UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): October 20, 2015

KURA ONCOLOGY, INC.
(Exact name of Registrant as Specified in Its Charter)

Delaware	**000-53058**	**61-1547851**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

11119 N. Torrey Pines Road, Suite 125, La Jolla, CA	**92037**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (858) 500-8800

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 5.05 Amendments to the Registrant's Code of Ethics, or Waiver of a Provision of the Code of Ethics.

On October 20, 2015, the board of directors of Kura Oncology, Inc. (the "Company") adopted a Code of Business Conduct and Ethics, contingent upon and effective as of the closing of the Company's proposed public offering, that applies to all of the Company's directors, officers and employees. A copy of the Code of Business Conduct and Ethics is attached as Exhibit 14.1 to this report and incorporated herein by reference.

Item 8.01 Other Events.

The Company is filing certain information for the purpose of updating various aspects of the descriptions of the Company's business and risk factors contained in the Company's other filings with the Securities and Exchange Commission ("SEC"). A copy of this additional disclosure is attached as Exhibit 99.1 to this report and incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
14.1	Code of Business Conduct and Ethics.
99.1	Company disclosure.

Forward-Looking Statements

Certain statements contained in this report, other than statements of fact that are independently verifiable at the date hereof, may constitute forward-looking information and forward-looking statements (collectively "forward-looking statements" within the meaning of applicable securities laws). Such statements, based as they are on the current expectations of management of the Company and upon what management believes to be reasonable assumptions based on information currently available to it, inherently involve numerous risks and uncertainties, known and unknown, many of which are beyond the Company's control. Such statements can usually be identified by the use of words such as "may", "would", "believe", "intend", "plan", "anticipate", "estimate" and other similar terminology, or state that certain actions, events or results "may" or "would" be taken, occur or be achieved. Forward-looking statements in this report include, but are not limited to, statements about:

- the initiation, cost, timing, progress and results of our research and development activities, clinical trials and preclinical studies;
- the early stage of products under development;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product candidates, any product candidates that we may develop, and any related restrictions, limitations, and/or warnings in the label of any approved product candidates;
- our plans to research, develop and commercialize our future product candidates;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- our ability to successfully commercialize our product candidates;

- the size and growth of the markets for our product candidates and our ability to serve those markets;
- the rate and degree of market acceptance of any future products;
- the success of competing drugs that are or become available;
- government regulation;
- regulatory developments in the United States and other countries;
- the performance of our third-party suppliers and manufacturers and our ability to obtain alternative sources of raw materials;
- our ability to obtain additional financing;
- our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act;
- the accuracy of our estimates regarding expenses, future revenues, capital requirements and the need for additional financing; and
- our ability to attract and retain key management, scientific or clinical personnel.

Whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, assumptions and uncertainties, many of which are beyond our control, and the effects of which can be difficult to predict. These risks include those inherent in drug development, whether the Company will be able to obtain financing when needed or on favorable terms, and other risks described in the Company's filings with the SEC. In evaluating any forward-looking statements in this report, the Company cautions readers not to place undue reliance on any forward-looking statements. Unless otherwise required by applicable securities laws, the Company does not intend, nor does it undertake any obligation, to update or revise any forward-looking statements contained in this report to reflect subsequent information, events, results or circumstances or otherwise.

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 hereunto duly authorized.

Date: October 20, 2015

KURA ONCOLOGY, INC.

By: /s/ Annette North
Annette North
Senior Vice President and General Counsel

KURA ONCOLOGY, INC.

CODE OF BUSINESS CONDUCT AND ETHICS

INTRODUCTION

Kura Oncology, Inc. (the "***Company***") is committed to maintaining the highest standards of business conduct and ethics. This Code of Business Conduct and Ethics (this "***Code***") reflects the business practices and principles of behavior that support this commitment. We expect every employee, officer and director to read and understand this Code and its application to the performance of his or her business responsibilities. References in this Code to employees are intended to cover officers and, as applicable, directors.

Officers, managers and other supervisors are expected to develop in employees a sense of commitment to the spirit, as well as the letter, of this Code. Supervisors are also expected to ensure that all agents and contractors conform to Code standards when working for or on behalf of the Company. The compliance environment within each supervisor's assigned area of responsibility will be a factor in evaluating the quality of that individual's performance. In addition, any employee who makes an exemplary effort to implement and uphold our legal and ethical standards may be recognized for that effort in his or her performance review. Nothing in this Code alters the at-will employment policy of the Company.

This Code cannot possibly describe every practice or principle related to honest and ethical conduct. This Code addresses conduct that is particularly important to proper dealings with the people and entities with whom we interact, but reflects only a part of our commitment. From time to time we may adopt additional policies and procedures with which our employees, officers and directors are expected to comply, if applicable to them. However, it is the responsibility of each employee to apply common sense, together with his or her own highest personal ethical standards, in making business decisions where there is no stated guideline in this Code.

Action by members of your family, significant others or other persons who live in your household (referred to in this Code as "family members") also may potentially result in ethical issues to the extent that they involve the Company's business. For example, acceptance of inappropriate gifts by a family member from one of our suppliers could create a conflict of interest and result in a Code violation attributable to you. Consequently, in complying with this Code, you should consider not only your own conduct, but also that of your family members, significant others and other persons who live in your household.

You should not hesitate to ask questions about whether any conduct may violate this Code, voice concerns or clarify gray areas. Section 17 below details the compliance resources available to you. In addition, you should be alert to possible violations of this Code by others and report suspected violations, without fear of any form of retaliation, as further described in Section 17. Violations of this Code will not be tolerated. Any employee who violates the standards in this Code may be subject to disciplinary action, which, depending on the nature of the violation and the history of the employee, may range from a warning or reprimand up to and including termination of employment and, in appropriate cases, civil legal action or referral for regulatory or criminal prosecution.

1. HONEST AND ETHICAL CONDUCT

It is the policy of the Company to promote high standards of integrity by conducting our affairs in an honest and ethical manner. The integrity and reputation of the Company depends on the honesty, fairness and integrity brought to the job by each person associated with us. Unyielding personal integrity is the foundation of corporate integrity.

2. LEGAL COMPLIANCE

Obeying the law, both in letter and in spirit, is the foundation of this Code. Our success depends upon each employee operating within legal guidelines and cooperating with local, national and international authorities. We expect employees to understand the legal and regulatory requirements applicable to their business units and areas of responsibility. We hold or provide access to periodic training sessions or relevant education in order to ensure that all employees comply with the relevant laws, rules and regulations associated with their employment, including laws prohibiting insider trading (which are discussed in further detail in Section 3 below). While we do not expect you to memorize every detail of these laws, rules and regulations, we want you to be able to determine when to seek advice from others. If you do have a question in the area of legal compliance, it is important that you not hesitate to seek answers from your supervisor or the Compliance Officer (as described in Section 17).

Disregard of the law will not be tolerated. Violation of domestic or foreign laws, rules and regulations may subject an individual, as well as the Company, to civil and/or criminal penalties. You should be aware that conduct and records, including emails, are subject to internal and external audits, and to discovery by third parties in the event of a government investigation or civil litigation. It is in everyone’s best interests to know and comply with our legal and ethical obligations.

3. INSIDER TRADING

Employees who have access to confidential (or “inside”) information are not permitted to use or share that information for stock trading purposes or for any other purpose except to conduct our business. All non-public information about the Company or about companies with which we do business is considered confidential information. To use material non-public information in connection with buying or selling securities, including “tipping” others who might make an investment decision on the basis of this information, is not only unethical, it is illegal. Employees must exercise the utmost care when handling material inside information.

We have adopted a separate Insider Trading Policy with which you will be expected to comply as a condition of your employment with the Company. In addition, we have adopted a Window Period Policy that applies to our officers, directors and certain other employees. You should consult our Insider Trading Policy and, if applicable, our Window Period Policy, for more specific information on the definition of “inside” information and on buying and selling our securities or securities of companies with which we do business.

4. REGULATORY COMPLIANCE

The Company's business is subject to, or may in the future be subject to, a number of legal and regulatory requirements, including standards related to ethical procedures and proper scientific conduct. We expect employees to comply with all such requirements.

5. INTERNATIONAL BUSINESS LAWS

Our employees are expected to comply with the applicable laws in all countries to which they travel, in which they operate and where we otherwise do business, including laws prohibiting bribery, corruption or the conduct of business with specified individuals, companies or countries. The fact that in some countries certain laws are not enforced or that violation of those laws is not subject to public criticism will not be accepted as an excuse for noncompliance. In addition, we expect employees to comply with U.S. laws, rules and regulations governing the conduct of business by its citizens and corporations outside the U.S.

These U.S. laws, rules and regulations, which extend to all our activities outside the U.S., include:

- The Foreign Corrupt Practices Act, which prohibits directly or indirectly giving anything of value to a government official to obtain or retain business or favorable treatment, and requires the maintenance of accurate books of account, with all company transactions being properly recorded;
- U.S. Embargoes, which generally prohibit U.S. companies, their subsidiaries and their employees from doing business with, or traveling to, certain countries subject to sanctions imposed by the U.S. government (currently, Cuba, Iran, North Korea, Sudan and Syria), as well as specific companies and individuals identified on lists published by the U.S. Treasury Department;
- U.S. Export Controls, which restrict exports from the U.S. and re-exports from other countries of goods, software and technology to many countries, and prohibit transfers of U.S.-origin items to denied persons and entities; and
- Antiboycott Regulations, which prohibit U.S. companies from taking any action that has the effect of furthering or supporting a restrictive trade practice or boycott imposed by a foreign country against a country friendly to the U.S. or against any U.S. person.

If you have a question as to whether an activity is restricted or prohibited, seek assistance before taking any action, including giving any verbal assurances that might be regulated by international laws.

6. ANTITRUST

Antitrust laws are designed to protect the competitive process. These laws are based on the premise that the public interest is best served by vigorous competition and will suffer from illegal agreements or collusion among competitors. Antitrust laws generally prohibit:

- agreements, formal or informal, with competitors that harm competition or customers, including price fixing and allocations of customers, territories or contracts;
- agreements, formal or informal, that establish or fix the price at which a customer may resell a product; and
- the acquisition or maintenance of a monopoly or attempted monopoly through anti-competitive conduct.

Certain kinds of information, such as pricing, production and inventory, should not be exchanged with competitors, regardless of how innocent or casual the exchange may be and regardless of the setting, whether business or social.

Antitrust laws impose severe penalties for certain types of violations, including criminal penalties and potential fines and damages of millions of dollars, which may be tripled under certain circumstances. Understanding the requirements of antitrust and unfair competition laws of the various jurisdictions where we do business can be difficult, and you are urged to seek assistance from your supervisor or the Compliance Officer whenever you have a question relating to these laws.

7. ENVIRONMENTAL COMPLIANCE

Federal law imposes criminal liability on any person or company that contaminates the environment with any hazardous substance that could cause injury to the community or environment. Violation of environmental laws can involve monetary fines and imprisonment. We expect employees to comply with all applicable environmental laws.

It is our policy to conduct our business in an environmentally responsible way that minimizes environmental impacts. We are committed to minimizing and, if practicable, eliminating the use of any substance or material that may cause environmental damage, reducing waste generation and disposing of all waste through safe and responsible methods, minimizing environmental risks by employing safe technologies and operating procedures, and being prepared to respond appropriately to accidents and emergencies.

8. CONFLICTS OF INTEREST

We respect the rights of our employees to manage their personal affairs and investments and do not wish to impinge on their personal lives. At the same time, employees should avoid conflicts of interest that occur when their personal interests may interfere in any way with the performance of their duties or the best interests of the Company. A conflicting personal interest could result from an expectation of personal gain now or in the future or from a need to satisfy a

prior or concurrent personal obligation. We expect our employees to be free from influences that conflict with the best interests of the Company or might deprive the Company of their undivided loyalty in business dealings. Even the appearance of a conflict of interest where none actually exists can be damaging and should be avoided. Whether or not a conflict of interest exists or will exist can be unclear. Conflicts of interest are prohibited unless specifically authorized as described below.

If you have any questions about a potential conflict or if you become aware of an actual or potential conflict, and you are not an officer or director of the Company, you must discuss the matter with your supervisor or the Compliance Officer. Supervisors may not authorize conflict of interest matters or make determinations as to whether a problematic conflict of interest exists without first seeking the approval of the Compliance Officer and providing the Compliance Officer with a written description of the activity. If the supervisor is involved in the potential or actual conflict, you should discuss the matter directly with the Compliance Officer. Officers and directors must seek any authorizations and determinations from the Audit Committee (the "***Audit Committee***") of the Board of Directors of the Company (the "***Board***"), depending on the nature of the conflict of interest. Factors that may be considered in evaluating a potential conflict of interest are, among others:

- whether it may interfere with the employee's job performance, responsibilities or morale;
- whether the employee has access to confidential information;
- whether it may interfere with the job performance, responsibilities or morale of others within the organization;
- any potential adverse or beneficial impact on our business;
- any potential adverse or beneficial impact on our relationships with our customers or suppliers or other service providers;
- whether it would enhance or support a competitor's position;
- the extent to which it would result in financial or other benefit (direct or indirect) to the employee;
- the extent to which it would result in financial or other benefit (direct or indirect) to one of our customers, suppliers or other service providers; and
- the extent to which it would appear improper to an outside observer.

Although no list can include every possible situation in which a conflict of interest could arise, the following are examples of situations that may, depending on the facts and circumstances, involve problematic conflicts of interests:

- *Employment by (including consulting for) or service on the board of a competitor, customer or supplier or other service provider.* Activity that enhances or supports the position of a competitor to the detriment of the Company is prohibited, including employment by or service on the board of a competitor. Employment by or service on the board of a customer or supplier or other service provider is generally discouraged and you must seek authorization in advance if you plan to take such a position.
- *Owning, directly or indirectly, a significant financial interest in any entity that does business, seeks to do business or competes with us.* In addition to the factors described above, persons evaluating ownership in other entities for conflicts of interest will consider the size and nature of the investment; the nature of the relationship between the other entity and the Company; the employee's access to confidential information; and the employee's ability to influence the Company's decisions. If you would like to acquire a financial interest of that kind, you must seek approval in advance.
- *Soliciting or accepting gifts, favors, or any other benefit or benefits (including reputational), loans or preferential treatment from any person or entity that does business or seeks to do business with us.* See Section 12 for further discussion of the issues involved in this type of conflict.
- *Soliciting contributions for any charity or for any political candidate from any person or entity that does business or seeks to do business with us.*
- *Taking personal advantage of corporate opportunities.* See Section 9 for further discussion of the issues involved in this type of conflict.
- *Conducting our business transactions with your family member or a business in which you have a significant financial interest.* Related-person transactions covered by our Related-Person Transactions Policy must be reviewed in accordance with such policy and will be publicly disclosed to the extent required by applicable laws and regulations.
- *Exercising supervisory or other authority on behalf of the Company over a co-worker who is also a family member.* The employee's supervisor and/or the Compliance Officer will consult with our Human Resources department to assess the advisability of reassignment.

Loans to, or guarantees of obligations of, employees or their family members by the Company could constitute an improper personal benefit to the recipients of these loans or guarantees, depending on the facts and circumstances. Some loans are expressly prohibited by law, and applicable law requires that the Board approve all loans and guarantees to employees. As a result, all loans and guarantees by the Company must be approved in advance by the Board or the Audit Committee.

9. CORPORATE OPPORTUNITIES

You may not take personal advantage of opportunities for the Company that are presented to you or discovered by you as a result of your position with us or through your use of corporate property or information, unless authorized by your supervisor, the Compliance Officer or the Audit Committee, as described in Section 17. Even opportunities that are acquired privately by you may be questionable if they are related to our existing or proposed lines of business. Participation in an investment or outside business opportunity that is directly related to our lines of business must be pre-approved. You may not use your position with the Company or our corporate property or information for improper personal gain, nor should you compete with us in any way.

10. MAINTENANCE OF CORPORATE BOOKS, RECORDS, DOCUMENTS AND ACCOUNTS; FINANCIAL INTEGRITY; PUBLIC REPORTING

The integrity of our records and public disclosure depends upon the validity, accuracy and completeness of the information supporting the entries to our books of account. Therefore, our corporate and business records should be completed accurately and honestly. The making of false or misleading entries, whether they relate to financial results or test results, is strictly prohibited. Our records serve as a basis for managing our business and are important in meeting our obligations to customers, suppliers, creditors, employees and others with whom we do business. As a result, it is important that our books, records and accounts accurately and fairly reflect, in reasonable detail, our assets, liabilities, revenues, costs and expenses, as well as all transactions and changes in assets and liabilities. We require that:

- no entry be made in our books and records that intentionally hides or disguises the nature of any transaction or of any of our liabilities, or misclassifies any transactions as to accounts or accounting periods;
- transactions be supported by appropriate documentation;
- the terms of sales and other commercial transactions be reflected accurately in the documentation for those transactions and all such documentation be reflected accurately in our books and records;
- employees comply with our system of internal controls; and
- no cash or other assets be maintained for any purpose in any unrecorded or “off-the-books” fund.

Our accounting records are also relied upon to produce reports for our management, stockholders and creditors, as well as governmental agencies. In particular, we rely upon our accounting and other business and corporate records in preparing periodic and current reports that we file with the Securities and Exchange Commission (“***SEC***”). Securities laws require that these reports provide full, fair, accurate, timely and understandable disclosure and fairly present our financial condition and results of operations. Employees who collect, provide or analyze information for or otherwise contribute in any way in preparing or verifying these reports should

strive to ensure that our financial disclosure is accurate and transparent and that our reports contain all of the information about the Company that would be important to enable stockholders and potential investors to assess the soundness and risks of our business and finances and the quality and integrity of our accounting and disclosures. In addition:

- no employee may take or authorize any action that would intentionally cause our financial records or financial disclosure to fail to comply with generally accepted accounting principles, the rules and regulations of the SEC or other applicable laws, rules and regulations;
- all employees must cooperate fully with our Accounting Department, as well as our independent public accountants and counsel, respond to their questions with candor and provide them with complete and accurate information to help ensure that our books and records, as well as our reports filed with the SEC, are accurate and complete; and
- no employee should knowingly make (or cause or encourage any other person to make) any false or misleading statement in any of our reports filed with the SEC or knowingly omit (or cause or encourage any other person to omit) any information necessary to make the disclosure in any of our reports accurate in all material respects.

Any employee who becomes aware of any departure from these standards has a responsibility to report his or her knowledge promptly to a supervisor, the Compliance Officer, the Audit Committee, or one of the other compliance resources described in Section 17.

11. FAIR DEALING

We strive to outperform our competition fairly and honestly through superior performance and not through unethical or illegal business practices. Acquiring proprietary information from others through improper means, possessing trade secret information that was improperly obtained, or inducing improper disclosure of confidential information from past or present employees of other companies is prohibited, even if motivated by an intention to advance our interests. If information is obtained by mistake that may constitute a trade secret or other confidential information of another business, or if you have any questions about the legality of proposed information gathering, you must consult your supervisor or the Compliance Officer, as further described in Section 17.

You are expected to deal fairly with our suppliers, employees and anyone else with whom you have contact in the course of performing your job. Be aware that the Federal Trade Commission Act provides that “unfair methods of competition in commerce, and unfair or deceptive acts or practices in commerce, are declared unlawful.” It is a violation of the Federal Trade Commission Act to engage in deceptive, unfair or unethical practices, and to make misrepresentations in connection with sales activities.

Employees involved in procurement have a special responsibility to adhere to principles of fair competition in the purchase of products and services by selecting suppliers based exclusively on normal commercial considerations, such as quality, cost, availability, service and reputation, and not on the receipt of special favors.

12. GIFTS AND ENTERTAINMENT

Business gifts and entertainment are meant to create goodwill and sound working relationships and not to gain improper advantage with current or potential suppliers, vendors or partners or facilitate approvals from government officials. The exchange, as a normal business courtesy, of meals or entertainment (such as tickets to a game or the theatre or a round of golf) is a common and acceptable practice as long as it is not extravagant. Unless express permission is received from a supervisor, the Compliance Officer or the Audit Committee, gifts and entertainment cannot be offered, provided or accepted by any employee unless consistent with customary business practices and not excessive in value. This principle applies to our transactions everywhere in the world, even where the practice is widely considered "a way of doing business." Employees should not accept gifts or entertainment that may reasonably be deemed to affect their judgment or actions in the performance of their duties.

Under some statutes, such as the U.S. Foreign Corrupt Practices Act (further described in Section 5), giving anything of value to a government official to obtain or retain business or favorable treatment is a criminal act subject to prosecution and conviction. Discuss with your supervisor or the Compliance Officer any proposed entertainment or gifts if you are uncertain about their appropriateness.

13. PROTECTION AND PROPER USE OF COMPANY ASSETS

All employees are expected to protect our assets and ensure their efficient use. Theft, carelessness and waste have a direct impact on our financial condition and results of operations. Our property, such as office supplies, computer equipment, products, laboratory supplies, and office or laboratory space are expected to be used only for legitimate business purposes, although incidental personal use may be permitted. You may not, however, use our corporate name, any brand name or trademark owned or associated with the Company or any letterhead stationery for any personal purpose.

You may not, while acting on behalf of the Company or while using our computing or communications equipment or facilities, either:

- access the internal computer system (also known as "hacking") or other resource of another entity without express written authorization from the entity responsible for operating that resource; or
- commit any unlawful or illegal act, including harassment, libel, fraud, sending of unsolicited commercial email (also known as "spam") in violation of applicable law, trafficking in contraband of any kind, or espionage.

If you receive authorization to access another entity's internal computer system or other resource, you must make a permanent record of that authorization so that it may be retrieved for future reference, and you may not exceed the scope of that authorization.

