AMAG PHARMACEUTICALS INC.
Form 10-K
February 18, 2015

Use these links to rapidly review the document

Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the Fiscal Year Ended December 31, 2014

or

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

For the transition period from to
Commission file number 001-10865

AMAG Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**04-2742593**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)
1100 Winter Street Waltham, Massachusetts	**02451**
(Address of Principal Executive Offices)	(Zip Code)

(617) 498-3300
(Registrant's Telephone Number, Including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock, par value $0.01 per share	NASDAQ Global Select Market

Preferred Share Purchase Rights

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. **Yes ý No o**

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. **Yes o No ý**

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ý

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

Large accelerated filer o	Accelerated filer ý	Non-accelerated filer o (Do not check if a smaller reporting company)	Smaller Reporting Company o

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

The aggregate market value of the registrant's voting stock held by non-affiliates as of June 30, 2014 was approximately $453,000,000 based on the closing price of $20.72 of the Common Stock of the registrant as reported on the NASDAQ Global Select Market on such date. As of February 4, 2015, there were 25,615,978 shares of the registrant's Common Stock, par value $0.01 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Definitive Proxy Statement that the registrant intends to file in connection with the solicitation of proxies for the Annual Meeting of Stockholders within 120 days of the end of the fiscal year ended December 31, 2014 are incorporated by reference into Part III of this Annual Report on Form 10-K.

Table of Contents

AMAG PHARMACEUTICALS, INC.
FORM 10-K
FOR THE YEAR ENDED DECEMBER 31, 2014
TABLE OF CONTENTS

Table of Contents

PART I

Except for the historical information contained herein, the matters discussed in this Annual Report on Form 10-K may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Annual Report on Form 10-K terminology such as "may," "will," "could," "should," "would," "expect," "anticipate," "continue," "believe," "plan," "estimate," "intend" or other similar words and expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.

Unless the context suggests otherwise, references to "Feraheme" refer to both Feraheme (the trade name for ferumoxytol in the U.S. and Canada) and Rienso (the trade name for ferumoxytol in the EU and Switzerland).

Examples of forward-looking statements contained in this report include, without limitation, statements regarding the following: plans to pursue opportunities to make new advancements in patients' health and to enhance treatment accessibility; plans to diversify and grow our product portfolio; expectations and plans as to regulatory and commercial developments and activities, including with regard to label changes for Feraheme and our plan to work with the FDA to finalize the Feraheme label, the pursuit, if any, of a broader indication for Feraheme, commercialization efforts, if any, for Feraheme outside of the U.S., requirements and initiatives for clinical trials and studies, post-approval commitments for our products and the lifecycle management program for Makena; expectations as to what impact recent regulatory developments will have on our business and competition, including recent changes to our product information and label, and other risk minimization measures in the EU; the market opportunities for each of our products; the amount of resources that we intend to dedicate to the commercialization of Feraheme; expected transitioning activities with Takeda Pharmaceutical Company Limited ("Takeda") and the impact of Takeda's withdrawal of the application for Type II Variation to vary the marketing authorization for Rienso in the EU or our mutual decision with Takeda to initiate withdrawal of Rienso's current marketing authorizations in the EU and Switzerland; our expectations regarding the results of discussions with Health Canada, including our belief that approval of the broader indication for Feraheme in such territory is unlikely without additional clinical data and the possibility that Health Canada will impose additional restrictions on the current Feraheme CKD indication; beliefs about compounding pharmacies and the impact of recent legislation focused on compounding pharmacies; beliefs regarding possible entry of generic competitors, including timing, for both Makena and Feraheme; plans regarding our sales and marketing initiatives, including our contracting strategy and efforts to increase patient compliance and access; the impact of government regulations on our business and the pricing and reimbursement for our products, including the Branded Drug Fee under the Healthcare Reform Act and the Medicare reimbursement rate and estimates for Medicaid rebates; our expectations regarding the timing for enrollment in and commencement of our clinical trials and studies; our expectation of costs to be incurred in connection with and revenue sources to fund our future operations; our expectation for the patient populations for Makena and Feraheme; our expectations regarding the contribution of Makena and Feraheme sales to the funding of our on-going operations; the magnitude of costs and timing of integrating Lumara Health into our current business; expectations regarding the manufacture of all drug substance and drug products at our third-party manufacturers; plans to increase headcount; our expectations regarding customer returns and other revenue-related reserves and accruals; estimates regarding our net operating loss carryforwards and other tax attributes; initiatives to improve the reputation of Makena and educate industry participants on the benefits of Makena; the impact of accounting pronouncements; the effect of product price increases; expected increases in research and development expenses; expectations regarding our financial results, including revenues, cost of product sales, selling, general and administrative expenses, restructuring costs and net income (expense); the impact on revenues from the termination of our license arrangement with Takeda; our investing activities; expectations regarding our cash, cash equivalents and investments balances and capital needs; the impact and outcomes of our legal proceedings; our beliefs regarding the validity of our

Table of Contents

ferumoxytol patent portfolio; estimates and beliefs related to our debt, including our Convertible Notes and the Term Loan Facility; expected customer mix and utilization rates for our products; the impact of volume rebates and other incentives; provider purchase patterns and use of competitive products; the valuation of certain intangible assets, goodwill, contingent consideration, debt and other assets and liabilities, including our methodology and assumptions regarding fair value measurements; our gross to net sales adjustments; our expectations regarding competitive pressures and the impact on growth on our product sales; our plans regarding manufacturing; the timing of our planned research and development projects; the manner in which we intend or are required to settle the conversion of our Convertible Notes; plans to submit the NOL Amendment to our Rights Plan to our shareholders for approval; and our expectations for our cash, revenue, cash equivalents and investments balances and information with respect to any other plans and strategies for our business. Our actual results and the timing of certain events may differ materially from the results discussed, projected, anticipated or indicated in any forward-looking statements.

Any forward-looking statement should be considered in light of the factors discussed in Part I, Item 1A below under "Risk Factors" and elsewhere in this Annual Report on Form 10-K. We caution readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the U.S. Securities and Exchange Commission, to publicly update or revise any such statements to reflect any change in company expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

ITEM 1. BUSINESS:

Overview

Product Portfolio Overview

AMAG Pharmaceuticals, Inc., a Delaware corporation, was founded in 1981. We are a specialty pharmaceutical company with a focus on maternal health, anemia and cancer supportive care. We currently market Makena® (hydroxyprogesterone caproate injection), Feraheme® (ferumoxytol) Injection for Intravenous ("IV") use and MuGard® Mucoadhesive Oral Wound Rinse. The primary goal of our company is to bring to market therapies that provide clear benefits and improve patients' lives.

Currently, our two primary sources of revenue are from the sale of *Makena* and *Feraheme.* On November 12, 2014, we acquired Lumara Health Inc. ("Lumara Health"), a privately held pharmaceutical company specializing in women's health, for approximately $600.0 million in upfront cash consideration (subject to finalization of certain adjustments related to Lumara Health's financial position at the time of closing, including adjustments related to net working capital, net debt and transaction expenses as set forth in the definitive agreement with Lumara Health (the "Lumara Agreement")) and approximately 3.2 million shares of our common stock having a fair value of approximately $112.0 million at the time of closing. The Lumara Agreement includes future contingent payments of up to $350.0 million in cash (or upon mutual agreement between us and the former Lumara Health security holders, future contingent payments may also be made in common stock or some combination thereof) payable by us to the former Lumara Health security holders based upon the achievement of certain sales milestones through calendar year 2019. In connection with the acquisition of Lumara Health, we acquired *Makena*, a progestin indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth. We sell *Makena* to specialty pharmacies and distributors, who, in turn sell *Makena* to healthcare providers, hospitals, government agencies and integrated delivery systems. Additional details regarding the Lumara Agreement can be found in Note C, "*Business Combinations,*" to our consolidated financial statements included in this Annual Report on Form 10-K.

Table of Contents

Feraheme was approved for marketing in the U.S. in June 2009 by the U.S. Food and Drug Administration (the "FDA") for use as an IV iron replacement therapy for the treatment of iron deficiency anemia ("IDA") in adult patients with chronic kidney disease ("CKD"). We began selling *Feraheme* in the U.S. in July 2009 through our commercial organization, including a specialty sales force. We sell *Feraheme* to authorized wholesalers and specialty distributors, who in turn, sell *Feraheme* to healthcare providers who administer *Feraheme* primarily within hospitals, hematology and oncology centers, and nephrology clinics.

In addition to continuing to pursue opportunities to make new advancements in patients' health and to enhance treatment accessibility, we intend to continue to expand and diversify our portfolio through the in-license or purchase of additional pharmaceutical products or companies. We are seeking complementary products that will leverage our corporate infrastructure, sales force call points and commercial expertise, with a particular focus on maternal health specialists, hematology and oncology centers, nephrology clinics and hospitals. We are evaluating and plan to pursue commercial products as well as late-stage development assets. In addition, we are contemplating transactions that allow us to realize cost synergies to increase cash flows, as well as transactions that potentially optimize after-tax cash flows.

Regulatory Developments Overview

In June 2014, we proposed changes to the FDA related to our current U.S. label of *Feraheme* based on a review of global post-marketing data to strengthen the warnings and precautions section of the label and mitigate the risk of serious hypersensitivity reactions, including anaphylaxis, in order to enhance patient safety. After considering our June 2014 submission and other information, in January 2015, the FDA notified us that it believes new safety information should be included in the labeling for *Feraheme*, including, among other things, a boxed warning to highlight the risks of serious hypersensitivity/anaphylaxis reactions and revisions that *Feraheme* should only be administered through an IV infusion (*i.e.*, not by IV injection) and should be contraindicated for patients with any known history of drug allergy. The FDA's recommended label changes go beyond what we proposed in June 2014. We plan to work with the FDA to finalize an updated U.S. *Feraheme* label.

In December 2012, we submitted a supplemental new drug application ("sNDA") to the FDA seeking approval for *Feraheme* for the treatment of IDA in adult patients who had failed or could not use oral iron. In January 2014, we received a complete response letter from the FDA for the sNDA informing us that our sNDA could not be approved in its present form and stating that we have not provided sufficient information to permit labeling of *Feraheme* for safe and effective use for the proposed broader indication. The FDA indicated that its decision was based on the cumulative ferumoxytol data, including the global Phase III IDA program and global post-marketing safety reports for the currently indicated CKD patient population. The FDA suggested, among other things, that we submit additional clinical trial data in the proposed broad IDA patient population with a primary composite safety endpoint of serious hypersensitivity/anaphylaxis, cardiovascular events and death, events that are included in the labels of *Feraheme* and other IV irons and that have been reported in the post-marketing environment for *Feraheme*. Additionally, the FDA proposed potentially evaluating alternative dosing and/or administration of *Feraheme* as well as potential changes to labeling that would be intended to reduce the risk of serious hypersensitivity reactions associated with *Feraheme*. In June 2014, we met with the FDA to discuss our proposed approach to resolving the points that were raised in the complete response letter. Based on the FDA's feedback, we submitted a revised proposal that includes the design of a potential clinical trial, a safety endpoint for such trial and alternative methods of administration of *Feraheme*. We expect to receive feedback from the FDA during 2015 and expect thereafter to be able to assess and determine the path forward, if any, for *Feraheme* in the broad IDA patient population in the U.S., including the related timing and cost of any clinical trials.

Table of Contents

Further, in October 2014, we filed with the FDA a prior approval supplement to the original *Makena* New Drug Application ("NDA") seeking approval of a 1 mL preservative-free vial of *Makena* and we are seeking to expand *Makena's* formulations and drug delivery technologies as part of the product's lifecycle management program.

Outside of the U.S., ferumoxytol has been granted marketing approval in the European Union ("EU"), Canada and Switzerland for use as an IV iron replacement therapy for the treatment of IDA in adult patients with CKD. In March 2010, we entered into a License, Development and Commercialization Agreement (the "Takeda Agreement"), which was amended in June 2012 (the "Amended Takeda Agreement") with Takeda. On December 29, 2014, we entered into an agreement with Takeda to terminate the Amended Takeda Agreement and we will regain all worldwide development and commercialization rights for *Feraheme* following the transfer of marketing authorizations from Takeda to us (the "Takeda Termination Agreement"). Under the Amended Takeda Agreement, Takeda had an exclusive license to market and sell ferumoxytol in the EU, Canada, and Switzerland, as well as certain other geographic territories. The EU marketing authorization for *Rienso* is valid in the 28 EU Member States as well as in Iceland, Liechtenstein and Norway. The trade name for ferumoxytol in Canada is *Feraheme* and outside of the U.S. and Canada the trade name is *Rienso*. Additional details regarding the Takeda Termination Agreement can be found in Note R, "*Collaborative Agreements,*" to our consolidated financial statements included in this Annual Report on Form 10-K.

Sales of *Feraheme/Rienso* outside of the U.S. do not and are not expected to materially contribute to our revenues. As such, and in light of the Takeda Termination Agreement, we have been assessing various commercialization strategies for *Rienso* in the EU and Switzerland and *Feraheme* in Canada. A number of considerations influence our analysis of our commercialization opportunities outside of the U.S., including (i) regulatory developments and the potential cost of post-approval clinical trial commitments and post-marketing obligations required by regulatory authorities outside of the U.S., (ii) the product's commercial viability (sales potential relative to the cost of maintaining the product on the market) in light of the current CKD label, the possible impact of future label changes, including any impact in the U.S., and the competitive landscape, and (iii) possible approaches in different geographies, which may include seeking a licensing or distribution partner or commercializing the product ourselves. Based on these considerations, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada.

In the future, we may decide to seek to obtain a new marketing authorization for ferumoxytol in the EU, particularly if we generate additional clinical data to support potential approval in the broader IDA indication. There can be no assurance that we will be able to develop an approach that would be economically viable for us or a commercialization partner.

Debt Obligations

In February 2014, we issued $200.0 million of 2.5% convertible senior notes due February 15, 2019 (the "Convertible Notes"). Interest is payable semi-annually in arrears on February 15 and August 15 of each year, beginning on August 15, 2014. The initial conversion rate is 36.9079 shares of our common stock per $1,000 principal amount of the Convertible Notes, which corresponds to an initial conversion price of approximately $27.09 per share of our common stock and represents a conversion premium of approximately 35% based on the last reported sale price of our common stock of $20.07 on February 11, 2014, the date the Convertible Notes offering was priced. In addition, in connection with the pricing of the Convertible Notes and in order to reduce the potential dilution to our common stock and/or offset cash payments due upon conversion of the Convertible Notes, we also entered into convertible bond hedge and warrant transactions in February 2014. The initial exercise price of the warrants is $34.12 per share, subject to adjustment upon certain events, which is 70% above the last reported sale price of our common stock of $20.07 on February 11, 2014.

Table of Contents

On November 12, 2014, in connection with the acquisition of Lumara Health, we entered into the Term Loan Facility, which provides for term loans in the aggregate principal amount of $340.0 million (the "Term Loan Facility"). We used $327.5 million of the Term Loan Facility proceeds to partially finance the $600.0 million cash portion of the Lumara Health acquisition. The Term Loan Facility bears interest, at our option, at either the Eurodollar rate plus a margin of 6.25% or the prime rate plus a margin of 5.25%. The Eurodollar rate is subject to a 1.00% floor and the prime rate is subject to a 2.00% floor. As of December 31, 2014, the stated interest rate was 7.25%. We must repay the Term Loan Facility in installments of (a) $8.5 million per quarter due on the last day of each quarter beginning with the quarter ending March 31, 2015 through the quarter ending December 31, 2015, and (b) $12.8 million per quarter due on the last day of each quarter beginning with the quarter ending March 31, 2016 through the quarter ending September 30, 2020, with the balance due in a final installment on November 12, 2020. The Term Loan Facility matures on November 12, 2020, except that the Term Loan Facility will mature on September 30, 2018 if:

(a) more than $25.0 million in aggregate principal amount of our Convertible Notes remain outstanding and not converted to common stock or refinanced and replaced with debt that matures following, and has no amortization prior to, the date that is six and one half years following the closing date; and

(b) the aggregate principal amount of the Term Loan Facility (including all undrawn incremental commitments) is greater than $50.0 million on and as of such date.

See Note S, "*Debt*," to our consolidated financial statements included in this Annual Report on Form 10-K for additional information regarding the Convertible Notes, the bond hedge and warrant transactions, as well as the Term Loan Facility.

Our common stock trades on the NASDAQ Global Select Market ("NASDAQ") under the trading symbol "AMAG."

Table of Contents

Products

The following table summarizes the current uses and, subject to regulatory approval, potential uses of our products, the current U.S. and foreign regulatory status, and the primary markets for our products.

Product	Uses/Potential Uses	U.S. Regulatory Status	Foreign Regulatory Status
Makena® (hydroxyprogesterone caproate injection) (5 mL multi-use vial)	A progestin indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth.	Approved and marketed.	Not approved outside of the U.S.
Makena® (hydroxyprogesterone caproate injection) (1 mL vial, preservative-free, single dose)	A progestin indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth.	Prior approval supplement submitted to the FDA in October 2014. Decision from the FDA expected in the second quarter 2015.	Not approved outside of the U.S.
Feraheme® (ferumoxytol)	IV iron replacement therapeutic agent for the treatment of IDA in adult patients with CKD.	Approved and marketed.	Approved and marketed as *Feraheme* in Canada. Approved and marketed as *Rienso* in the EU.* Approved in Switzerland and not currently marketed.*
Feraheme® (ferumoxytol)	IV iron replacement therapeutic agent for the treatment of patients with a history of unsatisfactory oral iron therapy or in whom oral iron cannot be used.	sNDA filed December 2012. Complete Response Letter received January 2014. Submitted proposal to FDA in 2014 that included the design of a potential clinical trial and we are awaiting feedback.	Application for Type II Variation filed with the European Medicines Agency ("EMA") in 2013 and withdrawn in January 2015. Decision from Health Canada on sNDS expected in the second half of 2015.
MuGard® Mucoadhesive Oral Wound Rinse	Management of oral mucocitis/stomatiits and all types of oral wounds.	Cleared and marketed.	We license only the U.S. commercial rights from PlasmaTech.

*

As discussed above, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. Our licensing arrangement with Takeda, and its termination, is discussed below under the heading "*Collaboration, License and Other Material Agreements Takeda*."

For a discussion of the substantive regulatory requirements applicable to the development and regulatory approval process in the U.S. and other countries, see "*Government Regulation*" below.

Makena

Overview

On November 12, 2014, we acquired Lumara Health, a privately held pharmaceutical company specializing in women's health, including its marketed drug product *Makena*, the only FDA-approved drug indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth. *Makena* is administrated intramuscularly by a healthcare professional at a dose of 250 mg (1 mL) weekly with treatment beginning between 16 weeks and

Table of Contents

20 weeks and six days and continuing until 37 weeks (through 36 weeks and six days) of pregnancy or delivery, whichever happens first.

Makena was approved by the FDA in February 2011 and was granted orphan drug exclusivity through February 3, 2018. 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, defined, in part, as a patient population of fewer than 200,000 in the U.S. In the U.S., the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application for the "same drug" for the same orphan indication during the exclusivity period, except in very limited circumstances. A designated orphan drug may not receive orphan drug exclusivity for an approved indication if that indication is for the treatment of a condition broader than that for which it received orphan designation. In addition, orphan drug exclusivity marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Preterm Birth

Preterm birth is defined as a birth prior to 37 weeks of pregnancy. According to the Centers for Disease Control and Prevention ("CDC"), in 2012, preterm births affected more than 450,000 babies, or one of every nine infants born in the U.S. Although, the causes of preterm births are not fully understood, certain women are at a greater risk for preterm birth, including those who have had a previous preterm birth, are pregnant with multiples or have certain uterine or cervical problems. *Makena* is indicated only for women with a history of spontaneous singleton preterm birth who are pregnant with a singleton, which accounts for approximately 140,000 pregnancies annually in the U.S. High blood pressure, pregnancy complications (such as placental problems) and certain other health or lifestyle factors may also be contributing factors. The last few weeks of a woman's pregnancy are important to the full development of many major organ systems, including the brain, lungs, and liver. Preterm births can increase the risk of infant death and can also result in serious long-term health issues for the child, including respiratory problems, gastrointestinal conditions, cerebral palsy, developmental delays, and vision and hearing impairments. According to a 2007 report by the Institute of Medicine (US) Committee on Understanding Premature Birth and Assuring Healthy Outcome, the annual societal economic cost associated with preterm birth is at least $26.2 billion and includes medical and healthcare costs for the baby, labor and delivery costs for the mother, early intervention and special education services, and costs associated with lost work and pay.

Post-Approval Commitments for Makena

Makena was approved under the provisions of the FDA's "Subpart H" Accelerated Approval regulations. The Subpart H regulations allow certain drugs, for serious or life-threatening conditions, to be approved on the basis of surrogate endpoints or clinical endpoints other than survival or irreversible morbidity. As a condition of approval under Subpart H, the FDA required that *Makena*'s sponsor perform certain adequate and well-controlled post-marketing clinical studies to verify and describe clinical benefit of *Makena* as well as fulfill certain other post-approval commitments. We are currently conducting the following clinical studies; (a) an ongoing efficacy and safety clinical study of *Makena*; (b) an ongoing follow-up study of the babies born to mothers from the efficacy and safety clinical study; and (c) a completed pharmacokinetic study of women taking *Makena.* Given the patient population (*i.e.*, women pregnant who are at an increased high risk for recurrent preterm delivery) and the informed risk of receiving a placebo instead of the active approved drug in the U.S., the pool of prospective subjects for such clinical trials in the U.S. is small and we are therefore seeking enrollment on a global scale.

Table of Contents

Lifecycle Management Program

We are pursuing a lifecycle management program for *Makena*, some elements of which may provide new intellectual property or data exclusivity beyond February 2018 by exploring new routes of administration and the use of new delivery technologies, as well as reformulation technologies. As part of this program, in October 2014, a prior approval supplement for a preservative-free, single-dose (1 mL) vial for *Makena* was filed with and is under review by the FDA. We expect a decision in the second quarter of 2015. *Makena* is currently available in a 5-dose (5 mL) vial.

Feraheme for the treatment of IDA in patients with CKD

Overview

In June 2009, *Feraheme* was approved for marketing in the U.S. by the FDA for use as an IV iron replacement therapy for the treatment of IDA in adult patients with all stages of CKD, Stage 1 through Stage 5 (end-stage renal disease). In July 2009, we began to market and sell *Feraheme* in the U.S. While *Feraheme* is approved for IDA in all stages of CKD, beginning in 2010, due to changes in the way the federal government reimburses providers for the care of dialysis patients, the utilization of *Feraheme* shifted to non-dialysis patients. The non-dialysis CKD IDA market is made up of a range of healthcare providers who administer IV iron, including nephrologists, hematologists, oncologists, hospitals and other end-users who treat patients with CKD. We anticipate the majority of all *Feraheme* utilization in the U.S. will continue to be in the non-dialysis CKD patient population if and until *Feraheme* receives a broader label to include non-CKD patients.

In June 2014, we proposed changes to the FDA related to our current U.S. label of *Feraheme* based on a review of global post-marketing data to strengthen the warnings and precautions section of the label and mitigate the risk of serious hypersensitivity reactions, including anaphylaxis, in order to enhance patient safety. After considering our June 2014 submission and other information, on January 7, 2015, the FDA notified us that it believes new safety information should be included in the labeling for *Feraheme*, including, among other things, a boxed warning to highlight the risks of serious hypersensitivity/anaphylaxis reactions and revisions that *Feraheme* should only be administered through an IV infusion (*i.e.*, not by IV injection) and should be contraindicated for patients with any known history of drug allergy. The FDA's recommended label changes go beyond what we proposed in June 2014. We plan to work with the FDA to finalize an updated U.S. *Feraheme* label.

In Europe, Takeda has been commercializing ferumoxytol since its approval in June 2012 under the trade name *Rienso*, currently in nine EU countries. *Rienso* is subject to periodic review by the EMA's Pharmacovigilance Risk Assessment Committee ("PRAC") and in February 2014 Takeda, as the marketing authorization holder (the "MAH") for *Rienso*, submitted to PRAC a Periodic Safety Update Report ("PSUR") concerning *Rienso* as part of such review. A PSUR is a pharmacovigilance document submitted by the MAH at defined intervals and is intended to provide a safety update permitting an evaluation of the risk-benefit balance of a medicinal product while it is commercialized.

