41                     |
| Non-vested, June 30, 2014     | 295,496          | \$<br>4.36                   |

At June 30, 2014, total unrecognized compensation costs related to unvested restricted stock outstanding amounted to \$0.7 million. The cost is expected to be recognized over a weighted-average period of 1.09 years.

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

# **Forward Looking Statements**

This quarterly report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. In particular, statements contained in this Form 10-Q, including but not limited to, statements regarding the costs and timing of our clinical trials and of the development and commercialization of our pipeline products and services; the sufficiency of our cash, investments and cash flows from operations and our expected uses of cash; our ability to finance our operations and business initiatives and obtain funding for such activities; our future results of operations and financial position, business strategy and plan prospects, projected revenue or costs and objectives of management for future research, development or operations, are forward-looking statements. These statements relate to our future plans, objectives, expectations and intentions and may be identified by words such as "may," "will," "should," "expects," "plans," "anticipates," "intends," "targets," "projects," "contemplates," "believes," "seeks," "goals," "estimates," "predicts," "potential" and "continue" or similar words. Readers are cautioned that these forward-looking statements are only predictions and are subject to risks, uncertainties, and assumptions that are difficult to predict, including those identified below, under Part II, Item 1A. "Risk Factors" and elsewhere herein. Therefore, actual results may differ materially and adversely from those expressed in any forward-looking statements. We undertake no obligation to revise or update any forward-looking statements for any reason.

# **Company Overview**

ZIOPHARM Oncology, Inc. is a biopharmaceutical company that seeks to acquire, develop and commercialize, on its own or with commercial partners, a diverse portfolio of cancer therapies that can address high unmet medical needs. Pursuant to an exclusive channel agreement with Intrexon Corporation, or Intrexon, we obtained exclusive rights to Intrexon's synthetic biology platform for use in the field of oncology, which included two existing clinical stage product candidates, Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex. The synthetic biology platform employs an inducible gene-delivery system that enables controlled delivery of genes that produce therapeutic proteins to treat cancer. Ad-RTS-IL-12 + veledimex is our lead drug candidate, which uses this gene delivery system to produce Interleukin-12, or IL-12, a potent, naturally occurring anti-cancer protein. We are currently studying Ad-RTS-IL-12 + veledimex in two Phase 2 studies, the first for the treatment of metastatic melanoma, and the second for the treatment of metastatic breast cancer, and expect to announce additional data from these Phase 2 studies in the fourth quarter of 2014. We plan to continue to combine Intrexon's synthetic biology platform with our capabilities to translate science to the patient setting to develop additional products to stimulate key pathways, including those used by the body's immune system to inhibit the growth and metastasis of cancers. We have numerous programs under development and expect to file multiple investigational new drug, or IND, applications through 2015. We also have a portfolio of small molecule drug candidates, which are no longer a strategic focus of our development activities for which we are seeking partners to pursue further development and potential commercialization.

# **Enabling Technology**

Synthetic biology entails the application of engineering principles to biological systems for the purpose of designing and constructing new biological systems or redesigning/modifying existing biological systems. Biological systems are governed by DNA, which control cellular processes by coding for the production of proteins and other molecules that have a functional purpose and by regulating the activities of these molecules. This regulation occurs via complex biochemical and cellular reactions working through intricate cell signaling pathways, and control over these molecules modifies the output of biological systems. Synthetic biology has been enabled by the application of information technology and advanced statistical analysis, also known as bioinformatics, to genetic engineering, as well as by improvements in DNA synthesis. Synthetic biology aims to engineer gene-based programs or codes to modify cellular function to achieve a desired biological outcome. Its application is intended to allow more precise control of drug concentration and dose, thereby improving the therapeutic index associated with the resulting drug.

On January 6, 2011, we entered into an Exclusive Channel Partner Agreement with Intrexon, which we refer to as the Channel Agreement, to develop and commercialize novel DNA-based therapeutics in the field of cancer treatment by combining Intrexon's synthetic biology platform with our capabilities to translate science to the patient setting. As a result, our DNA synthetic biology platform employs an inducible gene-delivery system that enables controlled delivery of genes that produce therapeutic proteins to treat cancer. The first example of this regulated controlled delivery is achieved by producing IL-12, a potent, naturally occurring anti-cancer protein, under the control of Intrexon's proprietary biological "switch" to turn on/off the therapeutic protein expression at the tumor site. We and Intrexon refer to this "switch" as the RheoSwitch Therapeutic System® or RTS® platform. Our initial drug candidates being developed using the synthetic biology platform are Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex with a current focus on Ad-RTS-IL-12 + veledimex.



We have demonstrated that we are able to simultaneously express multiple effectors under control of the RTS<sup>®</sup> platform from the same construct. In mice, we have also shown that we are able to express multigenic DNA constructs in an embedded, controlled bioreactor, by injecting into skeletal muscle and measuring the DNA-coded proteins in the blood. Furthermore, we have also demonstrated the ability to express these same three genes under RTS<sup>®</sup> platform control in mesenchymal stem cells, or MSCs.

More detailed descriptions of Ad-RTS-IL-12 + veledimex, DC-RTS-IL-12 + veledimex, our remaining small molecule programs, and our clinical development and commercialization plans for each are set forth in this report under the caption "Business – Product Candidates."

# **Recent Developments**

ZIOPHARM is currently conducting or planning Phase 2 studies of Ad-RTS-IL-12, alone or in combination with existing treatment standards, in melanoma and breast cancer, with plans to initiate a Phase 1 study in high-grade gliomas (brain cancer). We have numerous other programs under preclinical development and expect to file multiple investigational new drug, or IND, applications through 2015.

We presented at the American Society of Gene & Cell Therapy 17<sup>th</sup> annual meeting (ASGCT 2014) held May 21-24, 2014, that Ad-RTS-mIL-12 + veledimex exhibits controllable systemic immune activation in human subjects with melanoma and breast cancer. In an orthotopic murine glioma model we have demonstrated that Ad-RTS-mIL-12 + veledimex significantly reduces brain cancer stem cells and in another preclinical study we also demonstrated that Ad-RTS-mIL-12 + veledimex significantly reduces brain cancer and bevacizumab. These findings support the utility of localized, regulatable IL-12 production as an approach for the treatment of malignant glioma in human subjects.

# Product candidates

The following chart identifies our current synthetic biology product candidates and their stage of development, each of which are described in more detail below.

| Compound  | Pre Clinical | Phase 1 | Phase 2 |
|---|--------------|---------|---------|
| Ad-RTS-IL-12  |              | IND     |         |
| Melanoma  |              |         |         |
| Breast  |              |         |         |
| GBM   |              |         |         |
|   |              |         |         |
| Immuno-Oncology   |              |         |         |
| Cell-based programs<br>Immuno-Oncology<br>programs<br>Cell signal targeting |              |         |         |

# Synthetic Biology Programs:

*Ad-RTS-IL-12* + *veledimex*. Ad-RTS-IL-12 + veledimex is currently being tested in two Phase 2 studies, the first for the treatment of metastatic melanoma, and the second for the treatment of unresectable recurrent or metastatic breast cancer. Ad-RTS-IL-12 + veledimex is our lead drug candidate, which uses our gene delivery system to produce Interleukin-12, or IL-12, a potent, naturally occurring anti-cancer protein.

Interleukin-12 (IL-12) is a potent immunostimulatory cytokine which activates and recruits dendritic cells that facilitate the cross-priming of tumor antigenspecific T cells. Intratumoral administration of Ad-RTS-IL-12 + veledimex, which allows for adjustment of IL-12 gene expression upon varying the dose of veledimex, is designed to reduce the toxicity elicited by systemic delivery of IL-12, and increase efficacy through high intratumoral expression.

In March 2013, we announced the initiation of a Phase 2 clinical study of Ad-RTS-IL-12 + veledimex to treat metastatic breast cancer. The two-part, multicenter U.S. study is enrolling patients with unresectable, recurrent or metastatic breast cancer who have visible lesions or lesions accessible by injection. This study was designed to assess the safety and efficacy of the therapeutic effect of Ad-RTS-IL-12 + veledimex in this indication. The primary endpoint of the study is rate of progression-free survival at 16 weeks. Secondary endpoints include objective response rate, duration of response and evaluation of pharmacodynamic tumor markers.

In May 2013, we announced promising results from nonclinical and Phase 1 studies in metastatic melanoma using Ad-RTS-IL-12 + veledimex. In these studies, the controlled expression of IL-12, through a regulatable gene therapy strategy, was found to limit systemic toxicity while inducing biological and clinical activity. The findings were presented in an oral session at the 16th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT). In June 2013, updated results were presented at the 2013 American Society for Clinical Oncology (ASCO). Ad-RTS-IL-12 + veledimex induce production of IL-12 mRNA in the tumor microenvironment (switch on). Upon removal of veledimex, IL-12 mRNA levels return to baseline (switch off). Following treatment with Ad-RTS-IL-12 + veledimex, increases in TILs (CD8+, CD45RO+) were observed in the tumor microenvironment. Clinical activity was observed in injected and non-injected lesions primarily at the higher doses of veledimex. Inflammation, shrinkage, flattening, and depigmentation of lesions correlated with the elevated serum levels of IFN-g. Ad-RTS-IL-12 + veledimex therapy was generally well-tolerated and its safety profile is consistent with other immunotherapies.

We reported the controlled local expression of IL-12 as an immunotherapeutic treatment of glioma through the use of the RheoSwitch <sup>®</sup> at the October 2013 AACR-NCI-EORTC. Veledimex brain penetration was demonstrated in normal mice and monkeys with intact blood brain barriers. Treatment with Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex both demonstrated dose-related increase in survival in the mouse GL-261 glioma model with no adverse clinical signs observed. In December 2013, we announced the unanimous approval of the Recombinant DNA Advisory Committee of the National Institutes of Health (RAC/NIH) for the initiation of a Phase 1 study of Ad-RTS-IL-12 + veledimex, in subjects with recurrent or progressive high grade gliomas (brain cancer). We are currently discussing with the FDA nonclinical data in support of the Phase 1 study, and upon agreement, we anticipate initiation of the study during the second half 2014. Glioblastoma is by far the most frequent malignant brain tumor and is associated with a particularly aggressive course and dismal prognosis. The current standard of care is based in surgical resection to the maximum feasible extent, followed by radiotherapy and concomitant adjuvant temozolomide. Such aggressive treatment, however, is associated with only modest improvements in survival resulting in a very high unmet medical need. Newly diagnosed glioblastoma patients have a median overall survival, or OS, of 11-17 months.

In December 2013, we presented positive interim results from the ongoing Phase 1/2 study of Ad-RTS-IL-12 + veledimex in patients with advanced melanoma. The results from this multicenter study were presented at Melanoma Bridge 2013 Conference at the session "Best Abstracts on News in Immunotherapy", an international conference co-sponsored by Istituto Nazionale Tumori Fondazione, Sidra Medical and Research Center, and the Society for ImmunoTherapy of Cancer was held in Naples, Italy. In this study, 21 patients with unresectable, recurrent stage III/IV melanoma have been treated with intratumoral injections of Ad-RTS-IL-12 + veledimex and the oral activator veledimex. The purpose of the study is to evaluate the safety and tolerability of the Ad-RTS-IL-12 + veledimex therapy, determine tumor and immune response, and select the optimal dose and schedule of veledimex for future study. To date, expression of IL-12 mRNA in study subjects' tumors was determined to be controlled by veledimex. In addition, upon stopping veledimex dosing, expression of the IL-12 mRNA returned to baseline levels, demonstrating the "on" and "off" control of the RheoSwitch <sup>®</sup>. In this dose range, results to date demonstrate that Ad-RTS-IL-12 + veledimex therapy has potent biologic activity, as measured by on-mechanism and on-target toxicity and response in injected and non-injected lesions. Following treatment, 11 of 16 evaluable patients have demonstrated a response of stable disease or better on a per lesion basis. The most common severe adverse events (SAEs) were pyrexia, hypotension, mental status changes, and cytokine release syndrome. Four of seven patients with SAEs had veledimex dosing stopped during cycle one. Three had SAEs during subsequent cycles, and stopped veledimex dosing at that time. Importantly, all SAEs were reversed after veledimex dosing was stopped, demonstrating the "on" and "off" control of veledimex on gene expression.

Also in December 2013, we announced preliminary results from the ongoing Phase 2 clinical study of Ad-RTS-IL-12 + veledimex therapy in patients with unresectable recurrent or metastatic breast cancer. The findings were reported in a poster presentation at the San Antonio Breast Cancer (SABC) Symposium in San Antonio, Texas. This multicenter Phase 2 study is designed to evaluate the safety and efficacy of Ad-RTS-IL-12 + veledimex in subjects with recurrent/metastatic breast cancer with accessible tumor(s). The primary endpoint of the study is rate of progression-free survival at 16 weeks. Secondary objectives include objective response rate, duration of response and evaluation of pharmacodynamic tumor markers. Six patients were evaluable for safety at the time of presentation. The most common severe adverse events (SAEs) were neutropenia, AST elevation and pyrexia. Importantly, in the absence of disease progression, all SAEs were reversed after veledimex dosing was stopped, demonstrating the "on" and "off" control of veledimex on gene expression. Preliminary monotherapy PFS rate was reported for two patients to date, with one subject progressing at 12 weeks and a second at 16 weeks. Recruitment for the Phase 2 clinical trial is ongoing to refine the dose and dosing schedule. With this dosing data, the Company will initiate a Phase 1b/2 clinical trial of Ad-RTS-IL-12 + veledimex following first- or second-line standard treatment in subjects with locally advanced or metastatic breast cancer.

The Company continues to conduct Phase 2 monotherapy studies in melanoma and breast cancer using Ad-RTS-IL-12 + veledimex. Additionally, the Company is evaluating future trials with IL-12 in potential combination therapies with other immune-targeting agents in various cancers including melanoma and breast. Recently, as the treatment of advanced melanoma has undergone and continues to undergo a rapid evolution with the introduction and approval of highly promising new single and combination agents, the standard of care in this indication has become uncertain, resulting in a much more competitive and commercially unpredictable environment. As a result, the Company has prioritized brain cancer as our highest priority, followed by breast cancer, and then melanoma. The Company also plans to initiate a Phase 1 trial to evaluate Ad-RTS-IL-12 + veledimex as a single agent in the treatment of patients with brain cancer in the second half of 2014.

*DC-RTS-IL-12* + *veledimex*. We completed enrollment in a Phase 1 dose escalation study of DC-RTS-IL-12 + *veledimex* in the second quarter of 2012 in the United States. DC-RTS-IL-12 + *veledimex* employs intratumoral injection of modified dendritic cells from each patient and oral dosing of veledimex to turn on in vivo expression of IL-12. DC-RTS-IL-12 + *veledimex*, through the RTS® platform, controls the timing and level of transgene expression. The RTS® technology functions as a "gene switch" for the regulated expression of human IL-12 in the patients' dendritic cells which are transduced with a replication incompetent adenoviral vector carrying the IL-12 gene under the control of the RTS® platform. Currently, there are no actively enrolling studies using DC-RTS-IL-12 + *veledimex*, as we have prioritized our clinical development efforts on Ad-RTS-IL-12 + *veledimex*.

*Earlier Stage Programs*. At the October 2013 AARC-NCI-EORTC we also presented results showing systemic expression of three distinct immune effectors from a single RTS<sup>®</sup> regulated multigenic construct in mice, in vitro data demonstrating the potential use of MSCs for tumor-targeted delivery of single or multiple RTS<sup>®</sup> regulated cancer immunotherapies, and data demonstrating functional single chain variable fragment-Fc fusion proteins as an alternate approach to monoclonal antibodies which are more amenable for multi-genic therapies. We are also actively pursuing several other synthetic biology approaches, including additional gene delivery and multigenic approaches in our discovery pipeline to address unmet medical needs in cancer that are expected to result in multiple INDs through 2015.