Unsolicited commercial email is regulated by law in a number of jurisdictions. If you intend to send unsolicited commercial email to persons outside of the Company, either while acting on our behalf or using our computing or communications equipment or facilities, you should contact your supervisor or the Compliance Officer for approval.

All data residing on or transmitted through our computing and communications facilities, including email and word processing documents, is the property of the Company and subject to inspection, retention and review by the Company, with or without an employee's or third party's knowledge, consent or approval, in accordance with applicable law. Any misuse or suspected misuse of our assets must be immediately reported to your supervisor or the Compliance Officer.

14. CONFIDENTIALITY

One of our most important assets is our confidential information. As an employee of the Company, you may learn of information about the Company that is confidential and proprietary. You also may learn of information before that information is released to the general public. Employees who have received or have access to confidential information should take care to keep this information confidential. Confidential information includes non-public information that might be of use to competitors or harmful to the Company or its suppliers, vendors or partners if disclosed, such as business, marketing and service plans, financial information, product development, scientific data, manufacturing, laboratory results, designs, databases, customer lists, pricing strategies, personnel data, personally identifiable information pertaining to our employees, patients or other individuals (including, for example, names, addresses, telephone numbers and social security numbers), and similar types of information provided to us by our customers, suppliers and partners. This information may be protected by patent, trademark, copyright and trade secret laws.

In addition, because we interact with other companies and organizations, there may be times when you learn confidential information about other companies before that information has been made available to the public. You must treat this information in the same manner as you are required to treat our confidential and proprietary information. There may even be times when you must treat as confidential the fact that we have an interest in, or are involved with, another company.

You are expected to keep confidential information and proprietary information confidential unless and until that information is released to the public through approved channels (usually through a press release, an SEC filing or a formal communication from a member of senior management, as further described in Section 15). Every employee has a duty to refrain from disclosing to any person confidential or proprietary information about us or any other company learned in the course of employment here, until that information is disclosed to the public through approved channels. This policy requires you to refrain from discussing confidential or proprietary information with outsiders and even with other Company employees, unless those fellow employees have a legitimate need to know the information in order to perform their job duties. Unauthorized use or distribution of this information could also be illegal and result in civil liability and/or criminal penalties.

You should also take care not to inadvertently disclose confidential information. Materials that contain confidential information, such as memos, notebooks, computer disks and laptop computers, should be stored securely. Unauthorized posting or discussion of any information concerning our business, information or prospects on the Internet is prohibited, including on Internet forums, message boards, social media sites, “chat rooms” or blogs, regardless of whether you use your own name or a pseudonym. Be cautious when discussing sensitive information in public places like elevators, airports, restaurants and “quasi-public” areas within the Company, or in and around the Company’s facilities. All Company emails, voicemails and other communications are presumed confidential and should not be forwarded or otherwise disseminated outside of the Company, except where required for legitimate business purposes.

In addition to the above responsibilities, if you are handling information protected by any privacy policy published by us, then you must handle that information in accordance with the applicable policy.

15. MEDIA/PUBLIC DISCUSSIONS

It is our policy to disclose material information concerning the Company to the public only through specific limited channels to avoid inappropriate publicity and to ensure that all those with an interest in the Company will have equal access to information. All inquiries or calls from the press and financial analysts should be referred to our Chief Executive Officer or Chief Financial Officer. We have designated our Chief Executive Officer and Chief Financial Officer as our official spokespersons for questions concerning the financial performance, strategic direction or operating performance of the Company, and operational issues such as research and development, regulatory developments, sales and marketing, etc. Unless a specific exception has been made by our Chief Executive Officer or Chief Financial Officer, they are the only persons who may communicate with the press on behalf of the Company. You also may not provide any information to the media about us off the record, for background, confidentially or secretly, including, without limitation, by way of postings on internet websites, chat rooms or blogs.

16. WAIVERS

Any waiver of this Code for executive officers (including, where required by applicable laws, our principal executive officer, principal financial officer, principal accounting officer or controller (or persons performing similar functions)) or directors may be authorized only by our Board or, to the extent permitted by the rules of The Nasdaq Stock Market, a committee of the Board, and will be disclosed as required by applicable laws, rules and regulations.

17. COMPLIANCE STANDARDS AND PROCEDURES

Compliance Resources

To facilitate compliance with this Code, we have implemented a program of Code awareness, training and review that is part of our broader compliance programs overseen by our Audit Committee. We have established the position of Compliance Officer to oversee this

program. The Compliance Officer is a person to whom you can address any questions or concerns related to this Code or any other matters relating to legal or regulatory compliance. The Compliance Officer is our Senior Vice President, General Counsel, Annette North. In addition to fielding questions or concerns with respect to potential violations of this Code or any other matters relating to legal or regulatory compliance, the Compliance Officer is responsible for:

- investigating possible violations of this Code;
- training new employees in Code policies;
- conducting annual training sessions to refresh employees' familiarity with this Code;
- distributing copies of this Code annually via email to each employee with a reminder that each employee is responsible for reading, understanding and complying with this Code;
- updating this Code as needed and alerting employees to any updates, with appropriate approval of the Audit Committee, to reflect changes in the law, the Company's operations and in recognized best practices, and to reflect the Company's experience;
- overseeing the Company's compliance program and reporting to the Audit Committee material matters that may arise relating to the Company's legal and regulatory compliance efforts; and
- otherwise promoting an atmosphere of responsible and ethical conduct.

Your most immediate resource for any matter related to this Code is your supervisor. He or she may have the information you need, or may be able to refer the question to another appropriate source. There may, however, be times when you prefer not to go to your supervisor. In these instances, you should feel free to discuss your concern with the Compliance Officer. If you are uncomfortable speaking with the Compliance Officer because he or she works in your department or is one of your supervisors, please contact the Chief Executive Officer.

A toll-free compliance hotline and email address are also available to those who wish to ask questions about the Company's policy, seek guidance on specific situations, submit concerns regarding questionable accounting or auditing matters or report violations of this Code. The toll-free compliance hotline is [ ] and website for you to submit an anonymous email is [ ]. You may call the toll-free number or use the website if you prefer, although the Compliance Officer will be unable to obtain follow-up details from you that may be necessary to investigate the matter. Whether you identify yourself or remain anonymous, your contact with the toll-free compliance hotline or use of the compliance website will be kept strictly confidential to the extent reasonably possible within the objectives of this Code.

Clarifying Questions and Concerns; Reporting Possible Violations

If you encounter a situation or are considering a course of action and its appropriateness is unclear, discuss the matter promptly with your supervisor or the Compliance Officer; even the appearance of impropriety can be very damaging and should be avoided.

If you are aware of a suspected or actual violation of Code standards by others, you have a responsibility to report it. You are expected to promptly provide a compliance resource with a specific description of the violation that you believe has occurred, including any information you have about the persons involved and the time of the violation. Whether you choose to speak with your supervisor or the Compliance Officer, you should do so without fear of any form of retaliation. We will take prompt disciplinary action against any employee who retaliates against you, up to and including termination of employment.

Supervisors must promptly report any complaints or observations of Code violations to the Compliance Officer. If you believe your supervisor has not taken appropriate action, you should contact the Compliance Officer directly. The Compliance Officer will investigate all reported possible Code violations promptly and with the highest degree of confidentiality that is possible under the specific circumstances. Neither you nor your supervisor may conduct any preliminary investigation, unless authorized to do so by the Compliance Officer. Your cooperation in the investigation will be expected. As needed, the Compliance Officer will consult with our outside legal counsel and/or the Audit Committee. It is our policy to employ a fair process by which to determine violations of this Code.

With respect to any complaints or observations of Code violations, including, but not limited to, matters that may involve accounting, internal accounting controls and auditing concerns, the Compliance Officer shall promptly inform the chair of the Audit Committee, and the Audit Committee or such other persons as the Audit Committee determines to be appropriate under the circumstances shall be responsible for supervising and overseeing the inquiry and any investigation that is undertaken. In addition, any matters involving accounting, internal accounting controls and auditing concerns that are reported via the toll-free compliance hotline or compliance email address shall be routed to both the Compliance Officer and the Audit Committee.

If any investigation indicates that a violation of this Code has probably occurred, we will take such action as we believe to be appropriate under the circumstances. If we determine that an employee is responsible for a Code violation, he or she will be subject to disciplinary action up to, and including, termination of employment and, in appropriate cases, civil legal action or referral for regulatory or criminal prosecution. Appropriate action may also be taken to deter any future Code violations.

DESCRIPTION OF OUR BUSINESS

Overview

We are a clinical stage biopharmaceutical company discovering and developing personalized therapeutics for the treatment of solid tumors and blood cancers. We focus on the development of small molecule product candidates that target cell signaling pathways that are important to driving the progression of certain cancers. We aim to employ molecular diagnostics to identify patients with cancers who are likely to benefit from our targeted product candidates.

Advancements in cancer genetics and new molecular diagnostic tools are helping define why some patients respond to a particular therapy while other patients receive little to no clinical benefit. This new era in cancer drug discovery and development offers the potential for innovative treatments that are safer and more effective for patients with particular cancers. We aim to improve patient outcomes and contribute to the reduction in healthcare costs by matching targeted therapeutics to the patients who will benefit the most. We are developing drugs designed to inhibit the mutated or abnormally functioning cellular pathways that drive cancer growth and intend to pair them with molecular diagnostics to identify those patients with tumors most likely to respond to treatment.

We are developing our lead product candidate, tipifarnib, a farnesyl transferase inhibitor, in both solid tumors and blood cancers based on previously generated clinical data, preclinical data and our identification of potential molecular biomarkers. We in-licensed tipifarnib from Janssen Pharmaceutica NV, an affiliate of Johnson & Johnson, in December 2014. We initiated a Phase 2 clinical trial of tipifarnib in patients who have solid tumors with HRAS mutations in May 2015, and a Phase 2 clinical trial in patients with PTCL in September 2015. We plan to initiate a Phase 2 clinical trial in patients with lower risk MDS in the first half of 2016.

Our pipeline includes two preclinical programs. We are advancing KO-947, a small molecule inhibitor of ERK1/2 as a potential treatment for patients with tumors that have mutations in or other dysregulation of the MAPK signaling pathway, including pancreatic cancer, colorectal cancer, NSCLC and melanoma. We are also developing orally available, small molecule inhibitors of the menin-MLL interaction, which are currently in lead optimization as a potential treatment for patients with acute leukemias involving translocations or partial tandem duplications of the MLL gene.

Strategy

Our strategy is to acquire, develop, and commercialize innovative anti-cancer agents in oncology indications with significant unmet medical need. The critical components of our strategy include the following:

Focus on Oncology.

The oncology market is characterized by a number of disorders with high rates of disease recurrence and a limited response from current therapies or treatments. New oncology product candidates that address unmet medical needs or provide efficacy and safety profiles superior to those of standard of care have the potential for expedited development and regulatory review and, if approved, could be positioned to experience rapid adoption rates. We believe that the combination of molecularly-targeted cancer therapies and companion diagnostics to identify patients whose cancers are dependent on these targeted cell signaling pathways presents the potential for improved patient outcomes.

Focus on Compounds Where Improved Outcomes are Associated with Specific Biomarkers.

Our strategy is to prioritize those programs for which strong scientific and clinical hypotheses exist to link improved patient outcomes with specific biomarkers. Significant progress has been made in the identification of

molecular targets and pathways that more narrowly specify the causes of cancer and explain the variability in responses to different therapies by subsets of patients with a particular cancer or tumor type. We believe that the identification of such patient subsets and the correlation of their specific characteristics to the product candidate under development should increase the clinical benefit and the probability of success in our clinical trials. We believe such patient identification should also enable us to design smaller, more efficient clinical trials that, if successful, may achieve clinical outcomes for the targeted group that are more beneficial to the patients as well as more attractive to physicians and healthcare payors.

Leverage Companion Diagnostics to Realize Positive Clinical Outcomes.

Our development strategy is based on our belief that we can utilize effective companion diagnostics to identify patient subsets that will derive greater benefit from our product candidates. We intend to partner development of these companion diagnostics for use in clinical trials and, if successful, for commercialization of our product candidates. We have the ability to select from a number of diagnostic technology platforms and providers when choosing a partner for our programs under development.

Advance our Product Candidates in Clinical Proof-of-Concept Studies.

We initiated our first Phase 2 clinical trial of our lead product candidate, tipifarnib, in May 2015 in patients with solid tumors characterized by HRAS mutations, and our second Phase 2 clinical trial of tipifarnib in September 2015 in patients with PTCL. We plan to initiate a third Phase 2 clinical trial for tipifarnib in patients with lower risk MDS in the first half of 2016. We intend to maximize the likelihood of success in those trials by: (1) analyzing prior clinical data to identify one or more target patient populations that are more likely to respond to and benefit from tipifarnib and (2) evaluating biomarkers as potentially predictive of tipifarnib activity in new studies. We are also evaluating the potential for conducting additional company sponsored or investigator sponsored clinical trials of tipifarnib in certain patient subsets in other cancer indications. We intend to advance our ERK1/2 program and our menin-MLL program through to clinical development pending successful completion of research activities and preclinical studies.

Maintain Significant Development and Commercial Rights.

We believe it is important to maintain significant development and commercial rights to our product candidates. For many cancer indications, there are a relatively small number of oncologists practicing in each of the major pharmaceutical markets and an even smaller number of oncology key opinion leaders who significantly influence the types of drugs prescribed in cancer therapy. We believe that we can reach these oncology markets effectively with a relatively small sales and marketing organization focused on these physicians and oncology key opinion leaders. As a result, we plan to retain significant development and commercial rights to our products, which will enable us to retain the vast majority of the revenues from and commercial and economic value of our product candidates.

Build a Sustainable Product Pipeline

We have built our current pipeline of product candidates through in-licensing or acquisitions based on criteria driven by our corporate strategy. We intend to opportunistically evaluate product candidates that are complementary to our pipeline and have the potential to build value for the organization. Our decision to license or acquire additional product candidates will also be dependent on the scientific merits of the technology; costs of the transaction and other economic terms of the proposed license; the amount of capital required to develop the technology; and the economic potential of the drug candidate, should it be commercialized.

Cancer Background

Cancer is the second leading cause of death in the United States. The American Cancer Society, or ACS, estimated that, in 2015, there would be approximately 1.7 million new cases of cancer and approximately

589,000 deaths from cancer in the U.S. The World Health Organization estimated that 8.2 million people worldwide died of cancer in 2012. Despite advances in cancer diagnostics and treatment the unmet medical need remains high.

Despite significant disease variability, cancer in general originates from defects in the cell's genetic code, or DNA, which disrupt the mechanisms that normally prevent uncontrolled cell growth, proliferation, invasion and programmed cell death. Cancer cells that arise in organs or other tissues are referred to as solid tumors. Cancer cells that arise in the lymphatic system and bone marrow are referred to as hematological tumors. Increasingly, doctors are using diagnostic tests that identify genetic defects that may make a tumor more or less sensitive to a particular therapy in order to select better treatment options for patients with that disease. As genetic testing in cancer becomes a more routine practice, we are learning that many cancers arising in diverse sites in the body may share the same type of genetic alterations. For example, a mutation in a gene called BRAF is found in the majority of patients with metastatic melanoma, but it is also found in subsets of patients with colorectal cancer, lung cancer and other malignancies.

The most common methods of treating patients with cancer are surgery, radiation and drug therapy. A cancer patient often receives treatment with a combination of these methods. Surgery and radiation therapy are particularly effective when the disease is localized. Physicians generally use systemic drug therapies when the cancer has spread beyond the primary site or cannot otherwise be treated through surgery. The goal of drug therapy is to damage and kill cancer cells or to interfere with the molecular and cellular processes that control the development, growth and survival of cancer cells. In many cases, drug therapy entails the administration of several different drugs in combination. Over the past several decades, drug therapy has been evolving from non-specific drugs that kill both healthy and cancerous cells, such as cytotoxic therapies, to drugs that target specific molecular pathways or cellular processes involved in cancer and, more recently, to therapeutics that target specific activating alterations that are the "drivers" of cancer.

Advances in biology and understanding of cancer have led to the development of drugs, referred to as targeted therapeutics, which are designed to attack either a target that causes uncontrolled growth of cancer cells due to a specific genetic alteration primarily found in tumors but not in normal cells, or a target that cancer cells are more dependent on for their growth than normal cells. Targeted therapeutics are designed to preferentially kill cancer cells and spare normal cells and thus, in principle, they should exhibit enhanced efficacy and patients should experience fewer treatment-related side effects. Researchers and clinical oncologists now often incorporate genetic assessments into clinical trials and routine care with the hope of directing patients to medicines, which may have a greater chance of treating their cancers effectively. Furthermore, through the use of genetic testing, it is possible to develop drugs for defined subsets of patients, and to look for patients whose tumor types harbor genetically similar alterations. As such, doctors may begin to identify tumors and select therapies based on the type of mutations they share, rather than the part of the body from which they arise. Such a system should afford more efficient drug development, the opportunity for robust clinical responses and a better understanding of the underlying mechanisms of cancer.

Disease and Market Overview

We are focused on developing targeted therapeutics for the treatment of solid tumors and blood cancers. We are evaluating our lead product candidate, tipifarnib, a farnesyl transferase inhibitor, as a potential treatment for certain solid tumors, including thyroid cancer, head and neck cancers, urothelial carcinomas and salivary cancers, with HRAS mutations. Collectively, cancers that have an HRAS mutation are estimated to have an annual incidence of approximately 8,000 patients in the United States and, in general, patients with these cancers have poor prognosis and limited options for treatment. We commenced a Phase 2 clinical trial of tipifarnib in advanced solid tumors with the HRAS mutation in May 2015. We are also evaluating tipifarnib as a potential treatment for patients with PTCL. PTCL represents approximately 5-10% of non-Hodgkin's lymphomas, or NHL, which corresponds to an annual incidence of approximately 5,000 patients in the United States. Although several drugs have been approved by the FDA for treatment of relapsed or refractory PTCL, these drugs are

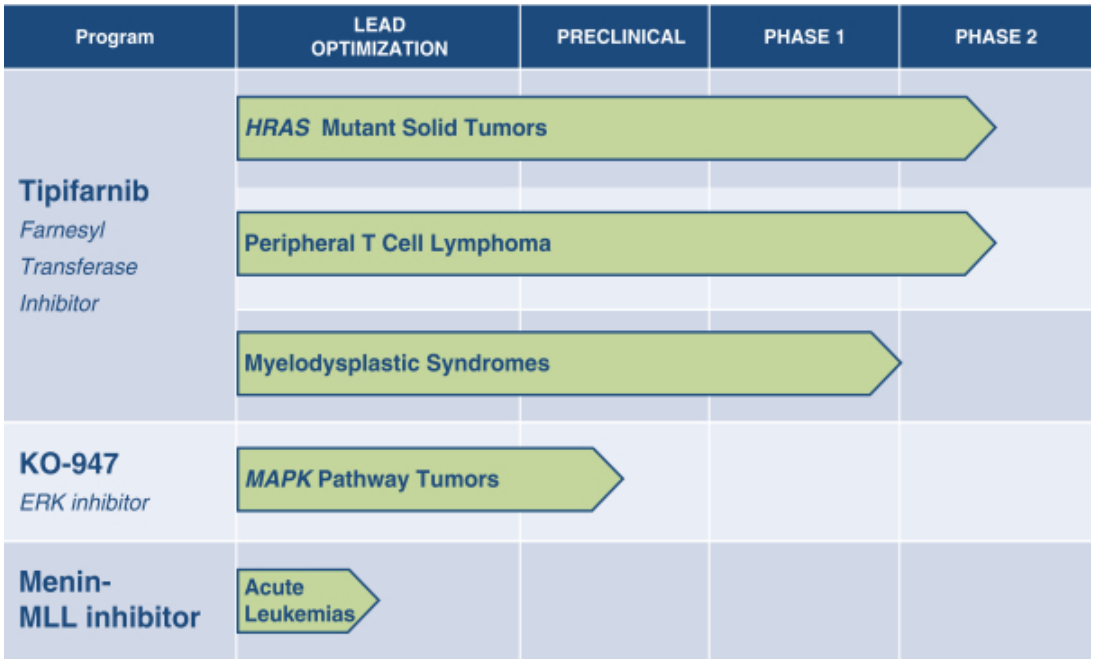
associated with relatively low objective response rates and relatively short durations of response. Accordingly, we believe the treatment of relapsed/refractory PTCL remains a significant unmet medical need. We commenced a Phase 2 clinical trial in PTCL in September 2015. Additionally, we are evaluating tipifarnib as a potential treatment for patients with MDS, which has an annual incidence of approximately 13,000 patients and an estimated prevalence of over 60,000 patients in the United States. Although the FDA has approved several drugs for treatment of select subsets of MDS patients, treatment options remain limited, and we believe a significant unmet need remains.

We are advancing KO-947, our development candidate that inhibits the activity of ERK1/2, as a potential treatment for patients with tumors that have mutations or other dysregulation in the MAPK pathway, including lung cancers, colorectal cancers, pancreatic cancers and melanoma. According to the ACS in 2015, there are estimated to be over 49,000 cases of pancreatic cancer, 133,000 cases of colorectal cancer and over 188,000 cases of non-small cell lung cancer, or NSCLC, diagnosed each year in the United States. We believe this corresponds to approximately 45,000 cases of KRAS mutant pancreatic cancer, 53,000 cases of KRAS mutant colorectal cancer, or CRC, and 23,000 cases of KRAS mutant NSCLC each year in the United States. According to the ACS, the annual incidence of melanoma patients is estimated at 74,000 patients in the United States, of which approximately 16% have metastatic disease. Approximately 40%-60% of melanoma patients have BRAF mutations and an additional 15-20% of those patients have NRAS mutations. As ERK inhibitors target the MAPK signaling pathway, which is activated with a BRAF mutation, they may also have the potential for activity not only in patients with BRAF-mutant metastatic melanoma but also in patients with tumors that harbor mutations in the NRAS gene, who currently have no adequate treatment option and poor prognosis.

