

GLYCOMIMETICS INC  
Form 10-Q  
July 31, 2014  
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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
**Washington, D.C. 20549**

**FORM 10-Q**

(Mark one)

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

**For the quarterly period ended June 30, 2014**

**OR**

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

**For the transition period from \_\_\_\_\_ to \_\_\_\_\_**

**Commission File Number 001-36177**

**GlycoMimetics, Inc.**

**(Exact name of registrant as specified in its charter)**

**Delaware**  
**(State or Other Jurisdiction of**  
**Incorporation or Organization)**

**06-1686563**  
**(I.R.S. Employer**  
**Identification No.)**

**401 Professional Drive, Suite 250**

**Gaithersburg, Maryland**  
**(Address of principal executive offices)**

**20879**  
**(Zip Code)**

**(240) 243-1201**

**(Registrant's telephone number, including area code)**

**N/A**

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of large accelerated filer, accelerated filer and smaller reporting company in Rule 12b-2 of the Securities Exchange Act of 1934.

Large accelerated filer

Accelerated filer

Non-accelerated filer  (Do not check if a smaller reporting company)

Smaller reporting company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Securities Exchange Act of 1934). Yes  No

The number of outstanding shares of the registrant's common stock, par value \$0.001 per share, as of the close of business on July 29, 2014 was 18,893,464.



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**GLYCOMIMETICS, INC.**

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**Table of Contents****Part I. FINANCIAL INFORMATION****Item 1. Financial Statements****GLYCOMIMETICS, INC.****Balance Sheets**

|  | <b>June 30,<br/>2014<br/>(unaudited)</b> | <b>December 31,<br/>2013</b> |
|--|--|------------------------------|
| <b>Assets</b>  |  |                              |
| Current assets:  |  |                              |
| Cash and cash equivalents  | \$ 66,217,242                            | \$ 2,310,603                 |
| Prepaid expenses and other current assets  | 881,714                                  | 2,573,072                    |
| <b>Total current assets</b>  | <b>67,098,956</b>                        | <b>4,883,675</b>             |
| Property and equipment, net  | 420,640                                  | 399,799                      |
| <b>Total assets</b>  | <b>\$ 67,519,596</b>                     | <b>\$ 5,283,474</b>          |
| <b>Liabilities &amp; stockholders equity</b>   |  |                              |
| Current liabilities:   |  |                              |
| Accounts payable   | \$ 472,161                               | \$ 1,144,895                 |
| Accrued bonuses  | 372,756                                  | 344,467                      |
| Accrued expenses   | 2,640,591                                | 685,746                      |
| Current portion of deferred rent   | 111,397                                  | 104,191                      |
| <b>Total current liabilities</b>   | <b>3,596,905</b>                         | <b>2,279,299</b>             |
| Deferred rent  | 38,837                                   | 96,756                       |
| <b>Total liabilities</b>   | <b>3,635,742</b>                         | <b>2,376,055</b>             |
| Stockholders equity:   |  |                              |
| Preferred Stock; \$0.001 par value; 5,000,000 shares authorized, no shares issued and outstanding at June 30, 2014 and December 31, 2013   |  |                              |
| Series A-1 Convertible Preferred Stock; \$0.001 par value; no shares authorized, issued or outstanding at June 30, 2014; 60,342,745 shares authorized, 30,726,326 shares issued and outstanding at December 31, 2013 |  | 30,726                       |
| Common stock; \$0.001 par value; 100,000,000 authorized, 18,893,464 issued and outstanding at June 30, 2014; 70,258,276 authorized; 1,426,593 shares issued and outstanding at December 31, 2013                     | 18,894                                   | 1,428                        |
| Additional paid-in capital   | 124,250,499                              | 66,150,674                   |
| Accumulated deficit  | (60,385,539)                             | (63,275,409)                 |
| <b>Total stockholders equity</b>   | <b>63,883,854</b>                        | <b>2,907,419</b>             |

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|  |               |              |
|--|---------------|--------------|
| Total liabilities and stockholders' equity | \$ 67,519,596 | \$ 5,283,474 |
|--|---------------|--------------|

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

**Table of Contents****GLYCOMIMETICS, INC.****Unaudited Statements of Operations and Comprehensive Income (Loss)**

|   | <b>Three Months Ended June 30,</b> |                | <b>Six</b>                   |                |
|---|------------------------------------|----------------|------------------------------|----------------|
|   | <b>2014</b>                        | <b>2013</b>    | <b>Months Ended June 30,</b> | <b>2013</b>    |
|   |                                    |                | <b>2014</b>                  | <b>2013</b>    |
| Revenue   | \$ 15,027,004                      | \$ 55,074      | \$ 15,027,004                | \$ 3,862,665   |
| Costs and expenses:   |                                    |                |                              |                |
| Research and development  | 5,357,696                          | 2,883,777      | 9,239,417                    | 5,626,709      |
| General and administrative  | 1,605,092                          | 668,253        | 2,829,995                    | 1,274,192      |
| Total costs and expenses  | 6,962,788                          | 3,552,030      | 12,069,412                   | 6,900,901      |
| Income (loss) from operations                                     | 8,064,216                          | (3,496,956)    | 2,957,592                    | (3,038,236)    |
| Other income (expense)  | 4,263                              | (104)          | 9,036                        | 865            |
| Income (loss) and comprehensive income (loss) before income taxes | 8,068,479                          | (3,497,060)    | 2,966,628                    | (3,037,371)    |
| Income tax expense  | 76,758                             |                | 76,758                       |                |
| Net income (loss) and net comprehensive income (loss)             | \$ 7,991,721                       | \$ (3,497,060) | \$ 2,889,870                 | \$ (3,037,371) |
| Basic net income (loss) per common share                          | \$ 0.42                            | \$ (3.70)      | \$ 0.16                      | \$ (3.24)      |
| Diluted net income (loss) per common share                        | \$ 0.39                            | \$ (3.70)      | \$ 0.15                      | \$ (3.24)      |
| Basic weighted average number of common shares                    | 18,807,675                         | 946,363        | 18,020,121                   | 938,446        |
| Diluted weighted average number of common shares                  | 20,238,343                         | 946,363        | 19,472,995                   | 938,446        |

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

**Table of Contents****GLYCOMIMETICS, INC.****Unaudited Statements of Cash Flows**

|  | <b>Six Months Ended June 30,</b> |                |
|--|----------------------------------|----------------|
|  | <b>2014</b>                      | <b>2013</b>    |
| <b>Operating activities</b>  |                                  |                |
| Net income (loss)  | \$ 2,889,870                     | \$ (3,037,371) |
| Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities: |                                  |                |
| Depreciation   | 73,472                           | 64,319         |
| Loss on disposal of property and equipment   | 1,424                            |                |
| Compensation expense from stock option grants  | 710,643                          | 217,674        |
| Changes in assets and liabilities:   |                                  |                |
| Prepaid expenses and other current assets  | 1,691,358                        | 237,551        |
| Accounts payable   | (672,734)                        | (215,635)      |
| Accrued expenses   | 1,983,134                        | 56,320         |
| Deferred revenue   |                                  | (3,862,665)    |
| Deferred rent  | (50,713)                         | (44,301)       |
| Net cash provided by (used in) operating activities  | 6,626,454                        | (6,584,108)    |
| <b>Investing activities</b>  |                                  |                |
| Purchases of property and equipment  | (95,737)                         | (30,492)       |
| Net cash used in investing activities  | (95,737)                         | (30,492)       |
| <b>Financing activities</b>  |                                  |                |
| Proceeds from issuance of common stock, net of issuance costs                                      | 57,248,219                       |                |
| Proceeds from exercise of stock options and warrants   | 127,703                          | 19,750         |
| Net cash provided by financing activities  | 57,375,922                       | 19,750         |
| Net change in cash and cash equivalents  | 63,906,639                       | (6,594,850)    |
| Cash and cash equivalents, beginning of period   | 2,310,603                        | 17,372,832     |
| Cash and cash equivalents, end of period   | \$ 66,217,242                    | \$ 10,777,982  |
| <b>Supplemental schedule of noncash financing activities:</b>                                      |                                  |                |
| Conversion of Series A-1 Convertible Preferred Stock to Common Stock                               | \$ 38,805,055                    | \$             |

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



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**GLYCOMIMETICS, INC.**

**Notes to Unaudited Financial Statements**

**1. Description of the Business Planned Commercial Operations Have Not Commenced**

GlycoMimetics, Inc. (the Company) is a business that has not commercialized any of its product candidates. The Company was incorporated in Delaware on April 4, 2003 and commenced operations on May 21, 2003. The Company is headquartered in Gaithersburg, Maryland.

The Company is a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using its expertise in carbohydrate chemistry and knowledge of carbohydrate biology, the Company is developing a pipeline of proprietary glycomimetics that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection.

The Company's executive personnel have devoted substantially all of their time to date to the planning and organization of the Company, the process of hiring scientists, initiating research and development programs and securing adequate capital for anticipated growth and operations. The Company is subject to a number of risks similar to those of other companies in similar development stages, including dependence on key individuals, the need to develop commercially viable products, competition from other companies, many of whom are larger and better capitalized, and the need to obtain adequate additional financing to fund the development of its product candidates. The Company has incurred significant operating losses since inception and has relied on its ability to fund its operations through private and public equity financings, and management expects operating losses and negative operating cash flows to continue for the foreseeable future. As the Company continues to incur losses, transition to profitability is dependent upon the successful development, approval, and commercialization of its product candidates and achieving a level of revenues adequate to support the Company's cost structure. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital. Management intends to fund future operations through additional public or private equity or debt offerings and may seek additional capital through arrangements with strategic partners or from other sources.

***Initial Public Offering***

On January 15, 2014, the Company completed an initial public offering of its common stock, which resulted in the sale of 8,050,000 shares, including all additional shares available to cover over-allotments, at a price to the public of \$8.00 per share. The Company received net proceeds of approximately \$57.2 million.

**2. Significant Accounting Policies**

***Basis of Accounting***

The accompanying financial statements were prepared based on the accrual method of accounting in accordance with U.S. generally accepted accounting principles (GAAP).

***Unaudited Financial Statements***

The accompanying balance sheet as of June 30, 2014, statements of operations and comprehensive income (loss) for the three and six months ended June 30, 2014 and 2013 and cash flows for the six months ended June 30, 2014 and 2013 are unaudited. These unaudited financial statements have been prepared in accordance with the rules and regulations for the United States Securities and Exchange Commission for interim financial information. Accordingly, they do not include all of the information and footnotes required by GAAP for complete annual financial statements. These financial statements should be read in conjunction with the audited financial statements and the accompanying notes for the year ended December 31, 2013 contained in the Company's Annual Report on Form 10-K filed with the SEC on March 31, 2014. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments (consisting of normal recurring adjustments) necessary to state fairly the Company's financial position as of June 30, 2014, the results of operations for the three and six months ended June 30, 2014 and 2013 and cash flows for the six months ended June 30, 2014 and 2013. The December 31, 2013 balance sheet included herein was derived from audited financial statements, but does not include all disclosures including notes required by GAAP for complete annual financial statements. The financial data and other information disclosed in these notes to the financial statements related to the three and six months ended June 30, 2014 and 2013 are unaudited. Interim results are not necessarily indicative of results for an entire year.

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### ***Segment Information***

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one segment, which is the identification and development of glycomimetic compounds.

### ***Use of Estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting periods. Although actual results could differ from those estimates, management does not believe that such differences would be material.

### ***Cash and Cash Equivalents***

Cash and cash equivalents consist of certificates of deposit and investment in money market funds with commercial banks and financial institutions. The Company considers all investments in highly liquid financial instruments with an original maturity of three months or less at the date of purchase to be cash equivalents. Cash equivalents are stated at amortized cost, plus accrued interest, which approximates fair value.

### ***Restricted Cash***

The Company is required to maintain certificates of deposit that serve as collateral for operating leases and credit card accounts. Amounts classified as restricted cash were \$83,000 at June 30, 2014 and December 31, 2013, and are presented within prepaid expenses and other current assets.

### ***Fair Value Measurements***

The Company's financial instruments include cash and cash equivalents. The fair values of the financial instruments approximated their carrying values at June 30, 2014 and December 31, 2013, due to their short-term maturities. The Company accounts for recurring and nonrecurring fair value measurements in accordance with ASC 820, *Fair Value Measurements*. ASC 820 defines fair value, establishes a fair value hierarchy for assets and liabilities measured at fair value, and requires expanded disclosures about fair value measurements. The ASC hierarchy ranks the quality of reliability of inputs, or assumptions, used in the determination of fair value, and requires assets and liabilities carried at fair value to be classified and disclosed in one of the following three categories:

Level 1 Fair value is determined by using unadjusted quoted prices that are available in active markets for identical assets and liabilities.

Level 2 Fair value is determined by using inputs, other than Level 1 quoted prices that are directly and indirectly observable. Inputs can include quoted prices for similar assets and liabilities in active markets or quoted prices for identical assets and liabilities in inactive markets. Related inputs can also include those used in valuation or other pricing models that can be corroborated by observable market data.

Level 3 Fair value is determined by inputs that are unobservable and not corroborated by market data. Use of these inputs involves significant and subjective judgments to be made by a reporting entity. In instances where the determination of the fair value measurement is based on inputs from different levels of fair value hierarchy, the fair value measurement will fall within the lowest level input that is significant to the fair value measurement in its entirety.

The Company periodically evaluates financial assets and liabilities subject to fair value measurements to determine the appropriate level at which to classify them each reporting period. This determination requires the Company to make subjective judgments as to the significance of inputs used in determining fair value and where such inputs lie within the ASC 820 hierarchy.

The Company had no assets or liabilities that were measured using quoted prices for similar assets and liabilities or significant unobservable inputs (Level 2 and Level 3 assets and liabilities, respectively) as of June 30, 2014 and December 31, 2013. The carrying value of cash held in money market funds of approximately \$64.1 million and \$2.2 million as of June 30, 2014 and December 31, 2013, respectively, is included in cash and cash equivalents and approximates market values based on quoted market prices (Level 1 inputs).

**Table of Contents*****Concentration of Credit Risk***

Credit risk represents the risk that the Company would incur a loss if counterparties failed to perform pursuant to the terms of their agreements. Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. Cash and cash equivalents consist of certificates of deposit and money market funds with major financial institutions in the United States. These deposits and funds may be redeemed upon demand and, therefore, bear minimal risk. The Company does not anticipate any losses on such balances.