As part of its assessment of the PSUR, PRAC reviewed various data, including the rate of hypersensitivity reactions with fatal outcomes with *Rienso*. Following that assessment, and in agreement with the EMA, Takeda issued a Direct Healthcare Professional Communication ("DHPC") letter in May 2014 to remind physicians in the EU of the existing risk minimization measures for all IV iron products to manage and minimize the risk of serious hypersensitivity reactions that were included in the special warnings and precautions sections of the *Rienso* label.

In July 2014 and again in January 2015, also in connection with the PSUR evaluation, PRAC confirmed that the benefit/risk balance of *Rienso* in the currently approved CKD indication remains favorable. These confirmations were subject to a number of proposed changes to the product information and label and other risk minimization measures, including, among others, that *Rienso*

Table of Contents

should be administered to patients by infusion over at least 15 minutes (replacing injection) and that it should be contraindicated in patients with any known history of drug allergy (the "July Recommendations"), that the label should caution that elderly patients or patients with multiple co-morbidities who experience a serious hypersensitivity reaction due to *Rienso* may have more severe outcomes (the "January Recommendations"), and related variations to the Summary of Product Characteristics ("SmPC"). The PRAC's recommendations were subsequently endorsed by the EMA's Committee for Medicinal Products for Human Use ("CHMP"). Takeda updated the product's label to reflect the July Recommendations and in August 2014 issued a DHPC letter informing physicians of these changes.

In December 2011, ferumoxytol was granted marketing approval in Canada, under the trade name *Feraheme*, for use as an IV iron replacement therapy for the treatment of IDA in adult patients with CKD. In August 2012, ferumoxytol was granted marketing approval in Switzerland under the trade name *Rienso* for use as an IV iron replacement therapy for the treatment of IDA in adult patients with CKD, but has subsequently been withdrawn from the market. As discussed above, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada.

Chronic kidney disease, anemia, and iron deficiency

CKD is the gradual and permanent loss of kidney function. It is a progressive illness that contributes to the development of many complications, including anemia, hypertension, fluid and electrolyte imbalances, acid/base abnormalities, bone disease and cardiovascular disease. According to the National Kidney Foundation, 26 million Americans are living with CKD and millions of others are at risk. Anemia, a common condition among CKD patients, is associated with cardiovascular complications, decreased quality of life, hospitalizations, and increased mortality. Anemia develops early during the course of CKD and worsens with advancing kidney disease. Patients with anemia can look pale, feel fatigued, experience shortness of breath, low energy, headaches, palpitations or chest pains, and have a loss of appetite, trouble sleeping and trouble concentrating. Anemia in CKD patients is most often considered to be caused by an insufficient production of erythropoietin, a hormone made by the kidneys which tells the body to produce red blood cells, and iron deficiency, due to inadequate iron intake, blood loss or because the body cannot use iron stores. Regardless of the cause of the iron deficiency, iron replacement therapy is essential to increase iron stores and raise hemoglobin levels. Iron is also essential for effective treatment with erythropoiesis stimulating agents ("ESAs"), which are commonly used in anemic patients to stimulate red blood cell production. Based on data contained in a 2009 publication in the *Journal of the American Society of Nephrology,* we estimate that there are approximately 1.6 million adults in the U.S. diagnosed with IDA and stages 3 through 5 CKD, who are patients in the mid to later stages of CKD but not yet on dialysis and could therefore benefit from receiving iron.

Currently there are two methods used to treat IDA in CKD patients: oral iron supplements and IV iron. The National Kidney Foundation's Kidney Disease Outcomes Quality Initiative guidelines recommend either oral or IV iron for peritoneal dialysis patients and non-dialysis patients with stages 1 through 5 CKD. Oral iron is currently the first-line iron replacement therapy of choice of most physicians in both the U.S. and abroad. However, oral iron supplements are poorly absorbed by many patients, which may adversely impact their effectiveness, and are associated with certain side effects, such as constipation, diarrhea, and cramping, that may adversely affect patient compliance in using such products. In addition, it can take an extended time for hemoglobin levels to improve following the initiation of oral iron treatment, and even then the targeted hemoglobin levels may not be reached. Conversely, iron given intravenously allows larger amounts of iron to be provided to patients while avoiding many of the side effects and treatment compliance issues associated with oral iron, and can result in faster rises in hemoglobin levels. The administration of IV iron has been shown to be effective

Table of Contents

in treating anemia either when used alone or in combination with an ESA. Current U.S. treatment guidelines indicate that treating first with iron alone may delay or reduce the need for ESA therapy. Iron supplementation is widely used in CKD patients to treat iron deficiency, prevent its development in ESA-treated patients, raise hemoglobin levels in the presence or absence of ESA treatment, and reduce ESA doses in patients receiving ESA treatment. We believe that a small fraction of non-dialysis CKD patients in the U.S. who are diagnosed with IDA are currently being treated with IV iron, and thus a significant opportunity remains to grow the market for IV iron in this patient population.

Post-Marketing Commitments of Feraheme in CKD

We have initiated a randomized, active-controlled pediatric study of *Feraheme* for the treatment of IDA in pediatric CKD patients to meet our FDA post-approval Pediatric Research Equity Act requirement to support pediatric labeling of *Feraheme* in the U.S. The study covers both dialysis-dependent and non-dialysis dependent CKD pediatric patients and will assess the safety and efficacy of *Feraheme* treatment as compared to oral iron in approximately 288 pediatric patients.

Our pediatric investigation plan, which was a requirement for submission of the marketing authorization application for ferumoxytol, was approved by the EMA in December 2009 and amended in 2012 and 2014. It includes the pediatric study, as described above, and two additional pediatric studies requested by the EMA. These additional studies include a rollover extension study in pediatric CKD patients and a study in pediatric patients with IDA regardless of the underlying cause. The rollover study is open for enrollment. The pediatric IDA study will commence once the appropriate dose of *Feraheme* is determined from the study data resulting from the pediatric study of *Feraheme*, described above.

As part of our post-approval commitments to the EMA, we are conducting a global multi-center clinical trial to determine the safety and efficacy of repeat doses of ferumoxytol for the treatment of IDA in patients with hemodialysis-dependent CKD. As part of the commitment we made to the EMA as a condition of the approval of the marketing authorization for ferumoxytol in the EU, this study includes a treatment arm with iron sucrose using a magnetic resonance imaging sub-analysis to evaluate the potential for iron to accumulate in the body following treatment with IV iron, specifically in the heart and liver, and, where possible, other major organs following repeated IV iron administration over a two year period (the "hd-CKD Study"). Enrollment has been completed.

We have assumed any post-marketing obligations of Takeda as part of the Takeda Termination Agreement, including costs that otherwise would have been Takeda's obligation under the Amended Takeda Agreement for the ongoing pediatric studies and the ongoing multi-center clinical trial discussed above. In connection with our decision to withdraw the marketing authorization for *Rienso* in the EU and Switzerland, we may modify or terminate clinical trials being conducted as part of our post-approval commitments to the EMA.

***Feraheme* for the treatment of IDA in a broad range of patients**

Overview

IDA not associated with CKD is widely prevalent in many different patient populations. For many of these patients, treatment with oral iron is unsatisfactory. In the U.S., approximately 900,000 grams of IV iron were administered for the treatment of non-dialysis patients with IDA in 2014. We believe that approximately half, or 450,000 grams, of the IV iron administered in the U.S. was for the treatment of non-dialysis patients with CKD and the other half was for non-CKD patients with IDA due to other causes, including patients with gastrointestinal diseases or disorders, abnormal uterine bleeding, inflammatory diseases, and chemotherapy-induced anemia. It is estimated that more than 4.5 million patients in the U.S. have IDA (CKD and non-CKD). We estimate that approximately 5% to 10% of these patients are currently treated with IV iron.

Table of Contents

As discussed above, in December 2012, we submitted an sNDA to the FDA seeking approval for *Feraheme* for the treatment of IDA in adult patients who had failed or could not use oral iron. The sNDA included data from two controlled, multi-center Phase III clinical trials ("IDA-301 and IDA-302"), including more than 1,400 patients, which evaluated the safety and efficacy of ferumoxytol for the treatment of IDA in this broader patient population. Both studies met the primary efficacy endpoints related to improvements in hemoglobin. In these studies no new safety signals were observed with *Feraheme* treatment and the types of reported adverse events were consistent with those seen in previous studies and those contained in the approved U.S. package insert for *Feraheme*. In addition, patients from IDA-301 were eligible to enroll in an open-label extension study ("IDA-303") and receive treatment with *Feraheme,* as defined in the protocol.

In January 2014, we received a complete response letter from the FDA for the sNDA informing us that our sNDA could not be approved in its present form and stating that we have not provided sufficient information to permit labeling of *Feraheme* for safe and effective use for the proposed broader indication. The FDA indicated that its decision was based on the cumulative ferumoxytol data, including the global Phase III IDA program and global post-marketing safety reports for the currently indicated CKD patient population. The FDA suggested, among other things, that we submit additional clinical trial data in the proposed broad IDA patient population with a primary composite safety endpoint of serious hypersensitivity/anaphylaxis, cardiovascular events and death, events that are included in the labels of *Feraheme* and other IV irons and that have been reported in the post-marketing environment for *Feraheme*. Additionally, the FDA proposed potentially evaluating alternative dosing and/or administration of *Feraheme* as well as potential changes to labeling that would be intended to reduce the risk of serious hypersensitivity reactions associated with *Feraheme*. In June 2014, we met with the FDA to discuss our proposed approach to resolving the points that were raised in the complete response letter. Based on the FDA's feedback, we submitted a revised proposal that includes the design of a potential clinical trial, a safety endpoint for such trial and alternative methods of administration of *Feraheme*. We expect to receive feedback from the FDA during 2015 and expect thereafter to be able to assess and determine the path forward, if any, for *Feraheme* in the broad IDA patient population in the U.S., including the related timing and cost of any clinical trials.

In June 2013, Takeda filed an application for Type II Variation to vary the marketing authorization for *Rienso* in the EU with the EMA to extend the therapeutic indication from adult patients with IDA associated with CKD to adult patients with iron deficiency from any underlying cause. During the course of CHMP's review of the Type II Variation, Takeda received inquiries and reports from regulators indicating that approval of the Type II Variation would be unlikely without additional confirmative clinical data. As a result, in January 2015, we and Takeda mutually agreed that Takeda withdraw the Type II Variation.

In addition, in October 2013, Takeda filed an sNDS with Health Canada seeking marketing approval for *Feraheme* for the treatment of IDA in a broad range of patients. In October 2014, Takeda received inquiries from Health Canada and in January 2015, we submitted a response to these inquiries. Based on these inquiries and interactions, we believe that approval in the broader indication is unlikely in Canada without additional clinical data. We believe that we will receive Health Canada's final decision on the sNDS in the second half of 2015, however we cannot guarantee that Health Canada will issue a final decision on the expected timeline. In addition, until we have further conversations with Health Canada, we cannot predict whether their concerns with regard to approval of the broader IDA indication, including with regard to the need for additional clinical data, will cause Health Canada to impose additional restrictions on the current CKD indication.

As discussed above, we are in the process of regaining all worldwide development and commercialization rights for *Feraheme* from Takeda and have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada.

Table of Contents

MuGard

In June 2013, we entered into the License Agreement with PlasmaTech Biopharmaceuticals, Inc. ("PlasmaTech") (formerly known as Access Pharmaceuticals, Inc.), under which we acquired the U.S. commercial rights to *MuGard* for the management of oral mucositis (the "MuGard License Agreement"). *MuGard* was launched in the U.S. by PlasmaTech in 2010 after receiving 510(k) clearance from the FDA and is indicated for the management of oral mucositis/stomatitis (that may be caused by radiotherapy and/or chemotherapy) and all types of oral wounds (mouth sores and injuries), including aphthous ulcers/canker sores and traumatic ulcers, such as those caused by oral surgery or ill-fitting dentures or braces. Mucositis is the painful inflammation and ulceration of the mucous membranes of the mouth and gastrointestinal tract that can be caused by high-dose chemotherapy and/or radiotherapy. Oral mucositis is a common and often debilitating complication of cancer treatment that may impair oral nutritional intake or result in delays, unplanned breaks or decreases in dose for chemotherapy and/or radiation treatments, leading to sub-optimal cancer treatment results. In the U.S., there are approximately 400,000 people per year who experience oral mucositis and approximately 80% of patients with mucositis experience severe oral pain. The incidence rate and severity of symptoms depends on the type of anti-cancer treatment and patient-related risk factors. For example, based on data reported in a 2001 article in *CA: A Cancer Journal for Clinicians*, the incidence of oral mucositis for patients undergoing radiation for the treatment of head and neck cancer could approximate 80%. The incidence of oral mucositis for bone marrow transplant patients undergoing high dose chemotherapy and/or radiation pre-conditioning and patients undergoing conventional chemotherapy is approximately 70% and 40%, respectively.

There are few effective treatments for oral mucositis and the products in this market remain mostly undifferentiated. There are a number of approaches used to treat or manage oral mucositis, including the use of ice chips during chemotherapy treatments, various medicinal mouthwashes, topical anesthetics and analgesics, and oral gel treatments. We sell *MuGard* through a distribution network of specialty pharmacies and wholesalers, who in turn supply it to hospitals or hematology/oncology clinics. Currently, *MuGard* is used by a small percentage of the oral mucositis patients in the U.S., which represents a significant opportunity for us to address an unmet medical need and grow the sales of *MuGard* in the oral mucositis market.

Our Core Proprietary Technology

Our core proprietary technology for ferumoxytol is based on coated superparamagnetic iron oxide particles and their characteristic properties. Our core competencies for ferumoxytol include the ability to design such particles for particular applications and to manufacture the particles in controlled sizes. Our technology and expertise enable us to synthesize, sterilize and stabilize these iron oxide particles in a manner necessary for use in pharmaceutical products such as IV iron replacement therapeutics.

Our iron oxide particles are composed of bioavailable iron that is easily utilized by the body and incorporated into the body's iron stores. As a result, our core technology for ferumoxytol is well-suited for use as an IV iron replacement therapy product.

Our rights to the technology are derived from and/or protected by license agreements, patents, patent applications and trade secret protections. See "*Patents and Trade Secrets*" below. There are no patents covering *Makena*. Our rights to *MuGard* are governed by the MuGard License Agreement. See Note C, "*Business Combinations*," to our consolidated financial statements included in this Annual Report on Form 10-K.

Table of Contents

Collaboration, License and Other Material Agreements

Takeda

In March 2010, we entered into the Takeda Agreement, as amended in June 2012, under which we granted exclusive rights to Takeda to develop and commercialize *Feraheme* as a therapeutic agent in certain agreed-upon territories. In February 2014, we entered into the Supply Agreement with Takeda, which provides the terms under which we sell *Feraheme* to Takeda in order for Takeda to meet its requirements for commercial use of *Feraheme* in its licensed territories. On December 29, 2014, we entered into the Takeda Termination Agreement, under which the Amended Takeda Agreement will be terminated and we will regain all worldwide development and commercialization rights for *Feraheme* following the transfer of the outstanding marketing authorizations. Pursuant to the Takeda Termination Agreement, we and Takeda have agreed to effectuate the termination of the Amended Takeda Agreement on a rolling basis, whereby the termination will be effective for a particular geographic territory (e.g., countries under the regulatory jurisdictions of Health Canada, the EMA and SwissMedic) upon the earlier of effectiveness of the transfer to us or a Withdrawal (as defined below) of the marketing authorization for such territory, with the final effective termination date to be on the third such effective date ("Termination Date").

In connection with each Termination Date and in accordance with the terms of the Takeda Termination Agreement, Takeda is obligated, with respect to the applicable terminated territory, to transfer and assign to us all applicable regulatory materials and approvals and certain product data, unlabeled inventory, third party contracts intellectual property rights and know-how to us, and to grant us an exclusive license for certain Takeda technology used and applied to commercialize *Feraheme* in the applicable territory. The Takeda Termination Agreement also details the regulatory activities each party is required to perform in connection with transferring the marketing authorization from Takeda to us in each of the territories and the allocation of the costs of such activities. We and Takeda have agreed to use commercially reasonable efforts to transfer all required activities to us on a territory-by-territory basis within 60 days after the applicable Termination Date (subject to a 30-day extension upon our request and Takeda's consent). In addition, Takeda is obligated pursuant to the Takeda Termination Agreement to provide transition assistance to us, at no cost to us, for up to 180 days after each Termination Date for the applicable termination territory. With Takeda's consent (which shall not be unreasonably withheld or delayed), we may extend the transition services period for a terminated territory for a period of time reasonably necessary to complete any services that cannot be reasonably transitioned to us during the initial 180-day period, which extension will not exceed an additional 180 days. If we request, and Takeda agrees to conduct, additional transition services after the end of the applicable transition services period, as may be extended, we will reimburse Takeda's fully burdened costs for such additional services plus 5%.

The Takeda Termination Agreement also provides that if the marketing authorization for the product is suspended in a particular territory and the parties are prevented from completing the transfer of such marketing authorization to us within 120 days after such suspension due to applicable laws or any regulatory requirements or restrictions, or if we do not fulfill our obligations to initiate marketing authorization transfer by the agreed-upon, territory-specific deadline, Takeda will have the right, in Takeda's sole discretion, to withdraw such marketing authorization (a "Withdrawal").

In consideration for the early termination of the Amended Takeda Agreement and the activities to be performed by us earlier than contemplated under the Amended Takeda Agreement, and in lieu of any future cost-sharing and milestone payments contemplated by the Amended Takeda Agreement, Takeda agreed to make certain payments to us, subject to certain terms and conditions, including up to approximately $6.7 million in connection with clinical study obligations, pharmacovigilance activities, regulatory filings and support, commercialization and back-office support and distribution expenditures and a $3.0 million milestone payment payable subject to certain regulatory conditions.

Table of Contents

Additionally, the Supply Agreement, which continues in effect until the expiration or termination of the Amended Takeda Agreement, will also terminate as of the respective Termination Date in the applicable geographic territory.

We have assumed any post-marketing obligations of Takeda as part of the Takeda Termination Agreement, including costs that otherwise would have been Takeda's obligation under the Amended Takeda Agreement for the ongoing pediatric studies, and the hd-CKD Study. As discussed above, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada. In connection with our decision to withdraw the marketing authorization for *Rienso* in the EU and Switzerland, we may modify or terminate clinical trials being conducted as part of our post-approval commitments to the EMA.

Additional details regarding the Takeda Termination Agreement and related revenue can be found in Note R, "*Collaborative Agreements,*" to our consolidated financial statements included in this Annual Report on Form 10-K.

PlasmaTech

In June 2013, we entered into the MuGard License Agreement under which PlasmaTech granted us an exclusive, royalty-bearing license, with the right to grant sublicenses, to certain intellectual property rights, including know-how, patents and trademarks, to use, import, offer for sale, sell, manufacture and commercialize *MuGard* in the U.S. and its territories (the "U.S. Territory") for the management of all diseases or conditions of the oropharyngeal cavity, including mucositis.

In consideration for the license, we paid PlasmaTech an upfront license fee of $3.3 million in June 2013. We are required to pay royalties to PlasmaTech on net sales of *MuGard* until the later of (a) the expiration of the licensed patents or (b) the tenth anniversary of the first commercial sale of *MuGard* in the U.S. Territory (the "Royalty Term"). These tiered, double-digit royalty rates decrease after the expiration of the licensed patents and are subject to off-set against certain of our expenses. After the expiration of the Royalty Term, the license shall become a fully paid-up, royalty-free and perpetual license in the U.S. Territory.

PlasmaTech remains responsible for the manufacture of *MuGard* and we have entered into a quality agreement and a supply agreement with PlasmaTech under which we purchase *MuGard* inventory from PlasmaTech. Our inventory purchases are at the price actually paid by PlasmaTech to purchase it from a third-party plus a mark-up to cover administration, handling and overhead.

PlasmaTech is responsible for maintenance of the licensed patents at its own expense, and we retain the first right to enforce any licensed patent against third party infringement. The MuGard License Agreement terminates at the end of the Royalty Term, but is subject to early termination by us for convenience and by either party upon an uncured breach by or bankruptcy of the other party.

3SBio

In 2008, we entered into the 3SBio License Agreement and the 3SBio Supply Agreement with 3SBio Inc. ("3SBio") for the development and commercialization of *Feraheme* as an IV iron replacement therapeutic agent in China. In consideration of the grant of the license, we received an upfront payment of $1.0 million. In late January 2014, we mutually terminated the agreement with 3SBio, effective immediately, due to the fact that, despite the best efforts of the parties, regulatory approval in China could not be obtained within the agreed upon time period.

Table of Contents

Manufacturing

We currently rely solely on third parties for the manufacture of *Feraheme* and *Makena* for our commercial and clinical use. Our third-party contract manufacturing facilities for *Feraheme* and *Makena* are subject to current good manufacturing practices ("cGMP"), regulations enforced by the FDA and equivalent foreign regulatory agencies through periodic inspections to confirm such compliance. Although we are currently working to establish and qualify alternative manufacturing facilities for both drug substance and drug product of *Feraheme* and drug product for *Makena,* we do not currently have alternative manufacturers for our *Feraheme* and *Makena* drug substance and drug product, as applicable. In addition, we currently do not have a supply agreement for *Makena* drug substance and, until we do, we plan to obtain *Makena* drug substance on a purchase order basis. We target to maintain sufficient inventory levels throughout our supply chain to meet our projected U.S. near-term demand of *Feraheme* and *Makena* drug product in order to minimize risks of supply disruption at points in our single source supply chain. We intend to continue to outsource the manufacture and distribution of *Feraheme* and *Makena* for the foreseeable future, and we believe this manufacturing strategy will enable us to direct more of our financial resources to the commercialization of our products. Under the terms of the MuGard License Agreement, PlasmaTech is responsible for all aspects of manufacturing *MuGard*. We have entered into a quality agreement and a supply agreement with PlasmaTech under which we purchase *MuGard* inventory from PlasmaTech.

To support the commercialization of our products, we have developed a fully integrated manufacturing support system, including quality assurance, quality control, regulatory affairs and inventory control policies and procedures. These support systems are intended to enable us to maintain high standards of quality for our products.

We have also established certain testing and release specifications with the FDA and other foreign regulatory agencies. This release testing must be performed in order to allow the finished product to be used for commercial sale.

Makena

The *Makena* drug product for our commercial and clinical use is currently manufactured by Hospira Worldwide, Inc. ("Hospira") under a Development and Supply Agreement, originally dated September 17, 2009, by and between Hologic, Inc. (from whom Lumara Health, then-named K-V Pharmaceutical Company ("K-V Pharmaceutical") originally purchased the worldwide rights to *Makena*) and Hospira, which was fully assigned to K-V Pharmaceutical in December 2012, and was amended on March 28, 2014 (as amended, the "Hospira Agreement"). Under the terms of the Hospira Agreement, Hospira was manufacturing *Makena* at certain agreed-upon pricing through December 31, 2014 and currently Hospira can increase the price (subject to certain limitations) of *Makena* for both commercial and clinical uses, upon advance written notice to us. In addition, under the terms of the Hospira Agreement we are obligated to make certain minimum purchase requirements. The term of the Hospira Agreement applies to the manufacture of certain dosage forms and provides for an option to extend the term based on the occurrence, timing and amount of certain forecasts and purchase orders related to other dosage forms. We cannot make any guarantees that we will be able to extend the term of the Hospira Agreement on favorable terms, if at all.

Lumara Health, as our wholly owned subsidiary following consummation of the acquisition, is subject to certain continuing obligations under a Consent Decree of Permanent Injunction (the "Consent Decree") among the FDA, Lumara Health's predecessor company, K-V Pharmaceutical and certain former officers and affiliates of K-V Pharmaceutical. In particular, Lumara Health is bound by a number of provisions and requirements in the Consent Decree including, but not limited to, inspection of Lumara Health's places of business by the FDA without prior notice, and notification of FDA of particular actions and events. If Lumara Health fails to comply with applicable provisions in the

Table of Contents

Consent Decree, the Federal Food, Drug, and Cosmetic Act (the "FDC Act") or the FDC Act's implementing regulations, the FDA may impose specific sanctions including, but not limited to, the requirement to cease any of Lumara Health's manufacturing operations, the imposition of substantial financial penalties, and the requirement to implement additional corrective actions.