We are also advancing a set of compounds that inhibit the interaction between the proteins menin and MLL for the treatment of MLL-rearranged, or MLL-r, and MLL-partial tandem duplications, or MLL-PTD, leukemias, two genetically-defined subsets of acute leukemias that affect both adults and children. The annual incidence of MLL-r and MLL-PTD patients is estimated to be 3,200 patients in the United States, and those patients currently have limited options other than chemotherapy.

Clinical Programs and Pipeline

The following table summarizes our current product pipeline:



Tipifarnib—An Oral Farnesyl Transferase Inhibitor

Overview

Tipifarnib is a new chemical entity we in-licensed in December 2014 from Janssen Pharmaceutica NV, an affiliate of Johnson & Johnson. Tipifarnib is a small molecule inhibitor of protein farnesylation, a key cell signaling process implicated in cancer initiation and development. Tipifarnib has been studied in more than 5,000 oncology patients and was generally well tolerated with a manageable side effect profile.

Although tipifarnib has demonstrated compelling and durable anti-cancer activity in certain patients and a well-established safety profile, its activity has not been sufficient in any prior clinical trial to support marketing approval by the FDA. An NDA was previously submitted to the FDA in January 2005 by a member of the Johnson & Johnson family of companies, for accelerated approval of tipifarnib for elderly patients with newly diagnosed, poor risk acute myeloid leukemia, or AML, who were not candidates for standard chemotherapy. At the FDA Oncology Drugs Advisory Committee meeting to review that NDA, the panel voted against accelerated and conventional approval and the FDA subsequently issued a non-approvable letter. However, clinical and preclinical data suggest that, in the right patient population, tipifarnib has the potential to provide significant benefit to cancer patients with limited treatment options.

Leveraging advances in next-generation sequencing, or NGS, as well as emerging information about cancer genetics, we will seek to identify patients most likely to benefit from tipifarnib. We initiated a Phase 2 clinical trial in patients who have tumors characterized by HRAS mutations in May 2015 and initiated a second Phase 2 clinical trial in patients with PTCL in September 2015. We also plan to initiate a Phase 2 clinical trial in patients with lower risk MDS in the first half of 2016. The preclinical studies and Phase 1–3 clinical trials in support of our IND for tipifarnib were conducted by affiliates of Johnson & Johnson and the National Cancer Institute. Efficacy and safety observations included in the IND are from 17 phase 1, 2 and 3 single-agent clinical trials

conducted prior to December 31, 2007. Regulatory sponsorship of the Janssen Pharmaceutica NV IND for tipifarnib has been transferred to us.

HRAS Mutant Tumors

Market Opportunity

RAS is a family of membrane-associated proteins that are involved in regulating cell division in response to growth factor stimulation. HRAS is a member of the RAS family, which includes two other proto-oncogenes: KRAS and NRAS. Collectively, the three RAS genes constitute one of the most frequently mutated families of oncogenes in human cancers. Although HRAS mutations are less common overall relative to KRAS and NRAS mutations, they have a relatively high prevalence in cancers of the upper digestive tract, skin, thyroid and urinary bladder. Collectively, cancers that have an HRAS mutation are estimated to have an annual incidence of approximately 8,000 patients in the United States.

HRAS as a Human Oncogene

The HRAS protein is involved in regulating cell division in response to growth factor stimulation. Growth factors act by binding cell surface receptors that span the cell's plasma membrane. Once activated, receptors stimulate signal transduction events in the cytoplasm, a process by which proteins and second messengers relay signals from outside the cell to the cell nucleus and instruct the cell to grow or divide. HRAS is localized in the plasma membrane, and is an early player in many signal transduction pathways. HRAS acts as a molecular on/off switch – once it is turned on it recruits and activates proteins necessary for the propagation of the receptor's signal. In certain tumors, mutations in HRAS or its upstream effectors cause it to be permanently on, resulting in persistent activation of downstream growth and proliferation signals that drive tumor cell growth. Farnesyl transferase inhibitors, or FTIs, work to prevent the aberrant growth and proliferation of cells that are dependent on these signaling pathways by inhibiting protein farnesylation and subsequent membrane localization of HRAS, thereby switching HRAS off.

FTIs such as tipifarnib prevent protein farnesylation, a type of protein modification known as prenylation, which along with other protein modifications, allows membrane localization of HRAS where it can receive and transmit extracellular signals implicated in cancer initiation and development. Tipifarnib has been shown to inhibit HRAS function. Specifically, by blocking HRAS farnesylation and subsequent membrane localization, tipifarnib inhibits oncogenic, HRAS-driven cellular transformation *in vitro* and *in vivo*. Earlier studies of FTIs were based on the hypothesis that FTIs would be generally active in RAS driven tumors. However, FTIs showed no significant antitumor activity in patients with advanced solid tumors such as lung, pancreatic and colon cancers, which mainly harbor KRAS mutations, and although the FTIs have demonstrated responses in certain patients with acute myeloid leukemia, the activity of the compound has not been shown to correlate with NRAS mutations. While KRAS and NRAS similarly utilize protein farnesylation, they can also utilize a related prenylation pathway that also leads to membrane localization and confers resistance to FTIs. We believe the refractory nature of RAS-driven tumors to treatment with FTIs has been attributed to this mechanism of resistance that is available to tumors with KRAS and NRAS mutations but not to those tumors with HRAS mutations. HRAS membrane localization is solely dependent on protein farnesylation, and therefore we believe that tipifarnib has the potential for the treatment of HRAS mutant solid tumors.

Clinical Significance of HRAS

The role of HRAS in patients with Costello syndrome, a rare genetic disorder, illustrates its potential as a human oncogene. At least five inherited mutations in the HRAS gene have been identified in people with Costello syndrome. Each of these mutations changes an amino acid in a critical region of the HRAS protein. The mutations associated with Costello syndrome lead to the production of an HRAS protein that is permanently active. Instead of triggering cell growth in response to particular signals from outside the cell, the overactive protein directs cells to grow and divide constantly. This uncontrolled cell division can result in the formation of noncancerous and cancerous tumors beginning in early childhood.

Transitional cell carcinoma of the bladder frequently occurs in adolescents with Costello syndrome, a presentation that is rare in the general population. Sporadic bladder tumors occurring in young patients without Costello syndrome also have a high frequency of HRAS mutation, but otherwise, lack extensive genetic alterations. Furthermore, HRAS mutations are present at all disease stages of bladder cancer and are detected in low-grade non-muscle invasive transitional tumors. These pieces of clinical evidence point to HRAS as a key protein involved in tumorigenesis in both Costello syndrome and, by extension, in the broader urothelial cancer population.

Clinical Development in HRAS Mutant Tumors

We initiated a Phase 2 clinical trial in May 2015 to test the hypothesis that tipifarnib can be used as a treatment for advanced tumors with a known HRAS mutation. We designed this trial based on preclinical data which demonstrated that tipifarnib inhibits HRAS mutant cell proliferation and HRAS tumor growth in mouse models. Sponsorship of the IND for tipifarnib previously filed by Janssen has been transferred to us. The trial is expected to enroll 2 cohorts of 18 patients each. Cohort 1 will enroll subjects with malignant thyroid tumors with HRAS mutations, independently of thyroid histology. Any subject with a non-hematological HRAS mutant tumor other than thyroid cancer who meets eligibility criteria may be enrolled in Cohort 2. This trial has a two-stage study design to minimize the number of study subjects treated if tipifarnib is not sufficiently efficacious. If one or no objective response is observed in a cohort after the first 11 evaluable patients, the cohort will be closed to further enrollment. If more than one response is observed in the cohort, 7 additional subjects will be enrolled (stage 2). The trial will be considered positive if at least 4 responses are observed in a cohort (out of 18 subjects). The primary endpoint is objective response rate, and tumor response assessments will be conducted according to the Response Evaluation Criteria in Solid Tumors version 1.1 criteria (confirmation of response is required). We anticipate receiving topline data from this trial in the second half of 2016.

Investigator Sponsored Trial in HRAS Mutant Urothelial Carcinoma

In addition to the company sponsored Phase 2 clinical trials, in the second half of 2015 we plan on initiating a Phase 2 investigator sponsored clinical trial of tipifarnib for the treatment of advanced, previously treated urothelial carcinomas that carry HRAS mutations. This clinical trial will be sponsored by the Samsung Medical Center, and designed to enroll at least 18 patients. The primary endpoint of this clinical trial will be objective response rate, and secondary endpoints include progression-free survival, duration of response, and safety.

Companion Diagnostics

Patients will be enrolled in the Phase 2 HRAS mutant tumor clinical trials based on information from the clinical sites on the patients' tumor HRAS mutation status. Most commonly this information will have been obtained by the clinical sites from the NGS panels used by the site to characterize patients' tumors. If the results of our Phase 2 clinical trials are positive, we plan to partner development and validation of a companion diagnostic test to aid in the selection of patients with HRAS mutant tumors in subsequent clinical trials of tipifarnib and to prepare and submit an investigational device exemption, or IDE, for use of the assay in the clinical trial. We expect that the companion diagnostic test will either be a qualitative PCR-based assay or an NGS-based assay. A qualitative PCR based assay would be technically very similar to the PCR-based assays already developed and approved by the FDA for KRAS. We expect that regulatory approval of tipifarnib as a treatment for patients with HRAS mutant tumors will require FDA approval of an HRAS assay in the form of a companion diagnostic test that has been validated for accuracy, precision and reproducibility.

Peripheral T-Cell Leukemia

Market Opportunity

We initiated a Phase 2 human clinical trial to evaluate tipifarnib as a treatment for patients with PTCL in September 2015.

Lymphoma is the most common blood cancer. The two main forms of lymphoma are Hodgkin lymphoma, or HL, and NHL. Lymphoma occurs when cells of the immune system called lymphocytes grow and multiply uncontrollably. Cancerous lymphocytes can travel to many parts of the body, including the lymph nodes, spleen, bone marrow, blood, or other organs, and form tumors. The body has two main types of lymphocytes that can develop into lymphomas: B-cells and T-cells.

PTCL consists of a group of rare and usually aggressive (fast-growing) NHLs that develop from mature T-cells. PTCLs collectively account for about 5 to 10 percent of all NHL cases, corresponding to an annual incidence of approximately 5,000 patients per year in the United States. By some estimates, the incidence of PTCL is growing significantly, and the increasing incidence may be driven by an aging population.

PTCLs are sub-classified into various subtypes, each of which are typically considered to be separate diseases based on their distinct clinical differences. Most of these subtypes are rare; the three most common subtypes are PTCL not otherwise specified, anaplastic large-cell lymphoma, or ALCL, and angioimmunoblastic T-cell lymphoma, that collectively account for approximately 70 percent of all PTCLs in the United States.

Treatment Options for PTCL

For most PTCL subtypes, the frontline treatment regimen is typically combination chemotherapy, such as CHOP (cyclophosphamide, doxorubicin, vincristine, prednisone), EPOCH (etoposide, vincristine, doxorubicin, cyclophosphamide, prednisone), or other multi-drug regimens.

Patients who relapse or are refractory to frontline treatments are typically treated with gemcitabine in combination with other chemotherapies, including vinorelbine (Navelbine®) and doxorubicin (Doxil®) in a regimen called GND, or other chemotherapy regimens such as DHAP (dexamethasone, cytarabine, cisplatin) or ESHAP (etoposide, methylprednisolone, cytarabine, and cisplatin).

Because most patients with PTCL will relapse, some oncologists recommend giving high-dose chemotherapy followed by an autologous stem cell transplant to some patients who had a good response to their initial chemotherapy. Recent, non-cytotoxic therapies that have been approved for relapsed or refractory PTCL, such as pralatrexate (Folotyn®), romidepsin (Istodax®) and belinostat (Beleodaq®), are associated with relatively low objective response rates (25-27% overall response rate, or ORR) and relatively short durations of response (8.2-9.4 months). Accordingly, we believe the treatment of relapsed/refractory PTCL remains a significant unmet medical need.

The five year survival for patients with PTCL is low—roughly 35% by most published records—and few treatment options are able to provide a durable treatment effect. Treatments in the relapsed or refractory setting are not very effective. Therefore, National Comprehensive Cancer Network guidelines currently recommend that patients seek participation in a clinical trial for the initial treatment.

Previous Phase 2 Experience with Tipifarnib in the Treatment of PTCL

A prior Phase 2 clinical trial of tipifarnib was sponsored by the National Cancer Institute and conducted at the Mayo Clinic and University of Iowa from 2004 to 2009 in adult patients with relapsed or refractory lymphoma. Ninety-three patients (42 aggressive, 15 indolent, and 36 HL/T-cell lymphoma) were enrolled in the study, and patients received tipifarnib 300 mg twice daily on days 1-21 of each 28-day cycle. The median age of patients was 62 years (range, 18-91 years). A total of 71% of patients had stage IV disease. The median number of prior regimens was five (range, 1-17). The majority of patients were diagnosed with diffuse large B-cell lymphoma, or DLBCL (40%; 37 of 93) or HL (20%; 19 of 93).

As shown in the table below, the ORR for all patients was 20.4%, with 7% complete responses, or CR, and 14% partial responses, or PR. In the groups of aggressive, indolent, and HL/T-cell types of lymphoma, the ORRs were 17%, 7%, and 31%, respectively.

In the 19 responders, the median response duration was 7.5 months with a mean of 15.8 months.

The highest ORR (31%) was demonstrated in the HL/T cell lymphoma group. Within that group, the ORR was 21% in patients with HL and 50% in the T-cell NHL indications of mycosis fungoides and peripheral T-cell lymphoma.

The median time to progression, or TTP, was 3.6 months for all patients and 3.2 months for the HL/T-cell lymphoma groups, respectively. Five patients in the HL/T-cell lymphoma group received treatment for more than 30 months with several patients receiving treatment for 60+ months.

The median overall survival, or OS, was 14.8 months for all patients and 6.4 months, 20.6 months, and 19.7 months for the aggressive, indolent, and HL/T-cell lymphoma groups, respectively.

Phase 2 Clinical Trial of tipifarnib in Adult Patients with Relapsed or Refractory Lymphoma.

Disease Type	n(%)	CR, n (%)	PR, n (%)	ORR, (%) (95% CI)	Median DR (95% CI)	Median TTP (95% CI)	Median OS (95% CI)
All patients	93	6 (7)	13 (14)	20 (13-30)	7.5 (4.9-18.5)	3.6 (2.1-4.5)	14.8 (7.6-17.8)
Aggressive B-cell lymphoma group	42	0	7 (17)	17 (7-31)	11.3 (4.9-17.1)	2.8 (1.7-4.2)	6.4 (4.1-10.7)
DLBCL	37 (88)	0	7 (19)	19	—	—	—
Mantle cell lymphoma	4 (10)	0	0	0	—	—	—
Folicular lymphoma, or FL, III	1 (2)	0	0	0	—	—	—
Indolent B-cell lymphoma group	15	0	1 (7)	7 (0.2-32)	2(NR)	5.2 (4-9.2)	20.6 (NR)
Chronic lymphocytic Leukemia/small lymphocytic lymphoma	5 (33)	0	0	0	—	—	—
Extranodal marginal zone	1 (7)	0	0	0	—	—	—
FL grade I	3 (20)	0	0	0	—	—	—
FL grade II	6 (40)	0	1	17	—	—	—
HL/T group	36	6 (17)	5 (14)	31 (16-48)	7.5 (3.2-29.8)	3.2 (1.9-5.8)	19.7 (9-60)
HL	19 (53)	2 (11)	2 (11)	21	—	—	—
Mycosis fungoides	4 (11)	0	2 (50)	50	—	—	—
Peripheral T-cell, unspecified	8 (22)	3 (38)	1 (13)	50	—	—	—
Anaplastic large cell, cutaneous	3 (8)	1 (33)	0	33	—	—	—
Anaplastic large cell, systemic	2 (6)	0	0	0	—	—	—

— not applicable; and NR, not reported
indicates

Tipifarnib was generally well tolerated on this dose and schedule. Three patients with aggressive lymphoma died on study of progressive disease, but there were no deaths related to tipifarnib treatment. The grade 3 or 4 toxicities were primarily reversible myelosuppression, with 11% anemia, 37% neutropenia, and 32% thrombocytopenia.

Of particular relevance to our Phase 2 clinical trial in PTCL are the results observed in the patients with T-cell NHL. Although the trial enrolled only small numbers of patients, a 41% response rate (7 responses out of 17 patients) was observed in patients with T-cell NHL, including 4 objective responses out of 8 patients with PTCL (3 CR and 1 PR). We believe the results observed from this Phase 2 clinical trial suggest that tipifarnib can be administered for prolonged periods and may produce durable responses as a single agent in relapsed lymphoma in a group of patients who were heavily pretreated, including those with PTCL.

Our Clinical Program in PTCL

Based on the promising results observed in the Phase 2 lymphoma study, we initiated a Phase 2 clinical trial in September 2015 to test the hypothesis that tipifarnib can be used as a treatment for patients with relapsed or refractory PTCL. This trial is being conducted under the IND that was transferred to us from Janssen. The current study protocol has a two-stage design for a total number of 18 eligible patients. If one or no objective response is

observed after the first 11 evaluable patients (stage 1), the study will be closed to further enrollment. If more than one response is observed, 7 additional patients will be enrolled (stage 2). The trial will be considered positive if at least 4 responses are observed (out of 18 patients). The primary endpoint is objective response rate, and tumor response assessments will be conducted according to the International Workshop Criteria for the assessment of responses in lymphoma. The study also includes a potential extension to up to a total study enrollment of 30 patients if 5 or more objective responses are observed at the end of stage 1. We anticipate receiving topline data from this trial in the first half of 2017.

Myelodysplastic Syndromes

We intend to initiate a Phase 2 clinical trial to evaluate tipifarnib as a treatment for patients with lower risk MDS in the first half of 2016.

Market Opportunity

MDS are a group of hematopoietic stem cell malignancies with significant morbidity and mortality. MDS is characterized by ineffective blood cell production, or hematopoiesis, leading to low blood cell counts, or cytopenias, and high risk of progression to AML. MDS is a highly heterogeneous disease, and the severity of symptoms and disease progression can vary widely among patients. The current standard clinical tool to evaluate risk stratification, including survival and risk for AML transformation, and treatment options is the revised International Prognostic Scoring System, or IPSS-R. The IPSS-R differentiates patients into five risk groups (Very Low, Low, Intermediate, High, Very High) based on evaluation of cytogenetics, percentage of blasts (undifferentiated blood cells) in the bone marrow, hemoglobin levels, and platelet and neutrophil counts.

According to the ACS, the annual incidence of MDS is approximately 13,000 patients in the United States, the majority of which are 60 years of age or older. The estimated prevalence is over 60,000 patients in the United States. Approximately 75% of patients fall into the IPSS-R risk categories of Very Low, Low, and Intermediate, collectively known as lower risk MDS, which is our target patient population for our planned Phase 2 MDS trial.

Treatment Options for MDS

Therapeutic options fall into three categories including supportive care, low intensity and high intensity therapy. Supportive care includes the use red blood cell and platelet transfusions and hematopoietic cytokines such as erythropoiesis stimulating agents or colony stimulating factors to improve blood counts. Low intensity therapies include hypomethylating agents such as azacytidine (Vidaza®) and decitabine (Dacogen®), biological response modifiers such as lenalidomide (Revlimid®), and immunosuppressive treatments such as cyclosporine A or antithymocyte globulin. High intensity therapies include chemotherapeutic agents such as idarubicin, cytarabine, fludarabine and topotecan, and hematopoietic stem cell transplants, or HSCT.

National Comprehensive Cancer Network, or NCCN, guidelines recommend that lower risk patients (IPSS-R groups Very Low, Low, Intermediate) receive supportive care or low intensity therapies with the major therapeutic goal of hematologic improvement, or HI. A substantial portion of lower risk MDS patients lack effective therapies and NCCN guidelines recommend clinical trials as additional therapeutic options. We believe that treatment of MDS remains a significant unmet need requiring the development of novel therapies.

Previous Phase 2 Experience with Tipifarnib in the Treatment of MDS

A prior Phase 2 clinical trial of tipifarnib was sponsored by Johnson & Johnson and conducted at 19 sites in seven countries from 2002 to 2006 in adult patients with intermediate to high risk MDS. This study also included

patients with chronic myelomonocytic leukemia. Eighty-two patients with International Prognostic Scoring System scores of Intermediate-1, Intermediate-2, and High risk MDS were enrolled in the study, and patients received tipifarnib 300 mg twice daily on days 1-21 of each 28-day cycle. The median age of patients was 67 (range 39-86 years). The median time since diagnosis was 8.8 months (range 0-128 months) and 37% (30 of 82) had been received prior therapy.

The ORR for all patients was 31.7% (26 of 82), with 14.6% (12 of 82) CR and 17.1% (14 of 82) HI. In the 12 complete responders, the median response duration was 11.5 months (range 2.0-21.9 months), and the median TTP was 12.4 months (3.9-23.8 months). Median duration of HI was 18 weeks (range 6 to 76 weeks). Median OS was 11.7 months for all patients.