# Small Molecule Programs

*Palifosfamide, ZIO-201.* The small molecule palifosfamide, or isophosphoramide mustard, is a proprietary active metabolite of the pro-drug ifosfamide. Because palifosfamide is the stabilized active metabolite of ifosfamide and a distinct pharmaceutical composition without the acrolein or chloroacetaldehyde metabolites we believe that the administration of palifosfamide may be an effective and well-tolerated agent to treat cancer. In addition to anticipated lower toxicity, palifosfamide may have other advantages over ifosfamide and cyclophosphamide. Palifosfamide cross-links DNA differently than the active metabolite of cyclophosphamide, resulting in a different activity profile. We are seeking transactions with third parties, including the possible out-license of palifosfamide.

*Soft Tissue Sarcoma*. Previously we have studied palifosfamide in combination with doxorubicin in patients with soft tissue sarcoma. In March 2013, we announced that the pivotal Phase 3 study, PICASSO 3, did not meet its primary endpoint of progression-free survival, and that we would terminate our development program in metastatic soft tissue sarcoma. PICASSO 3 study data was presented in September 2013 at the European Cancer Congress.

*Small-Cell Lung Cancer.* Small-Cell Lung Cancer, or SCLC, is almost exclusively associated with smoking. Standard of care for SCLC, which is etoposide and platinum therapy, has changed little in decades. Published studies of ifosfamide in combination with standard of care have evidenced enhanced efficacy but also with enhanced side effects, providing for an unfavorable benefit to risk association. We believe that combining palifosfamide with standard of care could offer a separation of enhanced efficacy from increased toxicity.



Data from a Phase 1 trial of palifosfamide in combination with etoposide and carboplatin informed appropriate dosing for initiating an adaptive Phase 3 trial in first-line, metastatic SCLC. In June 2012, the Company initiated an international, multi-center, open-label, adaptive, randomized study of palifosfamide in combination with carboplatin and etoposide, or PaCE, chemotherapy versus carboplatin and etoposide, or CE, alone in chemotherapy naïve patients with metastatic small cell lung cancer, which we refer to as MATISSE. The trial's primary endpoint is overall survival.

Based on the outcome of PICASSO 3 in soft tissue sarcoma and the resulting revision in the Company's development plans for palifosfamide, enrollment in this study was suspended with 188 patients enrolled. The interim analysis of overall survival events in MATISSE is forecasted to be reached during the second half of 2014.

*Darinaparsin, ZIO-101.* Darinaparsin is an anti-mitochondrial (organic arsenic) compound (covered by issued patents and pending patent applications in the United States and in foreign countries). Phase 1 testing of the intravenous, or IV, form of darinaparsin in solid tumors and hematological cancers was completed. We reported clinical activity and a safety profile from these studies as predicted by preclinical results. We subsequently completed Phase 2 studies in advanced myeloma, primary liver cancer and in certain other hematological cancers. At the May 2009 annual meeting of ASCO, we reported favorable results from the IV trial in lymphoma, particularly peripheral T-cell lymphoma, or PTCL. A Phase 1 trial in solid tumors with an oral form of darinaparsin has completed enrollment. We have obtained Orphan Drug Designation for darinaparsin in the United States and Europe for the treatment of PTCL and have entered into an amended and restated global licensing agreement with Solasia Pharma K.K. on July 31, 2014 (see Note 8 in the accompanying unaudited financial statements).

*Indibulin, ZIO-301.* Indibulin is a novel, small molecule inhibitor of tubulin polymerization and is potentially safer than other tubulin inhibitors as no neurotoxicity has been observed in preclinical studies or in Phase 1 clinical trials. Indibulin has a different pharmacological profile from other tubulin inhibitors currently on the market as it binds to a unique site on tubulin and is active in multi-drug-resistant (MDR-1, MRP-1) and taxane-resistant tumors. A Phase 1 study was conducted in late stage metastatic breast cancer and was found to be safe and tolerable. In connection with the Company's decision to focus on its synthetic biology programs, the Company will no longer pursue development of indibulin.

# Current Plans

Our current plans involve using our principal internal financial resources to develop the synthetic biology program, with the intention of ultimately partnering or otherwise raising additional capital to support further development activities for our strategic product candidates. As of June 30, 2014, we had approximately \$53.0 million of cash and cash equivalents. Based upon our current plans, we anticipate that our cash resources will be sufficient to fund our operations into the third quarter of 2015. This forecast of cash resources is forward-looking information that involves risks and uncertainties, and the actual amount of our expenses could vary materially and adversely as a result of a number of factors, including the factors discussed in the "Risk Factors" section of this report and the uncertainties applicable to our forecast for the overall sufficiency of our capital resources. We have based our estimates on assumptions that may prove to be wrong, and our expenses could prove to be significantly higher than we currently anticipate.

Furthermore, the successful development of our product candidates is highly uncertain. Product development costs and timelines can vary significantly for each product candidate, are difficult to accurately predict, and will require us to obtain additional funding, either alone or in connection with partnering arrangements. Various statutes and regulations also govern or influence the development, manufacturing, safety, labeling, storage, record keeping and marketing of each product. The lengthy process of seeking approval and the subsequent compliance with applicable statutes and regulations require the expenditure of substantial resources. Any failure by us to obtain, or any delay in obtaining, regulatory approvals could materially, adversely affect our business. To date, we have not received approval for the sale of any product candidates in any market and, therefore, have not generated any revenues from our product candidates.



#### **Financial Overview**

**Overview of Results of Operations** 

#### Three and six months ended June 30, 2014 compared to three and six months ended June 30, 2013

Revenue. Revenue during the three and six months ended June 30, 2014 and 2013 was as follows:

|                       | Three months ended<br>June 30, |        |        |        | Six months ended<br>June 30, |       |          |
|-----------------------|--------------------------------|--------|--------|--------|------------------------------|-------|----------|
| (\$ in thousands)     | 2014                           | 2013   | Change | 2014   | 2013                         | Chang | <u>e</u> |
| Collaboration revenue | \$ 200                         | \$ 200 | \$— 0% | \$ 400 | \$ 400                       | \$—   | 0%       |

Revenue for the three and six months ended June 30, 2014 was the same as the three and six months ended June 30, 2013. This is due to the continued recognition of income related to our entry into the collaboration agreement with Solasia Pharma K.K. on March 7, 2011. Under this agreement we received \$5.0 million in research and development funding which is earned over the period of effort, originally estimated to be 75 months. In accordance with the amended and restated License and Collaboration Agreement with Solasia (see note 8 to the accompanying unaudited financial statements), the Company is no longer obligated to continue their research and development efforts in connection with the upfront payment. Accordingly, the remaining deferred revenue of \$2.3 million at June 30, 2014 has been classified as current.

Research and development expenses. Research and development expenses during the three and six months ended June 30, 2014 and 2013 were as follows:

|                          | Three months ended<br>June 30, |          |           |       | Six months ended<br>June 30, |          |            |       |
|--------------------------|--------------------------------|----------|-----------|-------|------------------------------|----------|------------|-------|
| (\$ in thousands)        | 2014                           | 2013     | Chang     | e     | 2014                         | 2013     | Change     |       |
| Research and development | \$8,346                        | \$14,775 | \$(6,429) | (44%) | \$14,888                     | \$33,887 | \$(18,999) | (56%) |

Research and development expenses for the three months ended June 30, 2014 decreased by \$6.4 million when compared to the three months ended June 30, 2013. On March 26, 2013, we announced the decision to immediately terminate development of palifosfamide in first-line metastatic soft tissue sarcoma and during the quarter ended June 30, 2013, completed a workforce reduction plan to reduce costs (see Note 4 in the accompanying unaudited financial statements). This resulted in lower costs of \$0.6 million related to the Phase 3 palifosfamide study in SCLC as the decision was made to suspend enrollment pending further data, lower costs related to the Phase 3 palifosfamide study in soft tissue sarcoma ("STS") of \$5.3 million, lower other clinical costs of \$0.4 million, lower employee-related costs of \$0.5 million and lower manufacturing costs of \$2.1 million. The decrease was offset by an increase of \$2.4 million in discovery activities and \$0.1 million in nonclinical activities, all related to our synthetic biology program.

Research and development expenses for the six months ended June 30, 2014 decreased by \$19.0 million when compared to the six months ended June 30, 2013. On March 26, 2013, we announced the decision to immediately terminate development of palifosfamide in first-line metastatic soft tissue sarcoma and during the quarter ended June 30, 2013, completed a workforce reduction plan to reduce costs (see Note 4 in the accompanying unaudited financial statements). This resulted in lower costs of \$4.2 million related to the Phase 3 palifosfamide study in SCLC as the decision was made to suspend enrollment pending further data, lower costs related to the Phase 3 palifosfamide study in soft tissue sarcoma ("STS") of \$9.7 million, lower other clinical costs of \$1.7 million, lower employee-related costs of \$1.8 million and lower manufacturing costs of \$5.4 million. The decrease was offset by an increase of \$2.8 million in discovery activities, \$0.8 million in nonclinical activities, and \$0.2 million of other costs all related to our synthetic biology program.

Our research and development expense consists primarily of salaries and related expenses for personnel, costs of contract manufacturing services, costs of facilities and equipment, fees paid to professional service providers in conjunction with our clinical trials, fees paid to research organizations in conjunction with preclinical animal studies, costs of materials used in research and development, consulting, license and milestone payments and sponsored research fees paid to third parties.

We have not accumulated and tracked our internal historical research and development costs or our personnel and personnel-related costs on a program-byprogram basis. Our employee and infrastructure resources are allocated across several projects, and many of our costs are directed to broadly applicable research endeavors. As a result, we cannot state the costs incurred for each of our oncology programs on a program-by-program basis.

Our future research and development expenses in support of our current and future programs will be subject to numerous uncertainties in timing and cost to completion. We test potential products in numerous preclinical studies for safety, toxicology and efficacy. We may conduct multiple clinical trials for each product. As we obtain results from trials, we may elect to discontinue or delay clinical trials for certain products in order to focus our resources on more promising products or indications. Completion of clinical trials may take several years or more, and the length of time generally varies substantially according to the type, complexity, novelty and intended use of a product. It is not unusual for preclinical and clinical development of each of these types of products to require the expenditure of substantial resources.

We estimate that clinical trials of the type generally needed to secure new drug approval are typically completed over the following timelines:

The duration and the cost of clinical trials may vary significantly over the life of a project as a result of differences arising during clinical development, including, among others, the following:

- The number of clinical sites included in the trials;
- The length of time required to enroll suitable patents;
- The number of patients that ultimately participate in the trials;
- The duration of patient follow-up to ensure the absence of long-term product-related adverse events; and
- The efficacy and safety profile of the product.

As a result of the uncertainties discussed above, we are unable to determine the duration and completion costs of our programs or when and to what extent we will receive cash inflows from the commercialization and sale of a product. Our inability to complete our programs in a timely manner or our failure to enter into appropriate collaborative agreements could significantly increase our capital requirements and could adversely impact our liquidity. These uncertainties could force us to seek additional, external sources of financing from time-to-time in order to continue with our product development strategy. Our inability to raise additional capital, or to do so on terms reasonably acceptable to us, would jeopardize the future success of our business.

General and administrative expenses. General and administrative expenses during the three and six months ended June 30, 2014 and 2013 were as follows:

|                            | Three months ended<br>June 30, |          |         | Six months ended<br>June 30, |         |         |           |       |
|----------------------------|--------------------------------|----------|---------|------------------------------|---------|---------|-----------|-------|
| (\$ in thousands)          | 2014                           | 2013     | Chang   | ge <u> </u>                  | 2014    | 2013    | Change    | e     |
| General and administrative | \$3,031                        | \$ 3,721 | \$(690) | (19%)                        | \$6,473 | \$8,392 | \$(1,919) | (23%) |

General and administrative expenses for the three months ended June 30, 2014 decreased by \$0.7 million when compared to the three months ended June 30, 2013. The decrease was primarily due to lower employee-related costs of \$0.4 million as a result of our workforce reduction plan (see Note 4 in the accompanying unaudited financial statements) as well as \$0.3 million in non-employee contracted costs.

General and administrative expenses for the six months ended June 30, 2014 decreased by \$1.9 million when compared to the six months ended June 30, 2013. The decrease was primarily due to lower employee-related costs of \$0.9 million as a result of our workforce reduction plan (see Note 4 in the accompanying unaudited financial statements) as well as \$1.0 million in non-employee contracted costs.

Other income (expense). Other income (expense) for three and six months ended June 30, 2014 and 2013 was as follows:

| (\$ in thousands)                |          | nths ended<br>e 30,<br> | Change       | Six months ended<br>June 30,<br>2014 2013 | Change           |
|----------------------------------|----------|-------------------------|--------------|---|------------------|
| Other income, net                | \$ 1     | \$ 7                    | \$ (6) (86%) | ) \$ (8) \$                               | 3 \$ (11) (367%) |
| Change in fair value of warrants | 5,600    | (403)                   | 6,003 1490%  | 5,682 10,38                               | 5 (4,703) (45%)  |
| Total                            | \$ 5,601 | \$ (396)                | \$5,997      | \$5,674 \$10,38                           | 8 \$(4,714)      |

The increase in other income (expense) from the three months ended June 30, 2014 compared to the three months ended June 30, 2013 was due primarily to the change in the fair value of liability-classified warrants, which yielded a gain of \$5.6 million for the three months ended June 30, 2014 as compared to a loss of \$403 thousand for the three months ended June 30, 2013. The change in liability-classified warrants is primarily attributable to decreases in our stock price, remaining term and volatility.

The decrease in other income (expense) from the six months ended June 30, 2014 compared to the six months ended June 30, 2013 was due primarily to the change in the fair value of liability-classified warrants, which yielded a gain of \$5.7 million for the six months ended June 30, 2014 as compared to a gain of \$10.4 million for the six months ended June 30, 2013. The change in liability-classified warrants is primarily attributable to decreases in our stock price, remaining term and volatility.

# Liquidity and Capital Resources

As of June 30, 2014, we had approximately \$53.0 million in cash and cash equivalents, compared to \$68.2 million in cash and cash equivalents as of December 31, 2013. We anticipate that our cash resources will be sufficient to fund our operations into the third quarter of 2015. However, changes may occur that would consume our existing capital prior to that time, including the scope and progress of our research and development efforts and changes in governmental regulation. Actual costs may ultimately vary from our current expectations, which could materially impact our use of capital and our forecast of the period of time through which our financial resources will be adequate to support our operations. We have estimated the sufficiency of our cash resources based in part on the anticipated advancement of our synthetic biology product candidates in the clinic under our exclusive channel partnership with Intrexon, and we expect that the costs associated with these and additional product candidates will increase the level of our overall research and development expenses significantly going forward. Also included in the estimate are the discontinuation of the PICASSO 3 pivotal trial for first-line metastatic STS and our adaptive Phase 3 trial for first-line SCLC for IV palifosfamide and our current timing expectations for interim overall survival data in the MATISSE trial.

Although all human clinical trials are expensive and difficult to design and implement, we believe that due to complexity, costs associated with clinical trials for synthetic biology products are greater than the corresponding costs associated with clinical trials for small molecule candidates.

In addition to these factors, our actual cash requirements may vary materially from our current expectations for a number of other factors that may include, but are not limited to, changes in the focus and direction of our development programs, competitive and technical advances, costs associated with the development of our product candidates, our ability to secure partnering arrangements, and costs of filing, prosecuting, defending and enforcing our intellectual property rights. If we exhaust our capital reserves more quickly than anticipated, regardless of the reason, and we are unable to obtain additional financing on terms acceptable to us or at all, we will be unable to proceed with development of some or all of our product candidates on expected timelines and will be forced to prioritize among them.

We expect that we will need additional financing to support our long-term plans for clinical trials and new product development. We expect to finance our cash needs through the sale of equity securities, strategic collaborations and/or debt financings, or through other sources that may be dilutive to existing stockholders. There can be no assurance that we will be able to obtain funding from any of these sources or, if obtained, what the terms of such funding(s) may be, or that any amount that we are able to obtain will be adequate to support our working capital requirements until we achieve profitable operations. We have no current committed sources of additional capital. Recently, capital markets have experienced a period of instability that may severely hinder our ability to raise capital within the time periods needed or on terms we consider acceptable, if at all. If we are unable to raise additional funds when needed, we may not be able to continue development and regulatory approval of our products, or we could be required to delay, scale back or eliminate some or all our research and development programs.