***Property and Equipment***

Property and equipment are recorded at cost and depreciated on a straight-line basis over estimated useful lives ranging from one to five years. Upon retirement or disposition of assets, the costs and related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the results of operations. Expenditures for repairs and maintenance are charged to operations as incurred; major replacements that extend the useful life are capitalized. Depreciation and amortization are computed using the straight-line method over the following estimated useful lives:

|                        | <b>ESTIMATED USEFUL LIVES</b>        |
|------------------------|--------------------------------------|
| Furniture and fixtures | 2 5 years                            |
| Laboratory equipment   | 1 5 years                            |
| Office equipment       | 1 5 years                            |
| Computer equipment     | 1 5 years                            |
| Leasehold improvements | Shorter of lease term or useful life |

***Impairment of Long-Lived Assets***

The Company periodically assesses the recoverability of the carrying value of its long-lived assets in accordance with the provisions of ASC 360, *Property, Plant, and Equipment*. ASC 360 requires that long-lived assets and certain identifiable intangible assets be reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of the long-lived asset is measured by a comparison of the carrying amount of the asset to future undiscounted net cash flows expected to be generated by the asset. If the carrying value exceeds the sum of undiscounted cash flows, the Company then determines the fair value of the underlying asset. Any impairment to be recognized is measured by the amount by which the carrying amount of the assets exceeds the estimated fair value of the assets. Assets to be disposed of are reported at the lower of the carrying amount or fair value, less costs to sell. As of June 30, 2014 and December 31, 2013, the Company determined that there were no impaired assets and had no assets held for sale.

***Revenue Recognition***

From time to time, the Company is awarded reimbursement contracts for services and development grant contracts with government and non-government entities and philanthropic organizations. Under these contracts, the Company typically is reimbursed for the costs in connection with specific development activities. The Company recognizes revenue to the extent of costs incurred in connection with performance under such grant arrangements.

The Company has entered into a collaborative research and development agreement with Pfizer Inc. (Pfizer). The agreement is in the form of a license agreement. The agreement called for a nonrefundable up-front payment and milestone payments upon achieving significant milestone events. The agreement also contemplates royalty payments

on future sales of an approved product. There are no performance, cancellation, termination, or refund provisions in the arrangement that contain material financial consequences to the Company.

The primary deliverable under this arrangement is an exclusive worldwide license to the Company's rivipansel compound (previously referred to by the Company as GMI-1070), but the arrangement also includes deliverables related to research and preclinical development activities to be performed by the Company on Pfizer's behalf.

Collaborative research and development agreements can provide for one or more of up-front license fees, research payments, and milestone payments. Agreements with multiple components (deliverables or items) are evaluated according to the provisions of ASC 605-25, *Revenue Recognition Multiple-Element Arrangements*, to determine whether the deliverables can be separated into more than one unit of accounting. An item can generally be considered a separate unit of accounting if all of the following criteria are met: (1) the delivered item(s) has value to the customer on a stand-alone basis and (2) if the arrangement includes a general right of

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return relative to the delivered item(s) then delivery or performance of the undelivered item(s) is considered probable and substantially in control of the Company. Items that cannot be divided into separate units are combined with other units of accounting, as appropriate. Consideration received is allocated among the separate units based on selling price hierarchy. The selling price hierarchy for each deliverable is based on (i) vendor-specific objective evidence (VSOE), if available; (ii) third-party evidence (TPE) of selling price if VSOE is not available; or (iii) an estimated selling price, if neither VSOE nor third-party evidence is available. Management was not able to establish VSOE or TPE for separate unit deliverables, as the Company does not have a history of entering such arrangements or selling the individual deliverables within such arrangements separately. In addition, there may be significant differentiation in these arrangements, which indicates that comparable third-party pricing may not be available. Management determined that the selling price for the deliverables within the Pfizer collaboration agreement should be determined using its best estimate of selling price. The process of determining the best estimate of selling price involved significant judgment on the Company's part and included consideration of multiple factors such as estimated direct expenses, other costs, and available clinical development data.

The Company adopted the aforementioned accounting standard for multiple-element arrangements effective January 1, 2011. Pursuant to this standard, each required deliverable under the Pfizer collaboration agreement is evaluated to determine whether it qualifies as a separate unit of accounting. Factors considered in this determination include the research capabilities of Pfizer, the proprietary nature of the license and know-how, and the availability of the Company's glycomimetics technology research expertise in the general marketplace. Based on all relevant facts and circumstances and, most significantly, on the proprietary nature of the Company's technology and the related proprietary nature of the Company's research services, management concluded that stand-alone value does not exist for the license, and therefore, the license is not a separate unit of accounting under the contract and will be combined with the research and development services (including participation on a joint steering committee).

As such, the up-front payment received of \$22.5 million was recognized as revenue over the expected development period through March 2013. The determination of the length of the period over which to defer revenue and the methodology by which to recognize the related revenues is subject to judgment and estimation. Consistent with the research plan developed by and agreed to by both parties, management estimates that the research activities and participation on the joint steering committees will occur over a 1.5-year period. Revenues associated with the up-front license fee are recognized over this period using a straight-line method, which is consistent with expected completion of the research services.

Effective January 1, 2011, the Company also adopted ASC 605-28, *Revenue Recognition Milestone Method*. Under this guidance, at the inception of agreements that include milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether (a) the consideration is commensurate with either (1) the entity's performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone, (b) the consideration relates solely to past performance and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. In making this assessment, the Company evaluates factors such as scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the agreement. Non-refundable development and regulatory milestones that are expected to be achieved as a result of the Company's efforts during the period of substantial involvement are recognized as revenue upon the achievement of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive because the Company does not contribute effort to the achievement of such milestones are generally achieved after the period of substantial involvement and are recognized as revenue upon achievement of the milestone, as there are no undelivered elements remaining and no continuing performance obligation, assuming all

other revenue recognition criteria are met. In May 2014, the Company recognized \$15.0 million in revenue as a result of the first milestone payment received from Pfizer.

***Research and Development Costs***

Except for payments made in advance of services, research and development costs are expensed as incurred. For payments made in advance, the Company recognizes research and development expense as the services are rendered. Research and development costs primarily consist of salaries and related expenses for personnel, laboratory supplies and raw materials, sponsored research, depreciation of laboratory facilities and leasehold improvements, and utilities costs related to research space. Other research and development expenses include fees paid to consultants and outside service providers.

***Stock-Based Compensation***

Stock-based payments are accounted for in accordance with the provisions of ASC 718, *Compensation - Stock Compensation*. The fair value of stock-based payments is estimated, on the date of grant, using the Black-Scholes-Merton model. The resulting fair value is recognized ratably over the requisite service period, which is generally the vesting period of the option.



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The Company has elected to use the Black-Scholes-Merton option pricing model to value any options granted. The Company will reconsider use of the Black-Scholes-Merton model if additional information becomes available in the future that indicates another model would be more appropriate or if grants issued in future periods have characteristics that prevent their value from being reasonably estimated using this model.

A discussion of management's methodology for developing some of the assumptions used in the valuation model follows:

*Fair Value of Common Stock* Prior to the Company's initial public offering in January 2014, the Company had no active public market for its common stock. In the absence of a public trading market, and as a clinical-stage company with no significant revenues, the Company believed that it was appropriate to consider a range of factors to determine the fair market value of the common stock at each grant date. In determining the fair value of its common stock, the Company used methodologies, approaches, and assumptions consistent with the American Institute of Certified Public Accountants' (AICPA) Audit and Accounting Practice Aid Series: *Valuation of Privately Held Company Equity Securities Issued as Compensation* (the AICPA Practice Guide). In addition, the Company considered various objective and subjective factors, along with input from the independent third-party valuation firm. The factors included (1) the achievement of clinical and operational milestones by the Company; (2) the status of strategic relationships with collaborators; (3) the significant risks associated with the Company's stage of development; (4) capital market conditions for life science companies, particularly similarly situated, privately held, early-stage life science companies; (5) the Company's available cash, financial condition, and results of operations; (6) the most recent sales of the Company's preferred stock; and (7) the preferential rights of the outstanding preferred stock.

*Expected Dividend Yield* The Company has never declared or paid dividends and has no plans to do so in the foreseeable future.

*Expected Volatility* Volatility is a measure of the amount by which a financial variable such as share price has fluctuated (historical volatility) or is expected to fluctuate (expected volatility) during a period. The Company does not maintain an internal market for its shares, and prior to its initial public offering the shares were not traded publicly. The Company utilizes the historical volatilities of a peer group (e.g., several public entities of similar size, complexity, and stage of development) to determine its expected volatility.

*Risk-Free Interest Rate* This is the U.S. Treasury rate for the week of each option grant during the year, having a term that most closely resembles the expected life of the option.

*Expected Term* This is a period of time that the options granted are expected to remain unexercised. Options granted have a maximum term of 10 years. The Company estimates the expected life of the option term to be 6.25 years. The Company uses a simplified method to calculate the average expected term.

*Expected Forfeiture Rate* The forfeiture rate is the estimated percentage of options granted that is expected to be forfeited or canceled on an annual basis before becoming fully vested. The Company estimates the forfeiture rate based on turnover data with further consideration given to the class of the employees to whom the options were granted.

Equity instruments issued to nonemployees are accounted for under the provisions of ASC 718, *Compensation Stock Compensation*, and ASC 505-50, *Equity Equity-Based Payments to Non-Employees*. Accordingly, the estimated fair value of the equity instrument is recorded on the earlier of the performance commitment date or the date the services are completed and are marked to market during the service period.

***Net Income (Loss) Per Share***

Basic net income (loss) per common share is determined by dividing net income (loss) by the weighted-average number of common shares outstanding during the period, without consideration of common stock equivalents. Diluted net income (loss) per share is computed by dividing net income (loss) by the weighted-average number of common stock equivalents outstanding for the period. The treasury stock method is used to determine the dilutive effect of the Company's stock option grants and warrants and the if-converted method is used to determine the dilutive effect of the Company's Series A-1 convertible preferred stock.

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Basic and diluted income (loss) per common share is computed as follows:

|   | Three Months Ended |                | Six Months Ended June 30, |                |
|---|--------------------|----------------|---------------------------|----------------|
|   | June 30,<br>2014   | 2013           | 2014                      | 2013           |
| Net income (loss)                                     | \$ 7,991,721       | \$ (3,497,060) | \$ 2,889,870              | \$ (3,037,371) |
| Income (loss) per share-basic                         | \$ 0.42            | \$ (3.70)      | \$ 0.16                   | \$ (3.24)      |
| Income (loss) per share-diluted                       | \$ 0.39            | \$ (3.70)      | \$ 0.15                   | \$ (3.24)      |
| Basic weighted average common<br>shares outstanding   | 18,807,675         | 946,363        | 18,020,121                | 938,446        |
| Diluted weighted average common<br>shares outstanding | 20,238,343         | 946,363        | 19,472,995                | 938,446        |

The following common stock equivalents are included in the calculation of diluted net income (loss) per share:

|               | Three Months Ended June 30, |         | Six Months Ended June 30, |         |
|---------------|-----------------------------|---------|---------------------------|---------|
|               | 2014                        | 2013    | 2014                      | 2013    |
| Warrants      |                             | 611,698 |                           | 613,345 |
| Stock options |                             | 818,970 |                           | 839,529 |

The following potentially dilutive securities outstanding have been excluded from the computation of diluted weighted average shares outstanding, as they would be anti-dilutive:

|                           | Three Months Ended June 30, |           | Six Months Ended June 30, |           |
|---------------------------|-----------------------------|-----------|---------------------------|-----------|
|                           | 2014                        | 2013      | 2014                      | 2013      |
| Warrants                  | 1,544                       | 635,255   | 1,544                     | 635,255   |
| Stock options             | 919,188                     | 1,495,605 | 919,188                   | 1,495,605 |
| Converted preferred stock |                             | 9,305,359 |                           | 9,305,359 |

**Income Taxes**

The Company accounts for income taxes using the asset and liability method in accordance with ASC 740, *Income Taxes*. Deferred income taxes are recognized for the tax consequences in future years of differences between the tax bases of assets and liabilities and the financial reporting amounts at each year-end based on enacted tax laws and statutory tax rates applicable to the periods in which the differences are expected to affect taxable income. A valuation allowance is established when necessary to reduce deferred tax assets to the amount expected to be realized.

The Company accounts for uncertain tax positions pursuant to ASC 740. Financial statement recognition of a tax position taken or expected to be taken in a tax return is determined based on a more-likely-than-not threshold of that tax position being sustained. If the tax position meets this threshold, the benefit to be recognized is measured as the tax benefit having the highest likelihood of being realized upon ultimate settlement with the taxing authority. The Company recognizes interest accrued related to unrecognized tax benefits and penalties in the provision for income taxes.

**Comprehensive Income (Loss)**

Comprehensive income (loss) comprises net income (loss) and other changes in equity that are excluded from net income (loss). For the three and six months ended June 30, 2014 and 2013, the Company's net income (loss) equals comprehensive income (loss) and, accordingly, no additional disclosure is presented.

***Recently Issued Accounting Standards***

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, *Revenue from Contracts with Customers*. ASU 2014-09 will eliminate transaction- and industry-specific revenue recognition guidance under current U.S. GAAP and replace it with a principle-based approach for determining revenue recognition. ASU 2014-09 will require that companies recognize revenue based on the value of transferred goods or services as they occur in the contract. ASU 2014-09 will also require additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2016. Early application is not permitted. Entities can transition to the standard either retrospectively or as a cumulative-effect adjustment as of the date of adoption. Presently, the Company is assessing what effect the adoption of ASU 2014-09 will have on its financial statements and accompanying notes.

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In June 2014, the FASB issued ASU No. 2014-10, which amended Accounting Standards Codification (ASC) Topic 915 *Development Stage Entities*. The amendment eliminates certain financial reporting requirements surrounding development stage entities, including an amendment to the variable interest entities guidance in ASC Topic 810, *Consolidation*. The amendment removes the definition of a development stage entity from the ASC, thereby removing the financial reporting distinction between development stage entities and other entities from U.S. GAAP.

Consequently, the amendment eliminates the requirements for development stage entities to (1) present inception-to-date information in the statements of income, cash flows and shareholder equity, (2) label the financial statements as those of a development stage entity, (3) disclose a description of the development stage activities in which the entity is engaged, and (4) disclose the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage.

This amendment is effective for fiscal years beginning after December 15, 2014, and interim periods therein. Early application of each of the amendments is permitted for any annual reporting period or interim period for which the entity's financial statements have not yet been issued. The Company has made the election to early adopt this amendment effective June 30, 2014 and, as a result, the Company is no longer presenting or disclosing the information previously required under Topic 915. The early adoption was made to reduce data maintenance by removing all incremental financial reporting requirements for development stage entities. The adoption of this amendment alters the disclosure requirements of the Company, but it does not have any material impact on the Company's financial position or results of operations for the current or any prior reporting periods.

**3. Prepaid Expenses and Other Current Assets**

The following is a summary of the Company's prepaid expenses and other current assets:

|                                       | <b>June 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|---------------------------------------|--------------------------|------------------------------|
| Prepaid expenses                      | \$ 756,940               | \$ 172,814                   |
| Restricted deposits                   | 82,700                   | 82,700                       |
| Other receivables                     | 42,074                   | 94,754                       |
| Prepaid initial public offering costs |                          | 2,222,804                    |
|                                       | <b>\$ 881,714</b>        | <b>\$ 2,573,072</b>          |

**4. Property and Equipment**

Property and equipment consists of the following:

|                        | <b>June 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|------------------------|--------------------------|------------------------------|
| Furniture and fixtures | \$ 118,201               | \$ 108,815                   |
| Laboratory equipment   | 901,955                  | 826,821                      |
| Office equipment       | 10,467                   | 10,467                       |
| Computer equipment     | 173,129                  | 167,764                      |
| Leasehold improvements | 41,843                   | 41,843                       |

|                               |            |            |
|-------------------------------|------------|------------|
|                               | 1,245,595  | 1,155,710  |
| Less accumulated depreciation | (824,955)  | (755,911)  |
|                               | \$ 420,640 | \$ 399,799 |

Depreciation expense for the three months ended June 30, 2014 and 2013 was \$38,852 and \$32,378, respectively, and \$73,472 and \$64,319 for the six months ended June 30, 2014 and 2013, respectively.