Feraheme

We currently have the following contracts in place related to the manufacture of *Feraheme*:

Sigma-Aldrich, Inc.

In August 2012, we entered into a Commercial Supply Agreement, as amended in October 2013 and December 2014, with Sigma-Aldrich, Inc. ("SAFC") pursuant to which SAFC agreed to manufacture and we agreed to purchase from SAFC, the active pharmaceutical ingredient ("API") or the drug product intermediate ("DPI") for use in the finished product of ferumoxytol for U.S. commercial sale, for sale outside of the U.S., as well as for use in clinical trials (as amended, the "SAFC Agreement"). Subject to certain conditions, the SAFC Agreement provides that we purchase from SAFC certain minimum quantities of API or DPI each year, but we are not obligated to use SAFC as our sole supplier of API or DPI. In addition, the prices for each batch will decline as batches are produced in greater quantities throughout each year of the agreement. The SAFC Agreement has an initial term that ends December 31, 2020, which may be automatically extended thereafter for additional two year periods, unless cancelled by us or SAFC within an agreed-upon notice period.

The amendments to the SAFC Agreement provide updated pricing terms beginning on a certain date in the future, which are based on the amount of product produced by SAFC in a given calendar year. If SAFC is unable to offer these agreed-upon prices, we may terminate our minimum purchase commitments. In addition, if SAFC is unable to meet our actual demand requirements other than due to our acts, omissions or default, our minimum purchase commitment will be suspended for such period. Further, if after a certain date in the future, SAFC is unable to match a *bona fide* offer from a third party to manufacture and supply product to us on better terms than provided by SAFC pursuant to the SAFC Agreement then a reduced minimum purchase commitment will apply. We have the right to terminate the SAFC Agreement and any purchase orders under certain conditions and subject to certain notice requirements. The SAFC Agreement also specifies cost-sharing arrangements relating to future process changes or capital improvements to the manufacturing process for *Feraheme* under the SAFC Agreement.

Patheon, Inc. (formerly DSM Pharmaceuticals, Inc.)

In January 2010, we entered into a Pharmaceutical Manufacturing and Supply Agreement, as amended in July 2014, with Patheon, Inc. (formerly DSM Pharmaceuticals, Inc.) ("Patheon") pursuant to which Patheon agreed to manufacture ferumoxytol finished drug product for U.S. commercial sale, for sale outside of the U.S., as well as for use in clinical trials at a fixed price per vial (as amended, the "Patheon Agreement"). The Patheon Agreement will continue in force until December 31, 2015. The Patheon Agreement may be terminated at any time upon mutual written agreement by us and Patheon or at any time by us subject to certain notice requirements and early termination fees. In addition, the Patheon Agreement may be terminated by either us or Patheon in the event of a material breach of the agreement by the other party provided that the breaching party fails to cure such breach within an agreed-upon notice period.

Raw Materials

We and our third-party manufacturers currently purchase certain raw and other materials used to manufacture *Feraheme* and *Makena* from third-party suppliers and, at present, do not have long-term

Table of Contents

supply contracts with most of these third parties. Although certain of our raw or other materials are readily available, others may be obtained only from qualified suppliers. Certain materials used in *Feraheme* and *Makena* may from time to time be procured from a single source without a qualified alternative supplier of the high-quality standards imposed on our raw and other materials used to manufacture *Feraheme*, we may not be able to obtain such materials of the quality required to manufacture *Feraheme* or *Makena*. The qualification of an alternative source may require repeated testing of the new materials and generate greater expenses to us if materials that we test do not perform in an acceptable manner. In addition, we or our third-party manufacturers sometimes obtain raw or other materials from one vendor only, even where multiple sources are available, to maintain quality control and enhance working relationships with suppliers, which could make us susceptible to price inflation by the sole supplier, thereby increasing our production costs. As a result of the high-quality standards imposed on our raw or other materials, we or our third-party manufacturers may not be able to obtain such materials of the quality required to manufacture *Feraheme* or *Makena* from an alternative source on commercially reasonable terms, or in a timely manner, if at all.

Patents and Trade Secrets

We consider the protection of our technology to be material to our business. Because of the substantial length of time and expense associated with bringing new products through development and regulatory approval to the marketplace, we place considerable importance on obtaining patent and trade secret protection for our products. Our success depends, in large part, on our ability to maintain the proprietary nature of our technology and other trade secrets. To do so, we must prosecute and maintain existing patents, obtain new patents and ensure trade secret protection. We must also operate without infringing the proprietary rights of third parties or allowing third parties to infringe our rights.

Our policy is to aggressively protect our competitive technology position by a variety of means, including applying for patents in the U.S. and in appropriate foreign countries. We currently hold a number of U.S. and foreign patents, which expire at various times through 2023. One of our U.S. *Feraheme* patents is subject to a patent term extension under U.S. patent law and FDA regulations and will expire in June 2023. There are no patents covering *Makena.* We have a license to two U.S. patents relating to *MuGard*, that each expire in 2022. Our foreign patents may also be eligible for extension in accordance with applicable law in certain countries.

We also have patent applications pending in the U.S. and have filed counterpart patent applications in certain foreign countries directed to *Feraheme*. Although further patents may be issued on pending applications, we cannot be sure that any such patents will be issued on a timely basis, if at all. In addition, any issued patents may not provide us with competitive advantages or may be challenged by others, and the existing or future patents of third parties may limit our ability to commercialize *Feraheme*. For example, in July 2010, Sandoz GmbH ("Sandoz") filed with the European Patent Office (the "EPO") an opposition to a previously issued patent which covers ferumoxytol in EU jurisdictions. In October 2012, at an oral hearing, the Opposition Division of the EPO revoked this patent. In December 2012, our notice of appeal of that decision was recorded with the EPO, which suspended the revocation of our patent. On May 13, 2013, we filed a statement of grounds of appeal and on September 27, 2013, Sandoz filed a response to that statement. We filed a reply to that response on March 17, 2014 and oral proceedings for the appeal are scheduled for June 16, 2015. We continue to defend the validity of this patent throughout the appeals process, which we expect to take two to three years. However, in the event that we do not experience a successful outcome from the appeals process, under EU regulations ferumoxytol would still be entitled to eight years of data protection and ten years of market exclusivity from the date of approval, which we believe would create barriers to entry for any generic version of ferumoxytol into the EU market until sometime between 2020 and 2022.

Table of Contents

Our licensed patent rights to *MuGard* may not prevent competitors from independently developing and marketing a competing product that does not infringe our licensed patents or other intellectual property. Further, there are no patents covering *Makena* and thus the successful commercialization of *Makena* is significantly reliant on our ability to take advantage of its orphan drug exclusivity.

Frequently, the unpredictable nature and significant costs of patent litigation leads the parties to settle to remove any uncertainty related to the status of their patents. Settlement agreements between branded companies and generic applicants may allow, among other things, a generic product to enter the market prior to the expiration of any or all of the applicable patents covering the branded product, either through the introduction of an authorized generic or by providing a license to the applicant for the patents in suit.

Competition

The pharmaceutical and biopharmaceutical industries are intensely competitive and subject to rapid technological change. Many of our competitors for *Feraheme* are large, well-known pharmaceutical companies and may benefit from significantly greater financial, sales and marketing capabilities, greater technological or competitive advantages, and other resources. For *Makena*, most of our competition comes from pharmacies that compound non-FDA approved formulations of HPC (defined below), which are sold at a lower cost than *Makena*. In addition, generic *Feraheme* and *Makena* competitors could enter the market through approval of abbreviated new drug applications ("ANDAs") that use *Feraheme* or *Makena* as a reference listed drug, which would allow generic competitors to rely on *Feraheme's* or *Makena's* safety and efficacy trials instead of conducting their own studies. Because entry into the market can occur upon the expiration of the reference listed drug's exclusivity, we could face such competition in the near-term as *Feraheme's* U.S. market exclusivity expired in June 2014 and *Makena's* orphan drug exclusivity expires in February 2018. Our existing or potential new competitors for *Feraheme* and *Makena* may develop products that are more widely accepted than ours and may receive patent protection that dominates, blocks or adversely affects our product development or business.

Makena

Although *Makena* is the only FDA-approved drug indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth, it competes for market share with compounding pharmacies. Hydroxyprogesterone caproate ("HPC") is the active ingredient in *Makena.* Compounding pharmacies have been manufacturing formulations of HPC (which compounded formulations we refer to as "c17P") for many years and c17P formulations will likely remain available even though *Makena* has been granted orphan drug exclusivity until February 2018. We estimate that between approximately 40% and 50% of the at-risk patient population is treated with c17P. *Makena* currently has between approximately 20% and 30% of the market share of the at-risk patient population with at least 30% of the at-risk patient population being treated either with other therapies that are not approved for women pregnant with a singleton with a prior history of spontaneous preterm birth of a singleton, or not treated at all.

In March 2011, the FDA issued a press release announcing that, in order to ensure continued access for patients, the FDA intended to refrain from taking enforcement action with respect to compounding pharmacies producing c17P in response to individual prescriptions for individual patients, resulting in a reduction in commercial value of *Makena*'s orphan exclusivity protection and in the loss of substantial market share to compounding pharmacies. In June 2012, the FDA recommended using FDA-approved *Makena* instead of a compounded drug except when there is a specific medical need (e.g., an allergy) that cannot be met by the approved drug. In July 2014, the FDA issued another public statement affirming the position it took in its June 2012 press release recommending use of FDA-approved *Makena,* except when there is a specific need for a compounded drug. The FDA also

Table of Contents

stated that when it identifies a pharmacist that compounds regularly or in inordinate amounts of any drug products that are essentially copies of *Makena*, the FDA intends to take enforcement action as it deems appropriate. Despite recent negative publicity regarding compounding pharmacies, including the 2012 meningitis outbreak involving compounded drugs, the November 2013 enactment of the federal Drug Quality and Security Act ("DQSA") and recent enforcement actions against compounders violating the FDC Act, *Makena* will likely continue to face competition from c17P, especially in light of the long-standing availability of such compounded products, their lower cost and the criticism Lumara Health received in the past in connection with the pricing of *Makena,* as discussed below.

Lumara Health was criticized for the initial list pricing of *Makena* in numerous news articles and internet postings following the FDA's February 2011 approval of *Makena*. Although the list price of *Makena* was subsequently reduced in March 2011, *Makena* is still priced at a premium to c17P, which has negatively impacted coverage of *Makena* by some state Medicaid programs and by certain commercial payers. Although we are undertaking efforts to educate physicians and patients about progress made toward expanding coverage of *Makena* and about the benefits of FDA-approved *Makena*, certain doctors continue to choose to prescribe non-FDA approved purported substitute products made by pharmaceutical compounders in lieu of prescribing *Makena*. In addition, efforts to appropriately respond to future concerns raised by media, professional societies, advocacy groups, policymakers or regulatory agencies regarding patient access to *Makena* are costly and may not be successful.

Additionally, in 1956, the FDA-approved the drug Delalutin, which contained the same active ingredient as *Makena*. Delalutin was approved for conditions other than reducing the risk of preterm birth and was marketed by Bristol-Myers Squibb ("BMS"). BMS stopped marketing and manufacturing the FDA-approved product and it was withdrawn from the market in 1999. In 2010, in response to a citizen petition, the FDA determined that Delalutin was not withdrawn from sale for reasons of safety or effectiveness. As such, generic drug applications may reference the withdrawn Delalutin NDA.

Thus, before the expiration of *Makena's* orphan exclusivity, the FDA could determine that it has the authority to approve ANDAs that reference Delalutin so long as the ANDAs meet all relevant legal and regulatory requirements for approval and are labeled for the same indications as Delalutin (*i.e*., not for the risk of preterm birth). If such an approval is granted, doctors may elect to prescribe such approved drug off-label (*i.e.*, outside of FDA-approved indications) for *Makena's* orphan-protected indication, which could have an adverse impact on our business and results of operations.

Moreover, if one or more generic applicants were to receive approval to sell a generic or follow-on version of *Makena* for the orphan-protected indication, those generic products could potentially be approved as early as February 3, 2018 (the date on which *Makena's* orphan exclusivity ends) and we would become subject to increased competition at that time.

For a detailed discussions regarding the risks and uncertainties related to competition for *Makena*, please refer to our Risk Factor, "*Our ability to successfully commercialize Makena is dependent upon a number of factors, including maintaining the benefits of Makena's orphan drug exclusivity and the length of time before competitors begin selling generic versions of Makena.*"

Feraheme

Although *Feraheme* is approved in the U.S. for the treatment of IDA in adult patients with CKD, including both dialysis and non-dialysis CKD patients, our U.S. commercial strategy is entirely focused on growing the utilization of *Feraheme* in non-dialysis dependent adult CKD patients who are diagnosed with IDA. We believe there is a significant opportunity in the U.S. for *Feraheme* for the treatment of IDA in CKD patients not yet on dialysis. The U.S. non-dialysis IV iron market is comprised primarily of three sites of care where a substantial number of CKD patients are treated: hospitals, hematology and oncology centers, and nephrology clinics.

Table of Contents

Feraheme currently competes with the following IV iron replacement therapies in the U.S. for the treatment of IDA in CKD patients:

Venofer®, an iron sucrose complex, which is approved for use in hemodialysis, peritoneal dialysis, non-dialysis dependent CKD patients and pediatric CKD patients and is marketed in the U.S. by Fresenius Medical Care North America and American Regent Laboratories, Inc. ("American Regent") a subsidiary of Luitpold Pharmaceuticals, Inc. Venofer® is typically administered as a slow intravenous injection over two to five minutes in doses of 100 to 200 milligrams, thus requiring five to ten physician visits to reach a standard one gram therapeutic course;

Injectafer®, a ferric carboxymaltose injection, which is known as Ferinject® in Europe, was approved in the U.S. in July 2013 to treat IDA in adult patients who have intolerance to oral iron or have had an unsatisfactory response to oral iron. Injectafer® is also indicated for IDA in adult patients with non-dialysis dependent CKD. Injectafer® is marketed in the U.S. by American Regent, the same distributor of Venofer®. The labeled administration of Injectafer® is two slow injections or infusion of 750 milligrams each separated by at least seven days for a total cumulative dose of 1,500 milligrams, or one and a half grams per therapeutic course;

Ferrlecit®, a sodium ferric gluconate, which is marketed by Sanofi-Aventis U.S. LLC, is approved for use only in hemodialysis patients. The recommended dose of Ferrlecit® and the generic version of Ferrlecit® is 125 milligrams administered by intravenous infusion over one hour per dialysis session or undiluted as a slow intravenous injection per dialysis session, thus requiring eight physician visits to reach a standard one gram therapeutic course;

A generic version of Ferrlecit® marketed by Watson Pharmaceuticals, Inc. ("Watson");

INFeD®, an iron dextran product marketed by Watson, which is approved in the U.S. for the treatment of patients with documented iron deficiency in whom oral iron administration is unsatisfactory or impossible. The recommended dose of INFeD® is a slow push in 100 milligram doses, which would require up to ten physician visits to receive a standard one gram therapeutic course; and

Dexferrum®, an iron dextran product marketed by American Regent, which is approved in the U.S. for the treatment of patients with documented iron deficiency in whom oral iron administration is unsatisfactory or impossible. The recommended dose of INFeD® and Dexferrum® is a slow push in 100 milligram doses, which would require up to ten physician visits to receive a standard one gram therapeutic course.

As compared to the dosing regimens described above for *Feraheme's* U.S. competitors, *Feraheme* is currently administered as a 510 milligram injection or infusion followed by a second 510 milligram injection or infusion three to eight days later. In June 2014, we proposed changes to the FDA related to our current U.S. label of *Feraheme* based on a review of global post-marketing data to strengthen the warnings and precautions section of the label and mitigate the risk of serious hypersensitivity reactions, including anaphylaxis, in order to enhance patient safety. After considering our June 2014 submission and other information, in January 2015, the FDA notified us that it believes new safety information should be included in the labeling for *Feraheme*, including, among other things, a boxed warning to highlight the risks of serious hypersensitivity/anaphylaxis reactions and revisions that *Feraheme* should only be administered through an IV infusion (*i.e.*, not by IV injection) and should be contraindicated for patients with any known history of drug allergy. These or any future changes to the label/package could adversely impact our ability to successfully compete in the U.S. IV iron market.

Pharmacosmos A/S ("Pharmacosmos") the producer of another IV iron, Monofer® (iron isomaltoside 1000), which is approved and marketed in Europe, is also conducting clinical trials in the U.S. and may try to gain regulatory approval in the U.S. for Monofer®. In January 2015, the Helsinn

Table of Contents

Group and Pharmacosmos announced that they entered into an agreement for the exclusive U.S. commercialization rights to Monofer®.

Outside of the U.S., *Feraheme* also competes with a number of branded IV iron replacement products, including Venofer®, Ferrlecit®, Monofer®, Ferinject® (ferric carboxymaltose injection) (the brand name for Injectafer® outside the U.S.) and certain other iron dextran and iron sucrose products. Venofer® and Ferrlecit®, described above, have been marketed in many countries throughout the world, including most of Europe and Canada, for many years. Monofer® is an injectable iron preparation developed by Pharmacosmos, which is currently approved for marketing in approximately 30 countries, primarily in Europe, for the treatment of IDA. Ferinject® is an IV iron replacement therapy developed by Vifor Pharma, the pharmaceuticals business unit of the Galenica Group, and is currently approved for marketing in approximately 62 countries worldwide, for the treatment of iron deficiency where oral iron is ineffective or cannot be used.

Currently, all other IV iron products approved and marketed in the EU are approved for marketing to a broader group of patients with IDA. *Rienso* was approved only for use in CKD patients. In January 2015, we and Takeda mutually agreed to withdraw the application of Type II Variation for *Rienso* in the EU to extend the therapeutic indication from adult patients with IDA associated with CKD to adult patients with iron deficiency from any underlying cause. The limitation of *Rienso's* approved indication to CKD patients may put *Feraheme* at a competitive disadvantage if we were to pursue commercialization efforts in the EU with the product's currently labelled indicated patient population. In addition, based on PRAC's July and January Recommendations, Takeda issued a DHPC letter providing that, among other measures, *Riesno* be administered to patients by infusion over at least 15 minutes (replacing injection) and that it be contraindicated in patients with any known history of drug allergy, and we and Takeda are in the process of updating the label to caution that elderly patients or patients with multiple co-morbidities who experience a serious hypersensitivity reaction due to *Rienso* may have more severe outcomes, and related variations to the Summary of Product Characteristics ("SmPC"). These or any future changes to *Feraheme's* current indication could further put it at a disadvantage to its competitors. As discussed above, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada.

Feraheme may also face competition from generic IV iron replacement therapy products that achieve commercial success. For example, in 2011, Watson launched a generic version of Ferrlecit® in the U.S. which is approved for marketing in the U.S. for the treatment of IDA in adult patients and in pediatric patients age six years and older undergoing chronic hemodialysis who are receiving supplemental epoetin therapy. Sagent Pharmaceuticals, Inc. has also indicated its intention to introduce a generic iron sucrose in the U.S. in the future. Outside the U.S., there is currently a generic version of Venofer®.

The Drug Price Competition and Patent Term Restoration Act of 1984, as amended (the "Hatch-Waxman Act") requires an applicant whose subject drug is a drug listed in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," also known as the "Orange Book," to notify the patent-holder of their application and potential infringement of their patent rights. If an applicant for ferumoxytol notifies us of such application, we would have 45 days upon receipt of that notice to bring a patent infringement suit in federal district court against the applicant seeking approval of a product. If such a suit is commenced, the FDA is generally prohibited from granting approval of an application until the earliest of 30 months from the date the FDA accepted the application for filing, the conclusion of litigation in the generic's favor, or expiration of the patent(s). If the litigation is resolved in favor of the applicant or the challenged patent expires during the 30-month stay period, the stay is lifted and the FDA may thereafter approve the application based on the applicable standards for approval.

Table of Contents

A generic version of *Feraheme* can be marketed only with the approval of the FDA of the respective application for such generic version. In December 2012, the FDA issued draft guidance making recommendations regarding establishing bioequivalence with *Feraheme*, pursuant to which a party could seek approval of a generic version of *Feraheme* through an ANDA. The FDA generally publishes product-specific bioequivalence guidance after it has received an inquiry from a generic drug manufacturer about submitting an ANDA for the product in question; thus, it is possible that a generic drug manufacturer has approached the FDA requesting guidance about submitting an ANDA for ferumoxytol, the active ingredient in *Feraheme*, and that such an ANDA may be filed in the near future. The ANDA process is discussed in more detail below under the heading "*U.S. Approval Process Abbreviated New Drug Application."*

Companies that manufacture generic products typically invest far fewer resources in research and development than the manufacturers of branded products and can therefore price their products significantly lower than those branded products already on the market. Therefore, competition from generic IV iron products could limit our sales.

We believe that our ability to successfully compete with other IV iron products depends on a number of factors, including the actual or perceived safety and efficacy profile of *Feraheme* as compared to alternative iron replacement therapeutics, current and future limitations on *Feraheme's* approved indications and patient populations, our ability to obtain and maintain favorable pricing, insurance coverage and reimbursement rates and terms for *Feraheme*, our ability to implement effective marketing programs, the effectiveness of our sales force, our ability to maintain favorable patent protection for *Feraheme*, market acceptance of *Feraheme,* and our ability to manufacture sufficient quantities of *Feraheme* at commercially acceptable costs. For additional details on the risks and uncertainties regarding *Feraheme's* competition, see our Risk Factor, "*Market acceptance of Feraheme may suffer as a result of the widespread use of competing iron replacement therapy products, including Injectafer®, and as a result of the approval of generic drug products in the near-term, which would have a material adverse effect on our operations and our profitability.*"

Based on sales data provided to us in January 2015 by IMS Health Incorporated ("IMS"), we estimate that the size of the total 2014 U.S. non-dialysis IV iron replacement therapy market was approximately 900,000 grams, which represents an increase of approximately 6% over 2013. Based on this IMS data, the following represents the 2014 and 2013 U.S. market share allocation of the total non-dialysis IV iron market based on the volume of IV iron administered:

	2014 U.S. Non-dialysis IV Iron Market (900,000 grams)	**2013 U.S. Non-dialysis IV Iron Market (851,000 grams)**
Venofer®	43%	46%
INFeD®	20%	22%
Feraheme	16%	15%
Generic sodium ferric gluconate	10%	10%
Injectafer®	6%	<1%
Ferrlecit®	5%	6%
Dexferrum®	<1%	<1%

The market share data listed in the table above is not necessarily indicative of the market shares in dollars due to the variations in selling prices among the IV iron products.

MuGard

Up to 50% of certain new cancer patients develop oral mucositis each year for which there are currently few effective treatments. The market for treating oral mucositis is driven primarily by convenience, price and reimbursement and the products in this market remain mostly undifferentiated.

Table of Contents

There are a number of approaches used to treat or manage oral mucositis, including the use of ice chips during chemotherapy treatments, various medicinal mouthwashes, topical anesthetics and analgesics, and oral gel treatments. For example, many physicians use what is commonly known as "magic mouthwash", which may currently be the most commonly prescribed medication to manage oral mucositis or treat the pain associated with mucositis caused by radiation therapy or chemotherapy. Magic mouthwash is a combination of generic ingredients which are typically compounded in a pharmacy and is preferred by many physicians because of the availability of less expensive generic ingredients used to formulate the mouthwash. However, there is no clinical trial data to support the efficacy or safety of magic mouthwash. The efficacy of *MuGard* has been supported by a randomized, Phase IV multicenter, double-blind, sham-controlled trial.