In September 2014, approximately 2.3 million warrants to purchase shares of our common stock at \$2.04 per share will expire. If fully exercised, the warrants would result in net cash proceeds of up to approximately \$4.6 million. In December 2014, approximately 8.2 million warrants to purchase shares of our common stock at \$4.02 per share will expire. If fully exercised, the warrants would result in net cash proceeds of up to approximately \$3.1 million.

The following table summarizes our net increase (decrease) in cash and cash equivalents for the six months ended June 30, 2014 and 2013:

|  | Six mont<br>June |            |
|--|------------------|------------|
|  | 2014             | 2013       |
| (\$ in thousands)                                    |                  |            |
| Net cash provided by (used in):                      |                  |            |
| Operating activities                                 | \$(16,289)       | \$(34,964) |
| Investing activities                                 | (99)             | (115)      |
| Financing activities                                 | 1,185            | 705        |
| Net increase (decrease) in cash and cash equivalents | \$(15,203)       | \$(34,374) |

Net cash used in operating activities was \$16.3 million for the six months ended June 30, 2014 compared to \$35.0 million for the six months ended June 30, 2013. The \$18.7 million decrease in cash used was primarily due to a decrease in research and development expenses, in conjunction with our restructuring efforts (see note 4 to the accompanying unaudited financial statements).

Net cash used in investing activities was \$99 thousand for the six months ended June 30, 2014 compared to \$115 thousand for the six months ended June 30, 2013. The decrease was due to decreased spending on property, plant, and equipment.

Net cash provided by financing activities was \$1.2 million for the six months ended June 30, 2014 compared to \$0.7 million for the six months ended June 30, 2013. The change is primarily attributable to an increase in proceeds from stock option exercises.

# Operating capital and capital expenditure requirements

We anticipate that losses will continue for the foreseeable future. At June 30, 2014, our accumulated deficit was approximately \$356.1 million. Our actual cash requirements may vary materially from those planned because of a number of factors including:

- Changes in the focus, direction and pace of our development programs;
- Competitive and technical advances;
- Costs associated with the development of our product candidates;
- Our ability to secure partnering arrangements;
- Costs of filing, prosecuting, defending and enforcing any patent claims and any other intellectual property rights, or other developments, and
- Other matters identified under Part II Item 1A. "Risk Factors."

Working capital as of June 30, 2014 was \$43.2 million, consisting of \$54.0 million in current assets and \$10.8 million in current liabilities. Working capital as of December 31, 2013 was \$62.5 million, consisting of \$70.3 million in current assets and \$7.8 million in current liabilities.

# **Contractual obligations**

The following table summarizes our outstanding obligations as of June 30, 2014 and the effect those obligations are expected to have on our liquidity and cash flows in future periods:

| (\$ in thousands)           | Total   | Less than<br>1 year | <u>2 - 3 years</u> | <u>4 - 5 years</u> | More than<br>5 years |
|-----------------------------|---------|---------------------|--------------------|--------------------|----------------------|
| Operating leases            | \$3,763 | \$ 1,218            | \$ 1,866           | \$ 679             | \$ —                 |
| Royalty and license fees    | 2,125   | 1,275               | 550                | 300                | —                    |
| Contract milestone payments | 362     | 283                 | 79                 |                    |                      |
| Total                       | \$6,250 | \$ 2,776            | \$ 2,495           | \$ 979             | \$                   |

Our commitments for operating leases relate to the lease for our corporate headquarters in Boston, MA, and office space in New York, NY. Our commitments for royalty and license fees relate to our royalty agreements with Southern Research Institute, requiring minimum royalty payments, as well as our license agreement with The University of Texas M. D. Anderson Cancer Center, requiring payment upon the first patient treated in a pivotal trial in darinaparsin, currently being developed under the amended and restated License and Collaboration Agreement with Solasia. As part of the amended and restated License and Collaboration agreement (see note 8 to the accompanying unaudited financial statements), we will receive full reimbursement of this license payment. The contract milestone and contract installment payments relate to our agreement with Baxter Healthcare Corporation for the purchase of the assets relating to indibulin, and to our CRO agreements with Novella Clinical, Inc. The remaining contract installment payments to Baxter are comprised of four separate \$250 thousand payments on November 3, 2014-2017. The timing of the remaining contract milestone payments to Novella are dependent upon factors that are beyond our control, including our ability to recruit patients, the outcome of future clinical trials and any requirements imposed on our clinical trials by regulatory agencies. However, for the purpose of the above table, we have assumed that the payment of the milestones will occur within five years of June 30, 2014. On July 16, 2012, we decided to close our Germanton, Maryland office. In June 2013, we paid off the remainder of the Germantown, Maryland lease obligation. Included in the above table are obligations for the subleased portion of our Boston and New York offices as noted below and in Note 8 to the financial statements. We expect to receive a total of \$118 thousand in the next year and \$138 thousand in the next 2-3 years from our subtenant in the Boston office. We also expect to receive a total of

# **Off-balance sheet arrangements**

During the six months ended June 30, 2014 and 2013, we did not engage in any off-balance sheet arrangements.

# **Critical Accounting Policies and Estimates**

In our Annual Report on Form 10-K, as amended, for the fiscal year ended December 31, 2013, our most critical accounting policies and estimates upon which our financial status depends were identified as those relating to stock-based compensation; net operating losses and tax credit carryforwards; and impairment of long-lived assets. We reviewed our policies and determined that those policies remain our most critical accounting policies for the six months ended June 30, 2014.

# Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our exposure to market risk is limited to our cash. The goals of our investment policy are preservation of capital, fulfillment of liquidity needs and fiduciary control of cash. We also seek to maximize income from our investments without assuming significant risk. To achieve our goals, we maintain our cash in interest-bearing bank accounts in global banks, United States treasuries and other government-backed investments, which are subject to minimal interest rate risk.

#### Effect of Currency Exchange Rates and Exchange Rate Risk Management

We conduct clinical studies outside of the United States primarily in Western Europe. These business operations are not material at this time, therefore any currency fluctuations will not have a material impact on our financial position, results of operations or cash flows.

# **Item 4. Controls and Procedures**

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) as of the end of the period covered by this report. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of the end of such period, our disclosure controls and procedures were effective in ensuring that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported, on a timely basis, and is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

No change in our internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) occurred during the period covered by this quarterly report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

# Part II - Other Information

# **Item 1. Legal Proceedings**

In the ordinary course of business, we may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities. The results of litigation and claims cannot be predicted with certainty, and unfavorable resolutions are possible and could materially affect our results of operations, cash flows or financial position. In addition, regardless of the outcome, litigation could have an adverse impact on us because of defense costs, diversion of management resources and other factors.

While the outcome of these proceedings and claims cannot be predicted with certainty, there are no matters, as of June 30, 2014, that, in the opinion of management, might have a material adverse effect on our financial position, results of operations or cash flows.

#### Item 1A. Risk Factors

The following important factors could cause our actual business and financial results to differ materially from those contained in forward-looking statements made in this Quarterly Report on Form 10-Q or elsewhere by management from time to time. The risk factors in this report have been revised to incorporate changes to our risk factors from those included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013. The risk factors set forth below with an asterisk (\*) next to the title are new risk factors or risk factors containing changes, which may be material, from the risk factors previously disclosed in Item 1A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2013, as filed with the Securities and Exchange Commission.

# **RISKS RELATED TO OUR BUSINESS**

# \*We will require additional financial resources in order to continue ongoing development of our product candidates; if we are unable to obtain these additional resources, we may be forced to delay or discontinue clinical testing of our product candidates.

We have not generated significant revenue and have incurred significant net losses in each year since our inception. For the six months ended June 30, 2014, we had a net loss of \$15.3 million, and, as of June 30, 2014, we have incurred approximately \$356.1 million of cumulative net losses since our inception in 2003. We expect to continue to incur significant operating expenditures and net losses. Further development of our product candidates, including product candidates that we may develop under our Channel Agreement with Intrexon, will likely require substantial increases in our expenses as we:

- Continue to undertake clinical trials for product candidates;
- Scale-up the formulation and manufacturing of our product candidates;
- Seek regulatory approvals for product candidates;
- Work with regulatory authorities to identify and address program related inquiries;
- Implement additional internal systems and infrastructure; and
- Hire additional personnel.

We continue to seek additional financial resources to fund the further development of our product candidates. If we are unable to obtain sufficient additional capital, one or more of these programs could be placed on hold. Because we are currently devoting a significant portion of our resources to the development of synthetic biology and our adaptive Phase 3 trial for first-line SCLC for IV palifosfamide, MATISSE, further progress with the development of our other candidates may be significantly delayed and may depend on the licensing of those compounds to third parties.

We anticipate that our cash resources will be sufficient to fund our operations into the third quarter of 2015 and we have no current committed sources of additional capital. We do not know whether additional financing will be available on terms favorable or acceptable to us when needed, if at all. Our business is highly cash-intensive and our ability to continue operations after our current cash resources are exhausted depends on our ability to obtain additional financing and/or achieve profitable operations, as to which no assurances can be given. If adequate additional funds are not available when required, or if we are unsuccessful in entering into partnership agreements for the further development of our products, we will be required to delay, reduce or eliminate planned preclinical and clinical trials and may be forced to terminate the approval process for our product candidates from the FDA or other regulatory authorities. In addition, we could be forced to discontinue product development, forego attractive business opportunities or pursue merger or divestiture strategies. In the event we are unable to obtain additional financing, we may be forced to cease operations altogether.



### \*We need to raise additional capital to fund our operations. The manner in which we raise any additional funds may affect the value of your investment in our common stock.

As of June 30, 2014, we had incurred approximately \$356.1 million of cumulative net losses and had approximately \$53.0 million of cash and cash equivalents. We anticipate that our cash resources will be sufficient to fund our operations into the third quarter of 2015. Following negative results in our PICASSO 3 pivotal trial in first-line metastatic soft tissue sarcoma, or STS, in April 2013, we implemented a workforce reduction plan and other cost-cutting measures in an attempt to extend our cash resources as long as possible, though there are no assurances that such efforts will be effective. In addition, changes may occur that would consume our existing capital prior to the third quarter of 2015, including expansion of the scope of, and/or slower than expected progress of, our research and development efforts and changes in governmental regulation. Actual costs may ultimately vary from our current expectations, which could materially impact our use of capital and our forecast of the period of time through which our financial resources will be adequate to support our operations. We have estimated the sufficiency of our cash resources based in part on the discontinuation of the PICASSO 3 pivotal trial for first-line metastatic STS and our adaptive Phase 3 trial for first-line SCLC for IV palifosfamide and our current timing expectations for the interim analysis of data in the MATISSE trial. Also our estimates include the advancement of our synthetic biology product candidates in the clinic under our Channel Agreement with Intrexon, and we expect that the costs associated with these and additional product candidates will increase the level of our overall research and development expenses significantly going forward.

In addition to above factors, our actual cash requirements may vary materially from our current expectations for a number of other factors that may include, but are not limited to, changes in the focus and direction of our development programs, competitive and technical advances, costs associated with the development of our product candidates, our ability to secure partnering arrangements, and costs of filing, prosecuting, defending and enforcing our intellectual property rights. If we exhaust our capital reserves more quickly than anticipated, regardless of the reason, and we are unable to obtain additional financing on terms acceptable to us or at all, we will be unable to proceed with development of some or all of our product candidates on expected timelines and will be forced to prioritize among them.

The unpredictability of the capital markets may severely hinder our ability to raise capital within the time periods needed or on terms we consider acceptable, if at all. Moreover, if we fail to advance one or more of our current product candidates to later-stage clinical trials, successfully commercialize one or more of our product candidates, or acquire new product candidates for development, we may have difficulty attracting investors that might otherwise be a source of additional financing.

Our need for additional capital and limited capital resources may force us to accept financing terms that could be significantly dilutive to existing stockholders. To the extent that we raise additional capital by issuing equity securities, our stockholders may experience dilution. In addition, we may grant future investors rights superior to those of our existing stockholders. If we raise additional funds through collaborations and licensing arrangements, it may be necessary to relinquish some rights to our technologies, product candidates or products, or grant licenses on terms that are not favorable to us. If we raise additional funds by incurring debt, we could incur significant interest expense and become subject to covenants in the related transaction documentation that could affect the manner in which we conduct our business.

#### \*Clinical trials are very expensive, time-consuming, and difficult to design, initiate and implement.

Human clinical trials are very expensive and difficult to design, initiate and implement, in part because they are subject to rigorous regulatory requirements. The clinical trial start-up and process itself is also time-consuming and results are inherently uncertain. We estimate that clinical trials of our product candidates will take at least several years to complete. Furthermore, failure can occur at any stage of the trials, and we could encounter problems that cause us to delay the start, abandon or repeat clinical trials. The commencement and completion of clinical trials may be delayed by several factors, including:

- Additional nonclinical data requests by regulatory agencies;
- Unforeseen safety issues;
- Determination of dosing issues;
- Lack of effectiveness during clinical trials;
- Slower than expected rates of patient recruitment and enrollment;
- Inability to monitor patients adequately during or after treatment;
- Inability or unwillingness of medical investigators to follow our clinical protocols; and
- Regulatory determinations to temporarily or permanently cease enrollment for other reasons not related to patient safety.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. For example, despite positive findings in earlier clinical trials, our product candidate palifosfamide failed to meet the primary endpoint of the Phase 3 PICASSO 3 trail. In addition, we or the FDA may suspend our clinical trials at any time if it appears that we are exposing participants to unacceptable health risks or if the FDA finds deficiencies in our Investigational New Drug, or IND, submission or in the conduct of these trials.

See also "Risk Factors— Our product candidates are in various stages of clinical trials, which are very expensive and time-consuming. We cannot be certain when we will be able to file an NDA or BLA with the FDA and any failure or delay in completing clinical trials for our product candidates could harm our business."

#### We may not be able to obtain or maintain orphan drug exclusivity for our product candidates.

We have received Orphan Drug designations for darinaparsin for the treatment of peripheral T-cell lymphoma in both the United States and Europe, and we may be able to receive additional Orphan Drug designation from the FDA and the European Medicines Agency, or EMA, for other product candidates. In the United States, orphan designation is available to drugs intended to treat, diagnose or prevent a rare disease or condition that affects fewer than 200,000 people in the United States at the time of application for orphan designation. Orphan designation qualifies the sponsor of the product for a tax credit and marketing incentives. The first sponsor to receive FDA marketing approval for a drug with an orphan designation is entitled to a seven-year exclusive marketing period in the United States for that product for that indication and, typically, a waiver of the prescription drug user fee for its marketing application. However, a drug that the FDA considers to be clinically superior to, or different from, the approved orphan drug, even though for the same indication, may also obtain approval in the United States during the seven-year exclusive marketing period. Orphan drug exclusive marketing rights may also be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. There is no guarantee that any of our other product candidates will receive Orphan Drug designation or that, even if such product candidate is granted such status, the product candidate's clinical development and regulatory approval process will not be delayed or will be successful.



#### We may not be able to commercialize any products, generate significant revenues, or attain profitability.

To date, none of our product candidates have been approved for commercial sale in any country. The process to develop, obtain regulatory approval for, and commercialize potential drug candidates is long, complex, and costly. Unless and until we receive approval from the FDA and/or other regulatory authorities for our product candidates, we cannot sell our drugs and will not have product revenues. Even if we obtain regulatory approval for one or more of our product candidates, if we are unable to successfully commercialize our products, we may not be able to generate sufficient revenues to achieve or maintain profitability, or to continue our business without raising significant additional capital, which may not be available. Our failure to achieve or maintain profitability could negatively impact the trading price of our common stock.

#### Ethical, legal and social concerns about synthetic biologically engineered products could limit or prevent the use of our product candidates.