**Table of Contents****5. Accrued Expenses**

The following is a summary of the Company's accrued expenses:

|  | <b>June 30,<br/>2014</b> | <b>December 31,<br/>2013</b> |
|--|--------------------------|------------------------------|
| Accrued expenses                               | \$ 1,588,741             | \$ 356,345                   |
| Accrued research and development expenses      | 493,200                  | 114,005                      |
| Accrued consulting and other professional fees | 171,129                  | 54,211                       |
| Accrued employee benefits                      | 250,763                  | 155,935                      |
| Accrued taxes                                  | 136,758                  | 5,250                        |
|  | <b>\$ 2,640,591</b>      | <b>\$ 685,746</b>            |

**6. Operating Leases**

The Company leases its office and research space under a five-year operating lease that is subject to escalation clauses. The current lease expires in October 2015. In connection with its lease arrangement, the Company received rent abatement as a lease incentive. The rent abatement has been recognized as deferred rent that will be adjusted on a straight-line basis over the term of the lease. Deferred rent was \$150,234 and \$200,947 at June 30, 2014 and December 31, 2013, respectively. Total rent expense for the three months ended June 30, 2014 and 2013 was \$96,554 and \$96,317, respectively, and \$192,871 and \$177,307 for the six months ended June 30, 2014 and 2013, respectively.

Please refer to note 10 (Subsequent Event) for a description of the Company's lease agreement with BMR-Medical Center Drive LLC, which was entered into on July 23, 2014.

**7. Stockholders' Equity*****Convertible Preferred Stock******Series A-1 Convertible Preferred Stock***

On October 20, 2009, the Company entered into a Series A-1 Preferred Stock Purchase Agreement with certain investors. In connection with the financing, the Company issued 30,726,326 shares of Series A-1 Convertible Preferred Stock for an aggregate amount of \$38,979,412, which included the conversion of principal and accrued interest related to an earlier bridge financing of \$16,099,770. In connection with the Series A-1 Preferred Stock financing, all then-outstanding shares of Series A and Series B Preferred Stock were converted into common stock, and all then outstanding warrants to purchase Series B Preferred Stock were converted into warrants to purchase common stock. Immediately prior to the Series A-1 Preferred Stock financing, the Company effected a 1-for-10 reverse stock split of the outstanding common stock. All prior-period applicable share amounts have been retroactively adjusted to reflect the reverse stock split.

Upon closing of the Company's initial public offering, all outstanding shares of convertible preferred stock converted into an aggregate of 9,305,359 shares of common stock. In addition, immediately following the closing of the initial public offering, the Company's certificate of incorporation was amended to authorize 5,000,000 shares of undesignated preferred stock and 100,000,000 shares of common stock. Both the common stock and undesignated preferred stock

have a par value of \$0.001 per share.

***Common Stock***

As of June 30, 2014 and December 31, 2013, there were 100,000,000 and 70,258,276 shares of common stock, respectively, authorized to be issued. Certain of the outstanding shares of common stock are subject to stock restriction agreements (a Restriction Agreement). Pursuant to a Restriction Agreement, a stockholder shall not sell, assign, transfer, or otherwise dispose of any shares except to the Company or as expressly provided in the Restriction Agreement.

In connection with preparing for the initial public offering of the Company's common stock, the Company's board of directors and stockholders approved a 1-for-3.302 reverse stock split of the Company's common stock. The reverse stock split became effective on October 25, 2013. All share and per share amounts in the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse stock split, including reclassifying an amount to the reduction in par value of common stock to additional paid-in capital.

***Warrants to Acquire Company Stock***

On October 13, 2006, the Company issued a warrant to purchase 1,544 shares of common stock at an exercise price of \$25.92 per share to a commercial bank. This warrant, which was originally issuable for Series B Preferred Stock prior to the conversion of Series B Preferred Stock to common stock in 2009, was vested upon issuance and expires in October 2016. The fair value of the warrant, which was de minimis, was calculated using the Black-Scholes-Merton option pricing model.



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As part of the issuance of convertible unsecured promissory notes, the Company issued warrants to purchase an aggregate of 633,709 shares of common stock. In April 2014, one of the Company's warrant holders exercised its warrant to purchase 1,026 shares of common stock at an exercise of \$0.33 per share, resulting in proceeds to the Company of \$339.

The following common stock warrants were outstanding at June 30, 2014:

| <b>NUMBER OF SHARES</b> | <b>EXERCISE PRICE PER SHARE</b> | <b>EXPIRATION DATE</b> |
|-------------------------|---------------------------------|------------------------|
| 298,404                 | \$ 0.33                         | July 18, 2018          |
| 301,987                 | 0.33                            | January 16, 2019       |
| 17,042                  | 0.33                            | December 9, 2015       |
| 15,250                  | 0.33                            | January 30, 2019       |
| 1,544                   | 25.92                           | October 13, 2016       |

**2003 Stock Incentive Plan**

The 2003 Stock Incentive Plan (the 2003 Plan) provided for the grant of incentives and nonqualified stock options and restricted stock awards. The exercise price for incentive stock options must be at least equal to the fair value of the common stock on the grant date. Unless otherwise stated in a stock option agreement, 25% of the shares subject to an option grant will vest upon the first anniversary of the vesting start date and thereafter at the rate of one forty-eighth of the option shares per month as of the first day of each month after the first anniversary. Upon termination of employment by reasons other than death, cause, or disability, any vested options shall terminate 60 days after the termination date. Stock options terminate 10 years from the date of grant. The 2003 Plan expired on May 21, 2013. There were options for 9,297 shares granted under the 2003 Plan at a weighted average exercise price of \$3.73 during the six months ended June 30, 2013 prior to the expiration of the 2003 Plan.

A summary of the Company's stock option activity under the 2003 Plan for the six months ended June 30, 2014 is as follows:

|  | <b>OUTSTANDING OPTIONS</b> | <b>WEIGHTED-AVERAGE EXERCISE PRICE</b> | <b>WEIGHTED-AVERAGE REMAINING TERM (YEARS)</b> | <b>WEIGHTED-AVERAGE CONTRACTUAL VALUE (IN THOUSANDS)</b> |
|--|----------------------------|--|--|--|
| Outstanding as of December 31, 2013            | 972,860                    | \$ 1.18                                | 7.19   | \$ 7,000   |
| Options granted                                |                            |  |  |  |
| Options exercised                              | (110,486)                  | 1.16                                   |  |  |
| Options forfeited                              | (633)                      | 1.12                                   |  |  |
| Outstanding as of June 30, 2014                | 861,741                    | 1.26                                   | 5.70   | 6,180  |
| Vested or expected to vest as of June 30, 2014 | 859,829                    | 1.26                                   | 5.70   | 6,168  |

|                                 |         |      |      |       |
|---------------------------------|---------|------|------|-------|
| Exercisable as of June 30, 2014 | 802,034 | 1.20 | 5.56 | 5,799 |
|---------------------------------|---------|------|------|-------|

As of June 30, 2014, there was \$95,624 of total unrecognized compensation expense related to unvested options that will be recognized over a weighted-average period of approximately one year. Total intrinsic value of the options exercised during the six months ended June 30, 2013 was not material. The total fair value of shares underlying options which vested in the six months ended June 30, 2014 and 2013 was \$30,308 and \$180,295, respectively.

### ***2013 Equity Incentive Plan***

The Company's board of directors adopted, and its stockholders approved, its 2013 Equity Incentive Plan (the 2013 Plan). The 2013 Plan provides for the grant of incentive stock options within the meaning of Section 422 of the Internal Revenue Code (the Code), to the Company's employees and its parent and subsidiary corporations' employees, and for the grant of nonstatutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance stock awards and other forms of stock compensation to its employees, including officers, consultants and directors. The 2013 Plan also provides for the grant of performance cash awards to the Company's employees, consultants and directors.

**Table of Contents***Authorized Shares*

The maximum number of shares of common stock that may be issued under the 2013 Plan is 1,000,000 shares, plus any shares subject to stock options or similar awards granted under the 2003 Plan that expire or terminate without having been exercised in full or are forfeited to or repurchased by the Company. The number of shares of common stock reserved for issuance under the 2013 Plan will automatically increase on January 1 of each year, beginning on January 1, 2015 and ending on January 1, 2023, by 3% of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Company's board of directors. The maximum number of shares that may be issued pursuant to exercise of incentive stock options under the 2013 Plan is 20,000,000.

Shares issued under the 2013 Plan may be authorized but unissued or reacquired shares of common stock. Shares subject to stock awards granted under the 2013 Plan that expire or terminate without being exercised in full, or that are paid out in cash rather than in shares, will not reduce the number of shares available for issuance under the 2013 Plan. Additionally, shares issued pursuant to stock awards under the 2013 Plan that the Company repurchases or that are forfeited, as well as shares reacquired by the Company as consideration for the exercise or purchase price of a stock award or to satisfy tax withholding obligations related to a stock award, will become available for future grant under the 2013 Plan.

A summary of the Company's stock option activity under the 2013 Plan for the six months ended June 30, 2014 is as follows:

|  | OUTSTANDING<br>OPTIONS | EXERCISE<br>PRICE | TERM<br>(YEARS) | WEIGHTED-<br>AVERAGE<br>WEIGHTED<br>AVERAGE<br>CONTRACTUAL<br>VALUE<br>(IN<br>THOUSANDS) |
|--|------------------------|-------------------|-----------------|--|
| Outstanding as of December 31, 2013            |                        | \$                |                 | \$   |
| Options granted                                | 919,188                | 8.90              |                 |  |
| Options exercised                              |                        |                   |                 |  |
| Options forfeited                              |                        |                   |                 |  |
| Outstanding as of June 30, 2014                | 919,188                | 8.90              | 9.58            | 372  |
| Vested or expected to vest as of June 30, 2014 | 893,964                | 8.90              | 9.31            | 362  |
| Exercisable as of June 30, 2014                |                        |                   |                 |  |

The weighted-average fair value of the options granted during the six months ended June 30, 2014 was \$6.72 per share, applying the Black-Scholes-Merton option pricing model utilizing the following weighted-average assumptions:

**Six Months Ended  
June 30, 2014**

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|                         |        |
|-------------------------|--------|
| Expected term (years)   | 6.25   |
| Expected volatility     | 90.50% |
| Risk-free interest rate | 2.14%  |
| Expected dividend yield | 0%     |

As of June 30, 2014, there was \$5,322,601 of total unrecognized compensation expense related to unvested options that will be recognized over a weighted-average period of approximately 3.4 years.

Stock-based compensation expense was classified on the statement of operations as follows for the three and six months ended June 30, 2014 and 2013:

|   | Three Months Ended June 30, |                   | Six Months Ended June 30, |                   |
|---|-----------------------------|-------------------|---------------------------|-------------------|
|   | 2014                        | 2013              | 2014                      | 2013              |
| Research and development                      | \$ 127,658                  | \$ 49,560         | \$ 219,124                | \$ 98,310         |
| General and administrative                    | 290,771                     | 60,103            | 491,519                   | 119,364           |
| <b>Total stock-based compensation expense</b> | <b>\$ 418,429</b>           | <b>\$ 109,663</b> | <b>\$ 710,643</b>         | <b>\$ 217,674</b> |

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**Table of Contents****8. Income Taxes**

The Company recorded an income tax expense of \$76,758 and \$0 for the six months ended June 30, 2014 and 2013, respectively. The Company has provided a valuation allowance for the full amount of its net deferred tax assets since realization of any future benefit from deductible temporary differences, net operating loss carryforwards and research and development credits is not more-likely-than-not to be realized at June 30, 2014 and December 31, 2013. Under the Internal Revenue Code, certain substantial changes in the Company's ownership may result in a limitation on the amount of net operating loss carryforwards that can be used in future years. The Company's net operating loss carryforwards and research and development tax credits may be limited for use in a future annual period.

**9. Research and License Agreements**

In October 2011, the Company and Pfizer entered into a licensing agreement (the Pfizer Agreement) that provides Pfizer an exclusive worldwide license to rivipansel for vaso-occlusive crisis associated with sickle cell disease and for other diseases for which the drug candidate may be developed. The Company was responsible for completion of the Phase 2 trial, after which Pfizer will assume all further development and commercialization responsibilities. Upon execution of the Pfizer Agreement, the Company received an up-front payment of \$22.5 million. The Pfizer Agreement also provides for potential milestone payments of up to \$115.0 million upon the achievement of specified development milestones, including the dosing of the first patients in Phase 3 clinical trials for up to two indications and the first commercial sale of a licensed product in the United States and selected European countries for up to two indications; potential milestone payments of up to \$70.0 million upon the achievement of specified regulatory milestones, including the acceptance of our filings for regulatory approval by regulatory authorities in the United States and Europe for up to two indications; and potential milestone payments of up to \$135.0 million upon the achievement of specified levels of annual net sales of licensed products. In May 2014, the Company received a non-refundable payment of \$15.0 million from Pfizer as a partial milestone payment owed to the Company upon the dosing of the first patient in the Phase 3 clinical trial. Upon the dosing of the first patient in a Phase 3 clinical trial of rivipansel, the Company will be entitled to receive the remaining \$20.0 million of the scheduled milestone payment under the Pfizer Agreement.

The Company has determined that each potential future clinical, development and regulatory milestone is substantive. Although sales-based milestones are not considered substantive, they are still recognized upon achievement of the milestone (assuming all other revenue recognition criteria have been met) because there are no undelivered elements that would preclude revenue recognition at that time. The Company is also eligible to receive royalties on future sales contingent upon annual net sales thresholds. In addition, the Company and Pfizer have formed a joint steering committee that will oversee and coordinate activities as set forth in the research program. The \$22.5 million up-front payment was recognized over a period of 1.5 years through the six months ended March 31, 2013. During the six months ended June 30, 2013, the Company recorded revenue of \$3.8 million pursuant to the Pfizer Agreement in the Company's statement of operations. In May 2014, the Company received a non-refundable payment from Pfizer in the amount of \$15.0 million.

Pfizer has the right to terminate the Agreement by giving prior written notice. As of June 30, 2014, Pfizer and the Company are both in compliance with the provisions of the Agreement.

In February 2004, the Company entered into a research services agreement (the Research Agreement) with the University of Basel (the University) for biological evaluation of selectin antagonists. Certain patents covering the rivipansel compound are subject to provisions of the Research Agreement.

Under the terms of the Research Agreement, the Company is required to pay the University 10% payment of all milestone and royalty payments received from Pfizer with respect to rivipansel. During the three months ended June 30, 2014, the Company recorded an accrued expense of \$1.5 million due to the University, representing 10% of the \$15.0 million non-refundable cash milestone payment that the Company received from Pfizer in May 2014.