There are a number of companies in the U.S. commercializing products for the management or treatment of oral mucositis that may compete with *MuGard,* including the following marketed products:

NeutraSal® (supersaturated calcium phosphate rinse), a prescription mouth rinse marketed by Invado Pharmaceuticals, LLC and indicated to treat the painful symptoms associated with oral mucositis;

Caphosol®, a supersaturated calcium phosphate artificial saliva marketed by Jazz Pharmaceuticals, PLC, which is indicated as an adjunct to standard oral care in treating oral mucositis caused by radiation or high dose chemotherapy; and

Kepivance® (palifermin), an IV human growth factor manufactured by Amgen and marketed by Swedish Orphan Biovitrum AB, which is used to reduce the chances of developing severe mucositis and to shorten the time with severe mucositis in patients with cancer who receive high doses of chemotherapy and radiation therapy.

Further, there are several marketed products available which are indicated for the management of pain associated with oral mucositis including the following products:

Episil®, marketed by Cangene BioPharma, Inc., is indicated for the management of pain and relief from pain, by adhering to the mucosal surface of the mouth, soothing oral lesions of various etiologies, including oral mucositis/stomatitis that may be caused by chemotherapy or radio therapy;

Gelclair®, marketed by DARA BioSciences, Inc., is a viscous, concentrated, bio adherent oral gel, indicated for the management of painful symptoms of mucositis of the oropharyngeal cavity caused by chemo-radiotherapy; and

GelX® Oral Gel, marketed by Praelia Pharmaceuticals, Inc., is an oral gel indicated for the relief and management of pain by adhering to the mucosal surface of the mouth and soothing oral lesions of various etiologies, including oral mucositis/stomatitis (may be caused by chemotherapy or radiotherapy), irritation due to oral surgery, aging, and traumatic ulcers caused by braces or ill-fitting dentures, medication, or disease.

Based on data provided to us in January 2015 by IMS we estimate that the total number of prescriptions ("TRx's") filled in the U.S. in 2014 for the treatment or management of oral mucositis was approximately 16,500. The following represents the 2014 market share allocation based on TRx data to treat or manage oral mucositis, which accounts for approximately 75% of the total oral

Table of Contents

mucositis business. These figures do not include products purchased by hospitals or outpatient clinics, such as Kepivance®:

	2014 Oral Mucositis Market (16,500 TRx)	2013 Oral Mucositis Market (14,900 TRx)
Neutrasal®	49%	46%
Caphosol®	16%	23%
Gelclair®	14%	3%
MuGard	12%	13%
Episil®	7%	11%
GelX®	2%	4%

The market share data listed in the table above is not necessarily indicative of the market shares in dollars due to the variations in selling prices among the oral mucositis products.

Sales, Marketing and Distribution

Makena

In November 2014, we completed our acquisition of Lumara Health, including its commercialized drug *Makena*, a progestin indicated to reduce the risk of preterm birth in women with a singleton pregnancy who have a history of singleton spontaneous preterm birth. In connection with the acquisition, we retained the *Makena* commercial team, including 88 members of the Lumara Health sales team dedicated exclusively to the OB/GYN subspecialty. We sell *Makena* to specialty pharmacies and distributors, who, in turn sell *Makena* to healthcare providers, hospitals, government agencies and integrated delivery systems.

We estimate that *Makena* is currently used to treat between 20% and 30% of the at-risk patient population, allowing for significant potential to increase its market share. Our sales and marketing teams use a variety of common pharmaceutical marketing strategies and methods to promote *Makena*, including dedicating a separate reimbursement team to focus on health plans, both commercial and managed Medicaid as well as fee-for-service Medicaid programs.

In addition, we offer customer support through the Makena Care Connection, a support program for patients and healthcare providers that provides administrative, financial assistance and treatment support for *Makena.* Administrative and treatment support includes insurance benefit investigation, reimbursement and patient assistance programs. Because specialty injectable products like *Makena* are not typically carried by retail pharmacies, the process for facilitating prescriptions for *Makena* is managed by this dedicated customer support center. In December 2013, the Makena Care Connection initiated a pilot program in California designed to improve overall customer satisfaction by reducing the time from the prescription to the initiation of therapy, increasing the average number of injections per patient and increasing the number of paying patients. Favorable results from the pilot project led to a national roll out of the customer service initiative in 2014.

We also operate a patient assistance program for *Makena* that provides co-pay assistance (for insured patients), and financial assistance (for uninsured patients). Under the program, patients with a household income of $120,000 or less pay $20 or less per injection of *Makena*. This encompasses 85% of the U.S. based on 2009 U.S. census data. Clinically eligible patients who are uninsured and whose financial need is greatest will receive *Makena* at no cost. There are no upper-level income caps to qualify for the patient assistance program.

In early 2015, we plan to launch a telephonic 24/7 nursing services program to increase patient compliance (*i.e.*, following a weekly injection regime) via education and awareness of preterm birth and *Makena*'s benefits. The program will provide a registered nurse to each expectant mother who will be

Table of Contents

available to answer patient questions and guide the patient to her provider for necessary care ensuring all the patient's questions and concerns are addressed.

Feraheme

In July 2009, we began U.S. commercial sale of *Feraheme,* which is being marketed and sold in the U.S. through our commercial organization, including a specialty sales force. We sell *Feraheme* to authorized wholesalers and specialty distributors who, in turn, sell *Feraheme* to healthcare providers who administer *Feraheme* primarily within hospitals, hematology and oncology centers and nephrology clinics. Since many hospitals and hematology, oncology and nephrology practices are members of group purchasing organizations ("GPOs"), which leverage the purchasing power of a group of entities to obtain discounts based on the collective bargaining power of the group, we also routinely enter into pricing agreements with GPOs in these markets so the members of the GPOs have access to *Feraheme* and to the related discounts or rebates.

Our sales and marketing organization uses a variety of common pharmaceutical marketing strategies and methods to promote *Feraheme* including sales calls to purchasing entities, such as hospitals, hematology and oncology centers and nephrology practices in addition to individual physicians or other healthcare professionals, medical education symposia, personal and non-personal promotional materials, local and national educational programs, scientific meetings and conferences and informational and disease state awareness websites. In addition, we provide customer service and other related programs for *Feraheme* including physician reimbursement support services, a patient assistance program for uninsured or under-insured patients and a customer service call center.

Our commercial strategy currently focuses on the non-dialysis dependent CKD market in the U.S. We believe there is a significant opportunity in this market to provide IV iron to non-dialysis CKD patients, and our sales team has been working to educate physicians who treat CKD patients on the benefits of IV iron and the dosing profile of *Feraheme* in order to change existing treatment paradigms and expand the IV iron use in physicians' offices, clinics, and hospitals where CKD patients are treated for IDA.

Feraheme has been granted marketing approval in the EU, Canada, Iceland, Liechtenstein, Norway and Switzerland for use as an IV iron replacement therapy for the treatment of IDA in adult patients with CKD and was commercially launched in the EU, Canada, and Switzerland in 2012. In December 2014, we entered into the Takeda Termination Agreement with Takeda, under which we are in the process of regaining all worldwide development and commercialization rights for *Feraheme*. Prior to the Takeda Termination Agreement, Takeda was solely responsible for *Feraheme* commercialization efforts in these areas, including the deployment of a specialized sales force, pricing and reimbursement negotiations with national, provincial or local health authorities and customers, and development of market access strategies. Sales of *Feraheme* outside of the U.S. do not and are not expected to materially contribute to our revenues. As such, and in light of the Takeda Termination Agreement, we and Takeda have come to the mutual decision to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada.

MuGard

In June 2013, we acquired the U.S. rights to *MuGard* from PlasmaTech. We began comprehensive promotional activities related to *MuGard* in the third quarter of 2013, including training our sales force and developing new marketing materials, such as healthcare provider brochures, patient materials, reimbursement information and starter kits. To optimize the sales potential of both of our commercial products, our initial call targets for *MuGard* included current *Feraheme* prescribers as well as other high prescribing clinicians, including radiation oncologists who manage head and neck cancer patients undergoing radiation therapy where the incidence of oral mucositis could approximate 80%. Our current commercial strategy for *MuGard* includes differentiating *MuGard* from other currently used approaches for treating and managing oral mucositis, targeting oral mucositis prescribers and expanding reimbursement coverage for *MuGard*.

Table of Contents

Our sales and marketing teams use a variety of common pharmaceutical marketing strategies and methods to promote *MuGard,* including sales calls to providing entities, such as hospitals and hematology and oncology centers. In addition, other tactical programs may include personal and non-personal promotional materials to individual physicians or other healthcare professionals, sponsoring local and national educational programs, participation in scientific meetings and conferences and implementing informational product specific websites.

We market and sell *MuGard* to wholesalers and specialty pharmacies. Patients primarily receive *MuGard* through specialty pharmacies, which receive prescriptions from either our *MuGard* patient reimbursement and support center (the "HUB") or from physicians directly. We utilize the HUB as a centralized patient intake and referral management center to process insurance coverage issues and administer our patient assistance and copayment programs. In order to provide *MuGard* to patients as soon as possible, we have implemented a robust program that delivers a starter kit to clinicians, including a sample bottle and all pertinent information that the patient or caregiver needs to immediately begin *MuGard* therapy.

Product Supply Chain

We outsource a number of our product supply chain services for our products to third-party logistics providers, including services related to warehousing and inventory management, distribution, chargeback processing, accounts receivable management, sample distribution to our sales force, and customer service call center management.

Major Customers

The following table sets forth customers who represented 10% or more of our total revenues for 2014, 2013, and 2012. Revenues from Takeda include *Feraheme* collaboration revenue, milestone payments, revenues from product sales to Takeda and royalty payments, in each case in connection with the Amended Takeda Agreement.

	Years Ended December 31,		
	2014	**2013**	**2012**
AmerisourceBergen Drug Corporation	34%	41%	34%
McKesson Corporation	21%	24%	17%
Cardinal Health, Inc.	15%	16%	12%
Takeda Pharmaceuticals Company Limited	11%	11%	31%

In addition, approximately 26%, 30% and 32% of our *Feraheme* end-user demand in 2014, 2013 and 2012, respectively, was generated by members of a single GPO with which we have contracted.

The loss of any of these customers would have a material adverse effect on our business.

Government Regulation

Overview

Our activities are subject to extensive regulation by numerous governmental authorities in the U.S. and abroad. In the U.S., the FDC Act and other federal and state statutes and regulations govern, among other things, the research and development, manufacturing, quality control, labeling, recordkeeping, approval, storage, distribution, and advertising and promotion of pharmaceutical products and medical devices. Our activities outside of the U.S. are also subject to regulatory requirements governing the testing, approval, safety, effectiveness, manufacturing, labeling, and marketing of *Feraheme*.

Failure to comply with any of the applicable U.S. or foreign regulatory requirements may result in a variety of administrative or judicially imposed sanctions including, among other things, the regulatory

Table of Contents

agency's refusal to approve pending applications, suspension, variations or withdrawals of approval, clinical holds, warning letters, product recalls, product seizures, total or partial suspension of operations, injunctions, fines, civil penalties, or criminal prosecution.

U.S. Approval Process

Clinical Development

Before we may market a new human drug product in the U.S., we must obtain FDA approval of a NDA for that product. The FDA may approve an NDA if the safety and effectiveness of the drug candidate can be established based on the results of clinical trials.

Clinical testing proceeds in three phases. Phase I trials seek to establish initial data about safety, tolerability, and optimal dosing of the drug candidate in humans. The goal of Phase II trials is to provide evidence about the desired therapeutic efficacy of the product candidate in limited studies with small numbers of carefully selected subjects. Phase III trials generally consist of expanded, large-scale, randomized, double-blind, multi-center studies of the safety and effectiveness of the product in the target patient population.

Although we currently have no new unapproved drugs in development and our intention is to expand our portfolio with additional commercial-stage specialty products, we would be required to comply with the requirements for drug approval if we develop new or acquire earlier-stage products.

Submission and FDA Review of NDAs/sNDAs

Following the successful completion of clinical trials, the sponsor submits the results to the FDA as part of an NDA. The NDA must also include the results of pre-clinical tests and studies, information related to the preparation and manufacturing of the drug candidate, analytical methods, and proposed packaging and labeling. Pursuant to the Prescription Drug User Fee Act ("PDUFA"), the FDA has a goal of acting on most original NDAs within six months or ten months of the application filing date, depending on the nature of the drug. For drugs candidates intended to treat serious and life-threatening conditions, the FDA has a number of programs intended to help expedite testing, review, and approval. For example, under the provisions of the FDA's Subpart H accelerated approval is permitted for a new drug that is intended to treat a serious or life-threatening disease or condition and that fills an unmet medical need based on a surrogate endpoint.

If the FDA's evaluations of the NDA and of the sponsor's manufacturing facilities are favorable, the FDA will issue an approval letter, and the sponsor may begin marketing the drug in the U.S. for the approved indications, subject to any post-approval requirements described further below. If the FDA determines it cannot approve the NDA in its current form, it will issue a complete response letter indicating that the application will not be approved in its current form. The complete response letter usually describes the specific deficiencies that the FDA identified in the application and may require additional clinical or other data or impose other conditions that must be met in order to obtain approval of the NDA. Addressing the deficiencies noted by the FDA could be impractical and it is possible that approval may not be obtained, or may be costly and may result in significant delays prior to approval.

Where a sponsor wishes to expand the originally approved prescribing information, such as adding a new indication, it must submit and obtain approval of an sNDA. Changes to an indication generally require additional clinical studies, which can be time-consuming and require the expenditure of substantial additional resources. Under PDUFA, the target timeframe for the review of an sNDA to add a new clinical indication is ten months from the date of filing. As with an NDA, if the FDA determines that it cannot approve an sNDA in its current form, it will issue a complete response letter as discussed above. See the discussion above under *"Feraheme for the treatment of IDA in a broad range of patients*" for our ongoing post-marketing activities for *Feraheme*.

Table of Contents

Abbreviated New Drug Application

An ANDA is filed when approval is sought to market a generic equivalent of a drug product previously approved under an NDA and listed in the Orange Book. Rather than directly demonstrating the product's safety and effectiveness, as is required of an NDA, an ANDA must show that the proposed generic product is the same as the previously approved product in terms of active ingredient(s), strength, dosage form, route of administration and bioavailability. In addition, with certain exceptions, the generic product must have the same labeling as the product to which it refers.

NDA applicants and NDA holders must provide certain information about patents related to the branded drug for listing in the Orange Book. When an ANDA application is submitted, it must contain one of several possible certifications regarding each of the patents listed in the Orange Book for the branded product that is the reference listed drug. A certification that a listed patent is invalid or will not be infringed by the sale of the proposed product is called a Paragraph IV Certification.

Adverse Event Reporting

The FDA requires a sponsor to submit reports of certain information on side effects and adverse events ("AEs") associated with its products that occur either during clinical trials or after marketing approval. These requirements include specific and timely notification of certain serious, unexpected and/or frequent AEs, as well as regular periodic reports summarizing adverse drug experiences. Failure to comply with these FDA safety reporting requirements may result in FDA regulatory action that may include civil action or criminal penalties. In addition, as a result of these reports, the FDA could create a Tracked Safety Issue for a product in the FDA's Document Archiving, Reporting and Regulatory Tracking System, place additional limitations on an approved product's use, such as through labeling changes, or, potentially, could require withdrawal or suspension of the product from the market.

FDA Post-Approval Requirements

Even if initial approval of an NDA or sNDA is granted, such approval may be subject to post-market regulatory requirements, any or all of which may adversely impact a sponsor's ability to effectively market and sell the approved product. The FDA may require the sponsor to conduct Phase IV clinical trials, also known as post-marketing requirements or post-marketing commitments, to provide additional information on safety and efficacy. The results of such post-market studies may be negative and could lead to limitations on the further marketing of a product. Also, under the Pediatric Research Equity Act, the FDA may require pediatric assessment of certain drugs unless waived or deferred due to the fact that necessary studies are impossible or highly impractical to conduct or where the drug is not likely to be used in a substantial number of pediatric patients, for example. In addition, the FDA may require a sponsor to implement a Risk Evaluation Mitigation Strategy ("REMS"), a strategy to manage a known or potential serious risk associated with the product. Failure to comply with REMS requirements may result in civil penalties. Further, if an approved product encounters any safety or efficacy issues, including drug interaction problems, the FDA has broad authority to force the sponsor to take any number of actions, including but not limited to, undertaking post-approval clinical studies, implementing labeling changes, adopting a REMS, issuing DHPC letters, or removing the product from the market.

FDA Regulation of Product Marketing and Promotion

The FDA also regulates all advertising and promotional activities for prescription drugs, both prior to and after approval. Approved drug products must be promoted in a manner consistent with their terms and conditions of approval, including the scope of their approved use. The FDA may take enforcement action against a company for promoting unapproved uses of a product, or off-label promotion, or for other violations of its advertising and labeling laws and regulations. Failure to comply with these requirements could lead to, among other things, adverse publicity, product seizures, civil or

Table of Contents

criminal penalties, or regulatory letters, which may include warnings and require corrective advertising or other corrective communications to healthcare professionals.

Under the Subpart H regulations, until the *Makena* confirmatory post-marketing clinical trial is completed, we are subject to a special 30-day promotional material review by the FDA's Office of Promotional Drug Products ("OPDP"). This extra requirement means that there is a longer lead time before we are able to introduce new promotional material to the market for *Makena* and we are subject to increased scrutiny prior to using promotional pieces to ensure fair balance.

FDA Regulation of Manufacturing Facilities

Manufacturing procedures and quality control for approved drugs must conform to cGMP. Domestic manufacturing establishments must follow cGMP at all times, and are subject to periodic inspections by the FDA in order to assess, among other things, cGMP compliance. In addition, prior to approval of an NDA or sNDA, the FDA will perform a pre-approval inspection of the sponsor's manufacturing facility, including its equipment, facilities, laboratories and processes, to determine the facility's compliance with cGMP and other rules and regulations. Vendors that supply finished products or components to the sponsor that are used to manufacture, package, and label products are subject to similar regulation and periodic inspections. If the FDA identifies deficiencies during an inspection, it may issue a formal notice, which may be followed by a warning letter if observations are not addressed satisfactorily. FDA guidelines specify that a warning letter be issued only for violations of "regulatory significance" for which the failure to adequately and promptly achieve correction may be expected to result in an enforcement action. For example, as discussed above, Lumara Health is subject to certain continuing obligations under the Consent Decree, including, but not limited to, inspection of Lumara Health's places of business by the FDA without prior notice, and notification of FDA of particular actions and events. If Lumara Health fails to comply with applicable provisions in the Consent Decree, the FDC Act, or the FDC Act's implementing regulations, the FDA may impose specific sanctions including, but not limited to, the requirement to cease any of Lumara Health's manufacturing operations, the imposition of substantial financial penalties, and the requirement to implement additional corrective actions.

Product approval may be delayed or denied due to cGMP non-compliance or other issues at the sponsor's manufacturing facilities or contractor sites or suppliers included in the NDA or sNDA, and the complete resolution of these inspectional findings may be beyond the sponsor's control. If the FDA determines that the sponsor's equipment, facilities, laboratories or processes do not comply with applicable FDA regulations and conditions of product approval, the FDA may seek civil, criminal or administrative sanctions and/or remedies against the sponsor, including suspension of its manufacturing operations.

To supply products for use outside of the U.S., our third-party manufacturers must comply with cGMP and are subject to periodic inspection by the FDA or by regulatory authorities in certain other countries. In complying with these requirements, manufacturers, including a drug sponsor's third-party contract manufacturers, must continue to expend time, money, and effort in the area of production and quality to ensure compliance. Failure to maintain compliance with cGMP regulations and other applicable manufacturing requirements of various regulatory agencies could result in fines, unanticipated compliance expenditures, recall, total or partial suspension of production, suspension, variation or withdrawal of the marketing authorization for the product, suspension of the FDA's review of future sNDAs, enforcement actions, injunctions, or criminal prosecution.

Orphan Drug Exclusivity

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, defined, in part, as a patient population of fewer than 200,000 in the U.S. In the U.S., the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug

Table of Contents

for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application for the "same drug" for the same orphan indication during the exclusivity period, except in very limited circumstances. In addition, orphan drug exclusivity marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Drug Quality and Security Act

In November 2013, the DQSA legislation was implemented to amend the FDC Act with respect to the regulation and monitoring of the manufacturing of compounding drugs. Among other provisions of the DQSA, compounding pharmacies may now elect to register as an "outsourcing facility" under FDC Act 503B. Registration as an outsourcing facility requires that drugs be compounded according to cGMP standards; that facilities report adverse events to the FDA; and that facilities be subject to a risk-based inspection schedule, among other requirements. Additionally, FDC Act 503A describes the conditions that must be satisfied for drug products compounded by a licensed pharmacist or licensed physician to be exempt from approval, labeling, and cGMP requirements. To qualify for these exemptions, a compounded drug product must, among other things, be compounded for an identified patient based on a valid prescription or in limited quantities before the receipt of a prescription for such individual patient in certain circumstances. Under both 503A and 503B of the FDC Act, compounding pharmacies may not compound regularly or in inordinate amounts any drug products that are "essentially copies of commercially available drug products." Depending on how aggressively the FDA enforces this provision of the statute, pharmacy compounders may be significantly restricted in their future ability to make drug products that are copies or near-copies of FDA approved drugs.

Fraud and Abuse Regulation

Our general operations, and the research, development, manufacture, sale, and marketing of our products, are subject to extensive federal and state regulation, including but not limited to FDA regulations, the Federal Anti-Kickback Statute ("AKS"), the Federal False Claims Act ("FCA"), and the Foreign Corrupt Practices Act, and their state analogues, and similar laws in countries outside of the U.S., laws governing sampling and distribution of products and government price reporting laws.

The AKS makes it illegal to knowingly and willfully solicit, offer, receive, or pay any remuneration, directly or indirectly, in cash or in kind, in exchange for, or to induce, purchasing, ordering, arranging for, or recommending the purchase or order of any item or service, including the purchase or prescription of a particular drug, that is reimbursed by a federal healthcare program. Liability may be established without proving actual knowledge of the statute or specific intent to violate it. In addition, federal law now provides that the government may assert that a claim including items or services resulting from a violation of the AKS constitutes a false or fraudulent claim for purposes of the FCA, described below. Violations of the AKS carry potentially significant civil and criminal penalties, including imprisonment, fines, and exclusion from participation in federal healthcare programs. Many states have enacted similar anti-kickback laws, including in some cases laws that prohibit paying or receiving remuneration to induce a referral or recommendation of an item or service reimbursed by any payer, including private payers.

The FCA prohibits, among other things, anyone from knowingly presenting, or causing to be presented, claims for reimbursement of drugs or services to third-party payers such as Medicare or Medicaid, or other claims for payment of government funds, where those claims are false or fraudulent. The FCA also prohibits knowingly making, using, or causing to be made or used a false record or statement material to an obligation to pay money to the government or knowingly concealing or knowingly and improperly avoiding, decreasing, or concealing an obligation to pay money to the federal government. The FCA permits a private individual acting

Table of Contents

as a "whistleblower" to bring an action on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery. Government enforcement agencies and private whistleblowers have asserted liability under the FCA for, among other things, claims for items or services not provided as claimed or for medically unnecessary items or services, kickbacks, promotion of off-label uses, and misreporting of drug prices to federal agencies. Many states have enacted similar false claims laws, including in some cases laws that apply where a claim is submitted to any third-party payer, not just government programs.

The Foreign Corrupt Practices Act prohibits companies and their intermediaries from making, or offering or promising to make improper payments to non-U.S. officials for the purpose of obtaining or retaining business or otherwise seeking favorable treatment. Similar anti-bribery laws exist in other countries where we intend to commercialize *Feraheme*. For example, the U.K. Bribery Act imposes significant potential fines and other penalties for, among other things, giving, offering, or promising bribes in the public and private sectors, and bribing a foreign public official or private person.