Our products candidates use a synthetic biology platform. Public perception about the safety and environmental hazards of, and ethical concerns over, genetically engineered products could influence public acceptance of our product candidates. If we and our collaborators are not able to overcome the ethical, legal and social concerns relating to synthetic biological engineering, our product candidates may not be accepted. These concerns could result in increased expenses, regulatory scrutiny, delays or other impediments to the public acceptance and commercialization of our product candidates. Our ability to develop and commercialize products could be limited by public attitudes and governmental regulation.

#### Our use of synthetic biology to develop product candidates may become subject to increasing regulation in the future.

Most of the laws and regulations concerning synthetic biology relate to the end products produced using synthetic biology, but that may change. For example, the Presidential Commission for the Study of Bioethical Issues in December 2010 recommended that the federal government oversee, but not regulate, synthetic biology research. The Presidential Commission also recommended that the government lead an ongoing review of developments in the synthetic biology field and that the government conduct a reasonable risk assessment before the field release of synthetic organisms. Synthetic biology may become subject to additional government regulations as a result of the recommendations, which could require us to incur significant additional capital and operating expenditures and other costs in complying with these laws and regulations.

### The technology on which our Channel Agreement with Intrexon Corporation is based in part on early stage technology in the field of human oncologic therapeutics.

Our Channel Agreement with Intrexon contemplates our using Intrexon's advanced transgene engineering platform for the controlled and precise cellular production of anti-cancer effectors. The synthetic biology effector platform in which we have acquired rights represents early-stage technology in the field of human oncologic biotherapeutics, with DC-RTS-IL-12 + veledimex having completed a Phase 1 study in melanoma and Ad-RTS-IL-12 + veledimex currently in two Phase 2 studies, in melanoma and breast cancer. Although we plan to leverage Intrexon's synthetic biology platform for additional products targeting key pathways used by cancers to grow and metastasize, we may not be successful in developing and commercializing these products for a variety of reasons. The risk factors set forth herein that apply to our small molecule drug candidates, which are in various stages of development, also apply to product candidates that we seek to develop under our Channel Agreement with Intrexon.

#### We will incur additional expenses in connection with our Channel Agreement with Intrexon Corporation.

The synthetic biology platform, in which we have acquired rights for cancer from Intrexon, includes two existing product candidates, Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex. Upon entry into the Channel Agreement with Intrexon, we assumed responsibility for the clinical development of these product candidates, which we expect will increase the level of our overall research and development expenses significantly going forward. Although all human clinical trials are expensive and difficult to design and implement, we believe that due to complexity, costs associated with clinical trials for synthetic biology products are greater than the corresponding costs associated with clinical trials for small molecule candidates. In addition to increased research and development costs, prior to the adoption of our April 2013 workforce reduction plan we added headcount in part to support our Channel Agreement endeavors, and we may need to do so again in the future which would add to our general and administrative expenses going forward.

Although our forecasts for expenses and the sufficiency of our capital resources takes into account our plans to develop the Intrexon products, we assumed development responsibility for these products on January 6, 2011, and the actual costs associated therewith may be significantly in excess of forecasted amounts. In addition to the amount and timing of expenses related to the clinical trials, our actual cash requirements may vary materially from our current expectations for a number of other factors that may include, but are not limited to, changes in the focus and direction of our development programs, competitive and technical advances, costs associated with the development of our product candidates and costs of filing, prosecuting, defending and enforcing our intellectual property rights. If we exhaust our capital reserves more quickly than anticipated, regardless of the reason, and we are unable to obtain additional financing on terms acceptable to us or at all, we will be unable to proceed with development of some or all of our product candidates on expected timelines and will be forced to prioritize among them.

# We may not be able to retain the exclusive rights licensed to us by Intrexon Corporation to develop and commercialize products involving DNA administered to humans for expression of anti-cancer effectors for the purpose of treatment or prophylaxis of cancer.

Under the Channel Agreement, we use Intrexon's technology directed towards in vivo expression of effectors in connection with the development of Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex and generally to research, develop and commercialize products, in each case in which DNA is administered to humans for expression of anti-cancer effectors for the purpose of treatment or prophylaxis of cancer, which we collectively refer to as the Cancer Program. The Channel Agreement grants us a worldwide license to use patents and other intellectual property of Intrexon in connection with the research, development, use, importing, manufacture, sale, and offer for sale of products involving DNA administered to humans for expression of anti-cancer effectors for the purpose of treatment or prophylaxis of cancer, which we refer to collectively as the ZIOPHARM Products. Such license is exclusive with respect to any clinical development, selling, offering for sale or other commercialization of ZIOPHARM Products, and otherwise is non-exclusive. Subject to limited exceptions, we may not sublicense the rights described without Intrexon's written consent. Under the Channel Agreement, and subject to certain exceptions, we are responsible for, among other things, the performance of the Cancer Program, including development, commercialization and certain aspects of manufacturing of ZIOPHARM Products.

Intrexon may terminate the Channel Agreement if we fail to use diligent efforts to develop and commercialize ZIOPHARM Products or if we elect not to pursue the development of a Cancer Program identified by Intrexon that is a "Superior Therapy" as defined in the Channel Agreement. We may voluntarily terminate the Channel Agreement upon 90 days written notice to Intrexon. Upon termination of the Channel Agreement, we may continue to develop and commercialize any ZIOPHARM Product that, at the time of termination:

- Is being commercialized by us;
- Has received regulatory approval;
- Is a subject of an application for regulatory approval that is pending before the applicable regulatory authority; or
- Is the subject of at least an ongoing Phase 2 clinical trial (in the case of a termination by Intrexon due to an uncured breach or a voluntary termination by us), or an ongoing Phase 1 clinical trial in the field (in the case of a termination by us due to an uncured breach or a termination by Intrexon following an unconsented assignment by us or our election not to pursue development of a Superior Therapy).

Our obligation to pay 50% of net profits or revenue as described further in Note 8 in the accompanying unaudited financial statements under the heading *"License Agreements—Exclusive Channel Partner Agreement with Intrexon Corporation"* with respect to these "retained" products will survive termination of the Channel Agreement.

There can be no assurance that we will be able to successfully perform under the Channel Agreement and if the Channel Agreement is terminated it may prevent us from achieving our business objectives.

#### \*We have a limited operating history upon which to base an investment decision.

We have not demonstrated an ability to perform the functions necessary for the successful commercialization of any product candidates. The successful commercialization of any product candidates will require us to perform a variety of functions, including:

- Continuing to undertake preclinical development and clinical trials;
- Participating in regulatory approval process;
- Formulating and manufacturing products; and
- Conducting sales and marketing activities.

Our operations have been limited to organizing and staffing our company, acquiring, developing and securing our proprietary product candidates, and undertaking preclinical and clinical trials of our product candidates. These operations provide a limited basis for you to assess our ability to commercialize our product candidates and the advisability of investing in our securities.

# Because we currently neither have nor intend to establish internal research capabilities, we are dependent upon pharmaceutical and biotechnology companies and academic and other researchers to sell or license us their product candidates and technology.

Proposing, negotiating, and implementing an economically viable product acquisition or license is a lengthy and complex process. We compete for partnering arrangements and license agreements with pharmaceutical, biopharmaceutical, and biotechnology companies, many of which have significantly more experience than we do, and have significantly more financial resources. Our competitors may have stronger relationships with certain third parties including academic research institutions, with whom we are interested in collaborating and may have, therefore, a competitive advantage in entering into partnering arrangements with those third parties. We may not be able to acquire rights to additional product candidates on terms that we find acceptable, or at all.

We expect that any product candidate to which we acquire rights will require significant additional development and other efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All drug product candidates are subject to the risks of failure inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe or effective for approval by regulatory authorities. Even if our product candidates are approved, they may not be economically manufactured or produced, or be successfully commercialized.

We actively evaluate additional product candidates to acquire for development. Such additional product candidates, if any, could significantly increase our capital requirements and place further strain on the time of our existing personnel, which may delay or otherwise adversely affect the development of our existing product candidates. We must manage our development efforts and clinical trials effectively, and hire, train and integrate additional management, administrative, and sales and marketing personnel. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing.

#### We may not be able to successfully manage our growth.

In the future, if we are able to advance our product candidates to the point of, and thereafter through, clinical trials, we will need to expand our development, regulatory, manufacturing, marketing and sales capabilities or contract with third parties to provide for these capabilities. Any future growth will place a significant strain on our management and on our administrative, operational, and financial resources. Therefore, our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To manage this growth, we must expand our facilities, augment our operational, financial and management systems, and hire and train additional qualified personnel. If we are unable to manage our growth effectively, our business may be harmed.

#### Our business will subject us to the risk of liability claims associated with the use of hazardous materials and chemicals.

Our contract research and development activities may involve the controlled use of hazardous materials and chemicals. Although we believe that our safety procedures for using, storing, handling and disposing of these materials comply with federal, state and local laws and regulations, we cannot completely eliminate the risk of accidental injury or contamination from these materials. In the event of such an accident, we could be held liable for any resulting damages and any liability could have a materially adverse effect on our business, financial condition, and results of operations. In addition, the federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of hazardous or radioactive materials and waste products may require our contractors to incur substantial compliance costs that could materially adversely affect our business, financial condition, and results of operations.

### We rely on key executive officers and scientific and medical advisors, and their knowledge of our business and technical expertise would be difficult to replace.

We are highly dependent on Dr. Jonathan Lewis, our Chief Executive Officer, Caesar J. Belbel, our Executive Vice President and Chief Legal Officer and our principal scientific, regulatory, and medical advisors. Dr. Lewis' and Mr. Belbel's employment are governed by written employment agreements. The employment agreement with Dr. Lewis, as amended, provides for a term that expires in January 2015. Dr. Lewis and Mr. Belbel may terminate their employment with us at any time, subject, however, to certain non-compete and non-solicitation covenants. The loss of the technical knowledge and management and industry expertise of Dr. Lewis and Mr. Belbel, or any of our other key personnel, could result in delays in product development, loss of customers and sales, and diversion of management resources, which could adversely affect our operating results. We do not carry "key person" life insurance policies on any of our officers or key employees.

#### If we are unable to hire additional qualified personnel, our ability to grow our business may be harmed.

We will need to hire additional qualified personnel with expertise in preclinical and clinical research and testing, government regulation, formulation and manufacturing, and eventually, sales and marketing. We compete for qualified individuals with numerous biopharmaceutical companies, universities, and other research institutions. Competition for such individuals is intense and we cannot be certain that our search for such personnel will be successful. Attracting and retaining qualified personnel will be critical to our success. If we are unable to hire additional qualified personnel, our ability to grow our business may be harmed.

#### We may incur substantial liabilities and may be required to limit commercialization of our products in response to product liability lawsuits.

The testing and marketing of medical products entail an inherent risk of product liability. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our products, if approved. Even a successful defense would require significant financial and management resources. Regardless of the merit or eventual outcome, liability claims may result in:

- Decreased demand for our product candidates;
- Injury to our reputation;
- Withdrawal of clinical trial participants;
- Withdrawal of prior governmental approvals;
- Costs of related litigation;
- Substantial monetary awards to patients;
- Product recalls;
- Loss of revenue; and
- The inability to commercialize our product candidates.

We currently carry clinical trial insurance and product liability insurance. However, an inability to renew our policies or to obtain sufficient insurance at an acceptable cost could prevent or inhibit the commercialization of pharmaceutical products that we develop, alone or with collaborators.

#### Our business and operations would suffer in the event of system failures.

Despite the implementation of security measures, our internal computer systems and those of our current and future contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we are not aware of any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on third parties to manufacture our drug candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our drug candidates could be delayed.

#### RISKS RELATED TO THE CLINICAL TESTING, REGULATORY APPROVAL AND MANUFACTURING OF OUR PRODUCT CANDIDATES

#### If we are unable to obtain the necessary U.S. or worldwide regulatory approvals to commercialize any product candidate, our business will suffer.

We may not be able to obtain the approvals necessary to commercialize our product candidates, or any product candidate that we may acquire or develop in the future for commercial sale. We will need FDA approval to commercialize our product candidates in the United States and approvals from regulatory authorities in foreign jurisdictions equivalent to the FDA to commercialize our product candidates in those jurisdictions. In order to obtain FDA approval of any product candidate, we must submit to the FDA a New Drug Application, or NDA, or Biologics License Application, or BLA, demonstrating that the product candidate is safe for humans and effective for its intended use. This demonstration requires significant research and animal tests, which are referred to as preclinical studies, as well as human tests, which are referred to as clinical trials. Satisfaction of the FDA's regulatory requirements typically takes many years, depending upon the type, complexity, and novelty of the product candidate, and will require substantial resources for research, development, and testing. We cannot predict whether our research, development, and clinical approaches will result in drugs that the FDA will consider safe for humans and effective for their intended uses. The FDA has substantial discretion in the drug approval process and may require us to conduct additional preclinical and clinical testing or to perform post-marketing studies. The approval process may also be delayed by changes in government regulation, future legislation, or administrative action or changes in FDA policy that occur prior to or during our regulatory review. Delays in obtaining regulatory approvals may:

- · Delay commercialization of, and our ability to derive product revenues from, our product candidates;
- Impose costly procedures on us; and
- Diminish any competitive advantages that we may otherwise enjoy.

Even if we comply with all FDA requests, the FDA may ultimately reject one or more of our NDAs or BLAs. We cannot be sure that we will ever obtain regulatory approval for any of our product candidates. Failure to obtain FDA approval for our product candidates will severely undermine our business by leaving us without a saleable product, and therefore without any potential revenue source, until another product candidate can be developed. There is no guarantee that we will ever be able to develop or acquire another product candidate or that we will obtain FDA approval if we are able to do so.

In foreign jurisdictions, we similarly must receive approval from applicable regulatory authorities before we can commercialize any drugs. Foreign regulatory approval processes generally include all of the risks associated with the FDA approval procedures described above.

## Our product candidates are in various stages of clinical trials, which are very expensive and time-consuming. We cannot be certain when we will be able to submit an NDA or BLA to the FDA and any failure or delay in completing clinical trials for our product candidates could harm our business.

Our product candidates are in various stages of development and require extensive clinical testing. Notwithstanding our current clinical trial plans for each of our existing product candidates, we may not be able to commence additional trials or see results from these trials within our anticipated timelines. As such, we cannot predict with any certainty if or when we might submit an NDA or BLA for regulatory approval of our product candidates or whether such an NDA or BLA will be accepted. Because we do not anticipate generating revenues unless and until we submit one or more NDAs or BLAs and thereafter obtain requisite FDA approvals, the timing of our NDA or BLA submissions and FDA determinations regarding approval thereof, will directly affect if and when we are able to generate revenues.

#### The results of our clinical trials may not support our product candidate claims.

Even if our clinical trials are completed as planned, we cannot be certain that their results will support approval of our product candidates. The FDA normally expects two randomized, well-controlled Phase 3 pivotal studies in support of approval of an NDA or BLA. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be certain that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. For example, despite positive findings in earlier clinical trials, our product candidate palifosfamide failed to meet the primary endpoints of the Phase 3 PICASSO 3 trial, causing us to suspend clinical development of palifosfamide in soft tissue sarcoma. The clinical trial process may fail to demonstrate that our product candidates are safe for humans and effective for the indicated uses. This failure would cause us to abandon a product candidate and may delay development of other product candidates. Any delay in, or termination of, our clinical trials will delay the submission of our NDAs or BLAs with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenues. In addition, our clinical trials involve small patient populations. Because of the small sample size, the results of these clinical trials may not be indicative of future results.

# Our synthetic biology product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Currently, no gene therapy products have been approved in the United States and only one product has been approved in Europe.