Table B: Phase 2 Clinical Trial of tipifarnib in Adult Patients with Intermediate to High Risk MDS

	n	CR, n (%)	HI, n (%)	ORR, n (%)	Median DR	Median OS
All patients	82	12 (14.6)	14 (17.1)	26 (31.7)	11.5	11.7

Tipifarnib was generally well tolerated. Ten patients died during the treatment period with five deaths due to progressive disease and five due to an adverse event of which only one was considered drug-related. This death was due to coronary insufficiency triggered by anemia and severe internal bleeding in the context of nonresponsive MDS with persistent Grade 4 thrombocytopenia. Grade 3-4 adverse events were primarily neutropenia, thrombocytopenia and anemia, and were reported as possibly drug-related in 15 patients (18%), 26 patients (32%), and 15 patients (18%), respectively. We believe the results of this study suggest that tipifarnib may produce durable responses as a single agent in patients with intermediate to high risk MDS.

Clinical Development in MDS

We plan to initiate a Phase 2 clinical trial to investigate the anti-tumor activity of tipifarnib in patients with lower risk MDS in the first half of 2016. We have prioritized lower risk MDS because of the prevalence of this disease and our belief that treatment of lower risk MDS remains a significant unmet medical need. We expect that the activity of tipifarnib in lower risk MDS will be no less than the activity observed in the previously investigated intermediate/high risk setting, which is a more aggressive form of the disease. We anticipate that our Phase 2 study in lower risk MDS would aim to enroll approximately 70 patients, and have a primary endpoint of transfusion independence according to the adult Myelodysplastic/Myeloproliferative Neoplasms International Working Group criteria or related response assessment system. We expect this study will be conducted under the IND that was transferred to us from Janssen. We anticipate receiving topline data from this trial in the first half of 2017.

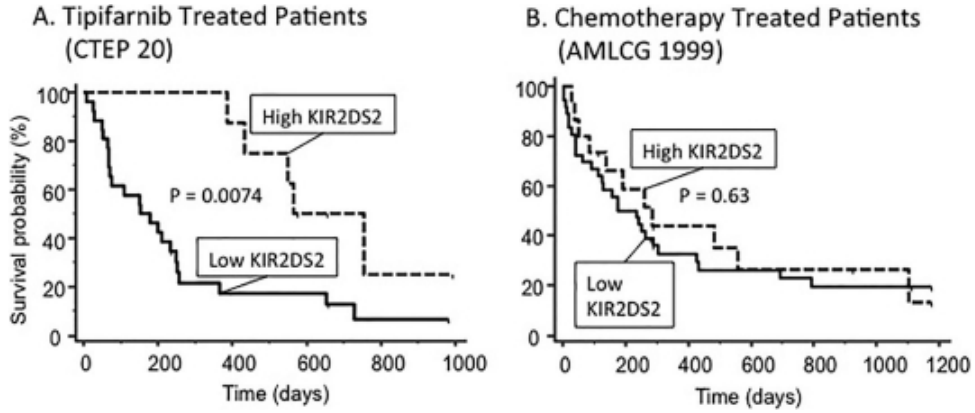
Exploratory Biomarkers

We have identified potential biomarkers that could be predictive of response to tipifarnib in MDS patients. One of these potential biomarkers is the killer cell immunoglobulin-like receptor 2DS2, or KIR2DS2, which is commonly expressed on natural killer, or NK, cells and some T-cells to regulate their activity. Autoimmunity is known to play a key role in the onset of lower risk MDS and KIR2DS2 has been shown to predispose to both MDS and autoimmune diseases.

Our interest in KIR2DS2 and other killer cell immunoglobulin-like receptors, or KIRs, was triggered by the results of our retrospective analysis of gene expression from bone marrow samples in 34 previously untreated poor-risk and elderly AML patients who were treated with tipifarnib in a prior Phase 2 clinical trial sponsored by the National Cancer Institute, or CTEP 20. 25 of these patients had prior MDS. We observed that expression of several markers, including KIR2DS2, strongly correlated with clinical benefit, including complete response rate and survival endpoints. Our analysis showed that patients in the upper (4th) quartile of KIR2DS2 expression had a median survival of 564 days whereas those in the 1st -3rd quartile of KIR2DS2 expression had a median survival of 153 days. Similar findings were observed with the expression of other NK specific genes such

as KIR2DS5 and GZMM, or granzyme M, as well as with the ratio of activatory to inhibitory KIRs (KIR2DS2/KIR2DL2, KIR2DS5/KIR2DL5). Granzyme M is an enzyme that is important for the activity of NK cells. KIR2DS5 is an activatory KIR that has been associated with the occurrence of certain types of MDS and of relapse after bone marrow transplantation.

Figure 1: Survival of AML Patients by KIR2DS2 Expression



Treatment (n)	Median Overall Survival (days)	KIR2DS2 Low 1st-3rd Quartile Median Survival (Days)	KIR2DS2 High 4th Quartile (Upper) Median Survival (Days)	Hazards Ratio
Tipifarnib (34)	233	153	564	0.30
Chemotherapy (51)	240	176	284	0.83

In contrast to the results with tipifarnib, we found no correlation between the expression of the markers, including KIR2DS2, and the clinical benefit derived from chemotherapy treatment in a subset of 51 previously untreated and elderly (>65 years) AML patients enrolled in the German AML Cooperative Group 1999 study, or AMLCG 1999.

Because it is known that KIR2DS2 signals in part through RAS, we hypothesize that tipifarnib may influence the signaling of KIR2DS2 through its inhibition of protein farnesylation, either of RAS proteins or other farnesylated proteins in the cell. Through this mechanism, we believe that tipifarnib could inhibit aberrant NK cell activity and improve patient outcomes. Because KIR2DS2 and KIR2DS5 are known to predispose to autoimmunity and the onset of MDS, we believe that tipifarnib could attenuate the autoimmune process that causes severe cytopenias in lower risk MDS. This hypothesis will be tested in the planned Phase 2 study in lower risk MDS.

Companion Diagnostics

If the results of our Phase 2 clinical trial in MDS are positive, and KIR2DS2 or other immune cell markers are shown to be predictive of response to tipifarnib, we would expect to partner development and validation of a companion diagnostic test to aid in the selection of patients in subsequent clinical trials of tipifarnib in this patient population. Genetic assays detecting the presence or absence of certain of these genes and markers are already available and used in some instances in bone marrow transplantation. We plan to investigate in our Phase 2 clinical trial whether these genetic assays will be sufficient to define the MDS patients susceptible to receive clinical benefit from tipifarnib or whether a PCR based assay defining biomarker expression levels will need to be developed including identification of the optimal biomarker cut-off criterion for patient selection.

Registration Strategy for Tipifarnib

We anticipate that we will have topline data from all three of our company sponsored Phase 2 clinical trials of tipifarnib by mid-2017. If the data from one or more of these trials is positive, we would plan to then initiate a registrational Phase 3 trial of tipifarnib in at least one disease indication. The use of regulatory pathways such as orphan drug or breakthrough therapy designation will be driven by the specific patient population and data from the Phase 2 clinical trials.

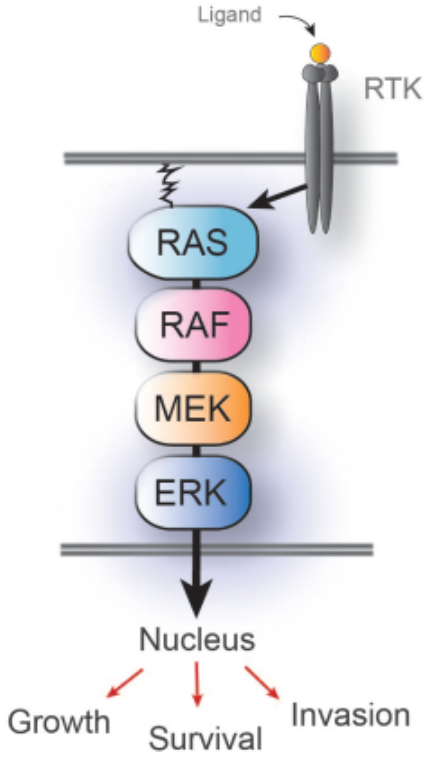
ERK Inhibitor Program

Overview

We are advancing KO-947, a small molecule inhibitor of ERK1/2, as a potential treatment for patients with tumors that have dysregulated activity due to mutations and other mechanisms in the MAPK pathway, including lung cancers, colorectal cancers, pancreatic cancers and melanoma. KO-947 and backup compounds represent new chemical entities we acquired pursuant to an agreement effective December 23, 2014 from Araxes Pharma LLC.

The high frequency of activating mutations in components of the MAPK pathway found in cancer provides strong rationale for targeting the MAPK pathway and, specifically, ERK. The MAPK pathway is responsible for receiving growth-promoting signals from outside the cell and translating these signals within the cell into programs that affect cell growth and proliferation. When external growth factors activate cell surface receptor tyrosine kinases, the MAPK pathway acts inside the cell to relay these growth signals through a series of signaling molecules, including the RAS, RAF, MEK, and ERK family of kinases. ERK kinase is the final signaling kinase of the MAPK pathway. See Figure 2.

Figure 2: MAPK pathway



Many cancers harbor genetic mutations in components of the MAPK pathway, especially in protein kinases, that lock transformed cells in a pro-growth state, even in the absence of external growth signals. Studies have shown that such aberrations in the MAPK pathway, including mutations in KRAS, BRAF, and other components of the pathway, are frequent contributors to the development of cancer in humans. Targeted cancer drugs, such as inhibitors of the proteins BRAF and MEK, that have been designed to turn off MAPK signaling by inhibiting specific protein kinases are effective, particularly in melanomas where the MAPK circuit is aberrantly active. We believe that a therapeutic product candidate that can block signaling of the MAPK pathway through inhibition of ERK should reduce or prevent cancer growth and may have a beneficial effect for patients.

As part of our ERK inhibitor program, we are advancing KO-947, which is an orally-available inhibitor of ERK that has nanomolar cellular potency in tumor cells with mutations in BRAF, NRAS or KRAS and induces tumor regressions at tolerable doses in xenograft mouse models. Because KO-947 targets ERK, a protein kinase essential to signaling through the MAPK pathway, it has the potential to selectively kill tumor cells bearing activating mutations in this critical pathway. KO-947 is currently in IND enabling studies, and we anticipate filing an IND in the first half of 2016.

Opportunity for Kura Oncology

We have focused on the discovery and development of ERK inhibitors and selected KO-947 as a potential product candidate because we believe that ERK inhibitors have two important potential advantages as therapeutics:

- Potential to effectively treat patients with mutations in the KRAS gene—a large and growing group of patients with lung, colorectal, pancreatic and other cancers who today have no effective therapy, and who have been identified with greater frequency due to recently approved diagnostic guidelines, and

- Potential to effectively treat patients with metastatic melanoma who receive “first-generation” BRAF or MEK inhibitors, but who develop resistance due to reactivation of ERK pathway signaling. KO-947 could prevent resistance through this mechanism and may thus cause responses of greater duration than the ones seen with first generation inhibitors and extend progression-free survival.

We acquired our ERK inhibitor program from Araxes Pharma based in La Jolla, California. Scientists at Araxes Pharma designed our ERK inhibitors using structure-guided drug discovery approaches to model chemical structures that would inhibit the ERK protein kinase but spare inhibition of closely related kinases. These molecules were then synthesized and tested in assays to verify their ability to inhibit ERK as well as to inhibit MAPK pathway signaling.

Solid Tumors with KRAS Mutations

Market Overview

Activating mutations in the KRAS gene are commonly found in a wide variety of tumor types. Among cancer indications with large patient populations, KRAS mutations are found in approximately 93 percent of pancreatic cancers, approximately 40 percent of colorectal cancers and approximately 12 percent of NSCLC. According to the ACS in 2015, there are estimated to be over 49,000 cases of pancreatic cancer, 133,000 cases of colorectal cancer and over 188,000 cases of NSCLC diagnosed each year in the United States. We believe this corresponds to approximately 45,000 cases of KRAS mutant pancreatic cancer, 53,000 cases of KRAS mutant CRC, and 23,000 cases of KRAS mutant NSCLC each year in the United States. These cancers typically present relatively late in their clinical course, when locally directed therapy (surgery and radiation) is not curative. The treatment of locally advanced and metastatic cancers represents a significant unmet medical need.

Therapeutic Rationale for KRAS Mutant Solid Tumors

In its normal, non-mutant form, the KRAS protein plays a key role in the promotion and regulation of cell growth and division. The KRAS protein initiates signaling of the MAPK pathway which is responsible for receiving growth-promoting signals from outside the cell and communicating those signals within the cell so that the cell can respond appropriately to the cell growth signals.

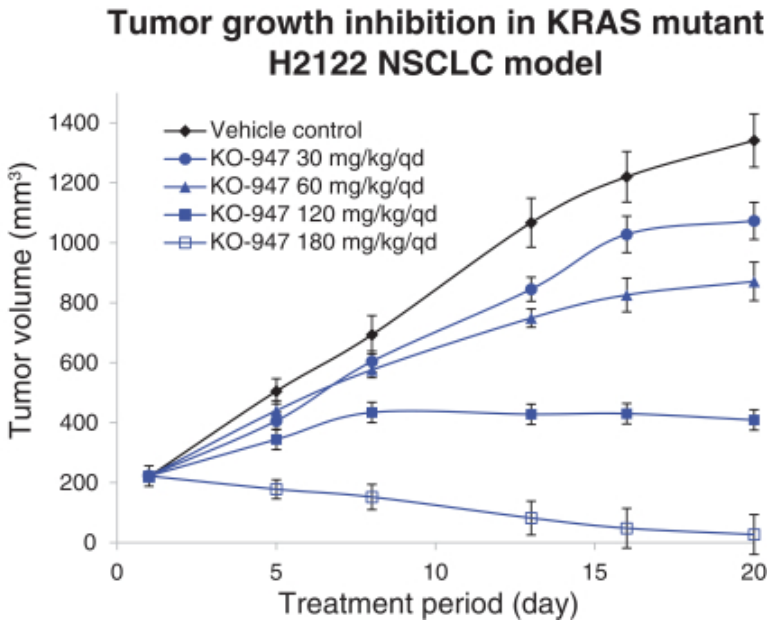
Studies have shown that disruptions to the MAPK pathway, either by mutations in KRAS or other components of the pathway, are frequent contributors to the development of cancer in humans. Certain mutations in KRAS promote cancer by putting the KRAS protein into a constitutively active state, which promotes the uncontrolled cell growth and division that are the hallmarks of cancer. We believe that a therapeutic product candidate that can inhibit signaling through the MAPK pathway should reduce or prevent cancer growth and may have a beneficial effect for patients.

Therapeutics have been successfully developed against other components of the MAPK pathway, including the BRAF inhibitors vemurafenib (ZELBORAF®) and dabrafenib (TAFINLAR®) and the MEK inhibitor trametinib (MEKINIST®), each of which has received approval from the FDA for treatment of BRAFV600E mutant melanoma. However, these drugs do not have potent activity in patients with KRAS mutations. Accordingly, oncologists and patients are still in need of a therapeutic agent that can inhibit signaling through the MAPK signaling pathway and provide benefit to patients. We believe the main challenge for MAPK pathway inhibitors has been to achieve and maintain drug exposures at tolerable doses sufficient to generate clinical benefit and that the properties of KO-947 may overcome the challenges of other MAPK pathway inhibition.

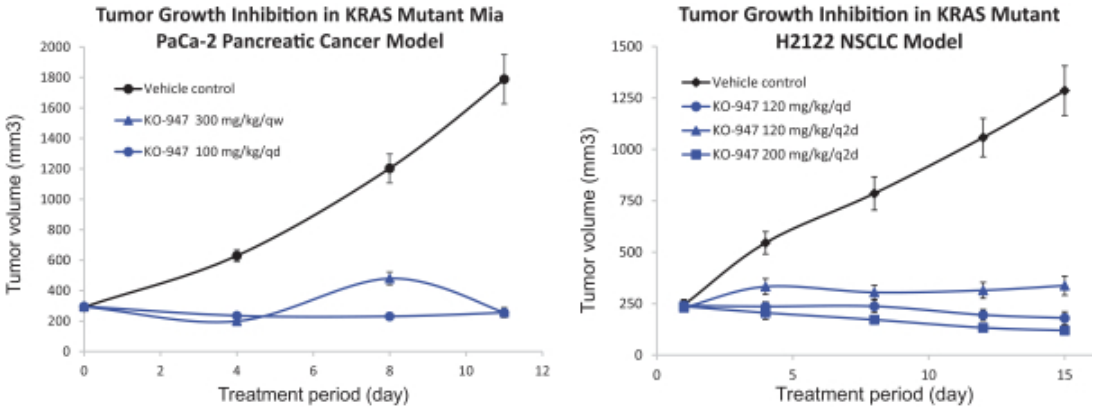
Preclinical Data for KO-947 for KRAS Mutant Solid Tumors

Our development candidate in our ERK inhibitor program, KO-947, demonstrates potent inhibition of the ERK kinase and high selectivity relative to a panel of approximately 400 kinases. KO-947 has also shown promising activity in both cell culture and xenograft animal models of KRAS mutant tumors.

In a preclinical study, xenograft tumors were grown subcutaneously in mice, followed by daily oral treatment with KO-947 or vehicle control. As shown below, treated animals in the 180 mg/kg group showed full tumor regression, while vehicle control treated animals showed rapid tumor growth. In addition, KO-947 was tolerated at all dose levels with no apparent body weight loss in the mice.



KO-947 has also shown promising activity in xenograft animal models of KRAS mutant tumors with intermittent dosing regimens. In these models, anti-tumor activity has been shown to be comparable when the compound is administered via multiple dosing schedules including once daily, once every other day, or once weekly. In the below graphs, we demonstrate that anti-tumor activity of KO-947 can be achieved by once a week dosing and every other day dosing. In addition, KO-947 was tolerated at these dose levels with no apparent body weight loss in the mice.



Melanoma Tumors with Acquired Resistance to BRAF and MEK Inhibitors

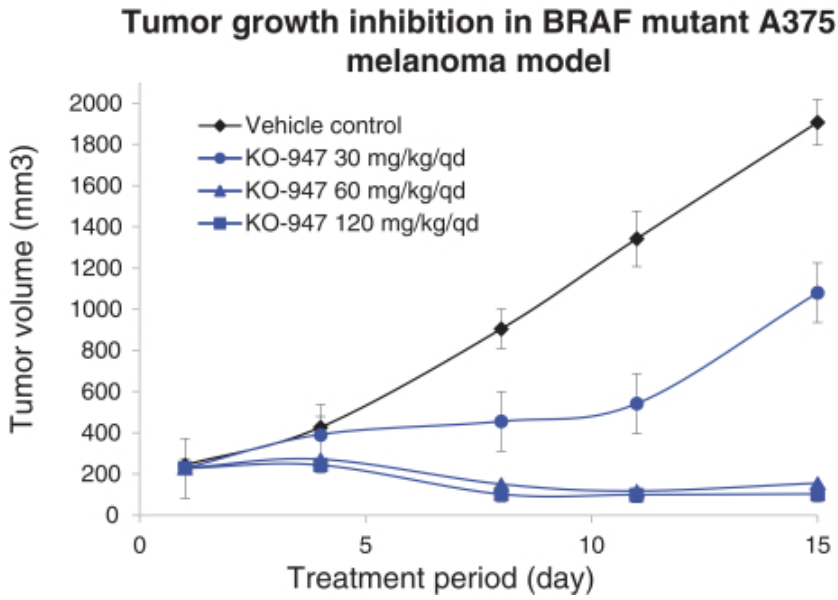
Market Overview

Specific inhibitors of RAF and MEK kinases have been developed to target BRAF- and RAS-mutant tumors. In particular, the FDA has approved the BRAF inhibitors vemurafenib (ZELBORAF®) and dabrafenib (TAFINLAR®) as well as the MEK inhibitor trametinib (MEKINIST®) for the treatment of BRAFV600E-mutant metastatic melanoma. Although these approvals are encouraging, durable responses in patients are limited, as median time to disease progression is approximately 6-7 months and resistance is often associated with pathway reactivation of the ERK signaling pathway.

According to the ACS in 2015, the annual incidence of diagnosed melanoma is 74,000 cases in the United States, of which approximately 16% have metastatic disease, and nearly 9,500 melanoma deaths occur in the each year in the United States. Mutations that activate the RAS/RAF/MEK/ERK pathway are common in melanoma, with BRAF mutations in 40% to 60%, and NRAS mutations in 15-20% of melanoma patients, suggesting the therapeutic potential for agents that target this pathway in metastatic melanoma. As ERK inhibitors target the RAS/RAF/MEK/ERK pathway, which is activated with BRAF mutation, they may also have the potential for activity not only in patients with BRAF-mutant melanoma, but also in patients with tumors that harbor mutations in the NRAS gene, who currently have no adequate treatment option and poor prognosis.

Preclinical Data for ERK Product Candidate for Melanoma with BRAF Mutation and Acquired Resistance to BRAF and MEK Inhibitors

KO-947 induced regression in a melanoma tumor model with BRAF mutation. In a pre-clinical study that we conducted, BRAF mutated melanoma tumors were grown subcutaneously in mice, followed by oral treatment with KO-947 or vehicle control. As shown below, treated animals showed full tumor regression at tolerated doses, while vehicle control and treated animals showed rapid tumor growth.



There is a strong rationale to develop ERK inhibitors for tumors that are resistant to other inhibitors of the MAPK pathway. Selective BRAF and MEK inhibitors have shown clinical efficacy in patients with melanoma. However, the majority of responses are transient, and resistance is often associated with reactivation of MAPK signaling pathway. In preclinical studies, ERK inhibitors have demonstrated promising activity in both cell

culture and xenograft animal models of tumors resistant to BRAF and MEK inhibitors. We believe KO-947 will also show anti-tumor activity in BRAF and MEK resistance models, as other ERK inhibitors have demonstrated.