Our activities relating to the sale and marketing of our products may be subject to scrutiny under these laws. Federal and state authorities continue to devote significant attention and resources to enforcement of these laws within the pharmaceutical industry, and private individuals have been active in bringing lawsuits on behalf of the government under the FCA. We have developed and implemented a corporate compliance program based on what we believe are current best practices in the pharmaceutical industry. However, these laws are broad in scope and there may not be regulations, guidance, or court decisions that definitively interpret these laws in the context of particular industry practices. We cannot guarantee that we, our employees, our consultants, or our contractors are or will be in compliance will all federal, state, and foreign regulations. If we or our representatives fail to comply with any of these laws or regulations, a range of fines, penalties, and/or other sanctions could be imposed on us, including, but not limited to, restrictions on how we market and sell our products, significant fines, exclusions from government healthcare programs, including Medicare and Medicaid, litigation, or other sanctions. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could also have an adverse effect on our business, financial condition and results of operations. Such investigations or suits may also result in related shareholder lawsuits, which can also have an adverse effect on our business.

In the EU, the advertising and promotion of our products are subject to EU level and EU Member States' national laws governing promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. These laws require that promotional materials and advertising in relation to medicinal products comply with the product's SmPC. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. It forms an intrinsic and integral part of the marketing authorization granted for the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion. The off-label promotion of medicinal products is prohibited in the EU. The applicable laws at EU level and in the individual EU Member States also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on our promotional activities with healthcare professionals.

Other U.S. Regulatory Requirements

In recent years, several states have enacted legislation requiring pharmaceutical companies operating within the state to establish marketing and promotional compliance programs or codes of conduct and/or file periodic reports with the state or make periodic public disclosures on sales,

Table of Contents

marketing, pricing, clinical trials and other activities. In addition, as part of the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (the "Health Care Reform Act") manufacturers of drugs are required to publicly report gifts and other payments or transfers of value made to U.S. physicians and teaching hospitals. Several states have also adopted laws that prohibit certain marketing-related activities, including the provision of gifts, meals or other items to certain healthcare providers. Many of these requirements are new and uncertain, and the likely extent of future enforcement for failure to comply with these requirements is unclear. However, compliance with these laws is difficult, time-consuming, and costly, and if we are found not to be in full compliance with these laws, we may face enforcement actions, fines, and other penalties, and we could receive adverse publicity which could have an adverse effect on our business, financial condition, and results of operations.

We are also subject to laws and regulations covering data privacy and the protection of health-related and other personal information. We obtain patient health information from most healthcare providers who prescribe our products and research institutions we collaborate with, and they are subject to privacy and security requirements that may affect us. Claims that we have violated individuals' privacy rights or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

Foreign Regulatory Process

In our efforts to market and sell *Feraheme* outside of the U.S., we are subject to foreign regulatory requirements. Approval of a drug by applicable regulatory agencies of foreign countries must be secured prior to the marketing of such drug in those countries. The regulatory approval process in countries outside of the U.S. vary widely from country to country and may in some cases be more rigorous than requirements in the U.S. Certain foreign regulatory authorities may require additional studies or studies designed with different clinical endpoints and/or comparators than those which we are conducting or have already completed. In addition, any adverse regulatory action taken by the FDA with respect to an approved product, or a product under review, in the U.S. may affect the regulatory requirements or decisions made by certain foreign regulatory bodies with regard to the regulatory approval of products outside of the U.S.

To obtain regulatory approval of a drug in the EU, marketing authorizations may be submitted through a centralized, mutual recognition or decentralized procedure or national procedure (single country). The legislators, policymakers and healthcare insurance funds in the EU Member States continue to propose and implement cost-containing measures to keep healthcare costs down, due in part to the attention being paid to healthcare cost containment and other austerity measures in the EU. Certain of these changes could impose limitations on the prices pharmaceutical companies are able to charge for their products. The amounts of reimbursement available from governmental agencies or third-party payers for these products may increase the tax obligations on pharmaceutical companies such as ours, or may facilitate the introduction of generic competition with respect to our products. Furthermore, an increasing number of EU Member States and other foreign countries use prices for medicinal products established in other countries as "reference prices" to help determine the price of the product in their own territory. Consequently, a downward trend in prices of medicinal products in some countries could contribute to similar downward trends elsewhere. In addition, the ongoing budgetary difficulties faced by a number of EU Member States have led and may continue to lead to substantial delays in payment and payment partially with government bonds rather than cash for medicinal products. Moreover, in order to obtain reimbursement of our medicinal products in some countries, including some EU Member States, sponsors may be required to conduct Health Technology Assessments ("HTAs") that compare the cost-effectiveness of the sponsors' products to other available therapies.

Table of Contents

The Canadian pharmaceutical industry is subject to federal regulation by Health Canada, the public health department of the Canadian government charged with overseeing healthcare-related regulatory matters, pursuant to the Canadian federal Food and Drugs Act. Health Canada's criteria for obtaining and maintaining marketing approval is generally similar to that of the FDA. Health Canada is also empowered to compel information, recall unsafe therapeutic products, disclose confidential business information and direct label change/package modification to address safety issues. In December 2011, *Feraheme* was granted marketing approval by Health Canada for use as an IV iron replacement therapy for the treatment of IDA in adult patients with CKD and commercially launched in late 2012.

The pharmaceutical industry in Switzerland is subject to federal regulation by Swissmedic. In August 2012, *Rienso* was granted marketing approval by Swissmedic and commercially launched in late 2012. *Rienso* is not currently being marketed in Switzerland. We are currently unable to predict when or if *Rienso* will be reintroduced into the Swiss market.

Medical Device Regulation

Medical devices, such as *MuGard*, are similarly subject to FDA approval and extensive post-approval regulation under the FDC Act. Authorization to commercially distribute a new medical device in the U.S. is generally received in one of two ways. The first, known as premarket notification, or the 510(k) process, requires a sponsor to demonstrate that the new medical device is substantially equivalent to a legally marketed medical device that is not subject to premarket approval. The second, more rigorous process, known as premarket approval, requires a sponsor to independently demonstrate that the new medical device is safe and effective.

Both before and after a device is commercially released, there are ongoing responsibilities under FDA regulations. The FDA reviews design and manufacturing practices, labeling and record keeping, and manufacturers' required reports of adverse experiences and other information to identify potential problems with marketed medical devices, similar to the reviews conducted in connection with drug product discussed above. If the FDA were to conclude that we are not in compliance with applicable laws or regulations, or that any of our medical devices are ineffective or pose an unreasonable health risk, the FDA could require us to notify health professionals and others that the devices present unreasonable risks of substantial harm to the public health, order a recall, repair, replacement, or refund of such devices, detain or seize adulterated or misbranded medical devices, or ban such medical devices. The FDA may also impose operating restrictions, enjoin and/or restrain certain conduct resulting in violations of applicable law pertaining to medical devices and assess civil or criminal penalties against our officers, employees, or us.

MuGard was launched in the U.S. by PlasmaTech in 2010 after receiving 510(k) clearance from the FDA. Under the terms of the MuGard License Agreement, PlasmaTech continues to hold the 510(k). *MuGard* is categorized as a pre-amendments device. This type of device has not been classified per se, but continues to be subject to regulatory review under the 510(k) premarket clearance process.

Pharmaceutical Pricing and Reimbursement

In both the U.S. and foreign markets, our ability to successfully commercialize our products is dependent, in significant part, on the availability and extent of reimbursement to end-users from third-party payers for the use of our products, including governmental payers, health maintenance organizations ("HMOs"), managed care organizations, and private health insurers. In the U.S., the federal government provides health insurance for people who are 65 or older, certain younger people with disabilities, and people with End-Stage Renal Disease through the Medicare program, and certain prescription drugs, including *Feraheme* and *Makena*, are covered under Medicare Part B. Medicaid, another program in the U.S., is a health insurance program for low-income children, families, pregnant women, and people with disabilities that is jointly funded by the federal and state governments, but administered by the states. In general, state Medicaid programs are required to cover drugs and biologicals of manufacturers that have entered into a Medicaid Drug Rebate Agreement, although such products and biologicals may be subject to prior authorization or other utilization controls. Both Medicare and Medicaid are administered by the Centers for Medicare and Medicaid Services ("CMS").

Table of Contents

We participate in and have certain price reporting obligations to the Medicaid Drug Rebate program, and we have obligations to report Average Sales Price ("ASP") for the Medicare program. Under the Medicaid Drug Rebate program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data reported by us on a monthly and quarterly basis to CMS. These data include the average manufacturer price and, in the case of innovator products such as *Feraheme* and *Makena*, the best price for each drug.

Federal law also requires that a company that participates in the Medicaid program report ASP information to CMS for certain categories of drugs that are paid under Part B of the Medicare program, such as *Feraheme* and *Makena*. This ASP information forms the basis for reimbursement for the majority of our current *Feraheme* business, and to a lesser extent, for our *Makena* business. Manufacturers calculate ASP based on a statutorily defined formula and interpretations of the statute by CMS as to what should or should not be considered in computing ASP. An ASP for each National Drug Code for a product that is subject to the ASP reporting requirement must be submitted to CMS no later than 30 days after the end of each calendar quarter. CMS uses these submissions to determine payment rates for drugs under Medicare Part B. Changes affecting the calculation of ASP could affect the ASP calculations for our products and the resulting Medicare payment rate, and could negatively impact our results of operations.

Federal law requires that any company that participates in the Medicaid rebate program also participate in the Public Health Service's 340B drug pricing discount program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B pricing program requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The 340B ceiling price is calculated using a statutory formula, which is based on the average manufacturer price and rebate amount for the covered outpatient drug as calculated under the Medicaid rebate program. Changes to the definition of average manufacturer price and the Medicaid rebate amount under the Healthcare Reform Act, as discussed below, and CMS's issuance of final regulations implementing those changes also could affect our 340B ceiling price calculations and negatively impact our results of operations.

The Healthcare Reform Act also expanded the Public Health Service's 340B drug pricing program to include additional types of covered entities: certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, each as defined by the Healthcare Reform Act. For example, the percentage of *Feraheme* sold to 340B institutions has grown from 11% in 2011 to 17% in 2014. Since these institutions are granted lower prices than those offered to our other customers, any further growth in our 340B business may have a negative impact on our sales price per gram and operating margins.

The Healthcare Reform Act exempts "orphan drugs," such as *Makena*, from the ceiling price requirements for the covered entity types newly added to the program by the Healthcare Reform Act. On July 21, 2014, the Health Resources and Services Administration ("HRSA"), which administers the 340B program, issued an interpretive rule to implement the orphan drug exception which interprets the orphan drug exception narrowly. It exempts orphan drugs from the ceiling price requirements for the newly eligible entities only when the orphan drug is used for its orphan indication. The newly eligible entities are entitled to purchase orphan drugs at the ceiling price when the orphan drug is not used for its orphan indication. A manufacturer trade group has filed a lawsuit challenging the interpretive rule as inconsistent with the statutory language. That challenge remains ongoing. The uncertainty regarding how the statutory orphan drug exception will be applied will increase the complexity of compliance, will

Table of Contents

make compliance more time-consuming, and could negatively impact our results of operations. If HRSA's narrow interpretation of the scope of the orphan drug exemption prevails, it could potentially negatively impact the price we are paid for *Makena* by certain entities and increase the complexity of compliance with the 340B program.

In order to be eligible to have our products paid for with federal funds under the Medicaid program and purchased by certain federal agencies and grantees, we participate in the Department of Veterans Affairs ("VA") Federal Supply Schedule ("FSS") pricing program. To participate, we are required to enter into an FSS contract with the VA, under which we must make our innovator "covered drugs" available to the "Big Four" federal agencies, including the VA, the Department of Defense ("DoD"), the Public Health Service, and the Coast Guard, at pricing that is capped pursuant to a statutory federal ceiling price ("FCP") formula set forth in Section 603 of the Veterans Health Care Act of 1992 ("VHCA"). The FCP is based on a weighted average non-federal average manufacturer price ("Non-FAMP"), which manufacturers are required to report on a quarterly and annual basis to the VA. If a company misstates Non-FAMPs or FCPs it must restate these figures. Pursuant to the VHCA, knowing provision of false information in connection with a Non-FAMP filing can subject a manufacturer to penalties of $100,000 for each item of false information.

FSS contracts are federal procurement contracts that include standard government terms and conditions, separate pricing for each product, and extensive disclosure and certification requirements. All items on FSS contracts are subject to a standard FSS contract clause that requires FSS contract price reductions under certain circumstances where pricing is reduced to an agreed "tracking customer." Further, in addition to the "Big Four" agencies, all other federal agencies and some non-federal entities are authorized to access FSS contracts. FSS contractors are permitted to charge FSS purchasers other than the Big Four agencies "negotiated pricing" for covered drugs that is not capped by the FCP; instead, such pricing is negotiated based on a mandatory disclosure of the contractor's commercial "most favored customer" pricing. We offer single pricing on our FSS contract.

In addition, pursuant to regulations issued by the DoD TRICARE Management Activity, now the Defense Health Agency, to implement Section 703 of the National Defense Authorization Act for Fiscal Year 2008, each of our covered drugs is listed on a Section 703 Agreement under which we have agreed to pay rebates on covered drug prescriptions dispensed to TRICARE beneficiaries by TRICARE network retail pharmacies. Companies are required to list their innovator products on Section 703 Agreements in order for those products to be eligible for DoD formulary inclusion. The formula for determining the rebate is established in the regulations and our Section 703 Agreement and is based on the difference between the annual Non-FAMP and the FCP (as described above, these price points are required to be calculated by us under the VHCA).

Reimbursement by third-party payers depends on a number of factors, including the third-party's determination that the product is competitively priced, safe and effective, appropriate for the specific patient, and cost-effective. Third-party payers are increasingly challenging the prices charged for pharmaceutical products and have instituted and continue to institute cost containment measures to control or significantly influence the purchase of pharmaceutical products. For example, to reduce expenditures associated with pharmaceutical products, many third-party payers use cost containment methods, including: (a) formularies, which limit coverage for drugs not included on a predetermined list; (b) variable co-payments, which may make a certain drug more expensive for patients as compared with a competing drug; (c) utilization management controls, such as requirements for prior authorization before the payer will cover the drug; and (d) other coverage policies that limit access to certain drugs for certain uses based on the payer-specific coverage policy.

For example, prior to the implementation of the DQSA, as discussed above, the reimbursement of *Makena* was often difficult to obtain in light of the less expensive compounding products. As a result of the provisions under the DQSA and efforts by Lumara Health to work with individual states, including

Table of Contents

entering into supplement rebate agreements, access to *Makena* has since expanded. However, Lumara Health has had to use the legal system to defend reimbursement practices related to Lumara Health. For example, in 2012, Lumara Health sued the Georgia Department of Community Health ("DCH") because they were requiring patients to provide documentation of medical necessity to approve *Makena* in favor of compounded versions of the active ingredient of *Makena*. During 2014, a permanent order was issued stating that DCH and their managed Medicaid plans must reimburse for *Makena* when prescribed by physicians for an on-label patient. Although, this case remains in the appeals process, this ruling would aid as precedent for other states to comply with current Medicaid laws.

In addition, U.S. and many foreign governments continue to attempt to curb healthcare costs through legislation, including legislation aimed at reducing the pricing and reimbursement of pharmaceutical products. The Healthcare Reform Act was enacted in the U.S. in March 2010 and includes certain cost containment measures including an increase to the minimum rebates for products covered by Medicaid programs from 15.1% to 23.1% of the average manufacturer price for most innovator products, and the expansion of the 340B Drug Discount Program under the Public Health Service Act. Effective March 2010, the Healthcare Reform Act expanded manufacturer rebate liability under the Medicaid program from fee-for-service Medicaid utilization to include the utilization of Medicaid managed care organizations as well. In addition, the Healthcare Reform Act and subsequent legislation changed the definition of average manufacturer price. Further, federal budgetary concerns could result in the implementation of significant federal spending cuts, including cuts in Medicare and other health related spending in the near-term. For example, recent legislative enactments have resulted in Medicare payments being subject to a two percent reduction, referred to as sequestration, until 2024. Finally, the Healthcare Reform Act required pharmaceutical manufacturers of branded prescription drugs to pay a branded prescription drug fee to the federal government. Each individual pharmaceutical manufacturer pays a prorated share of the branded prescription drug fee of $3.0 billion in 2015, based on the dollar value of its branded prescription drug sales to certain federal programs identified in the law. Sales of orphan drugs, such as *Makena* are excluded from the determination.

Some of the Healthcare Reform Act's significant reforms do not take effect until 2015. In 2012, CMS, issued proposed regulations to implement the changes to the drug rebate components of the Medicaid program under the Healthcare Reform Act but has not yet issued final regulations. CMS is currently expected to release the final regulations in 2015.

In addition, the heightened focus on the healthcare industry by the federal government could result in the implementation of significant federal spending cuts including cuts in Medicare and other health related spending in the near-term. In recent years, some states have also passed legislation to control the prices of drugs as well as begun a move toward managed care to relieve some of their Medicaid cost burden. These and any future changes in government regulation or private third-party payers' reimbursement policies may reduce the extent of reimbursement for our products and adversely affect our future operating results. For example, since almost half of *Makena* patients are Medicaid beneficiaries, the impact of future legislative changes may have a significant impact on *Makena* sales. Payers also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, average manufacturer price and actual acquisition cost. The existing data for reimbursement based on these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates. CMS has begun posting drafts of this retail survey price information on at least a monthly basis in the form of draft National Average Drug Acquisition Cost ("NADAC") files, which reflect retail community pharmacy invoice costs, and National Average Retail Price ("NARP") files, which reflect retail community pharmacy prices to consumers. In July 2013, CMS suspended the publication of draft NARP data, pending funding decisions. In November 2013, CMS moved to publishing final rather than draft NADAC data and has since made updated NADAC data publicly available on a weekly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement mechanics on the willingness of payers

Table of Contents

to cover our products. Any failure to cover our products appropriately, in addition to legislative and regulatory changes and others that may occur in the future, could impact our ability to maximize revenues in the federal marketplace.

Currently, in U.S. physician clinic and hospital settings, Medicare Part B generally reimburses for physician-administered drugs at a rate of 106% of the drug's ASP. ASP is defined by statute based on sales and price concession data, including rebates and chargebacks, for a defined period of time. As noted above, we submit the required information to CMS on a quarterly basis. In advance of the quarter in which the payment limit for drugs reimbursed under Medicare Part B program will go into effect, CMS calculates and publishes the payment limit. Under this methodology, payment rates change on a quarterly basis, and significant downward fluctuations in ASP, and therefore reimbursement rates, could negatively impact sales of a product. Because the ASP-based payment rate is defined by statute, and changes to Medicare payment methodologies require legislative change, it is unclear if and when ASP reimbursement methodology will change for the physician office setting. While the statute requires Medicare Part B payments for most drugs furnished in the physician office setting to be at 106% of ASP, the statute does not have a similar requirement for hospital outpatient departments. For that setting, the Medicare payment for many covered Part B drugs also is at 106% of ASP, but CMS could change that through regulations, without any intervening legislation. While Medicare is the predominant payer for *Makena* and *Feraheme* for treatment of patients with CKD, Medicare payment policy, in time, can also influence pricing and reimbursement in the non-Medicare markets, as private third-party payers and state Medicaid plans frequently adopt Medicare principles in setting reimbursement methodologies. We cannot predict the impact that any changes in reimbursement policies may have on our ability to compete effectively.

For example, in the U.S. hospital inpatient setting, most drugs are not reimbursed separately within the Medicare prospective payment system, based largely on the drug costs, but are bundled as a per discharge reimbursement based on the diagnosis and/or procedure rather than actual costs incurred in patient treatments, thereby increasing the incentive for a hospital to limit or control expenditures. As a result, we do not expect premium priced products, such as *Feraheme*, to be broadly used in the hospital inpatient setting.

In countries outside of the U.S., market acceptance may also depend, in part, upon the availability of reimbursement within existing healthcare payment systems. Generally, in the EU and other countries outside of the U.S., the government sponsored healthcare system is the primary payer of healthcare costs of patients and therefore enjoys significant market power. Some foreign countries also set prices for pharmaceutical products as part of the regulatory process, and we cannot guarantee that the prices set by such governments will be sufficient to generate substantial revenues or allow sales of *Feraheme* to be profitable in those countries. Moreover, in order to obtain reimbursement of our medicinal products in some countries, including some EU Member States, we may be required to conduct HTAs that compare the cost-effectiveness of our products to other available therapies. In addition, we may be unable to obtain favorable pricing and reimbursement approvals in certain EU Member States.

The legislators, policymakers and healthcare insurance funds in the EU Member States continue to propose and implement cost-containing measures to keep healthcare costs down, due in part to the attention being paid to healthcare cost containment and other austerity measures in the EU. Certain of these changes could impose limitations on the prices pharmaceutical companies are able to charge for their products. The amounts of reimbursement available for these products from governmental agencies or third-party payers, may increase the tax obligations on pharmaceutical companies such as ours, or may facilitate the introduction of generic competition with respect to our products. Furthermore, an increasing number of EU Member States and other foreign countries use prices for medicinal products established in other countries as "reference prices" to help determine the price of the product in their own territory. Consequently, a downward trend in prices of medicinal products in some countries could contribute to similar downward trends elsewhere. In addition, the ongoing budgetary difficulties faced

Table of Contents

by a number of EU Member States have led and may continue to lead to substantial delays in payment and payment partially with government bonds rather than cash for medicinal drug products, which could negatively impact our revenues and profitability. Moreover, in order to obtain reimbursement of our medicinal products in some countries, including some EU Member States, we may be required to conduct HTAs that compare the cost-effectiveness of our products to other available therapies. There can be no assurance that our medicinal products will obtain favorable reimbursement status in any country.

If adequate reimbursement levels are not maintained by government and other third-party payers for our products, our ability to sell our products may be limited and/or our ability to establish acceptable pricing levels for our products may be impaired, thereby reducing anticipated revenues and our profitability.

Backlog

We had a $4.3 million and $0.9 million product sales backlog as of December 31, 2014 and 2013, respectively. We expect to recognize the $4.3 million in 2015. These backlogs were largely due to timing of orders received from our third-party logistics providers. Generally, product orders from our customers are fulfilled within a relatively short time of receipt of a customer order.

Employees

As of February 4, 2015, we had 257 employees, including 108 employees of Lumara Health who accepted employment with us following our November 2014 acquisition of Lumara Health. We also utilize consultants and independent contractors on a regular basis to assist in the development and commercialization of our products. Our success depends to a significant extent on our ability to continue to attract, retain and motivate qualified sales, technical operations, managerial, scientific and medical personnel of all levels. Although we believe we have been relatively successful to date in obtaining and retaining such personnel, we may not be successful in the future. During 2014 and 2013, we expanded our leadership team and strengthened our commercial organization and medical affairs teams. We expect to continue these efforts in 2015 in support of the growth in our business.

None of our employees is represented by a labor union, and we consider our relationship with our employees to be good.

Foreign Operations

We have no foreign operations. Revenues from customers outside of the U.S. amounted to approximately 12%, 11% and 32% of our total revenues for 2014, 2013 and 2012, respectively, and were principally related to collaboration revenues recognized in connection with our agreement with Takeda, which is headquartered in Japan. During 2012, our revenues from customers outside of the U.S included approximately $20.0 million related to the recognition of upfront payments and milestones achieved under the Amended Takeda Agreement, which we entered into the Takeda Termination Agreement to terminate. Sales of *Feraheme* outside of the U.S. do not and are not expected to materially contribute to our revenues. As such, and in light of the Takeda Termination Agreement, we and Takeda have come to the mutual decision to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada. We have no plans to commercialize *Makena* outside of the U.S.

Research and Development

We have dedicated a significant portion of our resources to our efforts to develop our products and product candidates, particularly *Feraheme*. We incurred research and development expenses of $24.2 million, $20.6 million, and $33.3 million during 2014, 2013 and 2012, respectively. We expect our

Table of Contents

research and development expenses to increase in 2015 due to the timing of expenses related to our pediatric clinical studies and our hd-CKD Study as well as current clinical trials related to *Makena's* post approval commitments and its lifecycle management program. In addition, research and development expenses could increase further and significantly depending on the outcome of discussions with the FDA on the regulatory path forward for *Feraheme* in the broad indication and any resulting clinical trials or development efforts that we may undertake.

Segment Reporting

We conduct our operations in one business segment as further described in Note P, "*Business Segments,*" to our consolidated financial statements included in this Annual Report on Form 10-K.