#### **10. Subsequent Event**

On July 23, 2014, the Company and BMR-Medical Center Drive LLC (the Landlord) entered into a lease agreement for the Company's future corporate headquarters in Rockville, Maryland (the Lease). Under the Lease, which will have an 8-year term commencing on November 1, 2015, the Company will lease 33,843 square feet of office and laboratory space and will pay \$607,500 in annual base rent over the term of the Lease, subject to annual escalation clauses. The Company will also have the right to sublease or assign all or a portion of the premises, subject to the conditions set forth in the Lease. The Lease may be terminated early by either the Landlord or the Company in certain circumstances.

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The Company will continue to incur rent expenses for its current headquarters in Gaithersburg, Maryland under its current lease that expires in October 2015.

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**Table of Contents****ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS**

*Certain statements contained in this Quarterly Report on Form 10-Q may constitute 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. The words or phrases would be, will allow, intends to, will likely result, are expected to, will continue, is anticipated, estimate, project, or similar expressions, or the negative of such words or phrases, are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events. Because such statements include risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Factors that could cause or contribute to these differences include those below and elsewhere in this Quarterly Report on Form 10-Q, particularly in Part II Item 1A, Risk Factors, and our other filings with the Securities and Exchange Commission. Statements made herein are as of the date of the filing of this Form 10-Q with the Securities and Exchange Commission and should not be relied upon as of any subsequent date. Unless otherwise required by applicable law, we do not undertake, and we specifically disclaim, any obligation to update any forward-looking statements to reflect occurrences, developments, unanticipated events or circumstances after the date of such statement.*

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited financial statements and related notes that appear in Item 1 of this Quarterly Report on Form 10-Q and with our audited financial statements and related notes for the year ended December 31, 2013, which are included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 31, 2014.

**Overview**

We are a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using our expertise in carbohydrate chemistry and knowledge of carbohydrate biology, we are developing a pipeline of proprietary glycomimetics that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. We believe this represents an innovative approach to drug discovery to treat a wide range of diseases.

We are focusing our initial efforts on drug candidates for rare diseases that we believe will qualify for orphan drug designation. We are developing our lead drug candidate, rivipansel (previously referred to as GMI-1070), for the treatment of vaso-occlusive crisis, or VOC, one of the most severe complications of sickle cell disease. Rivipansel has received both orphan drug designation and fast track designation from the U.S. Food and Drug Administration, or FDA, for the treatment of VOC. In October 2011, we entered into a license agreement with Pfizer under which we were responsible for the clinical development of rivipansel through the completion of a Phase 2 clinical trial. Following our completion of the Phase 2 clinical trial in April 2013, Pfizer is now responsible for all further clinical development, regulatory approval and potential commercialization of rivipansel for all indications and Pfizer has commercial rights to rivipansel worldwide. Pfizer had advised us through the joint steering committee established under the license agreement that they intend to begin enrolling patients for a Phase 3 clinical trial of rivipansel in the second half of 2014. In July 2014, we were notified by Pfizer that an agreement had been reached with the FDA under a special protocol assessment, or SPA, for the Phase 3 trial.

Building on our experience with rivipansel, we are developing a pipeline of other glycomimetic drug candidates. Our second most advanced drug candidate, GMI-1271, is a specific E-selectin inhibitor, which we are developing to be



used in combination with chemotherapy to treat patients with acute myeloid leukemia, or AML, and potentially other hematologic cancers. In June 2014, we commenced a Phase 1 clinical trial to evaluate the safety, tolerability and pharmacokinetics of GMI-1271 for the treatment of AML. The single-site Phase 1 trial of GMI-1271 is a randomized, double-blind, placebo-controlled, single ascending intravenous dose study of GMI-1271 in 28 healthy adult subjects. Three dose levels of GMI-1271 will be tested. We anticipate the final data being available in the second half of 2014. Our next planned study is a Phase 1/2 trial of GMI-1271 as an adjunct to standard chemotherapy in patients with AML.

We are also developing a pipeline of other preclinical drug candidates based on our expertise in carbohydrate chemistry. We have retained the worldwide development and commercialization rights to all of our drug candidates other than rivipansel.

We commenced operations in 2003, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, developing our glycomimetics platform, identifying potential drug candidates, undertaking preclinical studies and, beginning in 2008, conducting clinical trials of rivipansel. To date, we have financed our operations primarily through private placements of our securities, upfront and milestone payments under our collaboration with Pfizer and the net proceeds from our initial public offering, or IPO, in January 2014. We have no products currently available for sale, and substantially all of our

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revenue to date has been revenue from the upfront and milestone payments from Pfizer, although we have received nominal amounts of revenue under research grants. Prior to our IPO, we raised an aggregate of \$86.6 million to fund our operations, of which \$22.5 million was an upfront payment under our collaboration with Pfizer and \$64.1 million was from the sale of our convertible promissory notes and convertible preferred stock. The IPO provided us with net proceeds of \$57.2 million, and we received a partial non-refundable milestone payment from Pfizer in May 2014 of \$15.0 million.

Since inception, we have incurred significant operating losses. Although we have generated cumulative revenue of \$38.6 million since our inception through June 30, 2014, primarily consisting of the \$22.5 million upfront payment from Pfizer in 2011 and the \$15.0 million milestone payment in May 2014, we had an accumulated deficit of \$60.4 million as of June 30, 2014 and we expect to continue to incur significant expenses and operating losses over at least the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our clinical trials, the receipt of milestone payments, if any, under our collaboration with Pfizer, and our expenditures on other research and development activities. We anticipate that our expenses will increase substantially as we:

prepare and initiate our planned Phase 1/2 clinical trials of GMI-1271 in 2014;

continue the research and development of our other drug candidates;

seek to discover and develop additional drug candidates;

seek regulatory approvals for any drug candidates other than rivipansel that successfully complete clinical trials;

ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any drug candidates other than rivipansel for which we may obtain regulatory approval;

maintain, expand and protect our intellectual property portfolio;

hire additional clinical, quality control and scientific personnel; and

add operational, financial and management information systems and personnel, including personnel to support our drug development and potential future commercialization efforts.

To fund further operations, we will need to raise capital in addition to the net proceeds we received from our IPO and additional milestone payments we may receive under our collaboration with Pfizer. We may obtain additional financing in the future through the issuance of our common stock, through other equity or debt financings or through collaborations or partnerships with other companies. We may not be able to raise additional capital on terms

acceptable to us, or at all, and any failure to raise capital as and when needed could compromise our ability to execute on our business plan. Although it is difficult to predict future liquidity requirements, we believe that our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations for at least the next 12 months. However, our ability to successfully transition to profitability will be dependent upon achieving a level of revenues adequate to support our cost structure. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

### **Our Collaboration with Pfizer**

In October 2011, we entered into the license agreement with Pfizer under which we granted Pfizer an exclusive worldwide license to develop and commercialize products containing rivipansel for all fields and uses. The license also covers specified back-up compounds along with modifications of and improvements to rivipansel that meet defined chemical properties. Pfizer is required to use commercially reasonable efforts, at its expense, to develop, obtain regulatory approval for and commercialize rivipansel for sickle cell disease in the United States. Under the terms of the agreement, we received a \$22.5 million upfront payment. We are also eligible to earn potential milestone payments of up to \$115.0 million upon the achievement of specified development milestones, including the dosing of the first patients in Phase 3 clinical trials for up to two indications and the first commercial sale of a licensed product in the United States and selected European countries for up to two indications, up to \$70.0 million upon the achievement of specified regulatory milestones, including the acceptance of our filings for regulatory approval by regulatory authorities in the United States and Europe for up to two indications, and up to \$135.0 million upon the achievement of specified levels of annual net sales of licensed products. We are also eligible to receive tiered royalties for each licensed product, with percentages ranging from the low double digits to the low teens, based on net sales worldwide, subject to reductions in specified circumstances.

The first potential milestone payment under the Pfizer agreement was \$35.0 million upon the initiation of dosing of the first patient in a Phase 3 trial of rivipansel by Pfizer. Under the collaboration, Pfizer made a \$15.0 million non-refundable payment to us in May 2014, and the dosing of the first patient in the Phase 3 clinical trial will trigger the remaining \$20.0 million milestone payment to us.

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Activities necessary to support the initiation of a Phase 3 trial in the second half of 2014 are currently underway. In addition to Pfizer's agreement with the FDA under an SPA for the Phase 3 trial, the steps that Pfizer has taken and is taking to prepare for a Phase 3 trial include manufacturing of the drug substance to be used in the Phase 3 trial, completion of toxicology studies that would support a Phase 3 trial and an NDA, engagement with regulatory authorities overseas to discuss plans for the conduct of a Phase 3 trial, planning and preparation for a so-called TQTc clinical trial to evaluate cardiac safety that would support a Phase 3 trial, contracting with a CRO to provide services in the conduct of a Phase 3 trial and convening clinical investigators in the United States and overseas to discuss plans for a Phase 3 trial.

Although Pfizer has taken and is taking a number of steps to prepare for Phase 3 initiation in the second half of 2014, there can be no assurance that Pfizer will proceed on that schedule, or at all. There also can be no assurance that the conditions to its obligation to make the remaining \$20.0 million milestone payment to us under the agreement will be satisfied.

We have a research services agreement with the University of Basel, or the University, under which University personnel have performed research services for us on an as-requested basis since 2004. The agreement includes one-year research terms, and we have no affirmative obligation to purchase any minimum amount of services in any year beyond what we commit to at the beginning of each term, if any. For each of the research terms ended in February 2013 and 2014, we paid the University approximately \$150,000. As part of the original consideration for entering into this agreement, we granted to the University the right to receive payments from us under specified circumstances. If we receive any future milestone payments or royalties from Pfizer with respect to rivipansel, we have agreed to pay 10% of those amounts to the University. As of June 30, 2014, we have recorded an accrued expense of \$1.5 million to the University, which is equal to 10% of the \$15.0 million non-refundable milestone payment we received from Pfizer in May 2014. Amounts paid to the University are recorded as research and development expense on our statements of operations.

## **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of revenue and expenses during the reporting periods. In accordance with GAAP, we base our estimates on historical experience and on various other assumptions that we believe are reasonable under the circumstances at the time such estimates are made. Actual results may differ materially from our estimates and judgments under different assumptions or conditions. We periodically review our estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates are reflected in our financial statements prospectively from the date of the change in estimate.

We define our critical accounting policies as those accounting principles generally accepted in the United States that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 2 to our financial statements appearing elsewhere in this Quarterly Report, we believe the following are the critical accounting policies used in the preparation of our financial statements that require significant estimates and judgments.

## **Revenue Recognition**

*Research Grant Contracts*

From time to time, we are awarded reimbursement contracts for services and development grant contracts with government and non-government entities and philanthropic organizations. Under these contracts, we are typically reimbursed for our costs in connection with specific research or development activities. We recognize revenue as and to the extent we incur costs in connection with performance under these arrangements.

*License and Collaboration Agreements*

We have entered into a license agreement with Pfizer. Under the agreement, Pfizer made a nonrefundable \$22.5 million upfront payment to us in 2011 and a \$15.0 million milestone payment in 2014. In addition, Pfizer may become obligated to make potential milestone payments to us upon the achievement of significant clinical development milestones, regulatory approvals and sales-based events. The agreement also contemplates royalty payments to us on any future net sales of rivipansel worldwide.

Collaborative research and development agreements can provide for one or more of upfront license fees, research payments and milestone payments. Agreements with multiple components, such as deliverables or similar items, are referred to as multi-element

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revenue arrangements and are evaluated according to the provisions of Accounting Standards Codification, or ASC, Topic 605-25, *Revenue Recognition Multiple-Element Arrangements*, which we adopted effective as of January 1, 2011, to determine whether the deliverables can be separated into more than one unit of accounting. An item can generally be considered to be a separate unit of accounting if both of the following criteria are met:

the delivered item(s) has value to our customer on a standalone basis; and

the arrangement includes a general right of return relative to the delivered item(s), and delivery or performance of the undelivered item(s) is considered probable and substantially in our control.

Items that cannot be divided into separate units are combined with other units of accounting, as appropriate. Consideration received is then allocated among the separate units based on a selling price hierarchy. The selling price hierarchy for each deliverable is based on vendor-specific objective evidence, or VSOE, if it is available; third-party evidence of selling price, or TPE, if VSOE is not available; or an estimated selling price, if neither VSOE nor TPE is available.

Our license agreement with Pfizer represents a multiple-element revenue arrangement. To account for this transaction, we determined the elements, or deliverables, included in the arrangement and allocated arrangement consideration to the various elements based on each element's relative selling price. The identification of individual elements in a multiple-element arrangement and the estimation of the selling price of each element involve significant judgment, including consideration as to whether each delivered element has standalone value to our collaborator.

The primary deliverable under our license arrangement with Pfizer is an exclusive worldwide license to rivipansel, which is currently being developed to treat people experiencing VOC. The arrangement also includes deliverables related to research and preclinical development activities to be performed by us on Pfizer's behalf and our participation on a joint steering committee. We concluded that these deliverables should be accounted for as a single unit of accounting, and we therefore determined to recognize the upfront payment of \$22.5 million as revenue over the expected development period of 1.5 years, which was the period over which we expected to provide our research and development services and participate on the joint steering committee under the arrangement. Our determination of the appropriate length of the period over which to recognize revenue was consistent with the research plan agreed to with Pfizer.

In reaching this conclusion, we evaluated whether the license to rivipansel has standalone value to Pfizer. Factors we considered in determining whether the license has standalone value included whether or not Pfizer can use the license for its intended purpose without the receipt of the remaining deliverables, the value of the license without the undelivered items, Pfizer's or other vendors' ability to provide the undelivered items, the proprietary nature of the license and know-how, and the availability of our glycomimetics expertise in the general marketplace. Based on all relevant facts and circumstances and, most significantly, on the proprietary nature of our platform and the related proprietary nature of our research services, we concluded that standalone value does not exist for the license and, therefore, the license is not a separate unit of accounting under the collaboration and should be combined with the research and development services we are obligated to provide, including our participation on the joint steering committee.

We also evaluated whether our participation on the joint steering committee is a substantive obligation and therefore a separate unit of accounting. The joint steering committee is responsible for overseeing the general working relationships, determining the protocols to be followed in the research and development performed and evaluating the

results from the continued development of the drug candidate. The factors we considered in determining if our participation on the joint steering committee is a substantive obligation included:

which party negotiated or requested the steering committee;

how frequently the steering committee meets;

whether or not there are any penalties or other recourse if we do not attend the steering committee meetings;

which party has decision-making authority on the steering committee; and

whether or not Pfizer has the requisite experience and expertise associated with the research and development of rivipansel.

We considered that we may terminate our participation on the joint steering committee at any point during the agreement. Further, the estimated selling price of our obligation was not material to the overall license agreement. Based on all relevant facts and circumstances, we concluded that our participation on the joint steering committee is not a substantive obligation and, therefore, is not a separate unit of accounting under the collaboration.

We were not able to establish VSOE or TPE for the separate unit deliverables under the arrangement with Pfizer, as we do not have a history of entering into such arrangements or selling the individual deliverables within such arrangements separately.