Ongoing IND-enabling Studies

Based on these preclinical efficacy data in KRAS, NRAS and BRAF mutant tumor models, we have advanced KO-947 into IND-enabling studies. The IND-enabling program includes toxicology studies to determine if select doses, schedules and modes of administration are able to achieve required drug exposures to generate tumor regression, which could be tolerable in the clinical setting.

We believe opportunities exist to advance both oral and intravenous, or IV, routes of administration of KO-947 into the clinic, however, we have elected to focus our initial efforts on the IV route. Our initial non-rodent toxicology studies have shown that IV administration may increase exposure and tolerability when compared to oral dosing. Based on our preclinical data we believe that we can maintain efficacy with intermittent IV dosing, which may translate into improved tolerability in the clinical setting. We anticipate filing an IND in the first half of 2016 and we intend to evaluate solid tumors with mutations or other dysregulation of the MAPK pathway.

Menin-MLL Program

Overview

We are developing orally bioavailable small molecule inhibitors of the menin-MLL interaction for the treatment of MLL-r and MLL-PTD acute leukemias, a genetically defined subtype of the two most common forms of acute leukemia, AML and acute lymphoblastic leukemia, or ALL.

Background on Mixed Lineage Leukemias

MLL-r leukemias are an aggressive subtype of two of the most common forms of acute leukemia, ALL and AML. The estimated five-year overall survival rate for adult patients with the MLL-r subtype of AML ranges from approximately 5% to 24%. Patients with MLL-r leukemias are routinely diagnosed using existing technologies that are commonly used in clinical settings. As a result, there is high awareness of MLL-r leukemias among oncologists. The disease predominantly occurs in two different demographics—an adult population and an infant/pediatric population. While they share a common genetic alteration, the adult disease is frequently a secondary leukemia resulting from prior chemotherapy for a different, unrelated cancer, and the childhood disease arises de novo. MLL-r leukemias are caused by a chromosomal translocation involving the MLL gene.

MLL-PTD is a subset of AML. MLL-PTD typically confers a worse prognosis with shortened overall and event free survival in childhood and adult AML.

The annual incidence of MLL-r and MLL-PTD patients is estimated to be 3,200 patients in the United States, and those patients currently have limited options other than chemotherapy. There are no approved therapies specifically indicated for either the MLL-r or MLL-PTD leukemias. Physicians treat these hematological cancers with therapies approved for other acute leukemias and malignancies. Patients with AML and ALL typically are treated with intensive multi-agent chemotherapy and high risk patients are treated with an allogeneic stem cell transplant. However, some patients, especially those who are older, are too fragile for any of these treatments and, as a result, have very few treatment options. Accordingly, we believe the treatment of MLL-r and MLL-PTD leukemias remains a significant unmet medical need.

Targeting the Menin-MLL Interaction

The MLL gene is a common target of chromosomal translocations found in patients with AML and ALL, which affects both children and adults. Fusion of MLL with one of over 50 different partner genes forms oncogenes encoding MLL fusion proteins, which play a causative role in the onset, development and progression of MLL.

The effect of MLL fusion proteins on the development and progression of leukemia is critically dependent on their direct interaction with menin, a protein encoded by the Multiple Endocrine Neoplasia 1 gene. Menin is a tumor suppressor protein, which directly controls cell growth in endocrine organs. Binding of menin to MLL fusion proteins upregulates expression of target genes involved in the malignant transformation of blood cells. In contrast, mutations to MLL fusion proteins that block association with menin abrogate the development of acute leukemia in mice. These findings demonstrate that menin functions as an essential oncogenic co-factor of MLL fusion proteins, and it implies that the menin-MLL interaction represents a valuable target for molecular therapy.

We have licensed from the University of Michigan a class of small molecule inhibitors of the menin-MLL fusion protein interaction that specifically bind to menin with nanomolar potency. By blocking menin—MLL fusion protein interactions, these compounds effectively reverse MLL fusion protein-mediated leukemic transformation by down-regulating the expression of target genes required for MLL-fusion protein oncogenic activity. These compounds also selectively block proliferation and induce both apoptosis and differentiation of leukemia cells harboring MLL translocations.

Opportunity for Kura Oncology

Our menin-MLL development program is aimed at identifying product candidates with the potential to effectively treat patients with MLL-r leukemias —a subset of adult and pediatric patients who today have no effective therapy—as well as MLL-PTD leukemias, a subset of acute myeloid leukemias that have no effective therapy.

License and Asset Purchase Agreements

Janssen Pharmaceutica NV

We entered into a license agreement with Janssen on December 18, 2014, which grants us exclusive global rights to develop and commercialize tipifarnib in the field of oncology and includes the right to grant sublicenses. We are obligated under the license agreement to use commercially reasonable efforts to develop and commercialize tipifarnib in oncology and, with the exception of the transfer to us without cost of Janssen's existing inventory of tipifarnib material, we are responsible for all future development and commercialization costs for tipifarnib in oncology. Under the license agreement, Janssen has a first right to negotiate for an exclusive license back from us to develop and commercialize tipifarnib on terms to be negotiated in good faith. Janssen may exercise this right of first negotiation during the 60-day period following completion of a Phase 2 clinical trial of tipifarnib in HRAS mutant patients in oncology and delivery by us to Janssen of a complete data package from such clinical trial.

Under the terms of the license agreement, on January 20, 2015 we issued a convertible promissory note in the principal amount of $1.0 million to Johnson & Johnson Innovation—JJDC, Inc., which automatically converted into shares of Prior Kura common stock in the Private Placement. When and if commercial sales of tipifarnib begin, we are obligated to pay Janssen tiered royalties of low double digit percentages of our net sales, depending on the amount of our net sales, with standard provisions for royalty offsets in the event of generic competition or compulsory licenses, on a product-by-product and country-by-country basis until the later of the expiration of the last to expire valid claim of the licensed patents covering the licensed product in the field in such country, the expiration of any regulatory exclusivity with respect to such product in such country, and ten years from our first commercial sale. We are also required to make regulatory milestone payments to Janssen of up to $25 million in the aggregate, if specified regulatory approvals are achieved for the first indication and additional payments for each subsequent indication if specified regulatory approvals are achieved. In addition, we are required to make sales milestone payments of up to $50 million in the aggregate if specified sales thresholds are surpassed. If we grant sublicenses under the license from Janssen, we are required to pay to Janssen a percentage of any upfront, lump-sum or milestone payments received from our sublicensee, subject to certain exclusions for regulatory milestone payments due under the license agreement.

The license agreement with Janssen will remain in effect until the expiration of all of our royalty and sublicense revenue obligations to Janssen, determined on a product-by-product and country-by-country basis, unless we elect to terminate the license agreement earlier. If we fail to meet our obligations under the license agreement and are unable to cure such failure within specified time periods, Janssen can terminate the license agreement, resulting in a loss of our rights to tipifarnib.

Araxes Pharma LLC

We entered into an asset purchase agreement with Araxes on December 23, 2014, under which we purchased all of Araxes' patent rights in the ERK program, including KO-947 and additional backup compounds, and related intellectual property. When and if commercial sales of a product candidate covered by the purchased patent rights begin, we are obligated to pay Araxes tiered royalties of low single digit percentages of our net sales, depending on the amount of our net sales with standard provisions for royalty offsets. We are also required to make development and regulatory milestone payments to Araxes of up to $9.7 million in the aggregate if specified development events and regulatory approvals are achieved. Under the terms of the asset purchase agreement, on December 23, 2014 we issued a convertible promissory note in the principal amount of $500,000 to Araxes, which automatically converted into shares of Prior Kura common stock in the Private Placement.

Competition

The development and commercialization of new products to treat cancer is intensely competitive and subject to rapid and significant technological change. While we believe that our knowledge, experience and scientific resources provide us with competitive advantages, we face substantial competition from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Many of our competitors have significantly greater financial, technical, and human resources. Smaller and early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. As a result, our competitors may discover, develop, license or commercialize products before or more successfully than we do.

We face competition with respect to our current product candidates, and will face competition with respect to future product candidates, from segments of the pharmaceutical, biotechnology and other related markets that pursue approaches to targeting molecular alterations and signaling pathways associated with cancer. Our competitors may obtain regulatory approval of their products more rapidly than we do or may obtain patent protection or other intellectual property rights that limit our ability to develop or commercialize our product candidates. Our competitors may also develop drugs that are more effective, more convenient, less costly, or possessing better safety profiles than our products, and these competitors may be more successful than us in manufacturing and marketing their products.

In addition, we will need to develop our product candidates in collaboration with diagnostic companies, and we will face competition from other companies in establishing these collaborations. Our competitors will also compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Furthermore, we also face competition more broadly across the market for cost-effective and reimbursable cancer treatments. The most common methods of treating patients with cancer are surgery, radiation and drug therapy, including chemotherapy, hormone therapy and targeted drug therapy or a combination of such methods. There are a variety of available drug therapies marketed for cancer. In many cases, these drugs are administered in combination to enhance efficacy. While our product candidates, if any are approved, may compete with these existing drug and other therapies, to the extent they are ultimately used in combination with or as an adjunct to these therapies, our product candidates may not be competitive with them. Some of these drugs are branded and subject to patent protection, and others are available on a generic basis. Insurers and other third-party payors may

also encourage the use of generic products or specific branded products. We expect that if our product candidates are approved, they will be priced at a premium over competitive generic, including branded generic, products. As a result, obtaining market acceptance of, and gaining significant share of the market for, any of our product candidates that we successfully introduce to the market will pose challenges. In addition, many companies are developing new therapeutics, and we cannot predict what the standard of care will be as our product candidates progress through clinical development.

Tipifarnib Competition

While there are currently no approved drugs targeting farnesyltransferase, we are aware of a number of compounds that are now or have previously been in clinical development, including Merck's lonafarnib, Bristol-Myers Squibb's BMS-214662, Astellas Pharma's (formerly OSI) CP-609,754, and AstraZeneca's AZD3409. Lonarfarnib is currently being investigated in a Phase 1 trial in combination with temozolomide in patients with malignant gliomas. To our knowledge, there are no other ongoing clinical trials evaluating any of these agents for the treatment of cancer. However, the initiation of clinical development of another of these agents in an oncology setting could become competitively significant, and if tipifarnib or our other product candidates do not offer sustainable advantages over competing products, we may not be able to successfully compete against current and future competitors.

Even if we are successful in developing our product candidates, the resulting products would compete with a variety of established drugs in the targeted therapeutic indications of PTCL and MDS. Competitive drugs currently approved for PTCL include belinostat (Beleodaq®) and pralatrexate (Folotyn®), marketed by Spectrum Pharmaceuticals, romidepsin (Istodax®), marketed by Celgene, and brentuximab vedotin (Adcetris®) (for ALCL), marketed by Seattle Genetics. Competitive drugs currently approved for MDS include azacytidine (Vidaza®) and lenalidomide (Revlimid®), marketed by Celgene, and decitabine (Dacogen®) marketed by Otsuka and Johnson & Johnson. Although there are currently no drugs approved specifically for the treatment of HRAS-mutant solid tumors, there are a number of targeted therapies approved for the treatment of thyroid cancer, including AstraZeneca's vandetanib (Caprelsa®), Bayer's sorafenib (Nexavar®), Exelixis' cabozantinib (Cometriq®) and Eisai's lenvatinib (Lenvima®). There are no targeted therapies approved for the treatment of urothelial cancer.

ERK Inhibitor Competition

While there are currently no approved drugs targeting ERK, we are aware of a number of compounds that are in clinical development, including Roche/Genentech's RG-7842/GDC-0994, Celgene's CC-90003, and BioMed Valley Discoveries' ulixertinib (BVD-523). Furthermore, it is possible that other companies are also engaged in discovery or preclinical development of compounds targeting ERK. These competitors, if successful in clinical development, may achieve clinical activity, regulatory approval and market adoption in advance of our compounds, constraining the ability of our compounds to gain significant market share. Although we believe that our ERK inhibitors, including KO-947, present several potential advantages relative to these aforementioned candidates, including potency as demonstrated in preclinical studies, these results may not translate to superior therapeutic benefit in clinical trials.

Menin-MLL Inhibitor Competition

There are no drugs approved or in clinical trials targeting the menin-MLL protein-protein interaction. Although there are no targeted therapies approved specifically for the treatment of MLL-r leukemias, there are a number of products in clinical development, including Epizyme's EPZ-5676 and Novartis's midostaurin, as well as Pfizer's palbociclib (IBRANCE®), which has received accelerated approval in combination with letrozole, for the treatment of postmenopausal women with estrogen receptor-positive, human epidermal growth factor receptor 2-negative (ER+/HER2-) advanced breast cancer as initial endocrine-based therapy for their metastatic disease.

Commercialization

We have not yet established a sales, marketing or product distribution infrastructure because our lead candidates are still in discovery, preclinical or early clinical development. We anticipate that we will aim to retain commercial rights in North America for any of our product candidates for which we may in the future receive marketing approvals. We may also seek to retain commercial rights in Europe for any of our product candidates for which we may in the future receive marketing approvals. We currently anticipate that, if and when appropriate, we will seek to access the North American or European oncology markets through a focused, specialized, internal sales force.

Subject to receiving marketing approvals, we expect to commence commercialization activities by building a focused internal sales and marketing team in North America to sell our products. We may also build a focused internal sales and marketing team in Europe to sell our products. We believe that such an approach will enable us to address the community of oncologists who are the key specialists in treating the patient populations for which our current product candidates are being developed. Outside of regions where we maintain commercial rights, we may enter into distribution and other marketing arrangements with third parties for any of our product candidates that obtain marketing approval in foreign jurisdictions.

We also aim to build a marketing and sales management force to create and implement marketing strategies for any products that we may in the future market through our own sales teams and to oversee and support our sales force. We anticipate that our goals for any such marketing force include developing educational initiatives with respect to any approved products and establishing relationships with thought leaders in relevant fields of medicine.

We currently expect that any third parties with which we may collaborate in the future on the development of any commercial companion diagnostics for use with our therapeutic products will most likely hold the commercial rights to those diagnostic products. We expect that we would coordinate closely with any future diagnostic collaborators in connection with the marketing and sale of such diagnostic products and our related therapeutic products.

Manufacturing

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates for preclinical and clinical testing as well as for commercial manufacture of any products that we may commercialize. Under our license agreement with Janssen, Janssen has provided us with its existing inventory of clinical supply of tipifarnib, which we believe will support our ongoing and planned Phase 2 clinical trials of tipifarnib. Janssen also provided us with its existing inventory of the crude drug substance and bulk key intermediate for manufacture of drug substance for tipifarnib. If needed, we aim to engage, by entering into a supply agreement or through another arrangement, third party manufacturers to provide us with additional tipifarnib clinical supply. For all of our product candidates, we aim to identify and qualify manufacturers to provide the active pharmaceutical ingredient and fill-and-finish services prior to submission of an NDA to the FDA.

We generally expect to rely on third parties for the manufacture of any companion diagnostics we or our collaborators may develop.

Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection for our product candidates and our core technologies, including novel biomarker and diagnostic discoveries and other know-how, to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary or intellectual property rights. We expect that we will seek to

protect our proprietary and intellectual property position by, among other methods, licensing or filing our own U.S., international and foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary and intellectual property position, which we generally seek to protect through contractual obligations with third parties.

We currently, and expect that we will continue to, file or license patent applications directed to our key product candidates in an effort to establish intellectual property positions regarding composition-of-matter of these product candidates, as well as formulations, processes and methods of using these product candidates in the treatment of various cancers. We also intend to seek patent protection, if available, with respect to biomarkers that may be useful in selecting the right patient population for use of any of our product candidates. We own or in-license a patent portfolio consisting of over 25 patent families, including issued U.S. patents and their respective counterparts in a number of foreign jurisdictions, pending U.S. patent applications, pending applications under the Patent Cooperation Treaty and corresponding pending patent applications in a number of foreign jurisdictions. In particular, we have exclusively licensed from Janssen a portfolio of approximately 20 patent families including composition-of-matter patents that cover tipifarnib as well as method-of-use patents covering tipifarnib for treating various cancers. These composition-of-matter and method-of-use patents are issued in major market countries including the United States, Europe, and Japan, and they are expected to expire in 2016 without patent term extension. We in-license from the University of Michigan or co-own approximately six families of patent applications pertaining to our menin-MLL program. Other patent applications we own include a composition-of-matter and method-of-use application covering our ERK product candidate. We currently, and expect that we will continue to, file for patents in the United States with counterparts in major market countries in Europe and other key markets in the rest of the world. We would expect that any patents that may issue from the pending U.S. patent applications directed to our ERK program and our menin-MLL program would likely start to expire in 2030; however, any and all of these patent applications may not result in issued patents.

In addition to the patent applications that we have filed to date, we plan to continue to expand our intellectual property portfolio by filing patent applications directed to dosage forms, methods of treatment and additional inhibitor compounds of oncology molecular targets and their derivatives. Specifically, we anticipate that we will seek patent protection in the United States and internationally for novel compositions of matter covering the compounds, the chemistries and processes for manufacturing these compounds, their intermediates and/or metabolites, the use of these compounds in a variety of therapies and the use of biomarkers for patient selection for these compounds. However, these or other patent applications that we may file or license from third parties may not result in the issuance of patents, and any issued patents may cover limited claims that reduce their value and/or may be challenged, invalidated or circumvented. See “Risk Factors—Risks Related to Our Intellectual Property.”

In addition to patents, we also rely upon unpatented trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, using confidentiality agreements with our collaborators, scientific advisors, employees and consultants, and invention assignment agreements with our employees and selected consultants, scientific advisors and collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed through a relationship with a third-party.

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA certain patents whose claims cover the applicant’s product. Upon approval, each of the patents listed in the application for the drug is then published in the FDA’s Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Any applicant who files an ANDA seeking approval of a generic

equivalent version of a drug listed in the Orange Book or a Section 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (1) no patent information on the drug product that is the subject of the application has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. A notice of the paragraph IV certification must be provided to each owner of the patent that is the subject of the certification and to the holder of the approved NDA to which the ANDA or Section 505(b)(2) application refers. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

If the NDA holder for the reference drug and/or patent owners assert a patent challenge directed to one of the Orange Book listed patents within 45 days of the receipt of the paragraph IV certification notice, the FDA is prohibited from approving the application until the earlier of 30 months from the receipt of the paragraph IV certification, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the applicant. The ANDA or Section 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the reference drug has expired as described in further detail below.

Non-Patent Exclusivity

In addition to patent exclusivity, the holder of an NDA for a listed drug may be entitled to a period of non-patent exclusivity, during which the FDA cannot approve an ANDA or Section 505(b)(2) application that relies on the listed drug. For example, a pharmaceutical manufacturer may obtain five years of non-patent exclusivity upon FDA approval of a new chemical entity, or NCE, which is a drug that contains an active moiety that has not been approved by the FDA in any other NDA. An "active moiety" is defined as the molecule or ion responsible for the drug substance's physiological or pharmacologic action. During the five year exclusivity period, the FDA cannot accept for filing any ANDA seeking approval of a generic version of that drug or any Section 505(b)(2) NDA for the same active moiety and that relies on the FDA's findings regarding that drug, except that the FDA may accept an application for filing after four years if the follow-on applicant makes a paragraph IV certification. Five-year NCE exclusivity does not block the submission, review or approval of a 505(b)(1) NDA.

Patent Term Extension

After NDA approval, owners of relevant drug patents may apply for up to a five-year patent extension. The allowable PTE is calculated as half of the drug's testing phase—the time between IND application and NDA submission—plus all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term, including the extension may not exceed 14 years from the date of NDA approval.

For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the U.S. PTO must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

Government Regulation

FDA Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by FDA. The Federal Food, Drug and Cosmetic Act and other federal and state statutes and regulations govern, among other things, the

research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending NDAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution.

Pharmaceutical product development for a new product or certain changes to an approved product in the U.S. typically involves preclinical laboratory and animal tests, the submission to FDA of an IND which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in compliance with good clinical practice, or GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors; as well as (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to FDA as part of the IND.

FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB’s requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses, and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to determine the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial

demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

After completion of the required clinical testing, an NDA is prepared and submitted to FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting an NDA is substantial, and the fees are typically increased annually.

FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, FDA begins an in-depth review. FDA has agreed to certain performance goals in the review of new drug applications to encourage timeliness. Most applications for standard review drug products are reviewed within twelve months from submission; most applications for priority review drugs are reviewed within eight months from submission. Priority review can be applied to drugs that FDA determines offer major advances in treatment, or provide a treatment where no adequate therapy exists. The review process for both standard and priority review may be extended by FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an outside advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

Before approving an NDA, FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, FDA will inspect the facility or the facilities at which the drug is manufactured. FDA will not approve the product unless compliance with current good manufacturing practice, or GMP—a quality system regulating manufacturing—is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for FDA to reconsider the application. If, or when, those deficiencies have been addressed to FDA's satisfaction in a resubmission of the NDA, FDA will issue an approval letter. FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Fast Track Designation and Accelerated Approval

FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the Fast Track program, the sponsor of a new product candidate may request that FDA designate the product candidate for a specific indication as a Fast Track drug concurrent with, or after, the filing of the IND for the product candidate. FDA must determine if the product candidate qualifies for Fast Track Designation within 60 days of receipt of the sponsor's request.

Under the Fast Track program and FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to priority review by FDA.

If a submission is granted Fast Track Designation, the sponsor may engage in more frequent interactions with FDA, and FDA may review sections of the NDA before the application is complete. This rolling review is available if the applicant provides, and FDA approves, a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, Fast Track Designation may be withdrawn by FDA if FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Breakthrough Therapy Designation

FDA is also required to expedite the development and review of the application for approval of drugs that are intended to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Under the Breakthrough Therapy program, the sponsor of a new product candidate may request that FDA designate the product candidate for a specific indication as a breakthrough therapy concurrent with, or after, the filing of the IND for the product candidate. FDA must determine if the product candidate qualifies for Breakthrough Therapy designation within 60 days of receipt of the sponsor's request.