We have recently focused our product research and development efforts on our synthetic biology product candidates under our Channel Agreement with Intrexon. These products, including Ad-RTS-IL-12 + veledimex and DC-RTS-IL-12 + veledimex, are based on gene therapy technology. Due to the novelty of this medical technology, there can be no assurance that any development problems we experience in the future related to our synthetic biology platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience unanticipated problems or delays in expanding our manufacturing capacity or transferring our manufacturing process to commercial partners, which may prevent us from completing our clinical studies or commercializing our synthetic biology product candidates on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. Currently, only one gene therapy product, UniQure's Glybera, which received marketing authorization from the EMA in 2012, has been approved in Europe but has not yet been launched for commercial sale, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or Europe. Approvals by the EMA may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical studies conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation. Conversely, the FDA can put an IND on clinical hold even if the RAC has provided a favorable review. Also, before a clinical trial can begin at an NIH-funded institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions. As we advance our synthetic biology product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected for oncology product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

### Because we are dependent upon clinical research institutions and other contractors for clinical testing and for research and development activities, the results of our clinical trials and such research activities are, to a certain extent, beyond our control.

We materially rely upon independent investigators and collaborators, such as universities and medical institutions, to conduct our preclinical and clinical trials under agreements with us. These collaborators are not our employees and we cannot control the amount or timing of resources that they devote to our programs. These investigators may not assign as great a priority to our programs or pursue them as diligently as we would if we were undertaking such programs ourselves. If outside collaborators fail to devote sufficient time and resources to our drug development programs, or if their performance is substandard, the approval of our FDA applications, if any, and our introduction of new products, if any, will be delayed. These collaborators may also have relationships with other commercial entities, some of whom may compete with us. If our collaborators assist our competitors to our detriment, our competitive position would be harmed.

### Our reliance on third parties to formulate and manufacture our product candidates exposes us to a number of risks that may delay the development, regulatory approval and commercialization of our products or result in higher product costs.

We do not have experience in drug formulation or manufacturing of drugs or biologics and do not intend to establish our own manufacturing facilities. Although we will work closely with and rely upon Intrexon on the manufacturing and scale-up of Intrexon product candidates, we lack the resources and expertise to formulate or manufacture our own product candidates. We currently are contracting for the manufacture of our product candidates. We intend to contract with one or more manufacturers to manufacture, supply, store, and distribute drug supplies for our clinical trials. If a product candidate we develop or acquire in the future receives FDA approval, we will rely on one or more third-party contractors or Intrexon to manufacture our products. Our anticipated future reliance on a limited number of third-party manufacturers exposes us to the following risks:

- We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA must approve any replacement contractor. This approval would require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- Our third-party manufacturers might be unable to formulate and manufacture our products in the volume and of the quality required to meet our clinical needs and commercial needs, if any.
- Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products.
- Drug manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration and
  corresponding state agencies to ensure strict compliance with good manufacturing practices, or cGMP, and other government regulations and
  corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- If any third-party manufacturer makes improvements in the manufacturing process for our products, we may not own, or may have to share, the intellectual property rights to the innovation.
- Our third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Each of these risks could delay our clinical trials, the approval, if any, of our product candidates by the FDA or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenues.

# Any drug candidate for which we obtain marketing approval could be subject to post-marketing restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any drug candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a drug candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy, or REMS, which could include requirements for a restricted distribution system. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the product.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of our approved products. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products outside of their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the FDCA relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.



In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- Litigation involving patients taking our drug;
- Restrictions on such products, manufacturers or manufacturing processes;
- Restrictions on the labeling or marketing of a product;
- Restrictions on product distribution or use;
- Requirements to conduct post-marketing studies or clinical trials;
- Warning letters;
- Withdrawal of the products from the market;
- Refusal to approve pending applications or supplements to approved applications that we submit;
- Recall of products;
- Fines, restitution or disgorgement of profits or revenues;
- Suspension or withdrawal of marketing approvals;
- Damage to relationships with existing and potential collaborators;
- Unfavorable press coverage and damage to our reputation;
- Refusal to permit the import or export of our products;
- Product seizure; or
- Injunctions or the imposition of civil or criminal penalties.

Noncompliance with similar European Union requirements regarding safety monitoring or pharmacovigilance can also result in significant financial penalties. Similarly, failure to comply with U.S. and foreign regulatory requirements regarding the development of products for pediatric populations and the protection of personal health information can also lead to significant penalties and sanctions.

#### RISKS RELATED TO OUR ABILITY TO COMMERCIALIZE OUR PRODUCT CANDIDATES

# If we are unable either to create sales, marketing and distribution capabilities or enter into agreements with third parties to perform these functions, we will be unable to commercialize our product candidates successfully.

We currently have no marketing, sales, or distribution capabilities. If and when we become reasonably certain that we will be able to commercialize our current or future product candidates, we anticipate allocating resources to the marketing, sales and distribution of our proposed products in North America and in certain other countries; however, we cannot assure that we will be able to market, sell, and distribute our product successfully. Our future success also may depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities and to encourage the collaborator's strategic interest in the products under development, and such collaborator's ability to successfully market and sell any such products. Although we intend to pursue certain collaborative arrangements regarding the sale and marketing of certain of our product candidates, there are no assurances that we will be able to establish or maintain relationships with third-party collaborators or develop in-house sales and distribution capabilities. To the extent that we depend on third parties for marketing and distribution, any revenues we receive will depend upon the efforts of such third parties, and there can be no assurance that such efforts will be successful. In addition, there can also be no assurance that we will be able to market and sell our product candidates in the United States or overseas.

If we are not able to partner with a third party and are not successful in recruiting sales and marketing personnel or in building a sales and marketing infrastructure, we will have difficulty commercializing our product candidates, which would harm our business. If we rely on pharmaceutical or biotechnology companies with established distribution systems to market our products, we will need to establish and maintain partnership arrangements, and we may not be able to enter into these arrangements on acceptable terms or at all. To the extent that we enter into co-promotion or other arrangements, any revenues we receive will depend upon the efforts of third parties that may not be successful and that will be only partially in our control.



# If we cannot compete successfully for market share against other drug companies, we may not achieve sufficient product revenues and our business will suffer.

The market for our product candidates is characterized by intense competition and rapid technological advances. If a product candidate receives FDA approval, it will compete with a number of existing and future drugs and therapies developed, manufactured and marketed by others. Existing or future competing products may provide greater therapeutic convenience or clinical or other benefits for a specific indication than our products, or may offer comparable performance at a lower cost. If our products fail to capture and maintain market share, we may not achieve sufficient product revenues and our business will suffer.

We will compete against fully integrated pharmaceutical companies and smaller companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Many of these competitors have products already approved or in development. In addition, many of these competitors, either alone or together with their collaborative partners, operate larger research and development programs or have substantially greater financial resources than we do, as well as significantly greater experience in:

- Developing drugs and biopharmaceuticals;
- Undertaking preclinical testing and human clinical trials;
- Obtaining FDA and other regulatory approvals of drugs and biopharmaceuticals;
- Formulating and manufacturing drugs and biopharmaceuticals; and
- Launching, marketing, and selling drugs and biopharmaceuticals.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products.

# If physicians and patients do not accept and use our product candidates, our ability to generate revenue from sales of our products will be materially impaired.

Even if the FDA approves our product candidates, physicians and patients may not accept and use them. Acceptance and use of our products will depend upon a number of factors including:

- Perceptions by members of the healthcare community, including physicians, about the safety and effectiveness of our drugs;
- Pharmacological benefit and cost-effectiveness of our products relative to competing products;
- Availability of coverage and adequate reimbursement for our products from government or other healthcare payors;
- · Effectiveness of marketing and distribution efforts by us and our licensees and distributors, if any; and
- The price at which we sell our products.

Because we expect sales of our current product candidates, if approved, to generate substantially all of our product revenues for the foreseeable future, the failure of a drug to find market acceptance would harm our business and could require us to seek additional financing in order to fund the development of future product candidates.

#### Our ability to generate product revenues will be diminished if our drugs do not obtain coverage adequate reimbursement from payors.

Our ability to commercialize our drugs, alone or with collaborators, will depend in part on the extent to which coverage and reimbursement will be available from government and health administration authorities, private health maintenance organizations and health insurers and other third-party payors.

Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

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

Third-party payors, whether foreign or domestic, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In addition, in the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that requires us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that approval will be obtained. If we are unable to obtain coverage of and adequate payment levels for our product candidates from third-party payors, physicians may limit how much or under what circumstances they will prescribe or administer them and patients may decline to purchase them. This in turn could affect our ability to successfully commercialize our products and impact our profitability, results of operations, financial condition, and future success.

In addition, in many foreign countries, particularly the countries of the European Union, the pricing of prescription drugs is subject to government control. In some non-U.S. jurisdictions, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. We may face competition for our product candidates from lower-priced products in foreign countries that have placed price controls on pharmaceutical products. In addition, there may be importation of foreign products that compete with our own products, which could negatively impact our profitability.

#### Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory proposals in recent years to change the healthcare system in ways that could impact our future ability to sell our product candidates profitably. For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 established a new Part D prescription drug benefit, which became effective January 1, 2006. Under the prescription drug benefit, Medicare beneficiaries can obtain prescription drug coverage from private sector plans that are permitted to limit the number of prescription drugs that are covered in each therapeutic category and class on their formularies. If any of our product candidates that are approved by the FDA are not widely included on the formularies of these plans, our ability to market our products to the Medicare population could suffer.

Furthermore, there have been and continue to be a number of initiatives at the federal and state level that seek to reduce healthcare costs. Most recently, in March 2010, President Obama signed into law the Patient Protection and Affordable Health Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the PPACA, which includes measures that significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the PPACA of importance to the pharmaceutical industry are the following:

- An annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, beginning in 2011;
- An increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23% and 13% of the average manufacturer price for most branded and generic drugs, respectively;
- A new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D, beginning in 2011;
- Extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations, effective March 23, 2010;
- Expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional
  individuals beginning in April 2010 and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of
  the Federal Poverty Level beginning in 2014, thereby potentially increasing both the volume of sales and manufacturers' Medicaid rebate
  liability; a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off
  negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's
  outpatient drugs to be covered under Medicare Part D, beginning in 2011;
- Expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program, effective in January 2010;
- A new requirement to annually report drug samples that manufacturers and distributors provide to physicians, effective April 1, 2012;
- Expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- A licensure framework for follow-on biologic products;
- A new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- Creation of the Independent Payment Advisory Board which, beginning in 2014, will have authority to recommend certain changes to the Medicare program that could result in reduced payments for prescription drugs and those recommendations could have the effect of law even if Congress does not act on the recommendations; and
- Establishment of a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending beginning by January 1, 2011.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee on Deficit Reduction did not achieve its targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. The full impact of these new laws, as well as laws and other reform and cost containment measures that may be proposed and adopted in the future, remains uncertain, but may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our future customers and accordingly, our ability to generate revenue, attain profitability, or commercialize our products.

### \*If we fail to comply with federal and state healthcare laws, including fraud and abuse and health information privacy and security laws, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other thirdparty payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We could be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- The federal Anti-Kickback Statute, which constrains our marketing practices, educational programs, pricing policies, and relationships with healthcare providers or other entities, by prohibiting, among other things, soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, either the referral of an individual or the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- Federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
- The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- New requirements to report certain financial arrangements with physicians and teaching hospitals, as defined in the PPACA and its implementing
  regulations, including reporting any "transfer of value" made or distributed to teaching hospitals, prescribers, and other healthcare providers and
  reporting any ownership and investment interests held by physicians and their immediate family members and applicable group purchasing
  organizations during the preceding calendar year, with data collection required as of August 1, 2013 and reporting to the Centers for Medicare &
  Medicaid Services, or CMS, required as of March 31, 2014 and by the 90th day of each subsequent calendar year;
- State and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers, and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has further strengthened these laws. For example, the PPACA, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

To the extent that any of our product candidates is ultimately sold in a foreign country, we may be subject to similar foreign laws and regulations. If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in United States federal or state health care programs, and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could materially adversely affect our ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

#### Our ability to use net operating loss carryforwards to reduce future tax payments may be limited or restricted.

We have generated significant net operating loss carryforwards, or NOLs, as a result of our incurrence of losses since inception. We generally are able to carry NOLs forward to reduce taxable income in future years. However, our ability to utilize the NOLs is subject to the rules of Section 382 of the Internal Revenue Code. Section 382 generally restricts the use of NOLs after an "ownership change." An ownership change occurs if, among other things, the stockholders (or specified groups of stockholders) who own or have owned, directly or indirectly, 5% or more of a corporation's common stock or are otherwise treated as 5% stockholders under Section 382 and the United States Treasury Department regulations promulgated thereunder increase their aggregate percentage ownership of that corporation's stock by more than 50 percentage points over the lowest percentage of the stock owned by these stockholders over the applicable testing period. In the event of an ownership change, Section 382 imposes an annual limitation on the amount of taxable income a corporation may offset with NOL carry forwards. This annual limitation is generally equal to the product of the value of the corporation's stock on the date of the ownership change, multiplied by the long-term tax-exempt rate published monthly by the Internal Revenue Service. Any unused annual limitation may be carried over to later years until the applicable expiration date for the respective NOL carry forwards. We may have experienced an "ownership change" within the meaning of Section 382 in the past and there can be no assurance that we have not experienced additional ownership changes. As a result, our NOLs may be subject to limitations and we may be required to pay taxes earlier and in larger amounts than would be the case if our NOLs were freely usable.

#### Our synthetic biology product candidates may face competition in the future from follow-on biologics.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as part of the Patient Protection and Affordable Care Act, an abbreviated pathway for the approval of follow-on biological products was created. The new abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" with an existing brand product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product was approved under a BLA. The new law is complex and is only beginning to be interpreted and implemented by the FDA. As a result, its ultimate impact is subject to uncertainty, and could have a material adverse effect on the future commercial prospects for our biological products.

#### RISKS RELATED TO OUR INTELLECTUAL PROPERTY

# If we or our licensors fail to adequately protect or enforce our intellectual property rights or secure rights to patents of others, the value of our intellectual property rights would diminish and our ability to successfully commercialize our products may be impaired.

Our success, competitive position, and future revenues will depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our products, methods, processes and other technologies, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights, and to operate without infringing the proprietary rights of third parties.

To date, we have exclusive rights to certain United States and foreign intellectual property with respect to our small molecule product candidates and with respect to the Intrexon technology, including the existing Intrexon product candidates. Under our Channel Agreement with Intrexon, Intrexon has the sole right to conduct and control the filings, prosecution and maintenance of the patents and patent applications licensed to us. Although under the agreement Intrexon has agreed to consider in good faith and consult with us regarding any comments we may have regarding these patents and patent applications, we cannot guarantee that our comments will be solicited or followed. Without direct control of the channel program patents and patent applications, we are dependent on Intrexon to keep us advised of prosecution, particularly in foreign jurisdictions where prosecution information may not be publicly available. We anticipate that we and Intrexon will file additional patent applications both in the United States and in other countries. However, we cannot predict or guarantee:

- The degree and range of protection any patents will afford us against competitors, including whether third parties will find ways to invalidate or otherwise circumvent our patents;
- If and when patents will be issued;
- Whether or not others will obtain patents claiming subject matter related to or relevant to our product candidates; or
- Whether we will need to initiate litigation or administrative proceedings that may be costly whether we win or lose.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost, in a timely manner, or in all jurisdictions. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. We may also require the cooperation of our licensors in order to enforce the licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States and we may fail to seek or obtain patent protection in all major markets. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all.

Changes in patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. In September 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law, resulting in a number of significant changes to United States patent law. These changes include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In addition, the United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the value of patents, once obtained, and with regard to our ability to obtain patents in the future. As the United States Patent and Trademark Office continues to implement the Leahy-Smith Act, and as the federal courts have the opportunity to interpret the Leahy-Smith Act, the laws and regulations governing patents, and the rules regarding patent procurement could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.