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Accordingly, we determined that the selling price for the deliverables under the Pfizer license agreement should be determined using the best estimate of selling price. The process of determining the best estimate of selling price involved significant judgment on our part and included consideration of multiple factors, including market conditions and company-specific factors, such as those factors contemplated in negotiating the agreement and internally developed models that included assumptions related to market opportunity, discounted cash flows, estimated development costs, probability of success and the time needed to commercialize a drug candidate pursuant to the license. In validating the best estimate of selling price, we considered whether changes in key assumptions used to determine the best estimate of selling price would have a significant effect on the allocation of the arrangement consideration between the multiple deliverables.

Our license agreement with Pfizer also includes contingent milestone payments related to specified development, regulatory and commercial milestones. We adopted ASC Topic 605-28, *Revenue Recognition Milestone Method*, effective as of January 1, 2011. Under this guidance, we may recognize revenue contingent upon the achievement of a substantive milestone in its entirety in the period the milestone is achieved. Milestones are considered substantive if all of the following conditions are met:

the milestone is nonrefundable;

achievement of the milestone was not reasonably assured at the inception of the arrangement;

substantive effort is involved to achieve the milestone;

the amount of the milestone appears reasonable in relation to the effort expended or the risk associated with achievement of the milestone; and

a reasonable amount of time passes between the upfront license payment and the first milestone payment, as well as between each subsequent milestone payment.

Our determination as to whether a payment meets these five conditions involves management's judgment. If any of these conditions are not met, the resulting payment would not be considered a substantive milestone and would instead be considered part of the consideration for the single unit of accounting. In addition, if we determine that one milestone is not substantive, it could prevent us from concluding that subsequent milestones are substantive and, as a result, any additional milestone payments could also be considered part of the consideration for the single unit of accounting and would be recognized as revenue as those performance obligations are performed under either the proportional performance method or the straight-line method.

We have evaluated whether each milestone under the Pfizer arrangement is substantive and at risk to both parties on the basis of the contingent nature of that milestone. This evaluation included an assessment of whether:

the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from our performance to



achieve the milestone;

the consideration relates solely to past performance; and

the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. Based on this evaluation, we concluded that the milestones under the Pfizer collaboration are substantive, due to the uncertainty of future clinical development success and the additional effort and time that is expected before the milestones could be achieved. Accordingly, each milestone will be recognized as revenue upon its achievement, assuming all other revenue recognition criteria are met. The \$15.0 million non-refundable milestone payment received in May 2014 was recorded as revenue upon receipt.

### ***Stock-Based Compensation***

We issue stock-based compensation awards to our employees and non-employee directors, including stock options. We measure stock-based compensation expense related to these awards based on the fair value of the award on the date of grant and recognize stock-based compensation expense, less estimated forfeitures, on a straight-line basis over the requisite service period of the awards, which generally equals the vesting period. We grant stock options with exercise prices equal to the estimated fair value of our common stock on the date of grant. We have selected the Black-Scholes-Merton option pricing model to determine the fair value of stock option awards, which requires the input of various assumptions that require management to apply judgment and make assumptions and estimates, including:

the expected life of the stock option award;

the expected volatility of the underlying common stock; and

the fair value of our common stock determined on the date of grant.

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The following table summarizes the assumptions we used for estimating the fair value of stock options granted for the periods indicated:

|  | <b>Six Months Ended</b> |             |
|--|-------------------------|-------------|
|  | <b>June 30,</b>         |             |
|  | <b>2014</b>             | <b>2013</b> |
| Risk-free interest rate                | 1.89%-2.16%             | 0.56%       |
| Expected life of option term           | 6.25 years              | 6.25 years  |
| Volatility                             | 83.82-91.91%            | 78.07%      |
| Estimated dividend yield               | 0%                      | 0%          |
| Weighted average grant date fair value | \$6.72                  | \$ 2.51     |

We have assumed no dividend yield because we do not expect to pay dividends in the future, which is consistent with our history of not paying dividends. The risk-free interest rate assumption is based on observed interest rates for constant maturity U.S. Treasury securities consistent with the expected life of our employee stock options. The expected life represents the period of time the stock options are expected to be outstanding and is based on the simplified method. Under the simplified method, the expected life of an option is presumed to be the midpoint between the vesting date and the end of the contractual term. We used the simplified method due to the lack of sufficient historical exercise data to provide a reasonable basis upon which to otherwise estimate the expected life of the stock options. Expected volatility is based on the historical volatilities of a peer group of comparable publicly traded companies with drug candidates in similar stages of development.

The amount of stock-based compensation expense recognized during a period is based on the value of the portion of the awards that are ultimately expected to vest. Our estimate of pre-vesting forfeitures, or forfeiture rate, is based on our analysis of historical behavior by stock option holders. The estimated forfeiture rate is applied to the total estimated fair value of the awards, as derived from the Black-Scholes-Merton model, to compute the stock-based compensation expense, net of pre-vesting forfeitures, to be recognized in our statements of operations. We estimate forfeitures for employee grants at the time of grant and revise the estimates, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

***Research and Development Expenses***

Research and development costs are charged to expense as incurred and include employee-related expenses, including salaries, benefits and travel, expenses incurred under agreements with CROs and investigative sites that conduct preclinical studies and clinical trials, as well as the cost of acquiring, developing and manufacturing clinical trial materials, facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies and costs associated with preclinical activities and regulatory operations.

We record costs for some development activities, such as clinical trials, based on our evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to us by our vendors on their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense, as the case may be.

***Income Taxes***

As part of the process of preparing our financial statements we are required to estimate our income taxes in each of the jurisdictions in which we operate. Our provision for income taxes is based upon the estimated annual effective tax rates. We recorded an income tax expense of \$76,758 and \$0 for the six months ended June 30, 2014 and 2013, respectively. We account for income taxes by the asset and liability method. Under this method, deferred income taxes are recognized for tax consequences in future years of differences between the tax bases of assets and liabilities and their financial reporting amounts at each year-end, based on enacted laws and statutory tax rates applicable to the periods in which the differences are expected to be recovered or settled. Valuation allowances are provided if, based upon the weight of available evidence, it is more likely than not that some of all of the deferred tax assets will not be realized. In consideration of historically generating net operating losses, as well as projected future taxable income and the availability of tax planning strategies, we have provided a valuation allowance for the full amount of our net deferred tax assets since the likelihood of realization of any future benefit from deductible temporary differences and net operating loss carry forwards cannot be determined at June 30, 2014 and December 31, 2013.

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### **Components of Operating Results**

#### ***Revenue***

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of drugs in the near future. Substantially all of our revenue recognized to date has consisted of the upfront and milestone payments under our agreement with Pfizer.

Since our inception, we have also recognized a nominal amount of revenue under research grant contracts, generally to the extent of our costs incurred in connection with specific research or development activities.

#### ***Research and Development***

Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for full-time research and development employees, facilities expenses, overhead expenses, cost of laboratory supplies, clinical trial and related clinical manufacturing expenses, fees paid to CROs and other consultants and other outside expenses. Other preclinical research and platform programs include activities related to exploratory efforts, target validation, lead optimization for our earlier programs and our proprietary glycomimetics platform.

To date, our research and development expenses have related primarily to the development of rivipansel and our other drug candidates. However, as of April 2013, when we completed our Phase 2 clinical trial of rivipansel, all further clinical development obligations associated with rivipansel have shifted to Pfizer. As of the date of this report, the majority of our current research and development expenses are associated with the manufacturing of GMI-1271 and the clinical costs associated with the Phase 1 trial we initiated in June 2014.

We do not currently utilize a formal time allocation system to capture expenses on a project-by-project basis because we are organized and record expense by functional department and our employees may allocate time to more than one development project. Accordingly, we only allocate a portion of our research and development expenses by functional area and by drug candidate, as shown below under Results of Operations Research and Development Expense.

Research and development costs are expensed as incurred. Non-refundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

Research and development activities are central to our business model. Drug candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later stage clinical trials. We expect our research and development expenses to increase over the next several years as we seek to progress GMI-1271 and our other drug candidates through clinical development. However, it is difficult to determine with certainty the duration and completion costs of our current or future preclinical studies and clinical trials of our drug candidates, or if, when or to what extent we will generate revenues from the commercialization and sale of any of our drug candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our drug candidates.

The duration, costs and timing of clinical trials and development of our drug candidates will depend on a variety of factors that include, but are not limited to:

per patient trial costs;

the number of patients that participate in the trials;

the number of sites included in the trials;

the countries in which the trial is conducted;

the length of time required to enroll eligible patients;

the number of doses that patients receive;

the drop-out or discontinuation rates of patients;

potential additional safety monitoring or other studies requested by regulatory agencies;

the duration of patient follow-up; and

the safety and efficacy profile of the drug candidate.

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In addition, the probability of success for each drug candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each drug candidate, as well as an assessment of each drug candidate's commercial potential.

**General and Administrative**

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities, potential commercialization of our drug candidates and the increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we have experienced, and expect to continue to experience, increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with NASDAQ listing rules and SEC requirements, insurance and investor relations costs. In addition, if any of our other drug candidates other than rivipansel obtains regulatory approval, we expect to incur expenses associated with building a sales and marketing team. However, we do not expect to receive any such regulatory approval for at least the next several years.

**Other Income**

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

**Results of Operations**

The following tables set forth our results of operations before income taxes for the three and six months ended June 30, 2014 and 2013:

| (in thousands)                    | THREE MONTHS ENDED<br>JUNE 30, |            | PERIOD-<br>TO<br>PERIOD<br>CHANGE |
|-----------------------------------|--------------------------------|------------|-----------------------------------|
|                                   | 2014                           | 2013       |                                   |
| Revenue                           | \$ 15,027                      | \$ 55      | \$ 14,972                         |
| Costs and expenses:               |                                |            |                                   |
| Research and development          | 5,358                          | 2,884      | 2,474                             |
| General and administrative        | 1,605                          | 668        | 937                               |
| Total costs and expenses          | 6,963                          | 3,552      | 3,411                             |
| Income (loss) from operations     | 8,064                          | (3,497)    | 11,561                            |
| Other income                      | 4                              |            | 4                                 |
| Income (loss) before income taxes | \$ 8,068                       | \$ (3,497) | \$ 11,565                         |

| <b>(in thousands)</b>             | <b>SIX MONTHS ENDED<br/>JUNE 30,</b> |             | <b>PERIOD-<br/>TO<br/>PERIOD<br/>CHANGE</b> |
|-----------------------------------|--------------------------------------|-------------|---|
|                                   | <b>2014</b>                          | <b>2013</b> |   |
| Revenue                           | \$ 15,027                            | \$ 3,863    | \$ 11,164                                   |
| Costs and expenses:               |                                      |             |   |
| Research and development          | 9,239                                | 5,627       | 3,612                                       |
| General and administrative        | 2,830                                | 1,274       | 1,556                                       |
| Total costs and expenses          | 12,069                               | 6,901       | 5,168                                       |
| Income (loss) from operations     | 2,958                                | (3,038)     | 5,996                                       |
| Other income                      | 9                                    | 1           | 8   |
| Income (loss) before income taxes | \$ 2,967                             | \$ (3,037)  | \$ 6,004                                    |

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The increase in revenue for the three and six months ended June 30, 2014 compared to the same periods in 2013 is due to the receipt of a \$15.0 million non-refundable milestone payment from Pfizer in May 2014. The \$3.9 million in revenue for the six months ended June 30, 2013 consisted primarily of the remaining deferred revenue related to the upfront payment from Pfizer in 2011. The upfront payment was fully recognized as of March 31, 2013 consistent with the completion of our development obligations under the collaboration.

**Research and Development Expense**

The following tables summarize our research and development expenses by functional area for the three and six months ended June 30, 2014 and 2013:

| (in thousands)                | THREE MONTHS ENDED |          |
|-------------------------------|--------------------|----------|
|                               | JUNE 30,           |          |
|                               | 2014               | 2013     |
| Clinical development          | \$ 350             | \$ 446   |
| Personnel related             | 1,164              | 972      |
| Consulting fees               | 89                 | 55       |
| Manufacturing and formulation | 1,251              | 666      |
| Institutional research        | 98                 | 86       |
| Preclinical research          | 445                | 269      |
| Laboratory costs              | 333                | 340      |
| Stock-based compensation      | 128                | 50       |
| Milestone license fees        | 1,500              |          |
|                               | \$ 5,358           | \$ 2,884 |

During the three months ended June 30, 2014, our research and development expense increased by \$2.5 million, or 86%, compared to the same period in 2013 due to the \$1.5 million milestone license fee due to the University of Basel as a result of the non-refundable \$15.0 million milestone payment received from Pfizer in May 2014, an increase in spending on GMI-1271 related manufacturing and development and the initiation of the Phase 1 clinical trial for GMI-1271 in AML. The increase in expenses was offset by the decrease in costs associated with rivicansel once the Phase 2 clinical trial was completed in April 2013 and Pfizer assumed the further costs of development.

| (in thousands)                | SIX MONTHS ENDED |        |
|-------------------------------|------------------|--------|
|                               | JUNE 30,         |        |
|                               | 2014             | 2013   |
| Clinical development          | \$ 1,018         | \$ 961 |
| Personnel related             | 2,209            | 1,937  |
| Consulting fees               | 254              | 143    |
| Manufacturing and formulation | 2,328            | 1,193  |
| Institutional research        | 192              | 168    |
| Preclinical research          | 896              | 544    |



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|                          |          |          |
|--------------------------|----------|----------|
| Laboratory costs         | 622      | 583      |
| Stock-based compensation | 220      | 98       |
| Milestone license fees   | 1,500    |          |
|                          | \$ 9,239 | \$ 5,627 |

During the six months ended June 30, 2014 our research and development expense increased by \$3.6 million, or 64%, compared to the same period in 2013. The increase in research and development expense was primarily attributable to the \$1.5 million milestone license fee due to the University of Basel, an increase in expenses related to the manufacturing and process development and toxicology studies for GMI-1271 and the initiation of the Phase 1 clinical trial, in which we enrolled the first subject in June 2014. The increase in expenses was offset by the decrease in costs associated with rivipansel following the completion of the Phase 2 clinical trial in April 2013.