Orphan Drug Designation and Exclusivity

The Orphan Drug Act provides incentives for the development of products intended to treat rare diseases or conditions. Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug available in the United States for this type of disease or condition will be recovered from sales of the product. If a sponsor demonstrates that a drug is intended to treat

a rare disease or condition, the FDA will grant orphan designation for that product for the orphan disease indication, assuming that the same drug has not already been approved for the indication for which the sponsor is seeking orphan designation. If the same drug has already been approved for the indication for which the sponsor is seeking orphan designation, the sponsor must present a plausible hypothesis of clinical superiority in order to obtain orphan designation. Orphan designation must be requested before submitting an NDA. After the FDA grants orphan designation, the FDA discloses the identity of the therapeutic agent and its potential orphan use.

Orphan designation may provide manufacturers with benefits such as research grants, tax credits, PDUFA application fee waivers, and eligibility for orphan drug exclusivity. If a product that has orphan designation subsequently receives the first FDA approval of the active moiety for that disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which for seven years prohibits the FDA from approving another product with the same active ingredient for the same indication, except in limited circumstances. Orphan drug exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan drug has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug has exclusivity.

In the European Union, orphan drug designation also entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following drug or biological product approval. This period may be reduced to 6 years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Post-Approval Requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports are required following FDA approval of an NDA. FDA also may require post-marketing testing, known as Phase 4 testing, risk evaluation and mitigation strategies, or REMS, and surveillance to monitor the effects of an approved product, or FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, drug manufacture, packaging and labeling procedures must continue to conform to current good manufacturing practices, or cGMPs, after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with FDA and certain state agencies. Registration with FDA subjects entities to periodic unannounced inspections by FDA, during which the Agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Pediatric Information

Under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. FDA may grant full or partial waivers, or deferrals, for submission of data. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month extension of any exclusivity—patent or non-patent—for a drug if certain conditions are met. Conditions for exclusivity include FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, FDA making a written request for pediatric studies, and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

FDA Regulation of Companion Diagnostics

Our drug products may rely upon in vitro companion diagnostics for use in selecting the patients that we believe will respond to our cancer therapeutics. If safe and effective use of a therapeutic product depends on an in vitro diagnostic, FDA generally will require approval or clearance of the diagnostic at the same time that FDA approves the therapeutic product. This policy is described in an August 2014 FDA guidance document.

FDA has required in vitro companion diagnostics intended to select the patients who will respond to cancer treatment to obtain a pre-market approval, or PMA, for that diagnostic simultaneously with approval of the drug. We believe that FDA will require PMA approval of one or more in vitro companion diagnostics to identify patient populations suitable for our cancer therapies. The review of these in vitro companion diagnostics in conjunction with the review of our cancer treatments involves coordination of review by FDA's Center for Drug Evaluation and Research and by FDA's Center for Devices and Radiological Health.

The PMA process, including the gathering of clinical and nonclinical data and the submission to and review by FDA, can take several years or longer. It involves a rigorous premarket review during which the applicant must prepare and provide FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA applications are subject to an application fee. In addition, PMAs for certain devices must generally include the results from extensive preclinical and adequate and well-controlled clinical trials to establish the safety and effectiveness of the device for each indication for which FDA approval is sought. In particular, for a diagnostic, the applicant must demonstrate that the diagnostic produces reproducible results when the same sample is tested multiple times by multiple users at multiple laboratories. As part of the PMA review, FDA will typically inspect the manufacturer's facilities for compliance with the Quality System Regulation, or QSR, which imposes elaborate testing, control, documentation and other quality assurance requirements.

PMA approval is not guaranteed, and FDA may ultimately respond to a PMA submission with a not approvable determination based on deficiencies in the application and require additional clinical trial or other data that may be expensive and time-consuming to generate and that can substantially delay approval. If FDA's evaluation of the PMA application is favorable, FDA typically issues an approvable letter requiring the applicant's agreement to specific conditions, such as changes in labeling, or specific additional information, such as submission of final labeling, in order to secure final approval of the PMA. If FDA concludes that the applicable criteria have been met, FDA will issue a PMA for the approved indications, which can be more limited than those originally sought by the applicant. The PMA can include post-approval conditions that FDA believes necessary to ensure the safety and effectiveness of the device, including, among other things, restrictions on labeling, promotion, sale and distribution.

After a device is placed on the market, it remains subject to significant regulatory requirements. Medical devices may be marketed only for the uses and indications for which they are cleared or approved. Device manufacturers must also establish registration and device listings with FDA. A medical device manufacturer's manufacturing processes and those of its suppliers are required to comply with the applicable portions of the QSR, which cover the methods and documentation of the design, testing, production, processes, controls, quality assurance, labeling, packaging and shipping of medical devices. Domestic facility records and manufacturing processes are subject to periodic unscheduled inspections by FDA. FDA also may inspect foreign facilities that export products to the United States.

Failure to comply with applicable regulatory requirements can result in enforcement action by FDA, which may include any of the following sanctions: warning letters, fines, injunctions, civil or criminal penalties, recall or seizure of current or future products, operating restrictions, partial suspension or total shutdown of production, denial of submissions for new products, or withdrawal of PMA approvals.

Clinical Trials and IDEs

A clinical trial is almost always required to support a PMA application. In some cases, one or more smaller IDE studies may precede a pivotal clinical trial intended to demonstrate the safety and efficacy of the investigational device.

All clinical studies of investigational devices must be conducted in compliance with the FDA's requirements. If an investigational device could pose a significant risk to patients pursuant to FDA regulations, the FDA must approve an IDE application prior to initiation of investigational use. IVD trials usually do not require an IDE, as the FDA does not judge them to be a significant risk because the results do not affect the patients in the study. However, for a trial where the IVD result directs the therapeutic care of patients with cancer, we believe that the FDA may consider the investigation to present significant risk and require an IDE application.

An IDE application must be supported by appropriate data, such as laboratory test results, showing that it is safe to test the device in humans and that the testing protocol is scientifically sound. The FDA typically grants IDE approval for a specified number of patients. A non-significant risk device does not require FDA approval of an IDE. Both significant risk and non-significant risk investigational devices require approval from IRBs at the study centers where the device will be used.

During the critical trial, the sponsor must comply with the FDA's IDE requirements for investigator selection, trial monitoring, reporting and record keeping. The investigators must obtain patient informed consent, rigorously follow the investigational plan and study protocol, control the disposition of investigational devices and comply with all reporting and record keeping requirements. Prior to granting PMA approval, the FDA typically inspects the records relating to the conduct of the study and the clinical data supporting the PMA application for compliance with applicable requirements.

Although the QSR does not fully apply to investigational devices, the requirement for controls on design and development does apply. The sponsor also must manufacture the investigational device in conformity with the quality controls described in the IDE application and any conditions of IDE approval that the FDA may impose with respect to manufacturing.

Foreign Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our product candidates to the extent we choose to sell any products outside of the United States. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by regulatory authorities of foreign countries before we can commence clinical trials

or marketing of the product in those countries. The approval process varies from country to country and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. As in the United States, post-approval regulatory requirements, such as those regarding product manufacture, marketing, or distribution would apply to any product that is approved outside the United States.

Government authorities in the United States, at the federal, state and local level, and in other countries, extensively regulate, among other things, the research, development, testing, manufacture, including any manufacturing changes, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, import and export of pharmaceutical products, such as those we are developing.

Additional Regulations and Environmental Matters

In addition to FDA restrictions on marketing of pharmaceutical products, we are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. These laws, which generally will not be applicable to us or our product candidates unless and until we obtain FDA marketing approval for any of our product candidates, include transparency laws, anti-kickback statutes, false claims statutes and regulation regarding providing drug samples, among others.

The federal Anti-Kickback Statute prohibits, among other things, individuals and entities from knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. Violations of the federal Anti-Kickback Statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal false claims laws and civil monetary penalties, including the False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws.

HIPAA imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters.

HIPAA, as amended by the HITECH Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. Many states and foreign jurisdictions also have laws and regulations that govern the privacy and security of individually identifiable health information, and such laws often vary from one another and from HIPAA.

The federal Physician Payment Sunshine Act requires certain 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 CMS, information related to payments or other transfers of value made to physicians and teaching hospitals, and ownership and investment interests held by the physicians and their immediate family members.

The majority of states also have statutes or regulations similar to the federal Anti-Kickback Law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. Our activities may also be certain state laws regarding the privacy and security of health information that may not be preempted by HIPAA, as well as additional tracking and reporting obligations regarding payments to healthcare providers and marketing expenditures.

In addition to regulatory schemes that apply, or may in the future apply, to our business, we are or may become subject to various environmental, health and safety laws and regulations governing, among other things, laboratory procedures and any use and disposal by us of hazardous or potentially hazardous substances in connection with our research and development activities. We do not presently expect such environmental, health and safety laws or regulations to materially impact our present or planned future activities.

Coverage and Reimbursement

Sales of any of our product candidates that may be approved will depend, in part, on the extent to which the cost of the product will be covered by third party payors. Third party payors may limit coverage to an approved list of products, or formulary, which might not include all drug products approved by the FDA for an indication. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Any product candidates for which we obtain marketing approval may not be considered medically necessary or cost-effective by third party payors, and we may need to conduct expensive pharmacoeconomic studies in the future to demonstrate the medical necessity and/or cost effectiveness of any such product. Nonetheless, our product candidates may not be considered medically necessary or cost effective. The U.S. government, state legislatures and foreign governments have shown increased interest in implementing cost containment programs to limit government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Continued interest in and adoption of such controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals such as the product candidates we are developing.

Health Reform

The United States and some foreign jurisdictions are considering or have enacted a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. 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. By way of example, in March 2010, the ACA was signed into law, which intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add transparency requirements for the healthcare and health insurance industries, impose taxes and fees on the health industry and impose additional health policy reforms. With regard to pharmaceutical products, among other things, the ACA expanded and increased industry rebates for drugs covered under Medicaid programs and made changes to the coverage requirements under the Medicare prescription drug benefit. We continue to evaluate the effect that the ACA has on our business.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year effective April 1, 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2024

unless additional Congressional action is taken. 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.

In the coming years, additional legislative and regulatory changes could be made to governmental health programs that could significantly impact pharmaceutical companies and the success of our product candidates.

Employees

As of September 30, 2015, we have 21 full-time employees and four part-time employees, including 10 employees with M.D. or Ph.D. degrees. None of our employees is represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Facilities

We occupy approximately 1,560 rentable square feet of office and laboratory space in La Jolla, California under a sublease that expires in August 2016. We also occupy approximately 3,677 square feet of office space in Cambridge, Massachusetts under a lease that expires in August 2021. We believe that our facilities are sufficient to meet our current needs and that suitable additional space will be available as and when needed.

Legal Proceedings

We are not currently a party to, nor is our property the subject of, any material legal proceedings.

RISK FACTORS

Risks Related to Our Financial Position and Need For Additional Capital

We expect to incur losses over the next several years and may never achieve or maintain profitability.

We expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To date, we have financed our operations primarily through equity and debt financings. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- continue development of our product candidates;
- initiate new clinical trials for our product candidates;
- seek marketing approvals for our product candidates;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur increased costs as a result of continued operations as a public company.

To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including completing clinical trials of our product candidates, obtaining marketing approval from the Food and Drug Administration, or FDA, for these product candidates and manufacturing, marketing and selling those products for which we may obtain marketing approval. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. 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 decrease the value of the company and could impair our ability to raise capital, maintain our discovery and preclinical development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We are a clinical-stage company with no approved products and no historical product revenue. Consequently, we expect that our financial and operating results will vary significantly from period to period.

We are a clinical-stage company that has incurred losses since its inception and expect to continue to incur substantial losses in the foreseeable future. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty. We expect our actual financial condition and operating results to fluctuate significantly from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations include:

- the success of our clinical trials through all phases of clinical development;
- delays in the commencement, enrollment and timing of clinical trials;

- our ability to secure and maintain collaborations, licensing or other arrangements for the future development and/or commercialization of our product candidates, as well as the terms of those arrangements;
- our ability to obtain, as well as the timeliness of obtaining, additional funding to develop our product candidates;
- the results of clinical trials or marketing applications for product candidates that may compete with our product candidates;
- competition from existing products or new products that may receive marketing approval;
- potential side effects of our product candidates that could delay or prevent approval or cause an approved drug to be taken off the market;
- any delays in regulatory review and approval of our product candidates;
- our ability to identify and develop additional product candidates;
- the ability of patients or healthcare providers to obtain coverage or sufficient reimbursement for our products;
- our ability, and the ability of third parties such as clinical research organizations, or CROs, to adhere to clinical study and other regulatory requirements;
- the ability of third-party manufacturers to manufacture our product candidates and key ingredients needed to conduct clinical trials and, if approved, successfully commercialize our products;
- the costs to us, and our ability as well as the ability of any third-party collaborators, to obtain, maintain and protect our intellectual property rights;
- costs related to and outcomes of any future intellectual property litigation;
- our ability to adequately support future growth;
- our ability to attract and retain key personnel to manage our business effectively; and
- our ability to build our finance infrastructure and, to the extent required, improve our accounting systems and controls.

Accordingly, the likelihood of our success must be evaluated in light of many potential challenges and variables associated with a clinical-stage company, many of which are outside of our control, and past operating or financial results should not be relied on as an indication of future results. Fluctuations in our operating and financial results could cause our share price to decline. It is possible that in some future periods, our operating results will be above or below the expectations of securities analysts or investors, which could also cause our share price to decline.

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

We are an early-stage clinical development company. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates, undertaking preclinical studies and preparing for and undertaking clinical studies of our most advanced product candidate, tipifarnib. We have not yet demonstrated our ability to commence or successfully complete any clinical trials, including those clinical trials in support of FDA approval, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third-party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Medicines, on

average, take 10 to 15 years to be developed from the time they are discovered to the time they are available for treating patients. Consequently, any predictions you make about our future success or viability based on our short operating history to date may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We may in the future need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We will need to obtain substantial additional capital in connection with our continuing operations. Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we will need to raise additional capital in connection with our continuing operations. We expect to finance our cash needs through a combination of equity offerings and debt financings. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect rights of our stockholders 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.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

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

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, 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 carryforwards and other pre-change tax attributes to offset its post-change income may be limited. As a result of our recently completed private placement, or the Private Placement, and other transactions that have occurred over the past three years, we may have triggered an “ownership change” limitation. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, including as a result of this offering. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards and other pre-change tax attributes to offset U.S. federal and state taxable income may be subject to limitations, which could potentially result in increased future tax liability to us.

Risks Related to the Discovery and Development of Our Product Candidates

Our discovery, preclinical and clinical development is focused on the development of targeted therapeutics for patients with genetically defined cancers, which is a rapidly evolving area of science, and the approach we are taking to discover and develop drugs is novel and may never lead to marketable products.

The discovery and development of targeted drug therapeutics for patients with genetically defined cancers is an emerging field, and the scientific discoveries that form the basis for our efforts to discover and develop product candidates are relatively new. The scientific evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. The patient populations for our product candidates are not completely defined but are substantially smaller than the general treated cancer population, and we will need to screen and identify these patients. Successful identification of patients is dependent on several factors, including achieving certainty as to how specific genetic alterations respond to our product candidates and developing companion diagnostics to identify such genetic alterations. Furthermore, even if we

are successful in identifying patients, we cannot be certain that the resulting patient populations will be large enough to allow us to successfully commercialize our products and achieve profitability. Therefore, we do not know if our approach of treating patients with genetically defined cancers will be successful. If our approach is unsuccessful, our business will suffer.

Our research and development programs and product candidates are at an early stage of development. As a result we are unable to predict if or when we will successfully develop or commercialize our product candidates.

Our clinical-stage product candidate, tipifarnib, as well as our other pipeline assets are at an early stage of development and will require significant investment and regulatory approvals prior to commercialization. We currently have no product candidates beyond Phase 2 clinical trials. We commenced a Phase 2 clinical trial of tipifarnib in advanced solid tumors with the HRAS mutation in May 2015, commenced a Phase 2 clinical trial in PTCL in September 2015 and anticipate commencing a Phase 2 clinical trial in patients with lower risk MDS in the first half of 2016. Our development candidate in our extracellular-signal-regulated kinases, or ERK, program, KO-947, is in IND-enabling pre-clinical development, and our other programs, including our menin-MLL program, are in earlier stages of discovery and development. Each of our product candidates will require additional clinical and preclinical development, management of clinical, preclinical and manufacturing activities, obtaining regulatory approval, obtaining manufacturing supply, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenues from product sales. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. In addition, our product development programs contemplate the development of companion diagnostics. Companion diagnostics are subject to regulation as medical devices and we may be required to obtain marketing approval for accompanying companion diagnostics before we may commercialize our product candidates.

We cannot be certain that clinical development of tipifarnib or any of our other product candidates will be successful or that we will obtain regulatory approval or be able to successfully commercialize any of our product candidates and generate revenue. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and the clinical trial process may fail to demonstrate that our product candidates are safe and effective for their proposed uses. Any such failure could cause us to abandon further development of any one or more of our product candidates and may delay development of other product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. Tipifarnib has been studied in more than 5,000 oncology patients and was generally well tolerated and exhibited a manageable side effect profile. However, there is no guarantee that unacceptable side effects will not be identified in our clinical trials of tipifarnib. In prior studies tipifarnib demonstrated anti-cancer activity in certain patient subsets. However the anti-cancer activity observed was not sufficient to support marketing approval by the FDA in the indication in which it was sought. Although we are designing our clinical trials to target the patient subsets who we believe are most likely to benefit from treatment with tipifarnib, there is no guarantee that our clinical trials will be successful. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Any delay in, or termination of, our clinical trials will delay and possibly preclude the filing of any new drug applications, or NDAs, with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenue.

We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our

product candidates, our revenues will be dependent, in part, upon our or our future collaborators' ability to obtain regulatory approval of the companion diagnostics to be used with our product candidates, if required, as well as the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

Difficulty in enrolling patients could delay or prevent clinical trials of our product candidates. We may find it difficult to enroll patients in our ongoing Phase 2 clinical trial for tipifarnib in HRAS mutant solid tumors or our ongoing Phase 2 clinical trial for tipifarnib in PTCL.

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends in part on the speed at which we can recruit patients to participate in testing our product candidates, and we may experience delays in our clinical trials if we encounter difficulties in enrollment. The patient population for our product candidates is not completely defined, but it is substantially smaller than other cancer indications, because we are looking for the same type of genetic alterations across different tumor types and the number of patients with these alterations may be small. For example, with respect to tipifarnib, we do not know how many patients will have the target HRAS mutations that tipifarnib is expected to inhibit. With regard to our Phase 2 clinical trial in PTCL, there are a limited number of patients with PTCL, as well as a limited number of clinical centers that treat these patients, and there is substantial competition to recruit these patients to clinical trials.

In addition to the potentially small populations, the eligibility criteria of our clinical trials will further limit the pool of available study participants as we will require that patients have specific characteristics that we can measure or to assure their disease is either severe enough or not too advanced to include them in a study. Additionally, the process of finding and diagnosing patients may prove costly. We also may not be able to identify, recruit and enroll a sufficient number of patients to complete our clinical studies because of the perceived risks and benefits of the product candidate under study including the number and frequency of study required procedures and tests, the availability and efficacy of competing therapies and clinical trials, the proximity and availability of clinical study sites for prospective patients, and the patient referral practices of physicians. If patients are unwilling to participate in our studies for any reason, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of potential products may be delayed.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process, and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may harm our business, financial condition, and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates, including:

- unforeseen safety issues or adverse side effects;
- failure of our companion diagnostics in identifying patients;
- modifications to protocols of our clinical trials resulting from FDA or institutional review board, or IRB, decisions; and
- ambiguous or negative interim results of our clinical trials, or results that are inconsistent with earlier results.

We may not be successful in our efforts to build a pipeline of product candidates.

A key element of our strategy is to build a pipeline of small molecule product candidates that inhibit cancer signaling targets where we believe outcomes can be improved by using molecular diagnostics to identify those patients whose tumors have the genetic mutations most likely to respond to treatment, and to progress those

product candidates through clinical development for the treatment of a variety of different types of cancer. We may not be able to develop product candidates that are safe and effective inhibitors of all or any of these targets. Even if we are successful in building a product pipeline, the potential product candidates that we identify may not be suitable for clinical development for a number of reasons, including causing harmful side effects or demonstrating other characteristics that indicate a low likelihood of receiving marketing approval or achieving market acceptance. If our methods of identifying potential product candidates fail to produce a pipeline of potentially viable product candidates, then our success as a business will be dependent on the success of fewer potential product candidates, which introduces risks to our business model and potential limitations to any success we may achieve.

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 product candidates.

The risk of failure for all of our product candidates is high. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Further, the results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage 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 product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. For instance, the FDA issued a non-approval letter for tipifarnib in acute myelogenous leukemia, in June 2005. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval.