Certain technologies utilized in our research and development programs are already in the public domain. Moreover, a number of our competitors have developed technologies, filed patent applications or obtained patents on technologies, compositions and methods of use that are related to our business and may cover or conflict with our owned or licensed patent applications, technologies or product candidates. Such conflicts could limit the scope of the patents that we may be able to obtain or may result in the rejection of claims in our patent applications. Because patent applications in the United States and many foreign jurisdictions are typically not published until eighteen months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind actual discoveries, neither we nor our licensors can be certain that others have not filed or maintained patent applications for technology used by us or covered by our pending patent applications without our being aware of these applications. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned patents or pending patent applications, or that we were the first to file for patent protection of such inventions, nor can we know whether those from whom we license patents were the first to make the inventions claimed or were the first to file. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, our own earlier filed patents and applications or those of Intrexon may limit the scope of later patents we obtain or may result in the rejection of claims in our later filed patent applications. If third parties filed patent applications or obtained patents on technologies, compositions and methods of use that are related to our business and that cover or conflict with our owned or licensed patent applications, technologies or product candidates, we may be required to challenge such protection, terminate or modify our programs impacted by such protection or obtain licenses from such third parties, which might not be available on acceptable terms, or at all.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

#### If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

Our success also depends upon the skills, knowledge, and experience of our scientific and technical personnel, our consultants and advisors, as well as our licensors and contractors. To help protect our proprietary know-how and our inventions for which patents may be unobtainable or difficult to obtain, and to maintain our competitive position, we rely on trade secret protection and confidentiality agreements. To this end, it is our general policy to require our employees, consultants, advisors, and contractors to enter into agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries, and inventions important to our business. These agreements may not provide adequate protection for our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure or the lawful development by others of such information. Moreover, we may not be able to obtain adequate remedies for any breaches of these agreements. Our trade secrets may also be obtained by third parties by other means, such as breaches of our physical or computer security systems. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets, know-how or other proprietary information is disclosed, the value of our trade secrets, know-how and other proprietary rights would be significantly impaired and our business and competitive position would suffer.

### Third-party claims of intellectual property infringement would require us to spend significant time and money and could prevent us from developing or commercializing our products.

In order to protect or enforce patent rights, we, or Intrexon, may initiate patent infringement litigation against third parties. Similarly, we may be sued by others for patent infringement. We also may become subject to proceedings conducted in the United States Patent and Trademark Office, including interference proceedings to determine the priority or derivation of inventions, or post-grant review, inter partes review, or reexamination proceedings reviewing the patentability of our patented claims. In addition, any foreign patents that are granted may become subject to opposition, nullity, or revocation proceedings in foreign jurisdictions having such proceedings. The defense and prosecution, if necessary, of intellectual property actions are costly and divert technical and management personnel away from their normal responsibilities.

Our commercial success depends upon our ability, and the ability of our collaborators, to develop, manufacture, market and sell our drug candidates without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. While no such litigation has been brought against us and we have not been held by any court to have infringed a third party's intellectual property rights, we cannot guarantee that our products or use of our products do not infringe third-party patents. It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States and elsewhere are published approximately 18 months after the earliest filing, which is referred to as the priority date. Therefore, patent applications covering our products or technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our products or the use of our products.

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be claimed to infringe patents or patent applications under which we do not hold licenses or other rights. Patents do not protect its owner from a claim of infringement of another owner's patent. Therefore, our patent position cannot and does not provide any assurance that we are not infringing the patent rights of another.

The patent landscape in the field of synthetic biology, which we are pursuing under our Channel Agreement with Intrexon, is particularly complex. We are aware of numerous United States and foreign patents and pending patent applications of third parties that cover compositions, methods of use and methods of manufacture of synthetic biology, including biotherapeutics involving the *in vivo* expression of human IL-12. In addition, there may be patents and patent applications in the field of which we are not aware. The technology we license from Intrexon is early-stage technology and we are just beginning the process of designing and developing products using this technology. Although we will seek to avoid pursuing the development of products that may infringe any patent claims that we believe to be valid and enforceable, we may fail to do so. Moreover, given the breadth and number of claims in patents and pending patent applications in the field of synthetic biology and the complexities and uncertainties associated with them, third parties may allege that we are infringing upon patent claims even if we do not believe such claims to be valid and enforceable.

If a claim for patent infringement is asserted, there can be no assurance that the resolution of the claim would permit us to continue marketing the relevant product on commercially reasonable terms, if at all. We may not have sufficient resources to bring these actions to a successful conclusion. If we do not successfully defend any infringement actions to which we become a party or are unable to have infringed patents declared invalid or unenforceable, we may have to pay substantial monetary damages, which can be tripled if the infringement is deemed willful, or be required to discontinue or significantly delay commercialization and development of the affected products.

Any legal action against us or our collaborators claiming damages and seeking to enjoin developmental or marketing activities relating to affected products could, in addition to subjecting us to potential liability for damages, require us or our collaborators to obtain licenses to continue to develop, manufacture, or market the affected products. Such a license may not be available to us on commercially reasonable terms, if at all.

An adverse determination in a proceeding involving our owned or licensed intellectual property may allow entry of generic substitutes for our products.

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

Periodic maintenance fees on any issued patent are due to be paid to the United States Patent and Trademark Office and foreign patent agencies in several stages over the lifetime of the patent. The United States Patent and Trademark Office and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

# If we breach any of the agreements under which we license rights to products or technology from others, we could lose license rights that are material to our business or be subject to claims by our licensors.

We license rights to products and technology that are important to our business, and we expect to enter into additional licenses in the future. For instance, we have exclusively licensed patents and patent applications under our Channel Agreement with Intrexon. Under these agreements, we are subject to a range of commercialization and development, sublicensing, royalty, patent prosecution and maintenance, insurance and other obligations.

Any failure by us to comply with any of these obligations or any other breach by us of our license agreements could give the licensor the right to terminate the license in whole, terminate the exclusive nature of the license or bring a claim against us for damages. Any such termination or claim could have a material adverse effect on our financial condition, results of operations, liquidity or business. Even if we contest any such termination or claim and are ultimately successful, such dispute could lead to delays in the development or commercialization of potential products and result in time-consuming and expensive litigation or arbitration. On termination we may be required to license to the licensor any related intellectual property that we developed.

In addition, in certain cases, the rights licensed to us are rights of a third party licensed to our licensor. In such instances, if our licensors do not comply with their obligations under such licenses, our rights under our license agreements with our licensor may be adversely affected.

# We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these employees or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.



#### OTHER RISKS RELATED TO OUR COMPANY

#### Our stock price has been, and may continue to be, volatile.

The market price of our common stock has been highly volatile. The stock market from time to time experiences significant price and volume fluctuations unrelated to the operating performance of particular companies. In addition, factors such as fluctuations in our operating results, future sales of our common stock, announcements of the timing and amount of product sales, announcements of the status of development of our products, announcements of technological innovations or new therapeutic products by us or our competitors, announcements regarding collaborative agreements, laboratory or clinical trial results, government regulation, FDA determinations on the approval of a product candidate NDA submission, developments in patent or other proprietary rights, public concern as to the safety of drugs developed by us or others, changes in reimbursement policies, comments made by securities analysts and general market conditions may have a substantial effect on the market price of our common stock.

#### We are subject to Sarbanes-Oxley and the reporting requirements of federal securities laws, which can be expensive.

As a public reporting company, we are subject to the Sarbanes-Oxley Act of 2002, as well as to the information and reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, and other federal securities laws. As a result, we incur significant legal, accounting, and other expenses that we would not incur as a private company, including costs associated with our public company requirements and corporate governance requirements. As an example of public reporting company requirements, we evaluate the effectiveness of disclosure controls and procedures and of our internal control over financing reporting in order to allow management to report on such controls. Sarbanes-Oxley generally requires that a public reporting company's independent registered public accounting firm attest to the effectiveness of the company's internal control over financial reporting as of the end of each fiscal year in the company's Annual Report on Form 10-K. In addition, any updates to our finance and accounting systems, procedures and controls, which may be required as a result of our ongoing analysis of internal controls, or results of testing by our independent auditor, may require significant time and expense. As a company with limited accounting resources, a significant amount of management's time and attention has been and will continue to be diverted from our business to ensure compliance with these regulatory requirements. This diversion of management's time and attention may have a material adverse effect on our business, financial condition and results of operations.

Management is working to continuously monitor and improve internal controls and has set in place controls to mitigate the potential segregation of duties risk. In the event significant deficiencies or material weaknesses are identified in our internal control over financial reporting that we cannot remediate in a timely manner, or if we are unable to receive a positive attestation from our independent registered public accounting firm with respect to our internal controls over financial reporting, investors and others may lose confidence in the reliability of our financial statements and the trading price of our common stock and ability to obtain any necessary equity or debt financing could suffer. In addition, in the event that our independent registered public accounting firm is unable to rely on our internal controls over financial reporting in connection with its audit of our financial statements, and in the further event that it is unable to devise alternative procedures in order to satisfy itself as to the material accuracy of our financial statements and related disclosures, we may be unable to file our periodic reports with the United States Securities and Exchange Commission, or SEC. This would likely have an adverse effect on the trading price of our common stock and our ability to secure any necessary additional equity or debt financing, and could result in the delisting of our common stock from the NASDAQ Capital Market, which would severely limit the liquidity of our common stock.

# Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

Provisions of our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us, even if doing so would benefit our stockholders. These provisions authorize the issuance of "blank check" preferred stock that could be issued by our board of directors to increase the number of outstanding shares and hinder a takeover attempt, and limit who may call a special meeting of stockholders. In addition, Section 203 of the Delaware General Corporation Law generally prohibits a publicly-held Delaware corporation from engaging in a business combination with a party that owns at least 15% of its common stock unless the business combination is approved by the company's board of directors before the person acquires the 15% ownership stake or later by its board of directors and two-thirds of its stockholders. In connection with our January 2011 issuance of shares of common stock to Intrexon in a private placement transaction, our board of directors waived the Section 203 prohibition with respect to a future business combination with Intrexon. Section 203 and this standstill provision could have the effect of delaying, deferring or preventing a change in control that our stockholders might consider to be in their best interests.

# Because we do not expect to pay dividends, you will not realize any income from an investment in our common stock unless and until you sell your shares at profit.

We have never paid dividends on our capital stock and we do not anticipate that we will pay any dividends for the foreseeable future. Accordingly, any return on an investment in us will be realized, if at all, only when you sell shares of our common stock.

## If securities and/or industry analysts fail to continue publishing research about our business, if they change their recommendations adversely or if our results of operations do not meet their expectations, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline. In addition, it is likely that in some future period our operating results will be below the expectations of securities analysts or investors. If one or more of the analysts who cover us downgrade our stock, or if our results of operations do not meet their expectations, our stock price could decline.

# Our stock price is volatile and may decline regardless of our operating performance, and you may not be able to resell your shares at or above the price at which you purchased such shares.

The market price for our common stock is volatile and may fluctuate significantly in response to a number of factors, most of which we cannot control, including:

- Price and volume fluctuations in the overall stock market;
- Market conditions or trends in our industry or the economy as a whole;
- Changes in operating performance and stock market valuations of other biopharmaceutical companies generally, or those that develop and commercialize cancer drugs in particular;
- The financial projections we may provide to the public, any changes in these projections or our failure to meet these projections;
- Changes in financial estimates or ratings by any securities analysts who follow our common stock, our failure to meet these estimates or failure of those analysts to initiate or maintain coverage of our common stock;
- The public's response to press releases or other public announcements by us or third parties, including our filings with the SEC and announcements relating to product development, litigation and intellectual property impacting us or our business;
- The sustainability of an active trading market for our common stock;
- Future sales of our common stock by our executive officers, directors and significant stockholders;
- Announcements of mergers or acquisition transactions;
- Our inclusion or deletion from certain stock indices;
- · Announcements of medical innovations or new products by our competitors;
- Announcements of changes in our senior management;
- Other events or factors, including those resulting from war, incidents of terrorism, natural disasters or responses to these events; and
- Changes in accounting principles.

In addition, the stock markets, and in particular the NASDAQ Capital Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many biopharmaceutical companies. Stock prices of many biopharmaceutical companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those companies. In the past, stockholders have instituted securities class action litigation following periods of market volatility. If we were involved in securities litigation, we could incur substantial costs and our resources and the attention of management could be diverted from our business.

# \*Our principal stockholders, executive officers and directors have substantial control over the company, which may prevent you and other stockholders from influencing significant corporate decisions and may harm the market price of our common stock.

As of June 30, 2014, our executive officers, directors and holders of five percent or more of our outstanding common stock, beneficially owned, in the aggregate, 28.8% of our outstanding common stock. These stockholders may have interests that conflict with our other stockholders and, if acting together, have the ability to influence the outcome of matters submitted to our stockholders for approval, including the election and removal of directors and any merger, consolidation or sale of all or substantially all of our assets. Accordingly, this concentration of ownership may harm the market price of our common stock by:

- Delaying, deferring or preventing a change in control;
- Impeding a merger, consolidation, takeover or other business combination involving us; or
- Discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us

#### Item 2. Unregistered Sale of Equity Securities and Use of Proceeds

#### **Issuer Purchases of Equity Securities**

During the six months ended June 30, 2014, we purchased 30,631 shares of common stock in settlement of employee tax withholding obligations due upon the vesting of restricted stock. The following table provides information about these purchases of restricted shares for the six months ended June 30, 2014:

| Period                 | Total Number of<br>Shares Purchased | Price Paid<br>hare (\$) |
|------------------------|-------------------------------------|-------------------------|
| January 1 to 31, 2014  | 16,031                              | \$<br>4.37              |
| February 1 to 28, 2014 | 14,600                              | \$<br>4.40              |
| March 1 to 31, 2014    | —                                   | \$<br>                  |
| April 1 to 30, 2014    | —                                   | \$<br>—                 |
| May 1 to 31, 2014      | —                                   | \$<br>                  |
| June 1 to 30, 2014     | —                                   | \$<br>—                 |
| Total                  | 30,631                              |                         |

#### Item 3. Defaults upon Senior Securities

Not applicable.

#### Item 4. Mine Safety Disclosures

Not applicable.

#### Item 5. Other Information

Not applicable.

#### Item 6. Exhibits

The exhibits listed in the Exhibit Index immediately preceding such exhibits are filed as part of this report and such Exhibit Index is incorporated herein by reference.



#### **SIGNATURES**

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

/s/ Jonathan Lewis

Jonathan Lewis, M.D., Ph.D. Chief Executive Officer (Principal Executive Officer) Dated: August 7, 2014

/s/ Kevin G. Lafond

Kevin G. Lafond Vice President, Chief Accounting Officer and Treasurer (Principal Financial and Accounting Officer) Dated: August 7, 2014



#### EXHIBIT INDEX

| 10.1*    | ZIOPHARM Oncology, Inc. 2012 Equity Incentive Plan, as amended             |
|----------|--|
| 31.1*    | Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 $$ |
| 31.2*    | Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002    |
| 32.1*    | Certifications pursuant to 18 U.S.C. Section 1350                          |
| 101.INS* | XBRL Instance Document   |
| 101.SCH* | XBRL Taxonomy Extension Schema Document                                    |
| 101.CAL* | XBRL Taxonomy Extension Calculation Linkbase Document                      |
| 101.DEF* | XBRL Taxonomy Definition Linkbase Document                                 |
| 101.LAB* | XBRL Taxonomy Extension Label Linkbase Document                            |
| 101.PRE* | XBRL Taxonomy Extension Presentation Linkbase Document                     |
|          |  |

\* Filed herewith.

#### ZIOPHARM ONCOLOGY, INC.

#### **2012 EQUITY INCENTIVE PLAN**

#### Adopted by the Board of Directors: March 7, 2012 Approved by the Stockholders: June 20, 2012 Amended by the Board of Directors: January 28, 2014 Amended by the Stockholders: June 18, 2014

#### 1. GENERAL.

(a) Status of Prior Plan. The Plan is intended as a new equity incentive plan that is separate from the ZIOPHARM Oncology, Inc. Amended and Restated 2003 Stock Option Plan (the "*Prior Plan*"). Following the Effective Date, no additional stock awards may be granted under the Prior Plan. Any shares of Common Stock that are set aside under the Prior Plan's share reserve but which are not subject to any outstanding stock awards under the Prior Plan as of 12:01 a.m. Eastern Standard Time on the Effective Date (the "*Prior Plan's Available Reserve*") will cease to be available for use under the Prior Plan at such time and will be added to this Plan's Share Reserve (as further described in Section 3(a)) and be then immediately available for issuance pursuant to Stock Awards. In addition, from and after 12:01 a.m. Eastern Standard Time on the Effective Date on or after 12:01 a.m. Eastern Standard Time on the Effective Date will remain subject to the terms of the Prior Plan. All Awards granted on or after 12:01 a.m. Eastern Standard Time on the Effective Date of this Plan will be subject to the terms of this Plan.