Code of Ethics

Our Board of Directors has adopted a code of ethics that applies to our officers, directors and employees. We have posted the text of our code of ethics on our website at *http://www.amagpharma.com* in the "Investors" section. We will provide to any person without charge a copy of such code of ethics, upon request in writing to Investor Relations, AMAG Pharmaceuticals, Inc., 1100 Winter Street, Waltham, MA 02451. In addition, should any changes be made to our code of ethics, we intend to disclose within four business days, on our website (or in any other medium required by law or the NASDAQ): (a) the date and nature of any amendment to our code of ethics that applies to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions and (b) the nature of any waiver, including an implicit waiver, from a provision of our code of ethics that is granted to one of these specified officers, the name of such person who is granted the waiver, and the date of the waiver.

Available Information

Our internet website address is *http://www.amagpharma.com*. Through our website, we make available, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy and registration statements, and all of our insider Section 16 reports (and any amendments to such filings), as soon as reasonably practicable after such material is electronically filed with, or furnished to, the U.S. Securities and Exchange Commission (the "SEC"). These SEC reports can be accessed through the "Investors" section of our website. The information found on our website is not part of this or any other report we file with, or furnish to, the SEC. Paper copies of our SEC reports are available free of charge upon request in writing to Investor Relations, AMAG Pharmaceuticals, Inc., 1100 Winter Street, Waltham, MA 02451. The content on any website referred to in this Form 10-K is not incorporated by reference into this Form 10-K unless expressly noted.

ITEM 1A. RISK FACTORS:

The following information sets forth material risks and uncertainties that may affect our business, including our future financial and operational results and could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Annual Report on Form 10-K and elsewhere as discussed in the introduction to Part I above. You should carefully consider the risks described below, in addition to the other information in this Annual Report on Form 10-K, before making an investment decision. The risks and uncertainties described below are not the only ones we face. Additional risks not presently known to us or other factors not perceived by us to present material risks to our business at this time also may impair our business operations.

Unless the context suggests otherwise, references to "Feraheme" refer to both Feraheme (the trade name for ferumoxytol in the U.S. and Canada) and Rienso (the trade name for ferumoxytol in the EU and Switzerland).

Table of Contents

Risks Related to Our Products

We are primarily dependent on revenues from our two principal products.

We currently derive substantially all of our revenue from sales of *Makena* and *Feraheme*. Although we may introduce additional products for commercialization to our product portfolio, we may be substantially dependent on sales of *Makena* and *Feraheme* for many years. Our financial condition will be materially adversely affected, we may have to restructure our current operations, and our business prospects will be limited if we experience any negative developments relating to *Makena* or *Feraheme*, including the following:

Actual or perceived safety or efficacy issues;

Restrictions on current or future labels;

The introduction or greater acceptance of competing products, including generic products, products that may be prescribed off-label and products made by compounding pharmacies;

Constraints on product pricing or price increases; and

Changes in reimbursement policies or adverse regulatory or legislative developments.

In the U.S., if *Makena* or *Feraheme* face any safety or efficacy issues, including drug interaction problems, under the Federal Food, Drug and Cosmetic Act (the "FDC Act"), the U.S. Food and Drug Administration ("FDA") has broad authority to force us to take any number of actions, including, but not limited to the following:

Requiring us to conduct post-approval clinical studies to assess known risks or signals of serious risks, or to identify unexpected serious risks;

Mandating labeling changes to a product;

Requiring us to implement a risk evaluation and mitigation strategy ("REMS") where necessary to assure safe use of the drug; or

Removing an already approved product from the market.

Similar laws and regulations exist in countries outside of the U.S. In addition, actual or perceived safety or drug interaction problems could result in product recalls, restrictions on the product's permissible uses, changes to the product label, a negative impact on regulatory applications (including supplemental new drug applications ("sNDAs") and applications for variations to the marketing authorization), suspension, variation or withdrawal of the marketing authorization for the product, or withdrawal of the product from the U.S. and/or foreign markets. Any such actions would adversely affect our results of operations.

The commercial success of our products depends upon the level of market adoption and continued use by physicians, hospitals, patients, and healthcare payers, including government payers, health maintenance organizations ("HMOs"), managed care organizations, group purchasing organizations ("GPOs") and specialty pharmacies. Our products might not be adopted if perceived to be no safer, less safe, no more effective, less effective, no more convenient, or less convenient than currently available products. In addition, the pricing and/or reimbursement rates and terms of our products may not be viewed as advantageous to potential prescribers and payers as the pricing and/or reimbursement rates and terms of other available products, including, in the case of *Makena*, compounded products. If our products do not achieve or maintain an

adequate level of market adoption for any reason, our profitability and our future business prospects will be adversely impacted.

Table of Contents

Competition in the pharmaceutical and biopharmaceutical industries, including from companies marketing generic products, is intense. If we fail to compete effectively, our business and market position will suffer.

The pharmaceutical and biopharmaceutical industries are intensely competitive and subject to rapid technological change. Many of our competitors for *Feraheme* are large, well-known pharmaceutical companies and may benefit from significantly greater financial, sales and marketing capabilities, greater technological or competitive advantages, and other resources. For *Makena*, most of our competition comes from pharmacies that compound a non-FDA approved version of *Makena*, which is sold at a much lower cost than *Makena*. Our existing or potential new competitors for *Feraheme* and *Makena* may develop products that are more widely accepted than ours and may receive patent protection that dominates, blocks or adversely affects our product development or business.

In addition, generic versions of *Feraheme* and *Makena* could enter the market through approval of abbreviated new drug applications ("ANDAs") that use *Feraheme* or *Makena* as a reference listed drug, which would allow generic competitors to rely on *Feraheme's* or *Makena's* safety and efficacy trials instead of conducting their own studies. Further, there are no patents covering *Makena.*

For example, in December 2012, the FDA published a draft guidance containing product-specific bioequivalence recommendations for drug products containing ferumoxytol. The FDA generally publishes product-specific bioequivalence guidance after it has received an inquiry from a generic drug manufacturer about submitting an ANDA for the product in question; thus, it is possible that a generic drug manufacturer has approached the FDA requesting guidance about submitting an ANDA for ferumoxytol, the active ingredient in *Feraheme*, and that such an ANDA may be filed in the near future. The published bioequivalence guidance could encourage a generic entrant seeking a path to approval of a generic ferumoxytol to file an ANDA. As a result, we could face generic competition in the near-term or have to engage in extensive litigation with a generic competitor to protect our patent rights, either of which could adversely affect our business and results of operations. Companies that manufacture generic products typically invest far fewer resources in research and development and marketing efforts than the manufacturers or marketers of branded products and can therefore price their products significantly lower than those branded products already on the market. As a result, competition from generic IV iron products could limit our sales, which would have an adverse impact on our business and results of operations.

The introduction by our competitors of alternatives to *Feraheme* or *Makena* that would be, or are perceived to be, more efficacious, safer, less expensive, easier to administer, available for a broader patient population, or provide more favorable insurance coverage or reimbursement, could reduce our revenues and the value of our product development efforts. For more information on *Feraheme* and *Makena* specific competition risks, please see Risk Factors "*Market acceptance of Feraheme may suffer as a result of the widespread use of competing iron replacement therapy products, including Injectafer®, and as a result of the approval of generic drug products in the near-term, which would have a material adverse effect on our operations and our profitability*" and "*Our ability to successfully commercialize Makena is dependent upon a number of factors, including maintaining the benefits of Makena's orphan drug exclusivity and the length of time before competitors begin selling generic versions of Makena*."

The success of our products depends on our ability to maintain the proprietary nature of our technology.

We rely on a combination of patents, trademarks and copyrights in the conduct of our business. The patent positions of pharmaceutical and biopharmaceutical firms are generally uncertain and involve complex legal and factual questions. We may not be successful or timely in obtaining any patents for which we submit applications. The breadth of the claims obtained in our patents may not provide sufficient protection for our technology. The degree of protection afforded by patents for proprietary or licensed technologies or for future discoveries may not be adequate to preserve our ability to protect or commercially exploit those technologies or discoveries. The patents issued to us may provide us with

Table of Contents

little or no competitive advantage. In addition, there is a risk that others will independently develop or duplicate similar technology or products or circumvent the patents issued to us.

One of our U.S. *Feraheme* patents is subject to a patent term extension under U.S. patent law and FDA regulations and will expire in June 2023. Our other U.S. patents relating to *Feraheme* expire in 2020. These and any other patents issued to or acquired by us may be contested or invalidated. There has been substantial litigation and other proceedings regarding patent and other intellectual property rights in the pharmaceutical and biotechnology industries. We may become a party to patent litigation and other proceedings, including interference and reexamination proceedings declared by the United States Patent and Trademark Office. There are no patents covering *Makena* and thus the successful commercialization of *Makena* is significantly reliant on our ability to take advantage of its orphan drug exclusivity, which risks are described in the Risk Factor "*Our ability to successfully commercialize Makena is dependent upon a number of factors, including maintaining the benefits of Makena's orphan drug exclusivity and the length of time before competitors begin selling generic versions of Makena*."

In addition, claims of infringement or violation of the proprietary rights of others may be asserted against us. If we are required to defend against such claims or to protect our own proprietary rights against others, it could result in substantial financial and business costs, including the distraction of our management. An adverse ruling in any litigation or administrative proceeding could result in monetary damages, injunctive relief or otherwise harm our competitive position, including by limiting our marketing and selling activities, increasing the risk for generic competition, limiting our development and commercialization activities or requiring us to obtain licenses to use the relevant technology (which licenses may not be available on commercially reasonable terms, if at all).

We also rely upon unpatented trade secrets and improvements, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our corporate licensees, collaborators, contract manufacturers, employees and consultants. These agreements, however, may be breached. We may not have adequate remedies for any such breaches, and our trade secrets might otherwise become known or might be independently discovered by our competitors. In addition, we cannot be certain that others will not independently develop substantially equivalent or superseding proprietary technology, or that an equivalent product will not be marketed in competition with our products, thereby substantially reducing the value of our proprietary rights.

Further, the laws of foreign countries may not protect our intellectual property rights to the same extent as do the laws of the U.S. and therefore our intellectual property rights may be subject to increased risk abroad, including opposition proceedings before the patent offices for other countries, such as the European Patent Office, or similar adversarial proceedings, regarding intellectual property rights with respect to *Rienso*.

We may not be able to further expand our product portfolio by entering into additional business development transactions, such as in-licensing arrangements, acquisitions, or collaborations or, if such arrangements are entered into, we may not realize the anticipated benefits and they could disrupt our business, decrease our profitability, result in dilution to our stockholders or cause us to incur significant additional debt or expense.

As part of our business strategy to expand our product portfolio, we are seeking to in-license or acquire additional pharmaceutical products or companies that leverage our corporate infrastructure and commercial expertise, such as our recent acquisition of Lumara Health Inc. ("Lumara Health"). We have limited experience with respect to these business development activities and there can be no assurance that we will be able to identify or complete any additional transactions in a timely manner, on a cost-effective basis, or at all.

Further, the valuation methods that we use for any acquired product or business requires significant judgment and assumptions. Actual results and performance of the products or businesses

Table of Contents

that we may acquire, including anticipated synergies and other financial benefits, could differ significantly from our original assumptions, especially during the periods immediately following the closing of the transaction. In addition, acquisitions may cause significant changes to our current organization and operations, may subject us to more rigid or constraining regulations or government oversight and may have negative tax and accounting consequences. These results could have a negative impact on our financial position or results of operations and result in significant charges in future periods.

In addition, proposing, negotiating and implementing collaborations, in-licensing arrangements or acquisition agreements is a lengthy and complex process. Other companies, including those with substantially greater financial, marketing and sales resources, may compete with us for these arrangements, and we may not be able to enter into such arrangements on acceptable terms or at all. Further, any such strategic transactions by us could result in large and immediate write-offs or the incurrence of additional debt and contingent liabilities, each of which may contain restrictive covenants that could adversely impact or limit our ability to grow our business, enter into new agreements, and adversely affect our operating results. Management of a license arrangement, collaboration, or other strategic arrangement and/or integration of an acquired asset or company may also disrupt our ongoing business, require management resources that otherwise would be available for ongoing development of our existing business, including our commercialization of *Feraheme* and *Makena*.

In addition, our cash, cash equivalents and investments may not be sufficient to finance any additional strategic transactions, and we may choose to issue shares of our common or preferred stock as consideration, which would result in dilution to our stockholders. Alternatively, it may be necessary for us to raise additional funds through public or private financings, and such additional funds may not be available on terms that are favorable to us, if at all, and our stockholders may experience significant dilution. Our Term Loan Facility, which provided us with $340.0 million to finance our acquisition of Lumara Health (the "Term Loan Facility") contains restrictions on our ability to acquire additional pharmaceutical products and companies, to enter into exclusive licensing arrangements, to incur additional indebtedness and will require us to use a portion of our free cash flow to repay indebtedness on an annual basis. These provisions may limit our ability to pursue attractive business development opportunities. If we are unable to successfully obtain rights to suitable products or if any acquisition or in-license arrangement we make is not successful, our business, financial condition and prospects for growth could suffer.

Further, even if we do acquire additional products or businesses, the integration of the operations of such acquired products or businesses requires significant efforts, including the coordination of information technologies, sales and marketing, operations, manufacturing, safety and pharmacovigilance, medical and finance. These efforts result in additional expenses and involve significant amounts of management's time. In addition, we may have to rely on the other parties with whom we may enter into a future agreement to perform certain regulatory filings, oversee certain functions, such as pharmacovigilance or the manufacture of the product we license from them, and any failure of such party to perform these functions for any reason, including ceasing doing business, could have a material effect on our ability to commercialize the licensed product. Similarly, we are relying on the *Makena* commercial team and other key Lumara Health personnel to assist with the integration and operations of Lumara Health and the commercialization of *Makena*. We may not realize the anticipated benefits of Lumara Health or any future acquisition, license or collaboration, any of which involves numerous risks including those discussed above and the following:

- Entry into markets in which we have no or limited direct prior experience, such as markets where we compete with non-traditional drug manufacturers, such as compounding pharmacies, and where competitors in such markets have stronger market positions;

Table of Contents

Our ability to train our sales force, and the ability of our sales force, to successfully incorporate new products into their call points, or to successfully integrate and leverage sales forces that we retain, such as the *Makena* commercial team;

Additional legal, compliance and/or accounting risks associated with such acquisitions, including liabilities assumed as part of the acquisition, which may be unknown or contingent; and

The introduction or wider acceptance of competitive products.

If we cannot successfully integrate the Lumara Health business, or other businesses or products we may acquire or in-license, into our company, we may experience material negative consequences to our business, financial condition or results of operations. We cannot be certain that, following any such acquisitions or in-licenses, including Lumara Health, we will achieve the expected synergies and other benefits that justify the purchase price of such transaction.

We are completely dependent on third parties to manufacture our commercial products and any difficulties, disruptions or delays, or the need to find alternative sources, could adversely affect our profitability and future business prospects.

We do not currently own or operate, and currently do not plan to own or operate, facilities for the manufacture of our products, and we do not plan to own or operate facilities for the manufacture of any commercial products we may acquire or in-license. We currently rely solely on third-party contract manufacturers to manufacture *Feraheme* and *Makena* for our commercial and clinical use. We do not currently have an alternative manufacturer for our *Feraheme* drug substance and finished drug product nor do we have an alternative manufacturer for *Makena* drug substance or drug product, and we may not be able to enter into agreements with second source manufacturers whose facilities and procedures comply with current good manufacturing practices ("cGMP") regulations and other regulatory requirements on a timely basis and with terms that are favorable to us, if at all.

Our ability to have our products manufactured in sufficient quantities and at acceptable costs to meet our commercial demand and clinical development needs is dependent on the uninterrupted and efficient operation of our third-party contract manufacturing facilities. Any difficulties, disruptions or delays in the manufacturing process could result in product defects or shipment delays, suspension of manufacturing or sale of the product, recall or withdrawal of product previously shipped for commercial or clinical purposes, inventory write-offs or the inability to meet commercial demand in a timely and cost-effective manner. Furthermore, our current third-party manufacturers do not manufacture for us exclusively and may exhaust some or all of their resources meeting the demand of other customers. In addition, securing additional third-party contract manufacturers for *Feraheme* or *Makena* will require significant time for transitioning the necessary manufacturing processes, gaining regulatory approval, and for having the appropriate oversight and may increase the risk of certain problems, including cost overruns, process reproducibility, stability issues, the inability to deliver required quantities of product that conform to specifications in a timely manner, or the inability to manufacture *Feraheme* or *Makena* in accordance with cGMP.

Further, we and our third-party manufacturers currently purchase certain raw and other materials used to manufacture *Feraheme* and *Makena* from third-party suppliers and, at present, do not have long-term supply contracts with most of these third parties. These third-party suppliers may cease to produce the raw or other materials used in *Feraheme* and *Makena* or otherwise fail to supply these materials to us or our third-party manufacturers or fail to supply sufficient quantities of these materials to us or our third-party manufacturers in a timely manner for a number of reasons, including but not limited to the following:

Unexpected demand for or shortage of raw or other materials;

Adverse financial developments at or affecting the supplier;

Table of Contents

Regulatory requirements or action;

An inability to provide timely scheduling and/or sufficient capacity;

Manufacturing difficulties;

Changes to the specifications of the raw materials such that they no longer meet our standards;

Lack of sufficient quantities or profit on the production of raw materials to interest suppliers;

Labor disputes or shortages; or

Import or export problems.

Any other interruption in our third-party supply chain could adversely affect our ability to satisfy commercial demand and our clinical development needs for *Feraheme*. In addition, there is only one FDA-approved supplier of the drug substance for *Makena* and, currently, we do not have a long-term supply agreement with that supplier. The supplier of drug substance may determine that it is not financially attractive for them to continue to supply drug substance for *Makena* at current prices, or at all, based on our expected purchasing volumes. The qualification of an alternative source may require repeated testing of the new materials and generate greater expenses to us if materials that we test do not perform in an acceptable manner. In addition, we or our third-party manufacturers sometimes obtain raw or other materials from one vendor only, even where multiple sources are available, to maintain quality control and enhance working relationships with suppliers, which could make us susceptible to price inflation by the sole supplier, thereby increasing our production costs. As a result of the high-quality standards imposed on our raw or other materials, we or our third-party manufacturers may not be able to obtain such materials of the quality required to manufacture *Feraheme* or *Makena* from an alternative source on commercially reasonable terms, or in a timely manner, if at all.

If we are unable to have *Feraheme* or *Makena* manufactured on a timely or sufficient basis because of the factors discussed above, we may not be able to meet commercial demand or our clinical development needs for *Feraheme* or *Makena*, or may not be able to manufacture *Makena* or *Feraheme* in a cost-effective manner. As a result, we may lose sales, fail to generate increased revenues or suffer regulatory setbacks, any of which could have an adverse impact on our profitability and future business prospects.

We rely on third parties in the conduct of our business, including our clinical trials and product distribution, and if they fail to fulfill their obligations, our commercialization and development plans may be adversely affected.

We rely on and intend to continue to rely on third parties, including clinical research organizations ("CROs"), third-party logistics providers, packaging, storage and labeling providers, wholesale distributors and certain other important vendors and consultants in the conduct of our business. In addition, we have contracted and plan to continue to contract with certain third parties to provide certain services, including site selection, enrollment, monitoring, data management and other services, in connection with the conduct of our clinical trials and the preparation and filing of our regulatory applications. We have limited experience conducting clinical trials outside the U.S., and, therefore, we are also largely relying on third parties such as CROs to manage, monitor and carry out these clinical trials. Although we depend heavily on these parties, we do not control them and, therefore, we cannot be assured that these third parties will adequately and timely perform all of their contractual obligations to us. If our third-party service providers cannot adequately fulfill their obligations to us in a timely and satisfactory manner, if the quality and accuracy of our clinical trial data or our regulatory submissions are compromised due to poor quality or failure to adhere to our protocols or regulatory requirements or if such third parties otherwise fail to adequately discharge their responsibilities or meet

Table of Contents

deadlines, our current and future development plans and regulatory submissions, or our commercialization efforts in current indications, may be delayed, terminated, limited or subject to additional expense, which would adversely impact our ability to generate revenues.

Further, in most cases, we do not currently have back-up suppliers or service providers to perform these tasks. If any of these third parties experience significant difficulties in their respective processes, fail to maintain compliance with applicable legal or regulatory requirements, fail to meet expected deadlines or otherwise do not carry out their contractual duties to us, or encounter physical or natural damages at their facilities, our ability to deliver our products to meet commercial demand could be significantly impaired. The loss of any of our third-party providers, together with a delay or inability to secure an alternate distribution source for end-users in a timely manner, could cause the distribution of our products to be delayed or interrupted, which would have an adverse effect on our business, financial condition and results of operations.

Additionally, we have limited experience independently commercializing multiple pharmaceutical products, including managing and maintaining a supply chain and distribution network for multiple products, and we are placing substantial reliance on third parties to perform this expanded network of product supply chain and distribution services for us. Any failure on our part to effectively execute on our multi-product commercial plans or to effectively manage our supply chain and distribution network would have an adverse impact on our business.

We depend, to a significant degree, on the availability and extent of reimbursement from third-party payers for the use of our products, and a reduction in the availability or extent of reimbursement could adversely affect our sales revenues and results of operations.

Our ability to successfully commercialize our products is dependent, in significant part, on the availability and extent of reimbursement to end-users from third-party payers for the use of our products, including governmental payers, HMOs, managed care organizations and private health insurers. Reimbursement by third-party payers depends on a number of factors, including the third-party's determination that the product is competitively priced, safe and effective, appropriate for the specific patient, and cost-effective. Third-party payers are increasingly challenging the prices charged for pharmaceutical products and have instituted and continue to institute cost containment measures to control or significantly influence the purchase of pharmaceutical products, such as through the use of prior authorizations and step therapy. If these entities do not provide coverage and reimbursement for our products or provide an insufficient level of coverage and reimbursement, physicians and other healthcare providers may choose to use alternative products, which would have an adverse effect on our ability to generate revenues.

In addition, U.S. and many foreign governments continue to propose and pass legislation designed to reduce the cost of healthcare for patients. The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (the "Healthcare Reform Act") includes certain cost containment measures including an increase to the minimum rebates for products covered by Medicaid programs, the extension of such rebates to drugs dispensed to Medicaid beneficiaries enrolled in Medicaid managed care organizations and the expansion of the 340B Drug Discount Program under the Public Health Service Act. In addition, federal budgetary concerns could result in the implementation of significant federal spending cuts, including cuts in Medicare and other health related spending in the near-term. Please see our discussion above under the heading, "*Pharmaceutical Pricing and Reimbursement*" in Item 1. Business for a more detailed discussion of such changes. The magnitude of the impact of these laws on our business is uncertain. Further, in recent years, some states have also passed legislation to control the prices of drugs as well as begun a move toward managed care to relieve some of their Medicaid cost burden. Given that almost half of *Makena* patients are Medicaid beneficiaries, the impact of future legislative changes may have a significant and adverse impact on *Makena* sales. Further, while Medicare is the predominant payer for *Feraheme*,

Table of Contents

Medicare payment policy, in time, can also influence pricing and reimbursement in the non-Medicare markets, as private third-party payers and state Medicaid plans frequently adopt Medicare principles in setting reimbursement methodologies. These and any future changes in government regulation or private third-party payers' reimbursement policies may reduce the extent of reimbursement for our products and adversely affect our future operating results.

Risks Related to *Makena*

Our ability to successfully commercialize Makena is dependent upon a number of factors, including maintaining the benefits of Makena's orphan drug exclusivity and the length of time before competitors begin selling generic versions of Makena.

Makena has been granted orphan drug exclusivity in the U.S. until February 3, 2018 for prevention of recurrent preterm birth in singleton pregnancies. 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, defined, in part, as a patient population of fewer than 200,000 in the U.S. In the U.S., the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application for the "same drug" for the same orphan indication during the exclusivity period, except in very limited circumstances. In addition, orphan drug exclusivity marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. Finally, FDA may approve a subsequent drug that is the same as a currently approved orphan drug for the same orphan indication during the exclusivity period if the sponsor of the subsequent drug can demonstrate that the drug is clinically superior to the already approved drug. According to the FDA, clinical superiority may be demonstrated by showing that a drug is significantly more effective in a clinical trial, safer in a substantial portion of the target population, or provides a major contribution to patient care relative to the currently approved drug.