We may experience delays in our clinical trials and we do not know whether planned clinical trials will begin or enroll patients on time, need to be redesigned or be completed on schedule, if at all. If the FDA or IRBs have comments on our study plans for our ongoing or planned Phase 2 clinical trials of tipifarnib that we are required to address, such studies may be delayed. There can be no assurance that the FDA will not put any of our product candidates on clinical hold in the future. We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates. Clinical trials may be delayed, suspended or prematurely terminated because costs are greater than we anticipate or for a variety of reasons, such as:

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- delays in reaching, or failure to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- inability, delay, or failure in identifying and maintaining a sufficient number of trial sites, many of which may already be engaged in other clinical programs;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in having subjects complete a trial or return for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical studies and increased expenses associated with the services of our CROs and other third parties;

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to redesign or modify our clinical trial protocols, conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product 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;
- we may experience delays or difficulties in the enrollment of patients whose tumors harbor the specific genetic alterations that our product candidates are designed to target;
- 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;
- we may have difficulty partnering with experienced CROs that can screen for patients whose tumors harbor the applicable genetic alterations and run our clinical trials effectively;
- regulators or IRBs 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 supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; or
- there may be changes in governmental regulations or administrative actions.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these 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 product 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 that would reduce the potential market for our products or inhibit our ability to successfully commercialize our products;
- be subject to additional post-marketing restrictions and/or testing requirements; or
- have the product removed from the market after obtaining marketing approval.

Our product 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 need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

We may not be successful in advancing the clinical development of our product candidates, including tipifarnib.

In order to execute on our strategy of advancing the clinical development of our product candidates, we have designed our Phase 2 clinical trials of tipifarnib, and expect to design future trials, to include patients whose tumors harbor the applicable genetic alterations that we believe contribute to particular cancer subsets. Our goal

in doing this is to enroll patients who have the highest probability of responding to the drug, in order to show early and statistically significant evidence of clinical efficacy. If we are unable to include patients whose tumors harbor the applicable genetic alterations, or if our product fails to work as we expect, our ability to assess the therapeutic effect, seek participation in FDA expedited review and approval programs, including Breakthrough Therapy, Fast Track Designation, Priority Review and Accelerated Approval, or otherwise to seek to accelerate clinical development and regulatory timelines, could be compromised, resulting in longer development times, larger trials and a greater likelihood of not obtaining regulatory approval. In addition, because the natural history of different tumor types is variable, we will need to study our product candidates, including tipifarnib, in clinical trials specific for a given tumor type and this may result in increased time and cost. Even if our product candidate demonstrates efficacy in a particular tumor type, we cannot guarantee that any product candidate, including tipifarnib, will behave similarly in all tumor types, and we will be required to obtain separate regulatory approvals for each tumor type we intend a product candidate to treat. If any of our clinical trials are unsuccessful, our business will suffer.

Preclinical and clinical testing of tipifarnib that has been conducted to date may not have been performed in compliance with applicable regulatory standards, which could lead to increased costs or material delays for their further development.

We have only recently licensed the rights to develop our lead product candidate, tipifarnib, from Janssen Pharmaceutica NV, or Janssen, and the development of tipifarnib prior to our license was conducted wholly by Janssen or any third parties with which it had contracted. As a result, we were not involved with nor did we have any control over any of those development activities. Because we had no input on Janssen's development activities relating to tipifarnib, we may discover that all or certain elements of the trials and studies it performed have not been in compliance with applicable regulatory standards or have otherwise been deficient, particularly relative to current requirements as development of tipifarnib began in the 1990's. Any such deficiency in the prior development of tipifarnib may adversely affect our ability to obtain regulatory approval for tipifarnib. We and Janssen are in the process of transitioning the safety database from studies previously conducted by Janssen to us. We cannot assure you that our efforts to transition the database from Janssen will be completed on a timely basis, or at all. If we are unable to successfully complete the transition of Janssen's tipifarnib safety database to us on a timely basis, our development plans may be delayed, which could harm our business, prospects, financial condition and results of operations.

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

If our product candidates are associated with undesirable side effects in preclinical or clinical trials or have characteristics that are unexpected, we may need to interrupt, delay or abandon their development or limit 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. Tipifarnib has been studied in more than 5,000 oncology patients and was generally well tolerated and exhibited a manageable side effect profile. The most common hematologic adverse events of any grade were neutropenia (low white blood cell count), anemia and thrombocytopenia (low platelet count). The most common non-hematologic adverse events of any grade were gastrointestinal system disorders (nausea, anorexia, diarrhea and vomiting), fatigue and rash.

Treatment discontinuation across the prior tipifarnib clinical studies has been in the range of approximately 20-25%. There is no guarantee that additional or more severe side effects will not be identified through further clinical studies. Rights to develop tipifarnib in certain non-oncology indications have been granted by Janssen to EB Pharma, a subsidiary of Eiger BioPharmaceuticals. Janssen may grant rights to other non-oncology indications to other third parties. Undesirable side effects may be identified in clinical trials that EB Pharma or any other third party may conduct in non-oncology indications, which may negatively impact the development, commercialization or potential value of tipifarnib. These or other drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

Many compounds developed in the biopharmaceutical industry that initially showed promise in early-stage testing for treating cancer have later been found to cause side effects that prevented further development of the compound. Any of these occurrences may harm our business, financial condition and prospects significantly.

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

Because we have limited financial and managerial resources, we must focus on a limited number of research programs and product candidates and on specific indications. As a result, we may forego or delay pursuit of opportunities with other product 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 products or profitable market opportunities. Our spending on current and future discovery and preclinical development programs and product candidates for specific indications may not yield any commercially viable products.

Failure to successfully validate, develop and obtain regulatory approval for companion diagnostics for our product candidates could harm our drug development strategy and operational results.

As one of the central elements of our business strategy and clinical development approach, we seek to screen and identify subsets of patients with a genetic alteration who may derive meaningful benefit from our drug product candidates. To achieve this, certain of our programs may be dependent on the development and commercialization of a companion diagnostic. We intend to partner development of companion diagnostics for use in clinical trials and, if successful, for commercialization of our product candidates. Companion diagnostics are developed in conjunction with clinical programs for the associated product and are subject to regulation as medical devices. Each agency that approves a product will independently need to approve the companion diagnostic before or concurrently with its approval of the product candidate, and before a product can be commercialized. The approval of a companion diagnostic as part of the product label will limit the use of the product candidate to only those patients who express the specific genetic alteration it was developed to detect. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners or negotiating insurance reimbursement plans, all of which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as medical devices and require separate clearance or approval prior to their commercialization. To date, the FDA has required premarket approval of all companion diagnostics for cancer therapies. We and our third-party collaborators may encounter difficulties in developing and obtaining approval for these companion diagnostics. Any delay or failure by us or third- party collaborators to develop or obtain regulatory approval of a companion diagnostic could delay or prevent approval of our related product candidates.

Failure by us or our third-party collaborators to successfully commercialize companion diagnostics developed for use with our product candidates could harm our ability to commercialize these product candidates.

Even if we or our companion diagnostic collaborators successfully obtain regulatory approval for the companion diagnostics for our product candidates, our collaborators:

- may not perform their obligations as expected;
- may not pursue commercialization of companion diagnostics for our therapeutic product candidates that achieve regulatory approval;
- may elect not to continue or renew commercialization programs based on changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

- may not commit sufficient resources to the marketing and distribution of such product or products; and
- may terminate their relationship with us.

Additionally, we or our collaborators may encounter production difficulties that could constrain the supply of the companion diagnostics, affect the ease of use, affect the price or have difficulties gaining acceptance of the use of the companion diagnostics in the clinical community.

If companion diagnostics for use with our product candidates fail to gain market acceptance, our ability to derive revenues from sales of our product candidates could be harmed. If we or our collaborators fail to commercialize these companion diagnostics, we may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative diagnostic test for use in connection with our product candidates or do so on commercially reasonable terms, which could adversely affect and delay the development or commercialization of our product candidates.

Risks Related to Regulatory Approval of Our Product Candidates and Other Legal Compliance Matters

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

Our product candidates must be approved by the FDA pursuant to an NDA in the United States and by the European Medicines Agency, or EMA, and similar regulatory authorities outside the United States prior to commercialization. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have no 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. 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 product 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, the regulatory authorities. Our product 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 obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are 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 product candidate. 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 product application, may also cause delays in or prevent the approval of an application.

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

We may not be able to benefit from available regulatory exclusivity periods if another company obtains regulatory approval for tipifarnib before we do.

As the composition of matter patents covering tipifarnib expire in 2016 in the United States and in countries in Europe, our commercial strategy for tipifarnib relies on obtaining patents covering methods of use of tipifarnib and on non-patent regulatory exclusivity. In the United States, a pharmaceutical manufacturer may obtain five

years of non-patent exclusivity upon FDA approval of an NDA for a new chemical entity, or NCE, which is a drug that contains an active moiety that has not been approved by the FDA in any other NDA. An "active moiety" is defined as the molecule or ion responsible for the drug substance's physiological or pharmacologic action. During the five year exclusivity period, the FDA cannot accept for filing any ANDA seeking approval of a generic version of that drug or any Section 505(b)(2) NDA for the same active moiety and that relies on the FDA's findings regarding that drug, except that the FDA may accept an application for filing after four years if the follow-on applicant makes a paragraph IV certification. EB Pharma has licensed rights from Janssen to develop tipifarnib in certain indications outside of our exclusive field of oncology and Janssen may license rights to other non-oncology indications to other third parties. If EB Pharma or another third party obtains regulatory approval for tipifarnib in a non-oncology indication before we obtain regulatory approval in one of our oncology indications, the five year exclusivity period would commence on the date upon which EB Pharma or another third party obtains regulatory approval, and as a result, the period of regulatory exclusivity to which we may be entitled may be reduced or eliminated and the commercial prospects for tipifarnib would be harmed as a result.

Additionally, if EB Pharma or another third party obtains approval of tipifarnib for another indication outside of oncology, EB Pharma or the other third party may sell tipifarnib at a lower price, which could adversely affect the price at which we could sell tipifarnib for oncology indications.

We may not be able to obtain orphan drug exclusivity for the product candidates for which we seek it, which could limit the potential profitability of such product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it receives the designation, then the product is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication during the exclusivity period. The applicable period is seven years in the United States and ten years in Europe. The European 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, if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

We expect that we may in the future pursue an orphan drug designation for at least some of our product candidates, including tipifarnib. However, obtaining an orphan drug designation can be difficult, and we may not be successful in doing so for any of our product candidates. Even if we were to obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from the competition of different drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same drug for the same condition if the FDA concludes that the later drug is shown to be safer, more effective or makes a major contribution to patient care. The failure to obtain an orphan drug designation for any product candidates we may develop for the treatment of rare cancers, and/or the inability to maintain that designation for the duration of the applicable exclusivity period, could reduce our ability to make sufficient sales of the applicable product candidate to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

If we obtain an orphan drug designation and FDA approval of tipifarnib for an oncology indication, we would be entitled to seven years of marketing exclusivity for that orphan drug indication. However, if a competitor obtained approval of a generic form of tipifarnib for another indication, physicians would not be

prevented from prescribing the generic drug for the orphan indication during the period of marketing exclusivity. Such prescribing practices could adversely affect the sales of tipifarnib for the orphan indication.

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

We do not currently have Fast Track Designation for any of our product candidates but intend to seek such designation. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for the FDA Fast Track Designation. The FDA has broad discretion whether or not to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. Many drugs that have received Fast Track Designation have failed to obtain drug approval.

A Breakthrough Therapy Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

We do not currently have Breakthrough Therapy Designation for any of our product candidates, but we may seek such designation. A Breakthrough Therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor can help to identify the most efficient path for development.

Designation as a Breakthrough Therapy is within the discretion of the FDA. Accordingly, even if we believe, after completing early clinical trials, that one of our product candidates meets the criteria for designation as a Breakthrough Therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as Breakthrough Therapies, the FDA may later decide that such product candidates no longer meet the conditions for qualification and rescind such designations.

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

In order to market and sell our products in the European Union and many other jurisdictions, we or our third-party 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 product be approved for reimbursement before the product can be approved for sale in that country. We or our third-party 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. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

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

Our product candidates and the activities associated with their development and commercialization, including their testing, manufacture, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by FDA and other regulatory authorities. These requirements include, without limitation, submissions of safety and other post-marketing information and reports, registration and listing requirements, current good manufacturing practices, or cGMP, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities, requirements regarding the distribution of samples to physicians, tracking and reporting of payments to physicians and other healthcare providers, and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. 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 use of their products and if we promote our products beyond their approved indications, we may be subject to enforcement action for off-label promotion. 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 products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in

significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our relationships with customers and third-party payors and our general business operations will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings, among other penalties.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare providers, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities 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 a federal healthcare program such as Medicare and Medicaid;
- the federal false claims and civil monetary penalties laws, including the civil False Claims Act, 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, 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, imposes criminal and civil liability for, among other things, 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, or the HITECH Act, and its implementing regulations, also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payments Sunshine Act requires applicable manufacturers of certain 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 and other transfers of value to physicians and teaching hospitals, as well as ownership and investment interests held by physicians or their immediate family; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some 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 will 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, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product 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 product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

For example, 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 the ACA, 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 the ACA of importance to our potential product candidates and our business are the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a 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 manufacturers’ Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report information regarding 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 the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013, that due to subsequent legislative amendments, will stay in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief

Act of 2012, which, among other things, reduced Medicare payments to certain 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 the ACA, 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 product. Any reduction in reimbursement from Medicare or other government 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 products.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product 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.

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

In some countries, particularly the countries of the European Union, 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 product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement for our products 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 discovery, preclinical 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 Our Dependence on Third Parties

We expect to rely on third-party contractors and organizations to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We will rely on third party contractors, clinical data management organizations, independent contractors, medical institutions and clinical investigators to support our pre-clinical development activities and conduct our clinical trials, including our Phase 2 clinical trials of tipifarnib. These agreements may terminate for a variety of reasons, including a failure to perform by the third parties. If we are required to enter into alternative arrangements, our product development activities would be delayed.

We compete with many other companies, some of which may be our competitors, for the resources of these third parties. Large pharmaceutical companies often have significantly more extensive agreements and relationships with such third-party providers, and such third-party providers may prioritize the requirements of such large pharmaceutical companies over ours. The third parties on whom we rely may terminate their engagements with us at any time, which may cause delay in the development and commercialization of our product candidates. If any such third party terminates its engagement with us or fails to perform as agreed, we may be required to enter into alternative arrangements, which would result in significant cost and delay to our product development program. Moreover, our agreements with such third parties generally do not provide assurances regarding employee turnover and availability, which may cause interruptions in the research on our product candidates by such third parties.

Our reliance on these third parties to conduct our clinical trials 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 and other regulatory authorities require 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 are also 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.

Additionally, we expect to rely substantially on third-party data managers for our clinical trial data. There is no assurance that these third parties will not make errors in the design, management or retention of our data or data systems. There is no assurance that these third parties will pass FDA or other regulatory audits, which could delay or prevent regulatory approval.

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 product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

We will depend on third parties for the manufacture of our product 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 product candidates or products at an acceptable cost and quality, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate facilities for the manufacture of our product candidates, and we do not have any manufacturing personnel. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. Janssen has provided us with its existing inventory of clinical supply of tipifarnib. Janssen also provided us with its existing inventory of crude drug substance and bulk key intermediate for manufacture of drug substance for tipifarnib. A portion of the clinical supply of tablets of tipifarnib provided by Janssen have a non-uniform surface where the film coating on the tablets has worn away to a varying degree. We

believe this surface erosion is a cosmetic defect only and has no impact on patient safety or the effectiveness of the tablets, and an insignificant impact on taste masking, and that this clinical supply will support our ongoing and planned Phase 2 clinical trials for tipifarnib. However there is no guarantee that clinical trial participants will accept all the tablets and that our existing clinical supply will be sufficient for our ongoing and planned Phase 2 clinical trials or for any unanticipated extension of our Phase 2 clinical trials. If we are required to manufacture additional clinical supplies our Phase 2 clinical trials may be delayed. We rely, and expect to continue to rely, on third parties, for the manufacture of our other product candidates for preclinical and clinical testing. We will rely on third parties as well for commercial manufacture if any of our product candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts. We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials.

We also expect to rely on third-party manufacturers or third-party collaborators for the manufacture of commercial supply of any other product candidates for which our collaborators or we obtain marketing approval.

Any performance failure on the part of our existing or future manufacturers or distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

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 nonrenewal 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 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 product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Our product candidates and any products that we may develop may compete with other product candidates and products 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.

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

Risks Related to the Commercialization of Our Product Candidates

Even if any of our product 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 product 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. For example, current cancer treatments like chemotherapy and radiation therapy are well established in the medical community, and doctors may continue to rely on these treatments to the exclusion of our product candidates. In addition, physicians, patients and third-party payors may prefer other novel products to ours. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety and potential advantages and disadvantages compared to alternative treatments;
- our ability to offer our products 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 our marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement, including patient cost-sharing programs such as copays and deductibles;
- our ability to develop or partner with third-party collaborators to develop companion diagnostics;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products together with other medications.

We currently have no marketing and sales force. If we are unable to establish effective sales or marketing capabilities or enter into agreements with third parties to sell or market our product candidates if they obtain regulatory approval, we may not be able to effectively sell or market our product candidates, if approved, or generate product revenues.

We currently do not have a marketing or sales team for the marketing, sales and distribution of any of our product candidates that are able to obtain regulatory approval. In order to commercialize any product candidates, we must build on a territory-by-territory basis marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. If our product candidates receive regulatory approval, we intend to establish an internal sales or marketing team with technical expertise and supporting distribution capabilities to commercialize our product candidates, which will be expensive and time consuming and will require significant attention of our executive officers to manage. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of any of our products that we obtain approval to market. With respect to the commercialization of all or certain of our product candidates, we may choose to collaborate, either globally or on a territory-by-territory basis, with third parties that have direct sales forces and established distribution systems, either to augment our own sales force and distribution systems or in lieu of our own sales force and distribution systems. If we are unable to enter into such arrangements when needed on acceptable terms or at all, we may not be able to successfully commercialize any of our product candidates that receive regulatory approval or any such commercialization may experience delays or limitations. If we are not successful in commercializing our product candidates, either on our own or through collaborations with one or more third parties, our future product revenue will suffer and we may incur significant additional losses.

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Specifically, there are a large number of companies developing or marketing treatments for cancer, including many major pharmaceutical and biotechnology companies, which may directly compete with tipifarnib, KO-947 and any other future product candidates. See “Description of Our Business—Competition.”

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market and or slow our regulatory approval. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. Generic products are currently on the market for the indications that we are pursuing, and additional products are expected to become available on a generic basis over the coming years. If our product candidates achieve marketing approval, we expect that they will be priced at a significant premium over competitive generic products.

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 products 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 and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties 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.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain coverage and adequate reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

The availability and extent of coverage and reimbursement by governmental and private payors is essential for most patients to be able to afford expensive treatments. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors often, but not always, follow CMS's decisions regarding coverage and reimbursement. It is difficult to predict what CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. We or our collaborators may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Nonetheless, our product candidates may not be considered medically necessary or cost-effective.

Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market.

In addition to CMS and private payors, professional organizations such as the National Comprehensive Cancer Network and the American Society of Clinical Oncology can influence decisions about reimbursement for new medicines by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our products.

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

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

- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;

- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Our current product liability insurance coverage 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 product 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 intellectual property protection for our technology and products, or if the scope of the intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be impaired.

We intend to rely upon a combination of regulatory exclusivity periods, patents, trade secret protection, confidentiality agreements, and license agreements to protect the intellectual property related to our current product candidates and development programs. If the breadth or strength of protection provided by any patents, patent applications or future patents we may own, license, or pursue with respect to any of our current or future product candidates or products is threatened, it could threaten our ability to commercialize any of our current or future product candidates or products. Further, if we encounter delays in our development efforts, the period of time during which we could market any of our current or future product candidates or products under any patent protection we obtain would be reduced. Given the amount of time required for the development, testing and regulatory review of new product candidates or products, patents protecting such candidates might expire before or shortly after such product candidates or products are commercialized.

Our patent rights may not protect our patent protected products and product candidates if competitors devise ways of making products that compete with us without legally infringing our patent rights. For example, our patent rights in tipifarnib are limited in ways that affect our ability to exclude third parties from competing against us. In particular, the patent term for the composition of matter patents covering the active pharmaceutical ingredient, or API, of tipifarnib expire in 2016 in the United States, countries in Europe and other jurisdictions. Composition of matter patents on APIs are generally considered to be the strongest form of intellectual property protection because such patents provide protection without regard to any particular method of use or manufacture or formulation of the API used. Patent term extension may be available in the U.S. to account for regulatory delays in obtaining human marketing approval for tipifarnib; however, only one patent may be extended per marketed compound. Under our license agreement with Janssen, we and Janssen agree to cooperate in obtaining available patent term extensions. We and Janssen may not reach agreement and no patent term extension may be obtained. Additionally, the applicable authorities, including the U.S. Patent and Trademark Office, or U.S. PTO, and the FDA, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to patents, or may grant more limited extensions than requested. If this occurs, our competitors who obtain the requisite regulatory approval can offer products with the same API as tipifarnib so long as the competitors do not infringe any method of use or formulations patents that we may hold. Competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

We expect that following expiration of composition of matter patents and any regulatory exclusivity we are able to obtain, competitors may manufacture and sell generic versions of tipifarnib, at a lower price, which would reduce tipifarnib's revenues. In certain jurisdictions, legislation mandates generic substitution for brand name drugs.

We depend on our licensors to prosecute and maintain patents and patent applications that are material to our business. Any failure by our licensors to effectively protect these intellectual property rights could adversely impact our business and operations.

We have licensed patent rights from third parties for some of our development programs, including tipifarnib from Janssen and compounds in our menin-MLL program from the University of Michigan. As a licensee of third parties, we rely on these third parties to file and prosecute patent applications and maintain patents and otherwise protect the licensed intellectual property under some of our license agreements. We have not had and do not have primary control over these activities for certain of our patents or patent applications and other intellectual property rights. We cannot be certain that such activities by third parties have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. Pursuant to the terms of the license agreements with some of our licensors, the licensors may have the right to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents and even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. Even if we are not a party to these legal actions, an adverse outcome could harm our business because it might prevent us from continuing to license intellectual property that we may need to operate our business.