(b) Eligible Award Recipients. Employees, Directors and Consultants are eligible to receive Awards.

(c) Available Awards. The Plan provides for the grant of the following types of Awards: (i) Incentive Stock Options; (ii) Nonstatutory Stock Options; (iii) Stock Appreciation Rights; (iv) Restricted Stock Awards; (v) Restricted Stock Unit Awards; (vi) Performance Stock Awards, (vii) Performance Cash Awards; and (viii) Other Stock Awards.

(d) **Purpose.** The Plan, through the granting of Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock.

#### 2. ADMINISTRATION.

(a) Administration by Board. The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine: (A) who will be granted Awards; (B) when and how each Award will be granted; (C) what type of Award will be granted; (D) the provisions of each Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Award; (E) the number of shares of Common Stock subject to, or the cash value of, an Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Award Agreement or in the written terms of a Performance Cash Award, in a manner and to the extent it will deem necessary or expedient to make the Plan or Award fully effective.

(iii) To settle all controversies regarding the Plan and Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which an Award may be exercised or vest (or at which cash or shares of Common Stock may be issued).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or an Award Agreement, suspension or termination of the Plan will not materially impair a Participant's rights under his or her then-outstanding Award without his or her written consent except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to Incentive Stock Options and certain nonqualified deferred compensation under Section 409A and/or to make the Plan or Awards granted under the Plan exempt from or compliant therewith, subject to the limitations, if any, of applicable law. However, if required by applicable law or listing requirements, and except as provided in Section 9(a) relating to Capitalization Adjustments, the Company will seek stockholder approval of any amendment of the Plan that (A) materially increases the number of shares of Common Stock available for issuance under the Plan, (B) materially expands the class of individuals eligible to receive Awards under the Plan, (C) materially increases the benefits accruing to Participants under the Plan, (D) materially reduces the price at which shares of Common Stock may be issued or purchased under the Plan, (E) materially extends the term of the Plan, or (F) materially expands the types of Awards available for issuance under the Plan. Except as provided in the Plan (including subsection (viii) below) or an Award Agreement, no amendment of the Plan will materially impair a Participant's rights under an outstanding Award without his or her written consent.

(vii) To submit any amendment to the Plan for stockholder approval, including, but not limited to, amendments to the Plan intended to satisfy the requirements of (A) Section 162(m) of the Code regarding the exclusion of performance-based compensation from the limit on corporate deductibility of compensation paid to Covered Employees, (B) Section 422 of the Code regarding incentive stock options, or (C) Rule 16b-3.

(viii) To approve forms of Award Agreements for use under the Plan and to amend the terms of any one or more Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion; *provided, however*, that a Participant's rights under any Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Awards without the affected Participant's consent: (A) to maintain the qualified status of the Award as an Incentive Stock Option under Section 422 of the Code; (B) to change the terms of an Incentive Stock Option under Section 422 of the Code; (C) to clarify the manner of exemption from, or to bring the Award into compliance with, Section 409A; or (D) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Employees, Directors or Consultants who are foreign nationals or employed outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

#### (c) Delegation to Committee.

(i) General. The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Committee may, at any time, abolish the subcommittee and/or revest in the Committee any powers delegated to the subcommittee. The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) Section 162(m) and Rule 16b-3 Compliance. The Committee may consist solely of two (2) or more Outside Directors, in accordance with Section 162(m) of the Code, or solely of two (2) or more Non-Employee Directors, in accordance with Rule 16b-3.

(d) Delegation to an Officer. The Board may delegate to one (1) or more Officers the authority to do one or both of the following (i) designate Employees who are not Officers to be recipients of Options and SARs (and, to the extent permitted by applicable law, other Stock Awards) and, to the extent permitted by applicable law, the terms of such rights and options, and (ii) determine the number of shares of Common Stock to be subject to such Stock Awards granted to such Employees; *provided, however*, that the Board resolutions regarding such delegation will specify the total number of shares of Common Stock that may be subject to the Stock Awards granted by such Officer and that such Officer may not grant a Stock Award to himself or herself. Any such Stock Awards will be granted on the form of Award Agreement most recently approved for use by the Committee or the Board, unless otherwise provided in the resolutions approving the delegation authority. The Board may not delegate authority to an Officer who is acting solely in the capacity of an Officer (and not also as a Director) to determine the Fair Market Value pursuant to Section 13(y)(iii) below.

(e) Effect of Board's Decision. All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

(f) Cancellation and Re-Grant of Stock Awards. Neither the Board nor any Committee will have the authority to: (i) reduce the exercise, purchase or strike price of any outstanding Options or SAR under the Plan; or (ii) cancel any outstanding Options or SARs that have an exercise price or strike price greater than the current Fair Market Value of the Common Stock in exchange for cash or other Stock Awards under the Plan, unless the stockholders of the Company have approved such an action within twelve (12) months prior to such an event.

#### 3. SHARES SUBJECT TO THE PLAN.

#### (a) Share Reserve.

(i) Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards from and after the Effective Date will not exceed (A) 9,000,000 shares (which number is the sum of (1) the number of shares (seventy-nine thousand eight hundred thirty-six (79,836)) subject to the Prior Plans' Available Reserve and (2) an additional eight million nine hundred twenty thousand one hundred sixty-four (8,920,164) new shares) (the "*Share Reserve*").

(ii) For clarity, the Share Reserve in this Section 3(a) is a limitation on the number of shares of Common Stock that may be issued pursuant to the Plan. Accordingly, this Section 3(a) does not limit the granting of Stock Awards except as provided in Section 7(a). Shares may be issued in connection with a merger or acquisition as permitted by NASDAQ Listing Rule 5635(c) or, if applicable, NYSE Listed Company Manual Section 303A.08, AMEX Company Guide Section 711 or other applicable rule, and such issuance will not reduce the number of shares available for issuance under the Plan.



(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion thereof (i) expires or otherwise terminates without all of the shares covered by such Stock Award having been issued or (ii) is settled in cash (*i.e.*, the Participant receives cash rather than stock), such expiration, termination or settlement will not reduce (or otherwise offset) the number of shares of Common Stock that may be available for issuance under the Plan. If any shares of Common Stock issued pursuant to a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will again become available for issuance under the Plan.

(c) Incentive Stock Option Limit. Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate maximum number of shares of Common Stock that may be issued pursuant to the exercise of Incentive Stock Options will be 9,000,000 shares of Common Stock.

(d) Section 162(m) Limitations. Subject to Section 9(a) relating to Capitalization Adjustments, at such time as the Company may be subject to the applicable provisions of Section 162(m) of the Code, the following limitations shall apply.

(i) A maximum of 2,000,000 shares of Common Stock subject to Options, SARs and Other Stock Awards whose value is determined by reference to an increase over an exercise or strike price of at least one hundred percent (100%) of the Fair Market Value on the date any such Stock Award is granted may be granted to any Participant during any calendar year. Notwithstanding the foregoing, if any additional Options, SARs or Other Stock Awards whose value is determined by reference to an increase over an exercise or strike price of at least one hundred percent (100%) of the Fair Market Value on the date the Stock Award are granted to any Participant during any calendar year, compensation attributable to the exercise of such additional Stock Awards will not satisfy the requirements to be considered "qualified performance-based compensation" under Section 162(m) of the Code unless such additional Stock Award is approved by the Company's stockholders.

(ii) A maximum of 2,000,000 shares of Common Stock subject to Performance Stock Awards may be granted to any one Participant during any one calendar year (whether the grant, vesting or exercise is contingent upon the attainment during the Performance Period of the Performance Goals).

(iii) A maximum of \$2,000,000 may be granted as a Performance Cash Award to any one Participant during any one calendar year.

If a Performance Stock Award is in the form of an Option, it will count only against the Performance Stock Award limit. If a Performance Stock Award could be paid out in cash, it will count only against the Performance Stock Award limit.

(e) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

#### 4. ELIGIBILITY.

(a) Eligibility for Specific Stock Awards. Incentive Stock Options may be granted only to employees of the Company or a "parent corporation" or "subsidiary corporation" thereof (as such terms are defined in Sections 424(e) and 424(f) of the Code). Stock Awards other than Incentive Stock Options may be granted to Employees, Directors and Consultants;*provided, however*, that Stock Awards may not be granted to Employees, Directors and Consultants;*provided, however*, that Stock Awards may not be granted to Employees, Directors and Consultants;*provided, however*, that Stock Awards may not be granted to Employees, Directors and Consultants; who are providing Continuous Service only to any "parent" of the Company, as such term is defined in Rule 405, unless (i) the stock underlying such Stock Awards is treated as "service recipient stock"

under Section 409A (for example, because the Stock Awards are granted pursuant to a corporate transaction such as a spin off transaction) or (ii) the Company, in consultation with its legal counsel, has determined that such Stock Awards are otherwise exempt from or alternatively comply with the distribution requirements of Section 409A.

(b) Ten Percent Stockholders. A Ten Percent Stockholder will not be granted an Incentive Stock Option unless the exercise price of such Option is at least one hundred ten percent (110%) of the Fair Market Value on the date of grant and the Option is not exercisable after the expiration of five (5) years from the date of grant.

#### 5. PROVISIONS RELATING TO OPTIONS AND STOCK APPRECIATION RIGHTS.

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be separately designated Incentive Stock Options or Nonstatutory Stock Options at the time of grant, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each type of Option. If an Option is not specifically designated as an Incentive Stock Option but some portion or all of the Option fails to qualify as an Incentive Stock Option under the applicable rules, then the Option (or portion thereof) will be a Nonstatutory Stock Option. The provisions of separate Options or SARs need not be identical; *provided, however*, that each Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Award Agreement or otherwise) the substance of each of the following provisions:

(a) **Term.** Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, no Option or SAR will be exercisable after the expiration of ten (10) years from the date of its grant or such shorter period specified in the Award Agreement.

(b) Exercise Price. Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, the exercise or strike price of each Option or SAR will be not less than one hundred percent (100%) of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Award is granted. Notwithstanding the foregoing, an Option or SAR may be granted with an exercise or strike price lower than one hundred percent (100%) of the Fair Market Value of the Common Stock subject to the Award is granted with an exercise or strike price lower than one hundred percent (100%) of the Fair Market Value of the Common Stock subject to the Award if such Award is granted pursuant to an assumption of or substitution for another option or stock appreciation right pursuant to a Corporate Transaction and in a manner consistent with the provisions of Section 409A and, if applicable, Section 424(a) of the Code. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft or money order payable to the Company;

(ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;

(iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;

(iv) if an Option is a Nonstatutory Stock Option, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; *provided, however*, that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of

the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or

(v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Award Agreement.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Award Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of the two or in any other form of consideration, as determined by the Board and contained in the Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) Restrictions on Transfer. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided herein, neither an Option nor a SAR may be transferred for consideration.

(ii) Domestic Relations Orders. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulation 1.421-1(b)(2). If an Option is an Incentive Stock Option, such Option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(iii) Beneficiary Designation. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, the executor or administrator of the Participant's estate will be entitled to exercise the Option or SAR and receive the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and therefore become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of Performance Goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

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(g) Termination of Continuous Service. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date three (3) months following the termination of the Participant's Continuous Service (or such longer or shorter period specified in the applicable Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if the exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of three (3) months (that need not be consecutive) after the termination of the Participant's Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement. In addition, unless otherwise provided in a Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Company's insider trading policy, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date twelve (12) months following such termination of Continuous Service (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Award Agreement for exercisability after the termination of the Participant's Continuous Service (for a reason other than death), then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant's death, but only within the period ending on the earlier of (i) the date eighteen (18) months following the date of death (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of such Option or SAR as set forth in the Award Agreement. If, after the Participant's death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant's Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant's termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR from and after the time of such termination of Continuous Service.

(I) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR will not be first exercisable for any shares of Common Stock until at least six (6) months following the date of grant of the Option or SAR (although the Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued, or substituted, (iii) upon a Change in Control, or (iv) upon the Participant's retirement (as such term may be defined in the Participant's Award Agreement, in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six (6) months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise of any shares under any other Stock Award will be exempt from the employee's regular rate of pay, the provisions of this Section 5(1) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

#### 6. PROVISIONS OF STOCK AWARDS OTHER THAN OPTIONS AND SARS.

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board deems appropriate. To the extent consistent with the Company's bylaws, at the Board's election, shares of Common Stock may be (i) held in book entry form subject to the Company's instructions until any restrictions relating to the Restricted Stock Award lapse, or (ii) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past services to the Company or an Affiliate, or (C) any other form of legal consideration (including future services) that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. Shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) Termination of Participant's Continuous Service. If a Participant's Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right any or all of the shares of Common Stock held by the Participant that have not vested as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) Transferability. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(v) Dividends. A Restricted Stock Award Agreement may provide that any dividends paid on Restricted Stock will be subject to the same vesting and forfeiture restrictions as apply to the shares subject to the Restricted Stock Award to which they relate.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board deems appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) **Payment**. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) Additional Restrictions. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) Dividend Equivalents. Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(vi) Termination of Participant's Continuous Service. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service.

#### (c) Performance Awards.

(i) Performance Stock Awards. A Performance Stock Award is a Stock Award (covering a number of shares not in excess of that set forth in Section 3(d)(ii) above) that is payable (including that may be granted, vest or exercised) contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Stock Award may, but need not, require the completion of a specified period of Continuous Service. The length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee (or, if not required for compliance with Section 162(m) of the Code, the Board), in its sole discretion. In addition, to the extent permitted by applicable law and the applicable Award Agreement, the Board may determine that cash may be used in payment of Performance Stock Awards.

(ii) **Performance Cash Awards**. A Performance Cash Award is a cash award (for a dollar value not in excess of that set forth in Section 3(d)(iii) above) that is payable contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Cash Award may also require the completion of a specified period of Continuous Service. At the time of grant of a Performance Cash Award, the length of any

Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee (or, if not required for compliance with Section 162(m) of the Code, the Board), in its sole discretion. The Board may specify the form of payment of Performance Cash Awards, which may be cash or other property, or may provide for a Participant to have the option for his or her Performance Cash Award, or such portion thereof as the Board may specify, to be paid in whole or in part in cash or other property.

(iii) Board Discretion. The Board retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for a Performance Period.

(iv) Section 162(m) Compliance. Unless otherwise permitted in compliance with the requirements of Section 162(m) of the Code with respect to an Award intended to qualify as "performance-based compensation" thereunder, the Committee will establish the Performance Goals applicable to, and the formula for calculating the amount payable under, the Award no later than the earlier of (A) the date ninety (90) days after the commencement of the applicable Performance Period, and (B) the date on which twenty-five percent (25%) of the Performance Period has elapsed, and in any event at a time when the achievement of the applicable Performance Goals remains substantially uncertain. Prior to the payment of any compensation under an Award intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Committee will certify the extent to which any Performance Goals and any other material terms under such Award have been satisfied (other than in cases where such relate solely to the increase in the value of the Common Stock). Notwithstanding satisfaction of any completion of any Performance Goals may be reduced by the Committee on the basis of such further considerations as the Committee, in its sole discretion, will determine.

(d) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (*e.g.*, options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

#### 7. COVENANTS OF THE COMPANY.

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy thenoutstanding Stock Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; *provided, however*, that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of an Award or the subsequent issuance of cash or Common Stock pursuant to the Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of an Award or a possible period in which the Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of an Award to the holder of such Award.

# 8. MISCELLANEOUS.

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Stock Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Awards. Corporate action constituting a grant by the Company of an Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Award Agreement as a result of a clerical error in the papering of the Award Agreement, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Award Agreement.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to an Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares of Common Stock under, the Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Award Agreement or any other instrument executed thereunder or in connection with any Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, including, but not limited to, Cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee) after the date of grant of any Award to the Participant, the Board has the right in its sole discretion to (i) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Award that is scheduled to vest or become payable after the date of such change in time commitment, and (ii) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Award. In the event of any such reduction, the Participant will have no right with respect to any portion of the Award that is so reduced or extended.