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The following tables summarize our research and development expenses by drug candidate for the three and six months ended June 30, 2014 and 2013:

| <b>(in thousands)</b>                          | <b>THREE MONTHS ENDED</b> |                 |
|--|---------------------------|-----------------|
|  | <b>JUNE 30,</b>           |                 |
|  | <b>2014</b>               | <b>2013</b>     |
| Rivipansel                                     | \$ 1,505                  | \$ 469          |
| GMI-1271                                       | 1,634                     | 734             |
| Other research and development                 | 927                       | 659             |
| Personnel related and stock-based compensation | 1,292                     | 1,022           |
|  | <b>\$ 5,358</b>           | <b>\$ 2,884</b> |

| <b>(in thousands)</b>                          | <b>SIX MONTHS ENDED</b> |                 |
|--|-------------------------|-----------------|
|  | <b>JUNE 30,</b>         |                 |
|  | <b>2014</b>             | <b>2013</b>     |
| Rivipansel                                     | \$ 1,534                | \$ 1,005        |
| GMI-1271                                       | 3,481                   | 1,424           |
| Other research and development                 | 1,799                   | 1,163           |
| Personnel related and stock-based compensation | 2,425                   | 2,035           |
|  | <b>\$ 9,239</b>         | <b>\$ 5,627</b> |

**General and Administrative Expense**

The following tables disclose the components of our general and administrative expenses for the three and six months ended June 30, 2014 and 2013:

| <b>(in thousands)</b>                             | <b>THREE MONTHS ENDED</b> |               |
|---|---------------------------|---------------|
|   | <b>JUNE 30,</b>           |               |
|   | <b>2014</b>               | <b>2013</b>   |
| Personnel related                                 | \$ 521                    | \$ 341        |
| Stock-based compensation                          | 291                       | 60            |
| Legal, consulting and other professional expenses | 698                       | 182           |
| Other   | 95                        | 85            |
|   | <b>\$ 1,605</b>           | <b>\$ 668</b> |

| <b>(in thousands)</b> | <b>SIX MONTHS ENDED</b> |             |
|-----------------------|-------------------------|-------------|
|                       | <b>JUNE 30,</b>         |             |
|                       | <b>2014</b>             | <b>2013</b> |
| Personnel related     | \$ 948                  | \$ 657      |

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|   |          |          |
|---|----------|----------|
| Stock-based compensation                          | 492      | 119      |
| Legal, consulting and other professional expenses | 1,183    | 349      |
| Other   | 207      | 149      |
|   | \$ 2,830 | \$ 1,274 |

General and administrative expense increased for the three and six months ended June 30, 2014 by \$0.9 million and \$1.6 million, respectively, compared to the same periods in 2013 primarily due to an increase in professional fees and other costs associated with our IPO and supporting public company operations. In addition, stock-based compensation increased for the three and six months ended June 30, 2014 compared to June 30, 2013 due to additional awards granted in 2014.

## Liquidity and Capital Resources

### *Sources of Liquidity*

Prior to our IPO in January 2014, we funded our operations primarily through private placements of our capital stock and upfront payments under our collaborative agreements. Through December 31, 2013, we raised an aggregate of \$86.6 million to fund our operations, of which \$22.5 million was the upfront payment under our license agreement with Pfizer and \$64.1 million was from

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the sale of convertible promissory notes and our convertible preferred stock. In January 2014, we received net proceeds of \$57.2 million from the IPO, and in May 2014, we received a non-refundable cash payment from Pfizer of \$15.0 million, of which we will pay \$1.5 million to the University of Basel under the terms of our research agreement with them. As of June 30, 2014, we had \$66.2 million in cash and cash equivalents.

We are potentially eligible to earn a significant amount of milestone payments and royalties under our agreement with Pfizer. Our ability to earn these payments and their timing is dependent upon the outcome of Pfizer's activities and is uncertain at this time.

### ***Initial Public Offering of Common Stock***

On January 15, 2014, we closed our IPO in which we sold an aggregate of 8,050,000 shares of common stock, including the full exercise of the underwriters' option to purchase additional shares, for net proceeds of \$57.2 million after deducting underwriting discounts and offering-related expenses. Upon closing of the IPO, all of our convertible preferred stock then outstanding automatically converted into 9,305,359 shares of common stock.

### ***Funding Requirements***

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses and general overhead costs.

The successful development of any of our drug candidates is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of GMI-1271 or our other drug candidates. We are also unable to predict when, if ever, material net cash inflows will commence from rivipansel or GMI-1271. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

successful enrollment in, and completion of, clinical trials;

receipt of marketing approvals from applicable regulatory authorities;

establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;

obtaining and maintaining patent and trade secret protection and regulatory exclusivity for drug candidates;  
and

launching commercial sales of drugs, if and when approved, whether alone or in collaboration with others. A change in the outcome of any of these variables with respect to the development of any of our drug candidates would significantly change the costs and timing associated with the development of that drug candidate. Because our drug candidates are in various stages of clinical and preclinical development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development and

commercialization of our drug candidates or whether, or when, we may achieve profitability. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity or debt financings and collaboration arrangements, including our existing collaboration with Pfizer. Except for Pfizer's obligation to make milestone payments under our agreement with them, we will not have any committed external source of liquidity.

To the extent that we raise additional capital through the future sale of equity or debt, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could contain covenants that would restrict our operations.

We may require additional capital beyond our currently anticipated amounts. Additional capital may not be available on reasonable terms, or at all. If we raise additional funds through collaboration arrangements in the future, we may have to relinquish valuable rights to our drug candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

**Table of Contents*****Outlook***

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect that our existing cash and cash equivalents as of June 30, 2014 will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months, without giving effect to any potential additional milestone payments we may receive under our license agreement with Pfizer. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. Additionally, the process of testing drug candidates in clinical trials is costly, and the timing of progress in these trials is uncertain.

***Cash Flows***

The following table represents a summary of our cash flows for the six months ended June 30, 2014 and 2013:

|   | <b>Six Months Ended<br/>June 30,</b> |             |
|---|--------------------------------------|-------------|
|   | <b>2014</b>                          | <b>2013</b> |
|   | <b>(In millions)</b>                 |             |
| Net cash provided by (used in) operating activities | \$ 6.6                               | \$ (6.6)    |
| Net cash used in investing activities               | (0.1)                                | (0.03)      |
| Net cash provided by financing activities           | 57.4                                 | 0.02        |

***Operating Activities***

Net cash provided by operating activities for the six months ended June 30, 2014 reflects the receipt of the \$15.0 million non-refundable milestone payment from Pfizer offset by the amounts used to conduct our preclinical studies and to support the manufacturing development of GMI-1271 in the first half of 2014. Net cash used in operating activities for the six months ended June 30, 2013 reflects, among other things, the amounts used to conduct our clinical trials for rivipansel as well as preclinical studies for GMI-1271.

***Investing Activities***

Net cash used in investing activities in both periods presented was primarily due to the acquisition of additional lab equipment needed to further our research and development activities.

***Financing Activities***

Net cash provided by financing activities of \$57.4 million during the six months ended June 30, 2014 reflects net proceeds received from our IPO of \$57.2 million with the remaining cash inflows provided from the exercise of employee stock options. Net cash provided by financing activities during the six months ended June 30, 2013 is comprised solely of employee stock option exercises.

***Contractual Obligations***

As of June 30, 2014, our only significant contractual obligations consisted of rent obligations under a non-cancelable lease for our office space in Gaithersburg, Maryland, with a term through October 2015. However, in July 2014, we entered into a lease agreement for our future corporate headquarters in Rockville, Maryland, which will have an 8-year term commencing on November 1, 2015.

As a result of entering into the new lease, we have updated the Contractual Obligations Table presented in our Annual Report on Form 10-K for the year ended December 31, 2013 as follows to reflect our significant contractual obligations as of June 30, 2014 after giving effect to the new lease:

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|                  | Payments Due by Period |                             |        |        |        |        | After<br>2018 |
|------------------|------------------------|-----------------------------|--------|--------|--------|--------|---------------|
|                  | Total                  | July to<br>December<br>2014 | 2015   | 2016   | 2017   | 2018   |               |
|                  | (In thousands)         |                             |        |        |        |        |               |
| Operating leases | \$ 5,971               | \$ 246                      | \$ 519 | \$ 610 | \$ 625 | \$ 641 | \$ 3,330      |

**Off-Balance Sheet Arrangements**

During the six months ended June 30, 2014, we did not have, and we do not currently have, any off-balance sheet arrangements, as defined under SEC rules.

**JOBS Act**

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107(b) of the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period, and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

**Item 3. Quantitative and Qualitative Disclosures about Market Risk**

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of June 30, 2014 and December 31, 2013, we had cash and cash equivalents of \$66.2 million and \$2.3 million, respectively. We generally hold our cash in interest-bearing money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents.

**Item 4. Controls and Procedures***(a) Evaluation of Disclosure Controls and Procedures*

The term disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act), refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Security and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information is accumulated and communicated to a company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and



procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2014, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of such date at the reasonable assurance level.

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*(b) Changes in Internal Controls Over Financial Reporting*

There have not been any changes in our internal controls over financial reporting during our fiscal quarter ended June 30, 2014 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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**PART II. OTHER INFORMATION**

**Item 1. Legal Proceedings**

From time to time, we are subject to litigation and claims arising in the ordinary course of business. We are not currently a party to any material legal proceedings and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results, cash flows or financial condition.

**Item 1A. Risk Factors**

*Our business is subject to numerous risks. You should carefully consider the following risks, as well as general economic and business risks, and all of the other information contained in this Quarterly Report on Form 10-Q, together with any other documents we file with the SEC. Any of the following risks could have a material adverse effect on our business, operating results and financial condition and cause the trading price of our common stock to decline.*

**Risks Related to Our Financial Position and Capital Needs**

*We have incurred significant losses since our inception. We expect to continue to incur losses over the next several years and may never achieve or maintain profitability.*

Since inception, we have incurred significant operating losses. As of June 30, 2014, we had an accumulated deficit of \$60.4 million. We have financed our operations to date with \$64.1 million raised in private placements of convertible debt and convertible preferred stock, an aggregate of \$37.5 million received from upfront and milestone payments under our license agreement with Pfizer and \$57.2 million from our IPO in January 2014. We have not generated any meaningful revenue since our inception other than from the upfront and milestone payments from Pfizer.

We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. We are still in the early stages of development of our drug candidates, and we have not completed development of any drugs. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. Although responsibility for further development, regulatory approval and potential commercialization of our lead drug candidate, rivipansel, has transferred to Pfizer under our collaboration with them following the recent completion of our Phase 2 clinical trial, we anticipate that our expenses will increase substantially as we:

commence and conduct clinical trials of GMI-1271;

continue the research and development of our other drug candidates;

seek to discover and develop additional drug candidates;

seek regulatory approvals for any drug candidates that successfully complete clinical trials;

ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any drugs other than rivipansel for which we may obtain regulatory approval;

maintain, expand and protect our intellectual property portfolio;

hire additional clinical, quality control and scientific personnel;

add operational, financial and management information systems and personnel, including personnel to support our drug development and planned future commercialization efforts; and

incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must succeed in developing and eventually commercializing drugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our drug candidates other than rivipansel, obtaining regulatory approval for these drug candidates and manufacturing and commercializing any drugs for which we may obtain regulatory approval, as well as discovering additional drug candidates. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenue that is significant enough to achieve profitability.

In the case of rivipansel, our ability to generate revenue is dependent upon the achievement of development, regulatory and commercial milestones and sales sufficient to generate royalties under our license agreement with Pfizer, and the achievement of such

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milestones is largely out of our control. If Pfizer fails, or chooses not to continue, to further develop, seek regulatory approval for or commercialize rivipansel, our ability to generate revenue with respect to rivipansel will be significantly reduced or eliminated. Because all of our drug candidates other than rivipansel are still in preclinical development, if we are unable to generate revenue from our license agreement with Pfizer, we may never become profitable, and we may not be able to invest in the further development of our other drug candidates.

Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of any of our drug candidates, our expenses could increase.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

***We will need substantial additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our drug development programs or potential commercialization efforts.***

We believe that our existing cash and cash equivalents as of June 30, 2014 will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months, without giving effect to any potential milestone payments we may receive under our agreement with Pfizer. However, we will need to obtain substantial additional funding in connection with our continuing operations. Our future capital requirements will depend on many factors, including:

our agreement with Pfizer remaining in effect and our ability to achieve milestones under this and any other license or collaboration agreement that we may enter into in the future, including the remaining \$20.0 million milestone payment upon dosing the first patient in a Phase 3 clinical trial;

the progress and results of the Phase 3 clinical trial of rivipansel that we expect Pfizer to commence in the second half of 2014, pending approval through Pfizer's governance process;

the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other drug candidates, including our planned Phase 1/2 clinical trials of GMI-1271;

the number and development requirements of other drug candidates that we may pursue;

the costs, timing and outcome of regulatory review of our drug candidates;

the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our drug candidates other than rivipansel for which we receive marketing approval;

any royalties we receive from Pfizer with respect to sales of rivipansel, if it receives marketing approval;

the revenue, if any, received from commercial sales of our drug candidates other than rivipansel for which we receive marketing approval;

the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims; and

the extent to which we acquire or in-license other drug candidates and technologies.

Identifying potential drug candidates and conducting preclinical testing and clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we and Pfizer or any future collaborators may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our drug candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will be derived from the sale of drugs that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

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***Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our drug candidates.***

Until such time, if ever, as we can generate substantial revenue from the sale of our drugs, we expect to finance our cash needs through a combination of equity offerings, debt financings and license and development agreements. We do not currently have any committed external source of funds other than possible milestone payments and possible royalties under our license agreement with Pfizer. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our research programs or drug candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements with third parties when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to third parties to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

***Our operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

We commenced operations in 2003, and our operations to date have been largely focused on raising capital, developing our expertise in carbohydrate chemistry and knowledge of carbohydrate biology, identifying potential drug candidates, undertaking preclinical studies and, with respect to rivipansel, conducting clinical trials. All but one of our drug candidates are still in preclinical development. We have not yet demonstrated our ability to successfully complete later stage clinical trials, obtain regulatory approvals, manufacture a commercial scale drug, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. With respect to our drug candidates other than rivipansel, we will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

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

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an ownership change, generally defined as a greater than 50% change by value in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss, or NOL, carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We may experience ownership changes in the future as a result of shifts in our stock ownership. As of December 31, 2013, we had federal and state NOL carryforwards of \$45.8 million, and research and development tax credit carryforwards of \$4.6 million, each of which could be limited if we

experience an ownership change.

**Risks Related to the Discovery and Development of Our Drug Candidates**

*Our research and development is focused on discovering and developing novel glycomimetic drugs, and we are taking an innovative approach to discovering and developing drugs, which may never lead to marketable drugs.*

A key element of our strategy is to use and expand our platform to build a pipeline of novel glycomimetic drug candidates and progress these drug candidates through clinical development for the treatment of a variety of diseases. The discovery of therapeutic drugs based on molecules that mimic the structure of carbohydrates is an emerging field, and the scientific discoveries that form the basis for our efforts to discover and develop drug candidates are relatively new. The scientific evidence to support the feasibility of developing drug candidates based on these discoveries is both preliminary and limited. Although our research and development efforts



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to date have resulted in a pipeline of glycomimetic drug candidates, we may not be able to develop drug candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential drug candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize drug candidates based upon our glycomimetics platform, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

***We are very early in our development efforts and have only one drug candidate, rivipansel, that has completed a clinical trial. All of our other drug candidates are still in preclinical or early clinical development. If we or our collaborators are unable to commercialize our drug candidates or experience significant delays in doing so, our business will be materially harmed.***

We are very early in our development efforts and have only one drug candidate, rivipansel, that has completed a clinical trial. All of our other drug candidates are still in preclinical or early clinical development. We have not completed the development of any drug candidates, we currently generate no revenue from the sale of any drugs and we may never be able to develop a marketable drug. We have invested substantially all of our efforts and financial resources in the development of our glycomimetics platform, the identification of potential drug candidates using that platform and the development of our drug candidates. Other than with respect to rivipansel, for which our collaborator Pfizer now has the responsibility for further development and commercialization, our ability to generate revenue from our other drug candidates, which we do not expect will occur for many years, if ever, will depend heavily on their successful development and eventual commercialization. The success of those drug candidates will depend on several factors, including:

successful completion of preclinical studies and clinical trials;

receipt of marketing approvals from applicable regulatory authorities;

obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our drug candidates;

making arrangements with third-party manufacturers for, or establishing, commercial manufacturing capabilities;

launching commercial sales of the drugs, if and when approved, whether alone or in collaboration with others;

acceptance of the drugs, if and when approved, by patients, the medical community and third-party payors;

effectively competing with other therapies;

obtaining and maintaining healthcare coverage and adequate reimbursement;

protecting our rights in our intellectual property portfolio; and

maintaining a continued acceptable safety profile of the drugs following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our drug candidates, which would materially harm our business.

***Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our drug candidates.***

All but one of our drug candidates are in preclinical or early clinical development, and their risk of failure is high. It is impossible to predict when or if any of our drug candidates will prove safe or effective in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we or a collaborator must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of the drug candidate in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of development. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs.

We or our current or future collaborators may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our or their ability to receive marketing approval or commercialize our drug candidates, including:

regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;

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we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;

clinical trials of our drug candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;

the number of patients required for clinical trials of our drug candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;

our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;

regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;

the cost of clinical trials of our drug candidates may be greater than we anticipate;

the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate; and

our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our drug candidates or other testing, if the results of these clinical trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

be delayed in obtaining marketing approval for our drug candidates;

not obtain marketing approval at all;

obtain approval for indications or patient populations that are not as broad as intended or desired;

obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;

be subject to additional post-marketing testing requirements; or

have the drug removed from the market after obtaining marketing approval.

Our drug development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring drugs to market before we do, and thereby impair our ability to successfully commercialize our drug candidates.

***If we or our collaborators experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.***

We or our collaborators may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. In particular, because rivipansel and GMI-1271 are intended to treat patients with sickle cell disease and AML, respectively, both of which represent a relatively low percentage of the population as compared to other diseases, our or our collaborators' ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. In addition, some of our competitors have ongoing clinical trials for drug candidates that treat the same or similar indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' drug candidates. Patient enrollment is also affected by other factors, including:

the severity of the disease or condition under investigation;

the eligibility criteria for the trial;

the perceived risks and benefits of the drug candidate;

the availability of drugs approved to treat the disease or condition under investigation;

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the efforts to facilitate timely enrollment in clinical trials;

the patient referral practices of physicians;

the ability to monitor patients adequately during and after treatment; and

the proximity and availability of clinical trial sites for prospective patients.

Our or our collaborators' inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us or them to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our drug candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

***If serious adverse or unacceptable side effects are identified during the development of our drug candidates, we may need to abandon or limit the development of some of our drug candidates.***

If our drug candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many drug candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented their further development.

***We may expend our limited resources to pursue a particular drug candidate or indication and fail to capitalize on drug candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and management resources, we focus on a limited number of research programs and drug candidates. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights.

## **Risks Related to Our Dependence on Third Parties**

***Our success is highly dependent on our existing collaboration with Pfizer, and future collaborations may also be important to us. If we are unable to maintain any of these collaborations, or if these collaborations are not successful, our business could be adversely affected.***

We have limited capabilities for drug development and do not yet have any capabilities for sales, marketing or distribution. Under our license agreement with Pfizer, Pfizer is responsible for all further development, regulatory approval and potential commercialization efforts with respect to rivipansel. All of our drug candidates other than rivipansel are still in preclinical or early stages of clinical development, and therefore our success is highly dependent on our collaboration with Pfizer. We cannot assure you that Pfizer will continue to develop rivipansel in a timely

manner, or at all, or, if it achieves regulatory approval, that Pfizer will successfully commercialize rivipansel.

Our Pfizer collaboration, and any future collaborations we might enter into, may pose a number of risks, including:

collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

collaborators may not perform their obligations as expected;

collaborators may not pursue the commercialization of any drug candidates that achieve regulatory approval or may elect not to pursue, continue or renew development or commercialization of drug candidates based on clinical trial results, changes in such collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;

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collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;

collaborators could independently develop, or develop with third parties, drugs that compete directly or indirectly with our drugs or drug candidates if such collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

drug candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own drug candidates or drugs, which may cause such collaborators to cease to devote resources to the commercialization of our drug candidates;

a collaborator with marketing and distribution rights to one or more of our drug candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such drug or drugs;

disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of drug candidates, might lead to additional responsibilities for us with respect to drug candidates or might result in litigation or arbitration, any of which would be time consuming and expensive;

collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;

collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable drug candidates.

If our collaboration with Pfizer or any other collaborations we might enter into in the future do not result in the successful development and commercialization of drugs, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration, including the remaining \$20.0 million payment for the next milestone under the Pfizer agreement. In addition, even if we are eligible to receive these payments, they could be substantially delayed. For example, under our license agreement, Pfizer has the option to commence another Phase 2 clinical trial of rivipansel, and such commencement would delay or inhibit our ability to receive some of the milestone payments we might otherwise have received under the agreement. If we do not receive the funding we expect under these agreements, the development of our drug candidates could be delayed and we may need additional resources to develop our drug candidates. All of the risks

relating to drug development, regulatory approval and commercialization described in this report also apply to the activities of our collaborators.

If Pfizer or a future collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate development or commercialization of any drug candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our reputation in the business and financial communities could be adversely affected. For our drug candidates other than rivipansel, we may in the future determine to collaborate with pharmaceutical and biotechnology companies for their development and potential commercialization. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of a collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a drug candidate, reduce or delay its development or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our drug candidates or bring them to market, which would impair our business prospects.

***We expect to rely on third parties to conduct our future clinical trials for drug candidates other than rivipansel, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.***

We currently expect to engage a third-party contract research organization, or CRO, to conduct our planned clinical trials for GMI-1271 and any of our other drug candidates that may progress to clinical development. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. Agreements with such third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that would delay our drug development activities.



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Our reliance on these third parties for research and development activities will reduce our control over these activities, but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our drug candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our drug candidates.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our drug candidates or commercialization of our drugs, producing additional losses and depriving us of potential revenue.

***We contract with third parties for the manufacturing of our drug candidates for preclinical and clinical testing and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs, or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.***

We do not have any manufacturing facilities or personnel. For our drug candidates other than rivipansel, for which manufacturing responsibility has shifted to Pfizer, we rely, and expect to continue to rely, on third parties for the manufacturing of our drug candidates for preclinical and clinical testing, as well as for commercial manufacture if any of our drug candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our ability to timely conduct our clinical trials or our other development or commercialization efforts.

We also expect to rely on third-party manufacturers or third-party collaborators for the manufacturing of commercial supply of any other drug candidates for which we or our collaborators obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

reliance on the third party for regulatory compliance and quality assurance;

the possible breach of the manufacturing agreement by the third party;

the possible misappropriation of our proprietary information, including our trade secrets and know-how; and

the possible termination or non-renewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practices, or 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 drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our drugs.

Our drug candidates and any drugs that we may develop may compete with other drug candidates and drugs for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. We are currently manufacturing GMI-1271 through a third party, but the drug supply to treat patients in our planned Phase 1/2 clinical trials is not yet available and there is no guarantee that it will become available in time for the anticipated start of that trial. If our current contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may incur added costs and delays in identifying and qualifying any such replacement.

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Our current and anticipated future dependence upon others for the manufacturing of our drug candidates or drugs may adversely affect our future profit margins and our ability to commercialize any drugs that receive marketing approval on a timely and competitive basis.

### **Risks Related to the Commercialization of Our Drug Candidates**

*Even if any of our drug candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.*

If any of our drug candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our drug candidates do not achieve an adequate level of acceptance, we may not generate significant revenue from drug sales and we may not become profitable. The degree of market acceptance of our drug candidates, if approved for commercial sale, will depend on a number of factors, including:

the efficacy and potential advantages compared to alternative treatments;

our ability to offer our drugs for sale at competitive prices;

the convenience and ease of administration compared to alternative treatments;

the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

the strength of marketing and distribution support;

the availability of third-party coverage and adequate reimbursement;

the prevalence and severity of any side effects; and

any restrictions on the use of our drugs together with other medications.

*If we are unable to establish sales, marketing and distribution capabilities for drug candidates other than rivipansel, we may not be successful in commercializing those drug candidates if and when they are approved.*

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical drugs. Under our collaboration with Pfizer, Pfizer is responsible for the commercialization of rivipansel, our lead drug candidate, if it receives regulatory approval. To achieve commercial success for any other drug candidate for which we may obtain marketing approval, we will need to establish a sales and marketing

organization to market or co-promote such drugs. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drugs on our own include:

our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;

the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;

the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more products; and

unforeseen costs and expenses associated with creating an independent sales and marketing organization. If we are unable to establish our own sales, marketing and distribution capabilities and enter into arrangements with third parties to perform these services, our revenue and our profitability, if any, are likely to be lower than if we were to sell, market and distribute any drugs that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our drug candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drugs effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug candidates.

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*We face substantial competition, which may result in others discovering, developing or commercializing drugs before or more successfully than we do.*

The development and commercialization of new drugs is highly competitive. We face competition with respect to our current drug candidates, and we will face competition with respect to any drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, academic institutions, governmental agencies and public and private research institutions. Should any competitors' drug candidates receive regulatory or marketing approval prior to ours, they may establish a strong market position and be difficult to displace or diminish the need for our drug candidates.

With respect to rivipansel, we are not aware of any therapies that have been approved for treatment of patients experiencing VOC. The only drug approved for the prevention of VOC, but not for the resolution of an ongoing VOC episode, is hydroxyurea, which is available in both generic and branded formulations. We are also aware of a company, Mast Therapeutics, Inc., that is developing a drug to treat an ongoing VOC episode. Mast is currently conducting a Phase 3 clinical trial in patients 4 to 65 years old experiencing VOC. If Mast's drug achieves regulatory approval before rivipansel, it could adversely affect commercialization of rivipansel if it is approved.

In addition to efforts to treat ongoing VOC episodes, we are aware of a number of companies developing therapies intended to prevent VOC from occurring in the first place. One company, Selexys Pharmaceuticals Corporation, is developing a therapy that, like our drug candidates, targets selectins. Selexys has announced that it has commenced enrollment in a Phase 2 clinical trial for its selectin antagonist drug candidate. Other companies are using different approaches to target a variety of biological mechanisms. We are also aware of efforts to develop cures for sickle cell disease through approaches such as bone marrow transplant and gene therapy. If any these approaches are successful and receive regulatory approval, it could limit the market for a drug such as rivipansel.

In addition, numerous non-profit and non-commercial foundations and interest groups also are committed to improving outcomes for patients with sickle cell disease. Advances in the understanding of the signaling pathways associated with sickle cell disease may lead to further interest and development of treatment options. There is also increasing interest among for-profit companies in developing drugs for rare diseases, which may have the effect of increasing the development of drug candidates to treat sickle cell disease generally or VOC in particular. Legislative action, such as the potential to expand the priority review voucher system to rare pediatric diseases, may generate further interest in developing drug candidates to treat sickle cell disease or VOC and increase competition. If an alternative effective treatment or cure for VOC or sickle cell disease receives regulatory approval, the potential commercial success of rivipansel could be jeopardized.

In addition, a competitor's drug candidate with an orphan drug designation may receive marketing approval prior to rivipansel. For example, Mast recently announced that it received orphan drug designation in Europe for its drug candidate intended to treat an ongoing VOC episode. If Mast receives orphan drug designation in the United States and marketing approval for its drug candidate prior to rivipansel receiving marketing approval, the commercial success of rivipansel could be significantly limited.

With respect to GMI-1271 and its development for treatment of AML and other hematologic cancers, there is substantial potential competition from other therapies currently in development. While some chemotherapies in development for AML could potentially be complementary to GMI-1271, there are also therapies in development that could be directly competitive with GMI-1271. For example, Mozobil, which is currently marketed by Sanofi, is being studied in combination with chemotherapy for the treatment of AML and myeloma. As the treatment landscape for AML changes, there is substantial risk that GMI-1271 might not provide additional benefit over other therapies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any drugs that we may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs 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, because we have no patents with respect to our glycomimetics platform, our competitors may use our methods, or acquire similar expertise, in order to develop glycomimetic drug candidates and progress these drug candidates through clinical development and commercialization, which could impair our ability to successfully commercialize our drug candidates or otherwise limit our commercial opportunities.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller

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and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These companies compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

*Even if we or our collaborators are able to commercialize any of our drug candidates, the drugs may become subject to unfavorable pricing regulations, third-party coverage and reimbursement policies or healthcare reform initiatives.*

Our and our collaborators' ability to commercialize any of our drug candidates successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these drugs and related treatments will be available from government payor programs at the federal and state levels authorities, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs. Coverage and reimbursement may not be available for any drug that we or our collaborators commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, any drug candidate for which we or our collaborators obtain marketing approval. Obtaining and maintaining adequate reimbursement for our drugs may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we or our collaborators may not be able to successfully commercialize any drug candidates for which marketing approval is obtained.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our or our collaborators' inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved drugs that we develop could adversely affect our operating results, our ability to raise capital needed to commercialize drugs and our overall financial condition.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drugs vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we or our collaborators might

obtain marketing approval for a drug in a particular country, but then be subject to price regulations that delay commercial launch of the drug, possibly for lengthy time periods, and negatively impact our ability to generate revenue from the sale of the drug in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more drug candidates, even if our drug candidates obtain marketing approval.

There can be no assurance that our drug candidates, if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication, that they will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our drug candidates profitably if they are approved for sale.

***Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any drugs that we may develop.***

We face an inherent risk of product liability exposure related to the testing of our drug candidates in human clinical trials, and will face an even greater risk if we commercially sell any drugs that we may develop. If we cannot successfully defend ourselves against claims that our drug candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

decreased demand for any drug candidates or drugs that we may develop;



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injury to our reputation and significant negative media attention;

withdrawal of clinical trial participants;

significant costs to defend the related litigation;

substantial monetary awards paid to trial participants or patients;

loss of revenue;

reduced resources of our management to pursue our business strategy; and

the inability to commercialize any drugs that we may develop.

We currently hold \$5.0 million of product liability insurance coverage in the aggregate, with a per incident limit of \$5.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our drug candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

**Risks Related to Our Intellectual Property**

***If we are unable to obtain and maintain patent protection for our drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize drug candidates similar or identical to ours, and our ability to successfully commercialize our drug candidates may be impaired.***

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our drug candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our drug candidates.

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 or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. 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, or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. 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. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. 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 that protect our drug candidates, in whole or in part, or which effectively prevent others from commercializing competitive drug candidates. 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.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The U.S. Patent and Trademark Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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Moreover, we may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, *inter partes* review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our drug candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

Even if our 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 patents by developing similar or alternative drug candidates in a non-infringing manner.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our 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 drug candidates, or limit the duration of the patent protection of our drug candidates. 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 patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our issued patents or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly.