With respect to the patent portfolio for tipifarnib, which is in-licensed from Janssen, Janssen maintains rights to prosecute and maintain patents and patent applications within the portfolio as well as to assert such patents against infringers within and outside the scope of our license, and to defend such patents against claims of invalidity and unenforceability. Although we have rights to consult with Janssen on actions taken as well as back-up rights of prosecution and enforcement, rights to tipifarnib granted to another licensee, such as EB Pharma, could potentially influence Janssen's interests in the exercise of its prosecution, maintenance and enforcement rights in a manner that may favor the interests of such other licensee as compared with us.

If we breach any of the agreements under which we license from third parties the commercialization rights to our product candidates, we could lose license rights that are important to our business and our operations could be materially harmed.

We have in-licensed from Janssen the use, development and commercialization rights in oncology indications for our lead product candidate, tipifarnib. We have also in-licensed rights to potential product candidates in other programs in our pipeline. As a result, our current business plans are dependent upon our satisfaction of certain conditions to the maintenance of the Janssen agreement and the rights we license under it and our other in-license agreements. The Janssen license agreement provides that we are subject to diligence obligations relating to the commercialization and development of tipifarnib, milestone payments, royalty payments and other obligations. If we fail to comply with any of the conditions or obligations or otherwise breach the terms of our license agreement with Janssen, or any of our other license agreements or license agreements we may enter into on which our business or product candidates are dependent, Janssen or other licensors may have the right to terminate the applicable agreement in whole or in part and thereby extinguish our rights to the licensed technology and intellectual property and/or any rights we have acquired to develop and commercialize certain product candidates, including, with respect to our license agreement with Janssen, tipifarnib. The loss of the rights licensed to us under our license agreement with Janssen, or our other license agreements or any future license agreement that we may enter granting us rights on which our business or

product candidates are dependent, would eliminate our ability to further develop the applicable product candidates and would materially harm our business, prospects, financial condition and results of operations.

The patent applications of pharmaceutical and biotechnology companies involve highly complex legal and factual questions, which, if determined adversely to us, could negatively impact our patent position.

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. For example, India and China do not allow patents for methods of treating the human body. 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 owned or licensed 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 which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

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. PTO 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.

Moreover, we may be subject to a third-party preissuance submission of prior art to the U.S. PTO, 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 technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products 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 product candidates.

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

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or

commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the U.S. PTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The U.S. PTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

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

Because competition in our industry is intense, competitors may infringe or otherwise violate our issued patents, patents of our licensors 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 in question. 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 also elect to enter into license agreements in order to settle patent infringement claims or to resolve disputes prior to litigation, and any such license agreements may require us to pay royalties and other fees that could be significant. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure.

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 product candidates and use our proprietary technologies 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 products and technology, including interference or derivation proceedings before the U.S. PTO. 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 products and technology. 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 technology or product. 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 product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may not be successful in obtaining or maintaining necessary rights for our development pipeline through acquisitions and in-licenses.

Presently we have rights to intellectual property to develop tipifarnib in the field of oncology, including patents and patent applications we exclusively licensed from Janssen, as well as exclusive worldwide licenses for all therapeutic indications for certain compounds in our other programs, including in our menin-MLL program. Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights. Additionally, a companion diagnostic may require that we or a third-party collaborator developing the diagnostic acquire use or proprietary rights held by third parties. We may be unable to acquire or in-license any compositions, methods of use, or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we may collaborate with U.S. and foreign academic institutions to accelerate our discovery and preclinical development work under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

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 some of our technology and product candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these 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 seek to protect our confidential proprietary information, in part, by entering into confidentiality and invention or patent assignment agreements with our employees and consultants, however, we cannot be certain that such agreements have been entered into with all relevant parties. Moreover, to the extent we enter into such agreements, 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.

Risks Related to Employee Matters, Managing Growth and Macroeconomic Conditions

We currently have a limited number of employees, are highly dependent on our Chief Executive Officer and our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are an early-stage clinical development company with a limited operating history, and, as of September 30, 2015, we had only 25 employees. We are highly dependent on the expertise of Troy E. Wilson, our President and Chief Executive Officer, Antonio Gualberto, our Chief Medical Officer, Yi Liu, our Chief Scientific Officer, and Pingda Ren, our Senior Vice President, Chemistry and Pharmaceutical Sciences, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment letter agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. Additionally, Dr. Wilson currently also serves as President and Chief Executive Officer of Avidity NanoMedicines, LLC. As a result, Dr. Wilson is not able to devote all of his business time and attention to our business. Conflicts may arise in the future if there are competing demands on Dr. Wilson's time and attention and our business may be harmed as a result.

Recruiting and retaining qualified scientific, clinical, manufacturing and 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 of and commercialize products. Competition to hire from this limited pool 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 discovery and preclinical 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.

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

Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including our ability to raise additional capital when needed on acceptable terms, if at all. This is particularly true in Europe, which is undergoing a continued severe economic crisis. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

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

Despite the implementation of security measures, our internal computer systems and those of our CROs, collaborators and third-parties on whom we rely are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and we may incur substantial costs to attempt to recover or reproduce the data. If any disruption or security breach resulted in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and/or the further development of our product candidates could be delayed.

Our operations are vulnerable to interruption by natural disasters, power loss, terrorist activity and other events beyond our control, the occurrence of which could materially harm our business.

Businesses located in California have, in the past, been subject to electrical blackouts as a result of a shortage of available electrical power, and any future blackouts could disrupt our operations. We are vulnerable to a major earthquake, wildfire and other natural disasters, and we have not undertaken a systematic analysis of the potential consequences to our business as a result of any such natural disaster and do not have an applicable recovery plan in place. We do not carry any business interruption insurance that would compensate us for actual losses from interruption of our business that may occur, and any losses or damages incurred by us could cause our business to materially suffer.

Risks Related to Ownership of our Common Stock and this Offering

Our stock price may fluctuate significantly and you may have difficulty selling your shares based on current trading volumes of our stock.

In connection with this offering, we have applied to have our common stock listed on the NASDAQ Global Select Market. Since September 16, 2015, shares of our common stock have been quoted for trading on the OTCQB in very limited volume and, as of October 19, 2015, the price per share of our common stock has ranged from a high of $15.00 to a low of $14.00. Prior to September 16, 2015, our common stock was not publicly-traded. We cannot predict the extent to which investor interest in our company will lead to the development of an active trading market on that stock exchange or any other exchange in the future. We have several stockholders, including affiliated stockholders, who hold substantial blocks of our stock. Sales of large numbers of shares by any of our large stockholders could adversely affect our trading price, particularly given our small historic

trading volumes. If stockholders holding shares of our common stock sell, indicate an intention to sell, or if it is perceived that they will sell, substantial amounts of their common stock in the public market, the trading price of our common stock could decline. Moreover, if there is no active trading market or if the volume of trading is limited, holders of our common stock may have difficulty selling their shares.

The price of our common stock may be volatile and may be influenced by numerous factors, some of which are beyond our control.

The market for our common stock could fluctuate substantially due to a variety of factors, some of which may be beyond our control. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this prospectus, these factors include:

- the product candidates we seek to pursue, and our ability to obtain rights to develop, commercialize and market those product candidates;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- actual or anticipated adverse results or delays in our clinical trials;
- our failure to commercialize our product candidates, if approved;
- unanticipated serious safety concerns related to the use of any of our product candidates;
- adverse regulatory decisions;
- additions or departures of key scientific or management personnel;
- changes in laws or regulations applicable to our product candidates, including without limitation clinical trial requirements for approvals;
- disputes or other developments relating to patents and other proprietary rights and our ability to obtain patent protection for our product candidates;
- our dependence on third parties, including CROs as well as our potential partners that produce companion diagnostic products;
- failure to meet or exceed any financial guidance or expectations regarding development milestones that we may provide to the public;
- actual or anticipated variations in quarterly operating results, liquidity or other indicators of our financial condition;
- failure to meet or exceed the estimates and projections of the investment community;
- overall performance of the equity markets and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies;
- market conditions or trends in the biotechnology and biopharmaceutical industries;
- introduction of new products offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to maintain an adequate rate of growth and manage such growth;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future, or the perception that such sales could occur;

- trading volume of our common stock;
- ineffectiveness of our internal control over financial reporting or disclosure controls and procedures;
- general political and economic conditions;
- effects of natural or man-made catastrophic events; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, and the stocks of small-cap biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. These events may also lead to securities litigation, which can be expensive and time-consuming to defend, regardless of the merit or outcome. The realization of any of the above risks or any of a broad range of other risks, including those described in these "Risk Factors," could have a dramatic and material adverse impact on the market price of our common stock.

There is a very limited public trading history for our common stock, so the public offering price in this offering does not reflect a reliable public trading price.

Since September 16, 2015, shares of our common stock have been quoted for trading on the OTCQB in very limited volume. Prior to September 16, 2015, our common stock was not publicly-traded. Because there is a very limited public trading history for our common stock, the public offering price in this offering is not a reliable indicator of the price at which our common stock will be publicly traded once it is listed on the NASDAQ Global Select Market.

We have broad discretion in the use of our cash and may not use our cash effectively, which could adversely affect our results of operations.

Our management has broad discretion in the application of our cash resources, including the net proceeds from this offering. Because of the number and variability of factors that will determine our use of our cash resources, our management might not apply our cash in ways that ultimately increase the value of our common stock. The failure by our management to apply our cash effectively could harm our business. Pending their use, we may invest our cash in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply our cash in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

Purchasers in this offering will experience immediate and substantial dilution in the book value of their investment.

The assumed public offering price is substantially higher than the net tangible book value per share of our common stock based on the total value of our tangible assets less our total liabilities immediately following this offering. Therefore, if you purchase shares of our common stock in this offering, you will experience immediate and substantial dilution of $ per share in the price you pay for shares of our common stock as compared to its pro forma as adjusted net tangible book value giving effect to this offering, assuming the issuance and sale of shares of our common stock at the assumed public offering price of $ per share, which is the last reported public sale price of our common stock on , 2015. To the extent outstanding options to purchase shares of common stock that are in-the-money are exercised, there will be further dilution. For further information on this calculation, see "Dilution" elsewhere in this prospectus.

The designation of our common stock as a "penny stock" would limit the liquidity of our common stock.

Our common stock may be deemed a "penny stock" (as that term is defined under Rule 3a51-1 of the Exchange Act) in any market that may develop in the future. Generally, a "penny stock" is a common stock that

is not listed on a securities exchange and trades for less than $5.00 per share. Prices often are not available to buyers and sellers and the market may be very limited. Penny stocks in start-up companies are among the riskiest equity investments. Broker-dealers who sell penny stocks must provide purchasers of these stocks with a standardized risk-disclosure document prepared by the SEC. The document provides information about penny stocks and the nature and level of risks involved in investing in the penny stock market. A broker must also provide purchasers with bid and offer quotations and information regarding broker and salesperson compensation and make a written determination that the penny stock is a suitable investment for the purchaser and obtain the purchaser's written agreement to the purchase. Many brokers choose not to participate in penny stock transactions. Because of the penny stock rules, there may be less trading activity in penny stocks in any market that develops for our common stock in the future and stockholders are likely to have difficulty selling their shares.

FINRA sales practice requirements may limit a stockholder's ability to buy and sell our stock.

The Financial Industry Regulatory Authority, or FINRA, has adopted rules requiring that, in recommending an investment to a customer, a broker-dealer must have reasonable grounds for believing that the investment is suitable for that customer. Prior to recommending speculative or low-priced securities to their non-institutional customers, broker-dealers must make reasonable efforts to obtain information about the customer's financial status, tax status, investment objectives and other information. Under interpretations of these rules, FINRA has indicated its belief that there is a high probability that speculative or low-priced securities will not be suitable for at least some customers. If these FINRA requirements are applicable to us or our securities, they may make it more difficult for broker-dealers to recommend that at least some of their customers buy our common stock, which may limit the ability of our stockholders to buy and sell our common stock and could have an adverse effect on the market for and price of our common stock.

Because we became a reporting company under the Exchange Act by means other than a traditional underwritten initial public offering, we may not be able to continue to attract the attention of research analysts at major brokerage firms.

Because we did not become a reporting company by conducting an underwritten initial public offering of our common stock, security analysts of brokerage firms may not continue to provide coverage of our company. In addition, investment banks may be less likely to agree to underwrite secondary offerings on our behalf than they might if we became a public reporting company by means of an underwritten initial public offering, because they may be less familiar with our company as a result of more limited coverage by analysts and the media, and because we became public at an early stage in our development. The failure to receive research coverage or support in the market for our shares will have an adverse effect on our ability to develop a liquid market for our common stock.

The resale of shares covered by our effective shelf registration statement could adversely affect the market price of our common stock in the public market, should one develop, which result would in turn negatively affect our ability to raise additional equity capital.

The sale, or availability for sale, of our common stock in the public market may adversely affect the prevailing market price of our common stock and may impair our ability to raise additional capital by selling equity or equity-linked securities. We filed a registration statement with the SEC, which was declared effective on July 21, 2015, to register the resale of 14,279,820 shares of our common stock, which represents substantially all of the shares of our common stock issued in connection with the Merger. The shelf registration statement permits the resale of these shares at any time, subject to applicable lock-up restrictions described in the "Certain Relationships and Related Person Transactions—Lock-Up Provisions in Registration Rights Agreement" section of this prospectus. The resale of a substantial number of shares of our common stock in the public market could adversely affect the market price for our common stock and make it more difficult for you to sell shares of our common stock at times and prices that you feel are appropriate. Furthermore, we expect that, because there are a

large number of shares registered pursuant to the shelf registration statement, the selling stockholders named in such registration statement will continue to offer shares covered by the shelf registration statement for a significant period of time, the precise duration of which cannot be predicted. Accordingly, the adverse market and price pressures resulting from an offering pursuant to the shelf registration statement may continue for an extended period of time and continued negative pressure on the market price of our common stock could have a material adverse effect on our ability to raise additional equity capital.

A significant portion of our total outstanding shares of common stock is 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 in the future. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. Our directors, executive officers and certain stockholders who own an aggregate of approximately 5,205,061 shares of our common stock are subject to a lock-up agreement with us contained in the Registration Rights Agreement and/or a separate lock-up agreement with the underwriters pursuant to which these persons have agreed, subject to specified exceptions, not to sell, transfer, dispose of, contract to sell, sell any option or contract to purchase, or otherwise transfer or dispose of, directly or indirectly, without the written consent of the underwriters, any shares of our common stock or any securities convertible into or exercisable or exchangeable for shares of our common stock for a period of 180 days after the date of this prospectus. Once these lock-up provisions expire, these shares, which are registered on our shelf registration statement that was declared effective on July 21, 2015, can be freely sold in the public market, which could cause the market price of our common stock to drop significantly.

At any time when our shelf registration statement may not be available, the liquidity and price of our common stock could significantly decline and it may be difficult for you to sell your shares, if at all.

Although we filed a registration statement with the SEC, which was declared effective on July 21, 2015, to register the resale of 14,279,820 shares of our common stock, which represents substantially all of the shares of our common stock issued in connection with the Merger, and the shelf registration statement permits the resale of these shares at any time, subject to applicable lock-up restrictions described in the "Certain Relationships and Related Person Transactions—Lock-Up Provisions in Registration Rights Agreement" section of this prospectus, such registration may not be available at all times. We are not currently eligible to register the resale of our common stock included in our shelf registration statement on Form S-3, and, therefore, have registered the resale of these securities on Form S-1. As a result, under certain circumstances, we must update the registration statement for the resale of such shares of our common stock by filing post-effective amendments to the registration statement that will not be effective until each is declared effective by the SEC. Between the time it is determined that the registration statement must be updated by a post-effective amendment and the time the SEC declares the applicable post-effective amendment effective, the registration statement will not be available for use and the price of our common stock could decline during that time. The SEC has broad discretion to determine whether any registration statement (including any post-effective amendment) will be declared effective and may delay or deny the effectiveness of any registration statement or post-effective amendment filed by us for a variety of reasons. Therefore, at any time when our shelf registration statement may not be available, the liquidity and price of our common stock could significantly decline and it may be difficult for you to sell your shares, if at all.

We will incur increased costs associated with, and our management will need to devote substantial time and effort to, compliance with public company reporting and other requirements.

As a public company, and particularly if and after we cease to be an "emerging growth company" or a "smaller reporting company," we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the rules and regulations of the SEC and NASDAQ impose numerous requirements on public companies, including requirements relating to our corporate governance practices, with

which we will need to comply. Further, we are required to, among other things, file annual, quarterly and current reports with respect to our business and operating results. Our management and other personnel will need to devote substantial time to gaining expertise regarding operations as a public company and compliance with applicable laws and regulations, and our efforts and initiatives to comply with those requirements could be expensive.

Prior Kura was not subject to requirements to establish, and did not establish, internal control over financial reporting and disclosure controls and procedures prior to the Merger. Our management team and board of directors will need to devote significant efforts to maintaining adequate and effective disclosure controls and procedures and internal control over financial reporting in order to comply with applicable regulations, which may include hiring additional legal, financial reporting and other finance and accounting staff and engaging consultants to assist in designing and implementing such procedures. Additionally, any of our efforts to improve our internal controls and design, implement and maintain an adequate system of disclosure controls may not be successful and will require that we expend significant cash and other resources.

If we fail to maintain proper and effective internal controls, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, our ability to operate our business and investors' views of us.

We are required to comply with certain aspects of the Sarbanes-Oxley Act. Section 404 of the Sarbanes-Oxley Act requires public companies to conduct an annual review and evaluation of their internal controls and attestations of the effectiveness of internal controls by independent auditors. Ensuring that we have adequate internal financial and accounting controls and procedures in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that will need to be evaluated frequently. Our failure to maintain the effectiveness of our internal controls in accordance with the requirements of the Sarbanes-Oxley Act could have a material adverse effect on our business. We could lose investor confidence in the accuracy and completeness of our financial reports, which could have an adverse effect on the price of our common stock. In addition, if our efforts to comply with new or changed laws, regulations, and standards differ from the activities intended by regulatory or governing bodies due to ambiguities related to practice, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

We are an emerging growth company and a smaller reporting company, which will allow us to take advantage of certain reduced disclosure obligations as a public reporting company that may make our common stock less attractive to investors.

We are an "emerging growth company" under the JOBS Act and a "smaller reporting company" as defined in applicable rules under the Exchange Act. As an emerging growth company and a smaller reporting company, we are eligible to take advantage of certain extended accounting standards and exemptions from various reporting requirements that are not available to public reporting companies that do not qualify for those classifications. For instance, we are exempt from any requirement that may be adopted by the Public Company Accounting Oversight Board, or PCAOB, regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and financial statements, commonly known as an "auditor discussion and analysis"; we are not required to hold a nonbinding advisory stockholder vote on executive compensation or any golden parachute payments not previously approved by stockholders; we are not required to comply with the requirement of auditor attestation of management's assessment of internal control over financial reporting, which is required for some other public reporting companies by Section 404 of the Sarbanes-Oxley Act of 2002; we are eligible for reduced disclosure obligations regarding executive compensation in our periodic and annual reports; and we are eligible for reduced financial statement disclosure in any registration statements under the Securities Act or reports under the Exchange Act that we may file. For as long as we continue to be an emerging growth company and/or a smaller reporting company, which we anticipate will be for the foreseeable future, we expect that we will take advantage of the reduced disclosure obligations

available to us as a result of those respective classifications. As a result, our publicly available disclosure may not be as robust or comprehensive as that of other public reporting companies that do not qualify for those classifications.

Management and our board of directors beneficially own a substantial amount of our outstanding equity securities and will be able to exert substantial control over us.

Our executive officers and directors beneficially own a substantial percentage of our outstanding equity securities. Accordingly, if they act as a group, our executive officers and directors will be able to significantly influence all business decisions, including with respect to such matters as amendments to our charter, other fundamental corporate transactions such as mergers, asset sales and the sale of us, and otherwise will be able to significantly influence our business and affairs.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, outstanding stock options or otherwise, could result in dilution to the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors in a prior transaction may be materially diluted by subsequent sales. Additionally, any such sales may result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to those of holders of our common stock. Further, any future sales of our common stock by us or resales of our common stock by our existing stockholders could cause the market price of our common stock to decline.

Pursuant to our 2014 plan, we are authorized to grant equity awards consisting of shares of our common stock to our employees, directors and consultants. As of September 30, 2015, we had 621,500 shares of common stock reserved for future issuance under our 2014 plan and options to purchase up to an aggregate of 410,000 shares of common stock outstanding, each at an exercise price of $6.32 per share. In addition, as of September 30, 2015, we had 25,000 shares of common stock reserved for future issuance under our 2015 ESPP. Any future grants of options, warrants or other securities exercisable or convertible into our common stock, or the exercise or conversion of such shares, and any sales of such shares in the market, could have an adverse effect on the market price of our common stock.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than 66 ⅔% of all outstanding shares of our voting stock then entitled to vote in the election of directors;

- a requirement of approval of not less than 66 ⅔% of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

We do not intend to pay cash dividends on our capital stock in the foreseeable future.

We have never declared or paid any dividends on our common stock and do not anticipate paying any dividends in the foreseeable future. Any future payment of cash dividends in the future would depend on our financial condition, contractual restrictions, solvency tests imposed by applicable corporate laws, results of operations, anticipated cash requirements and other factors and will be at the discretion of our board of directors. Our stockholders should not expect that we will ever pay cash or other dividends on our outstanding capital stock.