Additionally, in 1956, the FDA-approved the drug Delalutin, which contained the same active ingredient as *Makena*. Delalutin was approved for conditions other than reducing the risk of preterm birth and was marketed by Bristol-Myers Squibb ("BMS"). BMS stopped marketing and manufacturing the FDA-approved product and it was withdrawn from the market in 1999. In 2010, in response to a citizen petition, the FDA determined that Delalutin was not withdrawn from sale for reasons of safety or effectiveness. As such, generic drug applications may reference the withdrawn Delalutin New Drug Application ("NDA"). Thus, before the expiration of *Makena's* orphan exclusivity, the FDA could determine that it has the authority to approve ANDAs that reference Delalutin so long as the ANDAs meet all relevant legal and regulatory requirements for approval and are labeled for the same indications as Delalutin. If such an approval is granted, doctors may elect to prescribe such approved drug off-label (*i.e.*, outside of FDA-approved indications) for *Makena's* orphan-protected indication, which could have an adverse impact on our business and results of operations.

Moreover, if one or more ANDA filers or a generic manufacturer were to receive approval to sell a generic or follow-on version of *Makena* for the orphan indication, those generic products could potentially be approved as early as February 3, 2018 (the date on which *Makena's* orphan exclusivity ends) and we would become subject to increased competition at that time.

Table of Contents

Further, our ability to successfully commercialize *Makena* depends on a number of additional factors, including but not limited to the following:

- The possibility that the benefit of the remaining exclusivity period resulting from the designation of *Makena* as an orphan drug may not be realized as a result of off-label use by physicians of current or future FDA-approved drugs in the market where *Makena* competes;
- The level of enforcement by the FDA to ensure compounded copies of commercially available FDA-approved products manufactured by compounding pharmacies, including compounded copies of hydroxyprogesterone caproate ("HPC") that are in violation of the federal Drug Quality and Security Act ("DQSA"), as well as other relevant provisions of the FDC Act, are not distributed to patients;
- The size of the pool of patients who may be eligible to receive *Makena;*
- Actual or perceived safety and efficacy of *Makena*;
- Our ability to increase patient compliance in line with the current label;
- The successful integration and retention of the *Makena* commercial sales team and any other key employees into our business structure; and
- Our ability to successfully leverage Lumara Health's commercial organizations and distribution networks in marketing, selling and supplying *Makena*.

Failure to achieve any or all of these commercial objectives could have an adverse material effect on the growth of *Makena* and our ability to achieve our revenue forecasts which could impact our financial condition or results of operations.

We have no experience facing competition from compounded products and if we are unsuccessful in differentiating Makena from compounded HPC products, sales of Makena, and thus our profitability, could be materially adversely affected.

We are aware that formulations of HPC have been available from compounding pharmacies for many years (which compounded formulations of HPC we refer to as "c17P") and will likely remain available even though *Makena* has been granted orphan drug exclusivity until February 3, 2018, and we have no prior experience with facing such competition. In March 2011, the FDA communicated to Lumara Health and also separately issued a press release that, in order to ensure continued access for patients, the FDA intended to refrain from taking enforcement action with respect to compounding pharmacies producing c17P in response to individual prescriptions for individual patients. The FDA's statement had an adverse effect on Lumara Health's ability to realize the benefit of orphan drug exclusivity and its ability to grow sales of *Makena* following the launch of the product in March 2011. The failure by the FDA to take enforcement action against compounding pharmacies resulted in substantial sales of compounded copies of *Makena* and the effective loss of the value of marketing exclusivity for the affected period of time. In June 2012, the FDA recommended using an FDA-approved drug product, such as *Makena*, instead of a compounded drug except when there is a specific medical need (e.g., an allergy) that cannot be met by the approved drug. In July 2014, the FDA issued another public statement affirming the position it took in its June 2012 press release recommending use of FDA-approved *Makena* except when there is a specific need for a compounded drug. The FDA also stated that when it identifies a pharmacist that compounds regularly or in inordinate amounts of any drug products that are essentially copies of *Makena*, the FDA intends to take enforcement action as it deems appropriate. Despite recent negative publicity regarding compounding pharmacies, including the 2012 meningitis outbreak involving compounded drugs, the November 2013 enactment of the DQSA and recent enforcement actions against compounders violating the FDC Act, *Makena* may continue to face competition from c17P, especially in light of the

Table of Contents

long-standing availability of such compounded products, their lower cost and the criticism Lumara Health received in the past in connection with the pricing of *Makena*, as discussed below. Further, if any safety or efficacy concerns arise with respect to the c17P products, it may negatively impact sales of *Makena* if healthcare providers and patients do not distinguish between the compounded product and the FDA-approved *Makena*.

We may not be successful in implementing Makena's lifecycle management program, which could have a negative impact on our business.

In October 2014, we filed with the FDA a prior approval supplement to the original *Makena* NDA seeking approval of a 1 mL preservative-free vial of *Makena* (the "Single Dose Vial") and we are seeking to expand *Makena's* formulations and drug delivery technologies as part of the product's lifecycle management program. The lifecycle management program for *Makena* is an important strategy for our maternal health business, especially in light of the expiration of *Makena's* orphan drug exclusivity in February 2018. We have limited experience in the development of alternative formulations for *Makena* and in developing and implementing lifecycle management programs. We can make no assurance that our prior approval supplement for the Single Dose Vial will be approved on the expected timeline, or at all, or that our other lifecycle management activities will be successful in supporting our maternal health business. Further, the Single Dose Vial will not, and future activities may not, extend or grant exclusivity or provide patent protection, which will likely increase competition. If we are not successful in implementing *Makena's* lifecycle management program, or if such activities cannot be completed on anticipated timelines, our business will suffer.

The commercial success and growth prospects for Makena will be dependent upon perceptions related to pricing and access.

Lumara Health was criticized for the initial list pricing of *Makena* in numerous news articles and internet postings following the FDA's February 2011 approval of *Makena* for the prevention of recurrent preterm birth in certain at-risk women. Although the list price of *Makena* was subsequently reduced in March 2011, *Makena* is still priced at a premium to c17P, which has negatively impacted coverage of *Makena* by some state Medicaid programs and by certain commercial payers. Although we are undertaking efforts to educate physicians and patients about progress made toward expanding coverage of *Makena* and about the benefits of FDA-approved *Makena*, certain doctors continue to prescribe non-FDA approved purported substitute products made by pharmaceutical compounders in lieu of prescribing *Makena*. In addition, efforts to appropriately respond to future concerns about pricing and access raised by media, professional societies, advocacy groups, policymakers or regulatory agencies regarding patient access to *Makena*, are costly and may not be successful. If we are unable to increase usage of *Makena* by physicians and strengthen relationships with professional societies, advocacy groups, policymakers and regulatory agencies, some of whom have been previously critical of Lumara Health, our sales of *Makena* may suffer, which would have a materially adverse impact on revenues and our results of operations.

The FDA has required post-marketing studies to verify and describe the clinical benefit of Makena, and the FDA may limit further marketing of the product based on the results of these post-marketing studies, failure to complete these trials in a timely manner or evidence of safety risks or lack of effectiveness.

Makena was approved by the FDA in February 2011 under the provisions of the FDA's "Subpart H" Accelerated Approval regulations. The Subpart H regulations allow certain drugs, for serious or life-threatening conditions, to be approved on the basis of surrogate endpoints or clinical endpoints other than survival or irreversible morbidity. As a condition of approval under Subpart H, the FDA required that *Makena*'s sponsor perform certain adequate and well-controlled post-marketing clinical studies to verify and describe the clinical benefit of *Makena* as well as fulfill certain other

Table of Contents

post-marketing commitments. Given the patient population (*i.e.*, women pregnant and at an increased high risk for recurrent preterm delivery) and the informed risk of receiving a placebo instead of the active approved drug in the U.S., the pool of prospective subjects for such clinical trials in the U.S. is small, and we have therefore sought enrollment on a global scale. These factors make the enrollment process slow, difficult, time-consuming and costly. If the required post-marketing studies fail to verify the clinical benefit of the drug, if a sufficient number of participants cannot be enrolled, or if the applicant fails to perform the required post-marketing studies with due diligence, the FDA has the authority to withdraw approval of the drug following a hearing conducted under the FDA's regulations, which would have a materially adverse impact on our business. We cannot be certain of the results of the confirmatory clinical studies or what action the FDA may take if the results of those studies are not as expected based on clinical data that FDA has already reviewed or if such studies are not completed in a timely manner.

Risks Related to *Feraheme*

The market for Feraheme is limited because Feraheme is only indicated for the treatment of IDA in adult patients with CKD. Significant safety or drug interaction problems, or the evaluation or reevaluation of existing or future data by the FDA or other regulators, could have an adverse impact on Feraheme in this indication, which would adversely impact our future business prospects.

The market for *Feraheme* is limited because *Feraheme* is only indicated for the treatment of iron deficiency anemia ("IDA") in adult patients with chronic kidney disease ("CKD"). Although we intend to continue to dedicate significant resources to the commercialization of *Feraheme*, it may never receive approval for a broader indication and we may not be successful in our efforts to continue to successfully commercialize *Feraheme* in its current market, which would have a materially adverse effect on our results of operations and future business prospects.

Sales in the current indication may be limited or may decrease if label changes require us to provide additional warnings and/or restrictions related to *Feraheme*'s current or future indications or impose limitations or changes to the method of administering the drug, thereby giving rise to increased competitive pressures if *Feraheme* is viewed as less safe than other IV iron products. Significant safety or drug interaction problems with respect to *Feraheme*, including an increase in the severity or frequency of known adverse events or the discovery of previously unknown adverse events, or the evaluation or reevaluation of data, including pharmacovigilance data, by the FDA, the European Medicines Agency ("EMA"), the competent authorities of the European Union ("EU") Member States or other regulators, could result in lawsuits and increased regulatory scrutiny or a variety of adverse regulatory actions, including changes to the product label, the implementation of a REMS or any other enforcement actions. For example, the Committee for Medicinal Products for Human Use ("CHMP") recently issued opinions that, among other measures, *Rienso* be administered to patients by infusion over at least 15 minutes (replacing injection), that it be contraindicated in patients with any known history of drug allergy, that the label caution that elderly patients or patients with multiple co-morbidities who experience a serious hypersensitivity reaction due to *Rienso* may have more severe outcomes, and that related variations to the Summary of Product Characteristics ("SmPC") be implemented. Similarly, in June 2014, we proposed changes to FDA related to our current U.S. label of *Feraheme* based on a review of global post-marketing data to strengthen the warnings and precautions section of the label and mitigate the risk of serious hypersensitivity reactions, including anaphylaxis, in order to enhance patient safety. After considering our June 2014 submission and other information, in January 2015, the FDA notified us that it believes new safety information should be included in the labeling for *Feraheme*, including, among other things, a boxed warning to highlight the risks of serious hypersensitivity/anaphylaxis reactions and revisions that *Feraheme* should only be administered through an IV infusion (*i.e.*, not by IV injection) and should be contraindicated for patients with any known history of drug allergy. We plan to work with the FDA to finalize an updated U.S. *Feraheme* label.

Table of Contents

These or any future changes to the label/package could adversely impact our ability to successfully compete in the IV iron market and could have an adverse impact on potential sales of *Feraheme* and our future business prospects.

In addition, regulators may require us to conduct additional post-approval clinical trials or undertake other activities in order to maintain *Feraheme's* current indication. For example, CHMP recommended that amendments be made to the Risk Management Plan, including a Post Authorization Safety Study to be conducted to further characterize the risk of hypersensitivity with *Rienso* in patients with CKD and a non-clinical mechanistic study of hypersensitivity reactions. Pursuit of these or other studies are time-consuming and costly and the resulting data might not be as desired or expected, which could further limit the market for *Feraheme*.

Non-compliance with any recommendations or requirements from regulators could result in product recalls, restrictions on the product's permissible uses, changes to the product label, a negative impact on regulatory applications, suspension, variation or withdrawal of the marketing authorization for the product, or withdrawal of the product from the U.S. and/or foreign markets. Our business could be adversely affected if any such results occur.

Moreover, new safety or drug interaction issues may arise as *Feraheme* is used over longer periods of time by a wider group of patients, some of whom may be taking other medicines or have additional underlying health problems, which may require us to, among other things, provide additional warnings and/or restrictions on the label/package insert, including a boxed warning in the U.S. or similar warnings outside of the U.S., notify healthcare providers of new safety information, narrow our approved indications, change the rate or method of administration, alter or terminate current or future trials for additional uses of *Feraheme*, or even remove *Feraheme* from the market, any of which could have a significant adverse impact on potential sales of *Feraheme* or require us to expend significant additional funds. In the EU, *Rienso* is subject to additional monitoring by the EMA and the competent authorities of the EU Member States. In addition, if and as we conduct and complete other clinical trials for *Feraheme*, new safety issues may be identified, which could negatively impact our ability to successfully complete these studies and which could also negatively impact the use and/or regulatory status of *Feraheme* for the treatment of IDA in patients with CKD.

For additional details regarding these and other regulatory developments for *Feraheme's* current indication, please see the discussion under the heading "*Feraheme for the treatment of IDA in patients with CKD Overview*" in Item 1. Business.

We may never receive regulatory approval to market and sell Feraheme to the broader IDA patient population.

As discussed above in Item 1. Business under the heading "*Feraheme for the treatment of IDA in a broad range of patients Overview*", in January 2014, we received a complete response letter from the FDA informing us that our sNDA for the broad IDA indication could not be approved in its present form. In the letter, the FDA stated that we have not provided sufficient information to permit labeling of *Feraheme* for safe and effective use for the proposed indication. This decision by the FDA represents a significant set-back in our efforts to obtain U.S. approval for *Feraheme* for a broader indication as the issues raised and information requested by the FDA may be costly and time-consuming to address and generate. Further, there is no guarantee that any efforts that we decide to undertake will meet the FDA's requirements, and we may not receive approval at all for *Feraheme* in a broader indication despite such efforts.

Although we are continuing to work with the FDA, we may decide not to pursue regulatory approval for the broader indication. If we continue to pursue approval in the U.S. for the commercial marketing and sale of *Feraheme* for the broad IDA indication, we will have to demonstrate, through the submission of clinical study reports and data sets from one or more controlled clinical trials, that the benefit of *Feraheme* use in the proposed population would warrant the risks associated with *Feraheme*,

Table of Contents

including the potential for adverse events, including anaphylaxis, cardiovascular events, and death. The FDA has substantial discretion in the approval process and may decide that the results of any such additional trials and the information we submit seeking approval in the broader patient population or other information reviewed, such as post-marketing safety data, including reports of serious anaphylaxis, cardiovascular events, and death, or any information we provide in response to FDA requests, are insufficient for approval or that *Feraheme* is not effective or safe for the proposed broader indication. We have submitted proposed protocols for a clinical study to the FDA for potential pursuit of the broader indication and are awaiting the FDA's feedback. There is no guarantee that the FDA will support any protocols we propose or determine that the results of any clinical trials we undertake of *Feraheme* for the treatment of IDA in adult patients who have failed or could not tolerate oral iron will adequately support approval of *Feraheme* in this broader patient population, or any of the individual subpopulations of IDA patients.

If we do not obtain U.S. approval to market and sell *Feraheme* for the treatment of IDA in a broad range of patients, or if we experience additional significant delays or setbacks in obtaining approval, or if we receive approval with significant restrictions, or are required to incur significant costs as post-marketing commitments, our cash position, our ability to increase revenues, our ability to leverage our product portfolio, our profitability, and the future prospects of our business could be materially adversely affected.

Efforts to pursue a broader indication could also have a negative impact on the commercialization of *Feraheme* in its current indication if information submitted for purposes of the broader indication and any reevaluation of existing data, such as reports of serious anaphylaxis, cardiovascular events, and death, results in requirements to provide additional warnings and/or restrictions on our *Feraheme* label/package insert, change the rate or method of administration of *Feraheme*, notify healthcare providers of changes to the label/package insert, narrow the current indication, alter or terminate current or future trials for *Feraheme* or incur significant costs related to post-marketing requirements/commitments. Such adverse developments could put us at a disadvantage to our competitors and cause healthcare providers to choose to treat all of their IDA patients with competing IV irons based on the actual or perceived safety and efficacy of *Feraheme* in light of such activities.

Market acceptance of Feraheme may suffer as a result of the widespread use of competing iron replacement therapy products, including Injectafer®, and as a result of the approval of generic drug products in the near-term, which would have a material adverse effect on our operations and our profitability.

Market acceptance of *Feraheme* may suffer as a result of competing iron replacement therapy products, in part because most of these products have been on the market longer and are currently widely used by physicians in the U.S. and abroad, and because certain of these products are approved for the treatment of IDA in a broader group of patients. For example, in July 2013, Injectafer® was approved by the FDA for the treatment of IDA in adult patients who have an unsatisfactory response to oral iron or who have intolerance to oral iron, which is a broader indication than our current *Feraheme* indication. Injectafer® is approved in the U.S. with a recommended dose of two slow injections or infusions of 750 milligrams each separated by at least seven days apart for a total of 1,500 milligrams. Given potential label changes in the U.S., which could provide, among other changes, that *Feraheme* be administered to patients by infusion over at least 15 minutes (replacing injection), *Feraheme* could lose a competitive advantage to Injectafer® and other IV irons. Further, we may not be able to offer discounts, incentives or rebates to new or existing customers on terms as appealing as Injectafer® or other IV irons. Even if we continue to seek and eventually obtain labeling of *Feraheme* in a broader population, Injectafer® will have already been available for a considerable period of time. During this period, physicians may continue to increase their use of Injectafer®, new physicians may begin to use Injectafer®, and physicians will gain increased familiarity with the product, making it more difficult for us to cause these physicians to use *Feraheme* in the future. In addition, manufacturers of

Table of Contents

Injectafer® may enter into commercial contracts with key customers or GPOs during this period, which could prevent or make it more difficult for *Feraheme* to retain its existing customers, gain sales to new customers and gain market share in its existing indication with customers or GPOs, and may make entry into the non-CKD market difficult if we were to continue to seek and receive approval for the broader patient population in the future. We face similar challenges outside of the U.S., where our recent SmPC and label changes in the EU and Canada could cause *Feraheme* to lose market share to competitors such as Ferinject™ (the trade name of Injectafer® in the EU), causing *Feraheme* to be commercially unviable for us outside of the U.S. If we are not able to differentiate *Feraheme* from other marketed IV iron products, including Injectafer®, or convince physicians and other customers of *Feraheme's* safe and effective use, our ability to maintain a premium price, our ability to generate revenues and maintain profitability, our ability to pursue and support any commercialization efforts outside the U.S., and our long-term business prospects could be adversely affected.

Feraheme's ability to maintain its current market share, or gain wider market acceptance in the future, depends on a number of other factors, including but not limited to the following:

- Our ability to demonstrate to healthcare providers, particularly hematologists, oncologists, hospitals, nephrologists, and others who may purchase or prescribe *Feraheme*, the clinical efficacy and safety of *Feraheme* as an alternative to currently marketed IV iron products which treat IDA in CKD patients;

- Our ability to convince physicians and other healthcare providers to use IV iron, and *Feraheme* in particular, rather than oral iron, which is the current treatment of choice of most physicians for treating IDA in CKD patients;

- The actual or perceived safety and efficacy profile of *Feraheme* as compared to alternative iron replacement therapeutic agents;

- The relative price and level of reimbursement for *Feraheme* from payers, including government payers, such as Medicare and Medicaid, and private payers as compared to the price and level of reimbursement for alternative IV iron products;

- The actual or perceived convenience and ease of administration of *Feraheme* as compared to alternative iron replacement therapeutic agents, including iron administered orally, in light of recent or potential changes to the methods of administration;

- Our ability to execute on our contracting strategy and offer competitive discounts, rebates and other incentives, which can result in increasing the rebates we are required to pay under the Medicaid Drug Rebate program and the discounts we are required to offer under the 340B drug pricing program;

- Current and future limitations on the approved indications and patient populations for *Feraheme*;

- The introduction of generic versions of ferumoxytol, which may occur in the near-term given the FDA's December 2012 draft guidance containing product-specific bioequivalence recommendations for drug products containing ferumoxytol; and

- The effectiveness of our commercial organization and distribution networks in marketing, selling and supplying *Feraheme*.

Table of Contents

The key component of our U.S. commercialization strategy for *Feraheme* is to market and sell *Feraheme* for use in non-dialysis adult CKD patients. The current U.S. non-dialysis CKD market is comprised primarily of three sites of care where a substantial number of CKD patients are treated: hospitals, hematology and oncology centers, and nephrology clinics. Competition in these practices is intense and competitors such as Injectafer® are gaining market share, particularly in hematology practices. IV iron therapeutic products are not currently widely used by certain physicians who treat non-dialysis CKD patients in the U.S., particularly nephrologists, due to safety concerns and the inconvenience and often impracticability of administering IV iron therapeutic products in their offices. It is often difficult to change physicians' existing treatment paradigms even when supportive clinical data are available. In addition, our ability to effectively market and sell *Feraheme* in the U.S. hospital market depends in part upon our ability to achieve acceptance of *Feraheme* onto hospital formularies. Since many hospitals and hematology, oncology and nephrology practices are members of GPOs, which leverage the purchasing power of a group of entities to obtain discounts based on the collective bargaining power of the group, our ability to attract customers in these sites of care also depends in part on our ability to effectively promote *Feraheme* to and enter into pricing agreements with GPOs. The GPOs can also offer opportunities for competitors to *Feraheme* that provide the ability to quickly gain market share by offering a more attractive price or contracts and incentives that encourage the members of the GPO to use or switch to the competing product. If we are not successful in capturing a significant share of the U.S. non-dialysis CKD market or if we are not successful in securing and maintaining formulary coverage for *Feraheme*, or if we cannot maintain strong relationships and offer competitive contracts to key customers and GPOs, our profitability as well as our long-term business prospects could be adversely affected.

We derive a substantial amount of our Feraheme revenue from a limited number of customers and the loss of one or more of these customers, a change in their fee structure, or a decline in revenue from one or more of these customers could have an adverse impact on our results of operations and financial condition.

In the U.S., we sell *Feraheme* primarily to wholesalers and specialty distributors and therefore a significant portion of our revenues is generated by a small number of customers. The loss of any of our customers, including if a customer views *Feraheme* as having a higher risk profile as compared to other IV iron products, especially in light of recent regulatory developments, could have a materially adverse impact on our results of operations. Four customers accounted for 81% of our total *Feraheme* revenues during the year ended December 31, 2014, and two customers accounted for 57% of our *Feraheme* accounts receivable balance as of December 31, 2014. We pay these wholesalers and specialty distributors a fee for the services that they provide to us. Because our business is concentrated with such a small number of wholesalers and specialty distributors, we could be forced to accept increases in their fees in order to maintain the current distribution networks through which *Feraheme* is sold. Any increase in fees could have a negative impact on our current and future sales of *Feraheme* in the U.S. and could have a negative impact on the reimbursement rate an individual physician, hospital or clinic would realize upon using *Feraheme*.

In addition, a significant portion of our U.S. *Feraheme* sales are generated through a small number of contracts with GPOs. For example, approximately 26% of our *Feraheme* end-user demand during the year ended December 31, 2014 was generated by members of a single GPO with which we have contracted. As a result of the significant percentage of our end-user demand being generated by a single GPO, we may be at a disadvantage in future contract or price negotiations with such GPO and that GPO may be able to influence the demand for *Feraheme* from its members in a particular quarter through communications they make to their customers. In addition, competitors of *Feraheme* may be able to quickly gain market share if they are able to offer GPOs a more attractive price or contracts and incentives that encourage the members of the GPO to use or switch to the competing product, especially if such competing drug can be administered to a broader patient population. The loss of some or all of this demand to a competitor, a material reduction in sales volume, or a significant

Table of Contents

adverse change in our relationship with any of our key wholesalers, distributors or GPOs could have a material adverse effect on our revenue and results of operations.