***We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.***

A third party may hold intellectual property, including patent, rights that are important or necessary to the development of our drug candidates. It may be necessary for us to use patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially.

***Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.***

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. We may become party to, or

threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our drug candidates, including interference or derivation proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our drug candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing drug. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our drug candidates or force us to cease some of our business operations. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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***We may be subject to claims by third parties asserting that we or our employees 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. 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.

***Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.***

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

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

In addition to seeking patents for our drug candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. For example, our platform is based on trade secrets that consist largely of expertise in carbohydrate chemistry and knowledge of carbohydrate biology. We do not believe that we can obtain patent protection for our platform. Thus, our competitors may use our methods, or acquire similar expertise, in order to develop glycomimetic drug candidates and progress these drug candidates through clinical development and commercialization, which could impair our ability to successfully commercialize our drug candidates.

We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention

or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. 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 were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

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**Table of Contents****Risks Related to Regulatory Approval of Our Drug Candidates and Other Legal Compliance Matters**

*If we or our collaborators are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we or they will not be able to commercialize our drug candidates and our ability to generate revenue will be materially impaired.*

Our drug candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the European Medicines Agency, or EMA, and similar regulatory authorities outside the United States. Failure to obtain marketing approval for a drug candidate will prevent us or our collaborators from commercializing the drug candidate. We have not received approval to market any of our drug candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process for drug candidates other than rivipansel. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the drug candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, applicable regulatory authorities. Our drug candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our ability to obtain marketing approval or prevent or limit commercial use. 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 drug.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive and may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the drug candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations or changes in regulatory review for each submitted drug application may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application, or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a drug candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved drug not commercially viable.

For marketing exclusivity in the treatment of an ongoing VOC episode in sickle cell disease, we expect to rely primarily on the orphan drug designation that the FDA has granted us for rivipansel, which includes the treatment of the complications of sickle cell disease. However, in order to obtain marketing exclusivity, we must receive the first marketing approval for such indication. In addition, the orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If we experience delays in obtaining approval or if we fail to obtain approval of our drug candidates, the commercial prospects for our drug candidates may be harmed and our ability to generate revenue will be materially impaired.

*Even though we have obtained orphan drug designation for our most advanced drug candidate, rivipansel, we may not be able to obtain orphan drug marketing exclusivity for this drug candidate or any of our other drug candidates.*

Regulatory authorities in some jurisdictions, including the United States and the European Union, or EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. We have obtained orphan drug designation from the FDA and the EMA for rivipansel for the treatment of VOC, and we may seek orphan drug designation for our other drug candidates. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that time period. The applicable period is seven years in the United States and 10 years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a drug candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve another drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.



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***The FDA fast track designation for rivipansel may not actually lead to a faster development or regulatory review or approval process.***

If a drug is intended for the treatment of a serious or life-threatening disease or condition and the drug demonstrates the potential to address unmet medical needs for this disease or condition, the drug sponsor may apply for FDA fast track designation. If fast track designation is obtained, the FDA may initiate review of sections of a new drug application, or NDA, before the application is complete. This rolling review is available if the applicant provides, and the FDA approves, a schedule for submission of the individual sections of the application.

Although we have obtained a fast track designation from the FDA for rivipansel to treat VOC, we may not experience a faster development process, review or approval compared to conventional FDA procedures. Our fast track designation may be withdrawn by the FDA if it believes that the designation is no longer supported by data from our clinical development program. Our fast track designation does not guarantee that we will qualify for or be able to take advantage of the expedited review procedures or that we will ultimately obtain regulatory approval of rivipansel.

***Failure to obtain marketing approval in international jurisdictions would prevent our drug candidates from being marketed abroad.***

In order to market and sell our drugs in the EU and any other jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the drug be approved for reimbursement before it can be approved for sale in that country. We or our collaborators may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, failure to obtain approval in one jurisdiction may impact our ability to obtain approval elsewhere. We or our collaborators may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our drugs in any market.

***A variety of risks associated with marketing our drug candidates internationally could hurt our business.***

We or our collaborators may seek regulatory approval for rivipansel and our other drug candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

differing regulatory requirements in foreign countries;

the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market with low or lower prices rather than buying them locally;

unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;

economic weakness, including inflation or political instability in particular foreign economies and markets;

compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

foreign taxes, including withholding of payroll taxes;

foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations related to doing business in another country;

difficulties staffing and managing foreign operations;

workforce uncertainty in countries where labor unrest is more common than in the United States;

potential liability under the Foreign Corrupt Practices Act or comparable foreign regulations;

challenges enforcing our contractual and intellectual property rights, especially in foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;

production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and

business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our potential international operations may compromise our ability to achieve or maintain profitability.

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

Any drug candidate for which we obtain marketing approval, along with manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such drug candidate, 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 drug may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit its sales.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the drug. 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 do not market our drugs for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare 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 drugs, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

restrictions on such drugs, manufacturers or manufacturing processes;

restrictions on the labeling or marketing of a drug;

restrictions on product distribution or use;

requirements to conduct post-marketing studies or clinical trials;

warning letters;

recall or withdrawal of the drugs from the market;

refusal to approve pending applications or supplements to approved applications that we submit;

clinical holds;

fines, restitution or disgorgement of revenue or profit;

suspension or withdrawal of marketing approvals;

refusal to permit the import or export of our drugs;

product seizure; or

injunctions or the imposition of civil or criminal penalties.

Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of drugs for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the EU's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

***Our current and future relationships with customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.***

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which we sell, market and distribute any drugs for which we obtain marketing approval. In

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addition, we may be subject to transparency laws and patient privacy regulation by the U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include:

the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid;

federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for 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 their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the federal Open Payments program, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other transfers of value made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members, with data collection beginning on August 1, 2013, requirements for manufacturers to submit reports to CMS by March 31, 2014, and the 90th day of each subsequent calendar year, and disclosure of such information to be made by CMS on a publicly available website beginning by September 2014; and

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed

by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; 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.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business.

***Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our drug candidates and affect the prices we may obtain.***

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any drug candidates for which we obtain marketing approval.

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Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively PPACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of PPACA of importance to our potential drug candidates are:

an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;

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 non-compliance;

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 a manufacturer's outpatient drugs to be covered under Medicare Part D;

extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals 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 a manufacturer's Medicaid rebate liability;

expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

the new requirements under the federal Open Payments program and its implementing regulations;

a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. On March 1, 2013, the President signed an executive order implementing the 2% Medicare payment reductions, and on April 1, 2013, these reductions went into effect. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, 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. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and, accordingly, our financial operations.

We expect that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.



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***Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.***

In some countries, particularly in the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. To obtain coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available therapies. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

## **Risks Related to Employee Matters and Managing Our Growth**

***Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.***

We are highly dependent on the management, research and development, clinical, financial and business development expertise of Rachel King, our President and Chief Executive Officer; John Magnani, our Vice President of Research and Chief Scientific Officer; Helen Thackray, our Vice President of Clinical Development and Chief Medical Officer; and Brian Hahn, our Chief Financial Officer, as well as the other members of our scientific and clinical teams. In particular, we are dependent upon Dr. Magnani for key expertise in carbohydrate chemistry and knowledge of carbohydrate biology with respect to our glycomimetics platform, and the loss of his services would materially impair our future drug discovery efforts. Although we have entered into employment agreements with our executive officers, each of them may currently terminate their employment with us at any time. We do not maintain key person insurance for any of our executives or employees other than Ms. King.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our drug pipeline other than rivipansel toward scaling up for commercialization, sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval for and commercialize our drug candidates. Competition to hire qualified personnel in our industry is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of research, drug development, regulatory affairs and, if any of our drug candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and

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improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

***Our employees may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.***

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent improper activities may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

## **Risks Related to Ownership of Our Common Stock**

***An active trading market for our common stock may not continue to develop or be sustained.***

Prior to our January 2014 IPO, there was no public market for our common stock. Although our common stock is listed on The NASDAQ Global Market, we cannot assure you that an active trading market for our shares will continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for investors in our common stock to sell shares without depressing the market price for the shares or to sell the shares at all.

***The trading price of the shares of our common stock has been and is likely to continue to be volatile.***

Since our IPO, our stock price has been volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

announcements relating to development, regulatory approvals or commercialization of our drug candidates;

actual or anticipated variations in our operating results;

changes in financial estimates by us or by any securities analysts who might cover our stock;

conditions or trends in our industry;

changes in laws or other regulatory actions affecting us or our industry;

stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;

announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;

announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;

capital commitments;

investors' general perception of our company and our business;

disputes concerning our intellectual property or other proprietary rights;

recruitment or departure of key personnel; and

sales of our common stock, including sales by our directors and officers or specific stockholders.

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In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

***If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

***The issuance of additional stock in connection with financings, acquisitions, investments, our stock incentive plan, our employee stock purchase plan or otherwise will dilute all other stockholders.***

Our certificate of incorporation authorizes us to issue up to 100,000,000 shares of common stock and up to 5,000,000 shares of preferred stock with such rights and preferences as may be determined by our board of directors. Subject to compliance with applicable rules and regulations, we may issue our shares of common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition, investment, our stock incentive plan, our employee stock purchase plan or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

***A significant portion of our total outstanding shares are restricted from immediate resale, but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or if the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly. Upon the completion of our IPO, the 8,050,000 shares sold in the offering became freely tradable and the remaining outstanding shares of common stock are now available for sale in the public market following the July 2014 expiration of lock-up agreements between some of our stockholders and the underwriters.

In addition, we have filed a registration statement on Form S-8 registering the issuance of approximately 2.1 million shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under this registration statement on Form S-8 are available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and the restrictions of Rule 144 under the Securities Act in the case of our affiliates.

Additionally, some of the holders of our common stock who acquired their shares of our stock prior to the IPO, as well as some of the holders of outstanding warrants to purchase our common stock, or their transferees, have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include

their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

***Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.***

There are provisions in our certificate of incorporation and bylaws that may make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change in control was considered favorable by some or all of our stockholders. For example, our board of directors has the authority to issue up to 5,000,000 shares of preferred stock. The board of directors can fix the price, rights, preferences, privileges and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change in control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

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Our charter documents also contain other provisions that could have an anti-takeover effect, including:

only one of our three classes of directors is elected each year;

stockholders are not entitled to remove directors other than by a 66 2/3% vote and only for cause;

stockholders are not permitted to take actions by written consent;

stockholders cannot call a special meeting of stockholders; and

stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our certificate of incorporation also provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders.

***Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.***

Our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates beneficially own approximately 70% of our common stock. Further, funds controlled by one investor, New Enterprise Associates, or NEA, beneficially own approximately 48% of our common stock. As a result, NEA is able to control, and these other persons, acting together, are able to significantly influence, all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets or other significant corporate transactions. The interests of this group of stockholders may not coincide with our interests or the interests of other stockholders.

***We are an emerging growth company, and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.***

We are an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure;

not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;

not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;

reduced disclosure obligations regarding executive compensation; and

not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) December 31, 2019, (2) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.0 billion, (3) the last day of the fiscal year in which we are deemed to be a



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large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th and (4) any date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

***If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.***

We are subject to the reporting requirements of the Securities Exchange Act of 1934, the Sarbanes-Oxley Act and the rules and regulations of The NASDAQ Global Market. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Commencing with our current fiscal year, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This requires that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our initial public offering, we had never been required to test our internal controls within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

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

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

***We do not anticipate paying any cash dividends on our common stock in the foreseeable future and our stock may not appreciate in value.***

We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. There is no guarantee that shares of our common stock will appreciate in value or that the price at which our stockholders have purchased their shares will be able to be maintained.

***We will incur increased costs and demands upon management as a result of being a public company.***

As a newly public company listed in the United States, we have begun, and will continue, particularly after we cease to be an emerging growth company, to incur significant additional legal, accounting and other costs. These additional

costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and NASDAQ, may increase legal and financial compliance costs and make some activities more time consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies.

We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If we do not comply with new laws, regulations and standards, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

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

*(a) Sales of Unregistered Securities*

In April 2014, the holder of a warrant exercised its warrant to purchase 1,026 shares of common stock at an exercise of \$0.33 per share, resulting in proceeds to us of \$339. The issuance of these securities was exempt from registration under Section 4(a)(2) of the Securities Act.

*(b) Use of Proceeds from Public Offering of Common Stock*

On January 9, 2014, our Registration Statement on Form S-1, as amended (File No. 333-191567) was declared effective in connection with our initial public offering, pursuant to which we sold 8,050,000 shares of our common stock, including the full exercise of the underwriters' option to purchase additional shares, at a price to the public of \$8.00 per share. The offering closed on January 15, 2014, and, as a result, we received net proceeds of \$57.2 million (after underwriters' discounts and commissions of \$4.5 million and additional offering related costs of \$2.7 million). The joint managing underwriters of the offering were Jefferies LLC and Barclays Capital Inc.

No expenses incurred by us in connection with our initial public offering were paid directly or indirectly to (i) any of our officers or directors or their associates, (ii) any persons owning 10% or more of any class of our equity securities, or (iii) any of our affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board or board committee service.

There has been no material change in the planned use of proceeds from our initial public offering from that described in the final prospectus filed by us with the Securities and Exchange Commission on January 10, 2014.

**Table of Contents****Item 6. Exhibits**

| <b>Exhibit</b> |  |
|----------------|--|
| <b>No.</b>     | <b>Document</b>  |
| 3.1            | Amended and Restated Certificate of Incorporation of the Registrant (incorporated herein by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on January 15, 2014).                      |
| 3.2            | Amended and Restated Bylaws of the Registrant (incorporated herein by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on January 15, 2014).  |
| 4.1            | Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to Exhibit 4.2 to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 31, 2013). |
| 31.1*          | Certification of Principal Executive Officer under Section 302 of the Sarbanes-Oxley Act.  |
| 31.2*          | Certification of Principal Financial Officer under Section 302 of the Sarbanes-Oxley Act.  |
| 32.1**         | Certifications of Principal Executive Officer and Principal Financial Officer under Section 906 of the Sarbanes-Oxley Act.   |
| 101.INS***     | XBRL Instance Document   |
| 101.SCH***     | XBRL Taxonomy Extension Schema Document  |
| 101.CAL***     | XBRL Taxonomy Extension Calculation Linkbase Document  |
| 101.DEF***     | XBRL Taxonomy Extension Definition Linkbase Document   |
| 101.LAB***     | XBRL Taxonomy Extension Label Linkbase Document  |
| 101.PRE***     | XBRL Taxonomy Extension Presentation Linkbase Document   |

\* Filed herewith.

\*\* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

\*\*\* Pursuant to Rule 406T of Regulation S-T, the Interactive Data Files included in Exhibit 101 hereto are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and otherwise are not subject to liability under those sections.

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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.

GLYCOMIMETICS, INC.

Date: July 31, 2014

By: /s/ Brian M. Hahn  
Brian M. Hahn  
Chief Financial Officer  
*(On behalf of the Registrant and as Principal  
Financial Officer)*

**Table of Contents****Exhibit Index**

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