(f) Incentive Stock Option Limitations. To the extent that the aggregate Fair Market Value (determined at the time of grant) of Common Stock with respect to which Incentive Stock Options are exercisable for the first time by any Participant during any calendar year (under all plans of the Company and any Affiliates) exceeds one hundred thousand dollars (\$100,000) (or such other limit established in the Code) or otherwise does not comply with the rules governing Incentive Stock Options, the Options or portions thereof that exceed such limit (according to the order in which they were granted) or otherwise do not comply with such rules will be treated as Nonstatutory Stock Options, notwithstanding any contrary provision of the applicable Option Agreement(s).

(g) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and

business matters and that he or she is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Award for the Participant's own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Stock Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(h) Withholding Obligations. Unless prohibited by the terms of an Award Agreement, the Company may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to an Award by any of the following means (in addition to the Company's right to withhold from any compensation paid to the Participant by the Company) or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Stock Award; *provided, however*, that no shares of Common Stock are withheld with a value exceeding the minimum amount of tax required to be withheld by law (or such lesser amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from an Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Award Agreement.

(i) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at www.sec.gov (or any successor website thereto) or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(j) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A. Consistent with Section 409A, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(k) Compliance with Section 409A. Unless otherwise expressly provided for in an Award Agreement, the Plan and Award Agreements will be interpreted to the greatest extent possible in a manner that makes the Plan and the Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Award Agreement evidencing such Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code and to the extent an Award Agreement is silent on terms necessary for compliance, such terms are hereby incorporated by reference into the Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding an Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six (6) months following the date of such Participant's "separation from service" or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six (6) month period elapses, with the balance paid thereafter on the original schedule.

(I) Clawback/Recovery. All Awards granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company's securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Board may impose such other clawback, recovery or recoupment provisions in an Award Agreement as the Board determines necessary or appropriate, including but not limited to a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company.

### 9. ADJUSTMENTS UPON CHANGES IN COMMON STOCK; OTHER CORPORATE EVENTS.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a); (ii) the class(es) and maximum number of securities that may be issued pursuant to the exercise of Incentive Stock Options pursuant to Section 3(c); (iii) the class(es) and maximum number of securities that may be awarded to any person pursuant to Sections 3(d); and (iv) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution or Liquidation. Except as otherwise provided in the Stock Award Agreement, in the event of a dissolution or liquidation of the Company, all outstanding Stock Awards (other than Stock Awards consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company's right of repurchase) will terminate immediately prior to the completion of such dissolution or liquidation, and the shares of Common Stock subject to the Company's repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service; *provided, however*, that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the dissolution or liquidation is completed but contingent on its completion.

(c) Corporate Transaction. The following provisions will apply to Stock Awards in the event of a Corporate Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Corporate Transaction, then, notwithstanding any other provision of the Plan, the Board may take one or more of the following actions with respect to Stock Awards, contingent upon the closing or consummation of the Corporate Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the stockholders of the Company pursuant to the Corporate Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Corporate Transaction as the Board determines (or, if the Board does not determine such a date, to the date that is five (5) days prior to the effective date of the Corporate Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Corporate Transaction; *provided, however*, that the Board may require Participants to complete and deliver to the Company a notice of exercise before the effective date of a Corporate Transaction, which exercise is contingent upon the effectiveness of such Corporate Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Corporate Transaction, in exchange for such cash consideration, if any, as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Corporate Transaction, over (B) any exercise price payable by such holder in connection with such exercise. For clarity, this payment may be zero (\$0) if the value of the property is equal to or less than the exercise price. Payments under this provision may be delayed to the same extent that payment of consideration to the holders of the Company's Common Stock in connection with the Corporate Transaction is delayed as a result of escrows, earn outs, holdbacks or any other contingencies.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award prior to the earlier of (i) the effective time of the Corporate Transaction and (ii) the effectiveness of such action(s) with respect to the Awards.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant, but in the absence of such provision, no such acceleration will occur.

#### **10.** PLAN TERM; EARLIER TERMINATION OR SUSPENSION OF THE PLAN.

(a) The Board may suspend or terminate the Plan at any time. No Incentive Stock Option will be granted after the tenth (10th) anniversary of the earlier of (i) the date the Plan is adopted by the Board, or (ii) the date the Plan is approved by the stockholders of the Company. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

(b) No Impairment of Rights. Suspension or termination of the Plan will not impair rights and obligations under any Award granted while the Plan is in effect except with the written consent of the affected Participant or as otherwise permitted in the Plan.

#### **11. EFFECTIVE DATE OF PLAN.**

This Plan will become effective on the Effective Date.

### **12. CHOICE OF LAW.**

The laws of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state's conflict of laws rules.

13. DEFINITIONS. As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "*Affiliate*" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

(b) "Award" means a Stock Award or a Performance Cash Award.

(c) "Award Agreement" means a written agreement between the Company and a Participant evidencing the terms and conditions of an Award.

(d) "Board" means the Board of Directors of the Company.

(e) "*Capitalization Adjustment*" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Effective Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or any similar equity restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(f) "*Cause*" will have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term means, with respect to a Participant, the occurrence of any of the following events: (i) the willful misconduct, failure, disregard or refusal by such Participant to perform any of the material duties of his or her material duties of employment; *provided, however*, that such Participant will have one opportunity to cure any breach within five (5) business days ("*Cure Period*") of written notice to the Participant; (ii) any willful, intentional or grossly negligent act by such Participant having the effect of injuring, in a material way the business or reputation of the Company or an Affiliate; (iii) such Participant's conviction of any felony or a misdemeanor involving moral turpitude (including entry of a *nolo contendere* plea); (iv) the determination by the Company, after a reasonable and good-faith investigation by the Company following a written allegation by another employee of the Company, that such Participant engaged in some form of harassment prohibited by law (including, without limitation, harassment that constitutes age, sex or race discrimination); any misappropriation or embezzlement of the property of the Company or an Affiliate; (vi) breach by such Participant of any agreement between the Participant and the Company or an Affiliate which is not cured by the Participant within thirty (30) days after notice thereof is given by the Company or an Affiliate.

(g) "Change in Control" means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company, (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company's securities, or (C) solely because the level of Ownership held by any Exchange Act Person (the "*Subject Person*") exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than fifty percent (50%) of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction;



(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than fifty percent (50%) of the combined voting power of the voting securities of which are Owned by stockholders of the Company in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition; or

(iv) individuals who, on the date the Plan is adopted by the Board, are members of the Board (the "*Incumbent Board*") cease for any reason to constitute at least a majority of the members of the Board; *provided, however*, that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member will, for purposes of this Plan, be considered as a member of the Incumbent Board.

Notwithstanding the foregoing definition or any other provision of this Plan, (A) the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company, and (B) the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Awards subject to such agreement; *provided, however*, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the foregoing definition will apply; *provided, further*, that no Change in Control shall be deemed to occur upon announcement or commencement of a tender offer or upon a potential takeover or upon stockholder approval of a merger or other transaction, in each case without a requirement that the Change in Control actually occur. If required for compliance with Section 409A of the Code, in no event will a Change in Control be deemed to have occurred if such transaction is not also a "change in the ownership or effective control of" the Company or "a change in the ownership of a substantial portion of the assets of" the Company as determined under Treasury Regulation Section 1.409A-3(i)(5) (without regard to any alternative definition thereunder). The Board may, in its sole discretion and without a Participant's consent, amend the definition of "Change in Control" to conform to the definition of "Change in Control" under Section 409A of the Code, and the regulations thereunder.

(h) "Code" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(i) "Committee" means a committee of one (1) or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

- (j) "Common Stock" means the common stock of the Company.
- (k) "Company" means ZIOPHARM Oncology, Inc., a Delaware corporation.

(l) "Consultant" means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company's securities to such person.

(m) "*Continuous Service*" means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the Entity for which the Participant renders such service, provided that there is no interruption or termination of the

Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service; *provided, however*, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board, in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. For example, a change in status from an Employee of the Company to a Consultant of an Affiliate or to a Director will not constitute an interruption of Continuous Service. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in a Stock Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law. In addition, to the extent required for exemption from or compliance with Section 409A of the Code, the determination of whether there has been a termination of Continuous Service will be made, and such term will be construed, in a manner that is consistent with the definition of "separation from service" as defined under Treasury Regulation Section 1.409A-1(h) (without regard to any alternative definition thereunder).

(n) "*Corporate Transaction*" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board, in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of at least ninety percent (90%) of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

To the extent required for compliance with Section 409A of the Code, in no event will an event be deemed a Corporate Transaction if such transaction is not also a "change in the ownership or effective control of" the Company or "a change in the ownership of a substantial portion of the assets of" the Company as determined under Treasury Regulation Section 1.409A-3(i)(5) (without regard to any alternative definition thereunder).

(o) "Covered Employee" will have the meaning provided in Section 162(m)(3) of the Code.

(p) "Director" means a member of the Board.

(q) "*Disability*" means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than twelve (12) months, as provided in Sections 22(e)(3) and 409A(a)(2)(C)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(r) "*Effective Date*" means the effective date of this Plan document, which is the date of the annual meeting of stockholders of the Company held in 2012 provided this Plan is approved by the Company's stockholders at such meeting.

(s) "*Employee*" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(t) "Entity" means a corporation, partnership, limited liability company or other entity.

(u) "Exchange Act" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(v) "Exchange Act Person" means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to an offering of such securities, (iv) an Entity Owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the Effective Date, is the Owner, directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the combined voting power of the Company's then outstanding securities.

(w) "Fair Market Value" means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the date of determination, as reported in a source the Board deems reliable.

(ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

(iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

(x) "*Incentive Stock Option*" means an option granted pursuant to Section 5 of the Plan that is intended to be, and that qualifies as, an "incentive stock option" within the meaning of Section 422 of the Code.

(y) "Non-Employee Director" means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which disclosure would not be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("*Regulation S-K*")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3.

(z) "Nonstatutory Stock Option" means any option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(aa) "Officer" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.

(bb) "Option" means an Incentive Stock Option or a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(cc) "*Option Agreement*" means a written agreement between the Company and an Participant evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(dd) "Other Stock Award" means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(d).

(ee) "Outside Director" means a Director who either (i) is not a current employee of the Company or an "affiliated corporation" (within the meaning of Treasury Regulations promulgated under Section 162(m) of the Code), is not a former employee of the Company or an "affiliated corporation" who receives compensation for prior services (other than benefits under a tax-qualified retirement plan) during the taxable year, has not been an officer of the Company or an "affiliated corporation," and does not receive remuneration from the Company or an "affiliated corporation," either directly or indirectly, in any capacity other than as a Director, or (ii) is otherwise considered an "outside director" for purposes of Section 162(m) of the Code.

(ff) "Own," "Owned," "Owner," "Ownership" means a person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(gg) "Participant" means a person to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(hh) "Performance Cash Award" means an award of cash granted pursuant to the terms and conditions of Section 6(c)(ii).

(ii) "Performance Criteria" means the one or more criteria that the Board will select for purposes of establishing the Performance Goals for a Performance Period. The Performance Criteria that will be used to establish such Performance Goals may be based on any one of, or combination of, the following as determined by the Board: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) total stockholder return; (v) return on equity or average stockholder's equity; (vi) return on assets, investment, or capital employed; (vii) stock price; (viii) margin (including gross margin); (ix) income (before or after taxes); (x) operating income; (xi) operating income after taxes; (xii) pre-tax profit; (xiii) operating cash flow; (xiv) sales or revenue targets; (xv) increases in revenue or product revenue; (xvi) expenses and cost reduction goals; (xvii) improvement in or attainment of working capital levels; (xiii) economic value added (or an equivalent metric); (xix) market share; (xx) cash flow; (xxi) cash flow per share; (xxii) share price performance; (xxiii) debt reduction; (xxiv) implementation or completion of projects or processes; (xxv) customer satisfaction; (xxvi) stockholders' equity; (xxvii) capital expenditures; (xxiii) debt levels; (xxii) operating profit or net operating profit; (xxx) workforce diversity; (xxxi) growth of net income or operating income; (xxxi) billings; (xxxiii) achievement of drug development milestones; (xxxiv) regulatory achievements, including approval of a compound; (xxxv) progress of internal research or clinical programs; (xxxvi) progress of partner programs; (xxxvii) implementation or completion of projects and processes; (xxviii) clinical progress, (xxxix) in-licensing; and (xl) to the extent that an Award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by the Board.

(jj) "*Performance Goals*" means, for a Performance Period, the one or more goals established by the Board for the Performance Period based upon the Performance Criteria. Performance Goals may be based on a Company-wide basis, with respect to one or more business units, divisions, Affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise by the Board (i) in the Award Agreement at the time the Award is granted or (ii) in such other document setting forth the Performance Goals at the time the Performance

Goals are established, the Board will appropriately make adjustments in the method of calculating the attainment of Performance Goals for a Performance Period as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects, as applicable, for non-U.S. dollar denominated Performance Goals; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; and (5) to exclude the effects of any "extraordinary items" as determined under generally accepted accounting principles.

(kk) "*Performance Period*" means the period of time selected by the Board over which the attainment of one or more Performance Goals will be measured for the purpose of determining a Participant's right to and the payment of a Stock Award or a Performance Cash Award. Performance Periods may be of varying and overlapping duration, at the sole discretion of the Board.

(II) "Performance Stock Award" means a Stock Award granted under the terms and conditions of Section 6(c)(i).

(mm) "Plan" means this ZIOPHARM Oncology, Inc. 2012 Equity Incentive Plan.

(nn) "Restricted Stock Award" means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(oo) "*Restricted Stock Award Agreement*" means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(pp) "*Restricted Stock Unit Award*" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(qq) "Restricted Stock Unit Award Agreement" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(rr) "Rule 16b-3" means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(ss) "Rule 405" means Rule 405 promulgated under the Securities Act.

(tt) "Section 409A" means Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect.

(uu) "Securities Act" means the Securities Act of 1933, as amended.

(vv) "*Stock Appreciation Right*" or "*SAR*" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(ww) "*Stock Award*" means any right to receive Common Stock granted under the Plan, including an Incentive Stock Option, a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right, a Performance Stock Award or any Other Stock Award.

(xx) "Stock Award Agreement" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(yy) "*Subsidiary*" means, with respect to the Company, (i) any corporation of which more than fifty percent (50%) of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than fifty percent (50%).

(zz) "Ten Percent Stockholder" means a person who Owns (or is deemed to Own pursuant to Section 424(d) of the Code) stock possessing more than ten percent (10%) of the total combined voting power of all classes of stock of the Company or any Affiliate.

## CERTIFICATION

I, Jonathan Lewis, certify that:

1. I have reviewed this quarterly report on Form 10-Q of ZIOPHARM Oncology, Inc.;

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

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

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

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

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 7, 2014

/s/ Jonathan Lewis Jonathan Lewis, M.D., Ph.D. Chief Executive Officer (Principal Executive Officer)

## CERTIFICATION

I, Kevin G. Lafond, certify that:

1. I have reviewed this quarterly report on Form 10-Q of ZIOPHARM Oncology, Inc.;

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

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

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

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

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: August 7, 2014

/s/ Kevin G. Lafond Kevin G. Lafond Vice President, Chief Accounting Officer and Treasurer (Principal Financial and Accounting Officer)

# CERTIFICATION

In connection with the Quarterly Report on Form 10-Q of ZIOPHARM Oncology, Inc. (the "Company") for the period ended June 30, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), we Jonathan Lewis, the Principal Executive Officer of the Company and Kevin G. Lafond, the Principal Financial and Accounting Officer of the Company, hereby certify, pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to our knowledge:

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

Dated: August 7, 2014

/s/ Jonathan Lewis Jonathan Lewis, M.D., Ph.D. Chief Executive Officer (Principal Executive Officer)

Dated: August 7, 2014

/s/ Kevin G. Lafond

Kevin G. Lafond Vice President, Chief Accounting Officer and Treasurer (Principal Financial and Accounting Officer)