We have no experience commercializing Feraheme outside of the U.S. and we may be unable to undertake such efforts or find a collaboration partner to undertake such efforts, and we may be unsuccessful even if such efforts are undertaken.

Historically, Takeda Pharmaceutical Company Limited ("Takeda") had commercialized *Feraheme* in the EU, Canada and Switzerland (*Feraheme* is marketed as *Rienso* outside of the U.S. and Canada), but we have agreed to terminate our license arrangement with Takeda and are in the process of transitioning the product rights back to us, including the marketing authorizations for the EU and Canada. Sales of *Feraheme* outside of the U.S. do not and are not expected to materially contribute to our revenues even after we regain worldwide rights. For example, net sales of *Rienso* by Takeda in the EU were less than $0.5 million in 2014. A number of considerations influence our analysis of our commercialization opportunities outside of the U.S., including (i) regulatory developments and the potential cost of post-approval clinical trial commitments and post-marketing obligations required by regulatory authorities outside of the U.S., (ii) the product's commercial viability (sales potential relative to the cost of maintaining the product on the market) in light of the current CKD label, the possible impact of future label changes, including any impact in the U.S., and the competitive landscape, and (iii) possible approaches in different geographies, which may include seeking a licensing or distribution partner or commercializing the product ourselves. Based on these considerations, we have come to a mutual decision with Takeda to initiate withdrawal of the marketing authorization for *Rienso* in the EU and Switzerland. We are currently assessing the commercial opportunity for *Feraheme* in Canada. In order to continue commercialization efforts, we would have to assume the full cost of any post-marketing obligations required by the ex-U.S. regulatory authorities, both currently and in the future, some of which might have been Takeda's responsibility under a cost-sharing arrangement. Our U.S. sales could be negatively affected if patients or health-care providers in the U.S. perceive withdrawal from other markets as being the result of safety or efficacy, rather than commercial, reasons.

In the future, we may decide to seek to obtain a new marketing authorization for *ferumoxytol* in the EU, particularly if we generate additional clinical data to support potential approval in the broader IDA indication and we may decide to continue to pursue commercial efforts in Canada. If we do pursue commercialization efforts outside of the U.S., we may not have the resources, or be able to find a suitable collaboration partner, to undertake such activities on our behalf. If we choose to commercialize *Feraheme* outside of the U.S. ourselves, building the internal infrastructure would be costly and time-consuming, and may be distracting to management, and we may not be successful in our efforts. Our ability to commercialize *Feraheme* outside of the U.S. is also dependent upon the successful transition of the product and related materials back to us and we are relying on Takeda to perform certain services and make certain payments for our benefit in connection with such transition. If Takeda, for any reason, fails to provide such transition services or does not make the expected payments, our ability to commercialize *Feraheme* outside of the U.S. will be significantly handicapped. In addition, and in light of the termination of the Takeda licensing arrangement, recent or future changes to *Feraheme's* product label or product insert, withdrawal of the marketing authorization and the Type II Variation for *Rienso* and the fact that *Rienso* is approved for a narrower patient population than many of its competitors in the EU, could negatively impact the product's commercial potential outside of the U.S., and may lead us to withdraw the product or marketing authorization in additional geographics because it is not commercially viable. If we are not successful in commercializing or partnering, or choose not to commercialize, *Feraheme* outside of the U.S., our business may suffer, especially if we expend significant time and money pursuing such activities.

Table of Contents

Regulatory Risks

In the U.S. there have been, and we expect there will continue to be, a number of federal and state legislative initiatives implemented to reform the healthcare system in ways that could adversely impact our business and our ability to sell our products profitably.

In the U.S., there have been, and we expect there will continue to be, a number of legislative and regulatory proposals aimed at changing the U.S. healthcare system. For example, the Healthcare Reform Act contains a number of provisions that significantly impact the pharmaceutical industry and may negatively affect our business, including potential revenues. Changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under the health insurance exchanges, expansion of the 340B program, and fraud and abuse enforcement. For example, the percentage of *Feraheme* sold to 340B institutions has grown from 11% in 2011 to 17% in 2014. Since these institutions are granted lower prices than those offered to our other customers, any further growth in our 340B business may have a negative impact on our sales price per gram and operating margins. These changes will impact existing government healthcare programs and will result in the development of new programs, including Medicare payment for performance initiatives and improvements to the physician quality reporting system and feedback program. Substantial new provisions affecting compliance have also been added, which may require us to modify our business practices with healthcare providers and potentially incur additional costs. While we are continuing to evaluate this legislation and its potential impact on our business, this legislation may adversely affect the demand for *Feraheme* and *Makena* in the U.S. or cause us to incur additional expenses and therefore adversely affect our financial position and results of operations. Please see our discussion above under the heading, "*Pharmaceutical Pricing and Reimbursement*" in Item 1. Business for a more detailed discussion of such changes.

Further, although the Healthcare Reform Act exempts "orphan drugs," such as *Makena*, from 340B ceiling price requirements for the covered entity types added to the program by the Healthcare Reform Act, on July 21, 2014, the Health Resources and Services Administration ("HRSA"), which administers the 340B program, issued an interpretive rule to implement the orphan drug exception which interprets the orphan drug exception narrowly. It exempts orphan drugs from the ceiling price requirements for the newly added covered entity types, namely certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, only when the orphan drug is used for its orphan indication. The newly added entities are entitled to purchase orphan drugs at the ceiling price when the orphan drug is not used for its orphan indication. A manufacturer trade group has filed a lawsuit challenging the interpretive rule as inconsistent with the statutory language. That challenge remains ongoing. The uncertainty regarding how the statutory orphan drug exception will be applied will increase the complexity of compliance, will make compliance more time-consuming, and could negatively impact our results of operations. If HRSA's narrow interpretation of the scope of the orphan drug exemption prevails, it could potentially negatively impact the price we are paid for our *Makena* product by certain entities and increase the complexity of compliance with the 340B program.

In order to be eligible to have our products paid for with federal funds under the Medicaid and Medicare Part B programs and purchased by certain federal agencies and grantees, we participate in the Department of Veterans Affairs ("VA") Federal Supply Schedule ("FSS") pricing program. To participate, we are required to enter into an FSS contract with the VA, under which we must make our innovator "covered drugs" available to the "Big Four" federal agencies the VA, the Department of Defense, or DoD, the Public Health Service, and the Coast Guard at pricing that is capped pursuant to a statutory federal ceiling price ("FCP") formula set forth in Section 603 of the Veterans Health Care Act of 1992 ("VHCA"). The FCP is based on a weighted non-federal average manufacturer price, or Non-FAMP, which manufacturers are required to report on a quarterly and annual basis to the VA. In addition, pursuant to regulations issued by the DoD TRICARE Management Activity, now the Defense Health Agency, to implement Section 703 of the National Defense Authorization Act for

Table of Contents

Fiscal Year 2008, we are required to pay quarterly rebates on covered drug prescriptions dispensed to TRICARE beneficiaries by TRICARE network retail pharmacies. The formula for determining the rebate is established in the regulations and is based on the difference between the annual Non-FAMP and the FCP (as described above, these price points are required to be calculated by us under the VHCA).

If we overcharge the government in connection with our FSS contract or underpay our TRICARE rebates, whether due to a misstated FCP or otherwise, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges can result in allegations against us under the False Claims Act ("FCA") and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We expect that the Healthcare Reform Act, as currently enacted or as it may be amended in the future, and other healthcare reform measures that may be adopted in the future, could have a material adverse effect on our industry generally and on our ability to maintain or increase our product sales. In addition, various healthcare reform proposals have emerged at the state level in the U.S. We cannot predict the impact that newly enacted laws or any future legislation or regulation will have on us. We expect that there will continue to be a number of U.S. federal and state proposals to implement governmental pricing controls and limit the growth of healthcare costs. These efforts could adversely affect our business by, among other things, limiting the prices that can be charged for our products or the amount of reimbursement rates and terms available from governmental agencies or third-party payers, limiting the profitability of our products, increasing our rebate liability or limiting the commercial opportunities for our products, including its acceptance by healthcare payers.

If our products are marketed or distributed in a manner that violates federal, state or foreign healthcare fraud and abuse laws, marketing disclosure laws or other federal, state or foreign laws and regulations, we may be subject to civil or criminal penalties.

In addition to FDA and related regulatory requirements in the U.S. and abroad, our general operations, and the research, development, manufacture, sale and marketing of our products, are subject to extensive additional federal, state and foreign healthcare regulation, including the FCA, the Federal Anti-Kickback Statute, the Foreign Corrupt Practices Act, and their state analogues, and similar laws in countries outside of the U.S., laws, such as the U.K. Bribery Act of 2010 and governing sampling and distribution of products, and government price reporting laws as discussed above in Item 1. Business under the heading "*Government Regulation Fraud and Abuse Regulation*."

Our activities relating to the sale and marketing of our products may be subject to scrutiny under these laws and private individuals have been active in bringing lawsuits on behalf of the government under the FCA and similar regulations in other countries. We have developed and implemented a corporate compliance program based on what we believe are current best practices in the pharmaceutical industry; however, these laws are broad in scope and there may not be regulations, guidance or court decisions that definitively interpret these laws in the context of particular industry practices. We cannot guarantee that we, our employees, our consultants or our contractors are or will be in compliance with all federal, state and foreign regulations. If we or our representatives fail to comply with any of these laws or regulations, a range of fines, penalties and/or other sanctions could be imposed on us, including, but not limited to, restrictions on how we market and sell our products, significant fines, exclusions from government healthcare programs, including Medicare and Medicaid, litigation, or other sanctions. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could also have an adverse effect on our business, financial condition and

Table of Contents

results of operations. Such investigations or suits may also result in related shareholder lawsuits, which can also have an adverse effect on our business.

Further, the FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. For drug products like *Makena* that are approved by the FDA under the FDA's accelerated approval regulations, unless otherwise informed by the FDA, the sponsor must submit promotional materials at least 30 days prior to the intended time of initial dissemination of the promotional materials, which delays and may negatively impact our commercial team's ability to implement changes to *Makena's* marketing materials, thereby negatively impacting revenues. Moreover, under Subpart H, the FDA may also withdraw approval of *Makena* if, among other things, the promotional materials are false or misleading, or other evidence demonstrates that *Makena* is not shown to be safe or effective under its conditions of use.

The U.S. government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The government has also required companies to enter into complex corporate integrity agreements and/or non-prosecution agreements that can impose significant restrictions and other burdens on the affected companies. If we are found to have promoted such off-label uses, we may become subject to similar consequences.

In recent years, several U.S. states have enacted legislation requiring pharmaceutical companies to establish marketing and promotional compliance programs or codes of conduct and/or to file periodic reports with the state or make periodic public disclosures on sales, marketing, pricing, clinical trials, and other activities. In addition, as part of the Healthcare Reform Act, manufacturers of drugs are required to publicly report gifts and other payments or transfers of value made to U.S. physicians and teaching hospitals. Several states have also adopted laws that prohibit certain marketing-related activities, including the provision of gifts, meals or other items to certain healthcare providers. Many of these requirements are new and uncertain, and the likely extent of penalties for failure to comply with these requirements is unclear; however, compliance with these laws is difficult, time consuming and costly, and if we are found to not be in full compliance with these laws, we may face enforcement actions, fines and other penalties, and we could receive adverse publicity which could have an adverse effect on our business, financial condition and results of operations.

If we fail to comply with any federal, state or foreign laws or regulations governing our industry, we could be subject to a range of regulatory actions that could adversely affect our ability to commercialize our products, harm or prevent sales of our products, or substantially increase the costs and expenses of commercializing and marketing our products, all of which could have a material adverse effect on our business, financial condition and results of operations.

In addition, incentives exist under applicable U.S. law that encourage employees and physicians to report violations of rules governing promotional activities for pharmaceutical products. These incentives could lead to so-called whistleblower lawsuits as part of which such persons seek to collect a portion of moneys allegedly overbilled to government agencies as a result of, for example, promotion of pharmaceutical products beyond labeled claims. Such lawsuits, whether with or without merit, are typically time-consuming and costly to defend. Such suits may also result in related shareholder lawsuits, which are also costly to defend.

Table of Contents

If we fail to comply with our reporting and payment obligations under U.S. governmental pricing programs, we could be required to reimburse government programs for underpayments and could pay penalties, sanctions and fines which could have a material adverse effect on our business, financial condition and results of operations.

As a condition of reimbursement by various U.S. federal and state healthcare programs, we are required to calculate and report certain pricing information to U.S. federal and state healthcare agencies. For example, we participate in and have certain price reporting obligations to the Medicaid Drug Rebate program, and we have obligations to report average sales price ("ASP") for the Medicare program. Under the Medicaid Drug Rebate program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data reported by us on a monthly and quarterly basis to the Centers for Medicare and Medicaid Services ("CMS"), the federal agency that administers the Medicare and Medicaid programs. These data include the average manufacturer price and, in the case of innovator products such as *Feraheme* and *Makena*, the best price for each drug.

The Medicaid rebate amount is computed each quarter based on our submission to CMS of our current average manufacturer prices and best prices for the quarter. If we become aware that our reporting for a prior quarter was incorrect, or has changed as a result of recalculation of the pricing data, we are obligated to resubmit the corrected data for a period not to exceed 12 quarters from the quarter in which the data originally were due. Such restatements and recalculations increase our costs for complying with the laws and regulations governing the Medicaid program. Any corrections to our rebate calculations could result in an overage or underage in our rebate liability for past quarters, depending on the nature of the correction. Price recalculations also may affect the 340B "ceiling price." The 340B pricing program requires participating manufacturers to agree to charge statutorily defined covered entities, such as safety-net providers, no more than the 340B ceiling price for the manufacturer's covered outpatient drugs. The 340B ceiling price is calculated using a statutory formula, which is based on the average manufacturer price and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate program.

Federal law also requires that a company that participates in the Medicaid program report ASP information to CMS for certain categories of drugs that are paid under Part B of the Medicare program, such as *Feraheme* and *Makena*. This ASP information forms the basis for reimbursement for the majority of our current *Feraheme* business, and to a lesser extent, for the *Makena* business. Manufacturers calculate ASP based on a statutorily defined formula and interpretations of the statute by CMS as to what should or should not be considered in computing ASP. An ASP for each National Drug Code for a product that is subject to the ASP reporting requirement must be submitted to CMS no later than 30 days after the end of each calendar quarter. CMS uses these submissions to determine payment rates for drugs under Medicare Part B. Changes affecting the calculation of ASP could affect the ASP calculations for our products and the resulting Medicare payment rate, and could negatively impact our results of operations.

Price reporting and payment obligations are highly complex and vary among products and programs. The calculations are complex and are often subject to interpretation by us, governmental or regulatory agencies and the courts. The calculations of average manufacturer price, best price, and ASP include a number of inputs from our contracts with wholesalers, specialty distributors, GPOs and other customers. The calculations also require us to make an assessment of whether these agreements are deemed to be for *bona fide* services and whether the fees we pay for any bona fide services represent fair market value in our industry and for our products. These calculations are very complex and could involve the need for us to unbundle or reallocate discounts or rebates offered over multiple quarters or across multiple products. Our processes for estimating amounts due under these governmental pricing

Table of Contents

programs involve subjective decisions and estimates. For example, almost half of *Makena* sales are reimbursed through state Medicaid programs and are subject to the statutory Medicaid rebate, and in some cases, supplemental rebates offered by us. Often, state Medicaid programs may be slow to invoice pharmaceutical companies for these rebates resulting in a significant lag between the time a sale is recorded and the time the rebate is paid. This results in us having to carry a significant liability on our balance sheet for the estimate of rebate claims expected for Medicaid patients. If actual claims are higher than current estimates, our financial position and results of operations could be adversely affected. The unbundling of discounts and rebates across multiple reporting periods can also result in a restatement of government price reports and changes to the reimbursement rates for various customers covered under federal programs, such as Medicare, Medicaid or the 340B program.

If we have to restate our calculation of government price reports, we may be forced to refund certain monies back to payers to comply with federal pricing agreements. Such a restatement of our government price reports would also adversely impact our reported financial results of operations in the period of such restatement. As a result, our price reporting calculations remain subject to the risk of errors and our methodologies for calculating these prices could be challenged under the FCA or other laws. In addition, the Healthcare Reform Act modified the rules related to certain price reports, among other things. Presently, uncertainty exists as many of the specific determinations necessary to implement this new legislation have yet to be decided and communicated to industry participants. This uncertainty in the interpretation of the legislation increases the chances of an error in price reporting, which could in turn lead to a legal challenge, restatement or investigation. If we become subject to investigations, restatements, or other inquiries concerning our compliance with price reporting laws and regulations, we could be required to pay or be subject to additional reimbursements, penalties, sanctions or fines, which could have a material adverse effect on our business, financial condition and results of operations.

We are liable for errors associated with our submission of pricing data. In addition to retroactive adjustments to rebate amounts and the potential for 340B program refunds, if we are found to have knowingly submitted false average manufacturer price, average sales price, or best price information to the government, we may be liable for civil monetary penalties in the amount of $100,000 per item of false information. If we are found to have made a misrepresentation in the reporting of our ASP, the Medicare statute provides for civil monetary penalties of up to $10,000 for each misrepresentation for each day in which the misrepresentation was applied. Our failure to submit monthly/quarterly average manufacturer price, average sales price, and best price data on a timely basis could result in a civil monetary penalty of $10,000 per day for each day the information is late beyond the due date. Such failure also could be grounds for CMS to terminate our Medicaid drug rebate agreement, pursuant to which we participate in the Medicaid Drug Rebate program. In the event that CMS terminates our rebate agreement, federal payments may not be available under Medicaid or Medicare Part B for our covered outpatient drugs.

If we overcharge the government, we are required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges can result in allegations against us under the FCA and other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Table of Contents

We are subject to ongoing U.S. and foreign regulatory obligations and oversight of our products, and any failure by us to maintain compliance with applicable regulations may result in several adverse consequences including the suspension of the manufacturing, marketing and sale of our respective products, the incurrence of significant additional expense and other limitations on our ability to commercialize our respective products.

We are subject to ongoing regulatory requirements and review, including by periodic audits, both in the U.S. and in foreign jurisdictions pertaining to the development, manufacture, labeling, packaging, adverse event reporting, distribution, storage, marketing, promotion, record keeping and export of our respective products. Failure to comply with such regulatory requirements or the later discovery of previously unknown problems with our products or our third-party contract manufacturing facilities or processes by which we manufacture our products may result in restrictions on our ability to manufacture, market, distribute or sell our products, including potential withdrawal from the market. Any such restrictions could result in a decrease in our product sales, damage to our reputation or the initiation of lawsuits against us and/or our third-party contract manufacturers. We may also be subject to additional sanctions, including but not limited to:

Warning letters;

Civil or criminal penalties;

Variation, suspension or withdrawal of regulatory approvals;

Changes to the package insert of our products, such as additional warnings regarding potential side effects or potential limitations on the current dosage and administration;

Requirements to communicate with physicians and other customers about concerns related to actual or potential safety, efficacy, or other issues involving our products;

Implementation of risk mitigation programs and post-marketing obligations;

Restrictions on our continued manufacturing, marketing, distribution or sale of our products;

Temporary or permanent closing of the facilities of our third-party contract manufacturers;

Interruption of clinical trials; or

Recalls or a refusal by regulators to consider or approve applications for additional indications.

Any of the above sanctions could have a material adverse impact on our revenue generation and profitability and cause us to incur significant additional expenses.

Additionally, Lumara Health, as our wholly owned subsidiary following consummation of the acquisition, is subject to certain continuing obligations under a Consent Decree of Permanent Injunction ("Consent Decree") between the FDA, Lumara Health's predecessor company, K-V Pharmaceutical Company ("K-V Pharmaceutical") and certain former officers and affiliates of K-V Pharmaceutical. In particular, Lumara Health is bound by a number of provisions and requirements in the Consent Decree including, but not limited to, inspection of Lumara Health's places of business by the FDA without prior notice, and notification of FDA of particular actions and events. If Lumara Health fails to comply with applicable provisions in the Consent Decree, the FDC, or the FDC's implementing regulations, the FDA may impose specific sanctions including, but not limited to, the requirement to cease any of Lumara Health's manufacturing operations, the imposition of substantial financial penalties and the requirement to implement additional corrective actions.

61

Table of Contents

Regulators could determine that our clinical trials and/or our manufacturing processes, or those of our third parties, were not properly designed or are not properly operated, which could cause significant costs or setbacks for our commercialization activities.

We are obligated to conduct, and are in the process of conducting, certain post-approval clinical trials, and we may be required to conduct additional clinical trials, including if we pursue approval of additional indications, seek commercialization in other jurisdictions, or in support of our current indications. We may also determine to conduct additional clinical trials, including if we pursue new formulations or methods of administration for our products. The FDA could determine that our clinical trials and/or our manufacturing processes were not properly designed, did not include enough patients or appropriate administration, were not conducted in accordance with applicable laws and regulations, or were otherwise not properly managed. In addition, according to current good clinical practices regulations ("cGCP") we are responsible for conducting, recording and reporting the results of clinical trials to ensure that the data and results are credible and accurate and that the trial participants are adequately protected. The FDA may conduct inspections of clinical investigator sites which are involved in our clinical development programs to ensure their compliance with cGCP regulations. If the FDA determines that we, our CROs or our study sites fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may disqualify certain data generated from those sites or require us to perform additional clinical trials. Our clinical trials and manufacturing processes are subject to similar risks and uncertainties outside of the U.S. Any such deficiency in the design, implementation or oversight of our clinical development programs or post-approval clinical studies could cause us to incur significant additional costs, experience further delays or prevent us from commercializing *Makena* and *Feraheme* in their current indications, or obtaining marketing approval for additional indications, including the approval for use of *Feraheme* for the broad IDA indication, if such approval is pursued.

Further, our third-party contract manufacturing facilities are subject to cGMP regulations enforced by the FDA and equivalent foreign regulations and regulatory agencies through periodic inspections to confirm such compliance. Contract manufacturers must continually expend time, money and effort in production, record-keeping and quality assurance and control to ensure that these manufacturing facilities meet applicable regulatory requirements. Failure to maintain ongoing compliance with cGMP or similar foreign regulations and other applicable manufacturing requirements of various U.S. or foreign regulatory agencies could result in, among other things, the issuance of warning letters, fines, the withdrawal or recall of our products from the marketplace, total or partial suspension of product production, the loss of inventory, suspension of the review of our current or future sNDAs or equivalent foreign filings, enforcement actions, injunctions or criminal prosecution and suspension of manufacturing authorizations. A government-mandated recall or a voluntary recall could divert managerial and financial resources, could be difficult and costly to correct, could result in the suspension of sales of our products and could have a severe adverse impact on our profitability and the future prospects of our business. If any regulatory agency inspects any of these manufacturing facilities and determines that they are not in compliance with cGMP or similar regulations or our contract manufacturers otherwise determine that they are not in compliance with these regulations, as applicable, such contract manufacturers could experience an inability to manufacture sufficient quantities of product to meet demand or incur unanticipated compliance expenditures.

We have also established certain testing and release specifications with the FDA and other foreign regulatory agencies. This release testing must be performed in order to allow finished product to be used for commercial sale. If a finished product does not meet these release specifications or if the release testing is variable, we may not be able to supply product to meet our projected demand. We monitor annual batches of our finished product for ongoing stability after it has been released for commercial sale. If a particular batch of finished drug product exhibits variations in its stability or begins to generate test results that demonstrate an adverse trend against our specifications, we may

Table of Contents

need to conduct an investigation into the test results, quarantine the product to prevent further use, destroy existing inventory no longer acceptable for commercial sale, or recall the batch or batches. If we are unable to develop, validate, transfer or gain regulatory approval for the new release test, our ability to supply product to the EU will be adversely affected. Such setbacks could have an adverse impact on our revenues, our profitability and the future prospects of our business.

Risks Related to Our Business Generally

With our acquisition of Lumara Health we have significantly expanded the size of our organization and we may experience difficulties in managing this or future expansion.

With the acquisition of Lumara Health, we increased our headcount by 108 full time employees. Management, personnel, systems and facilities that we currently have in place may not be adequate to support this recent growth, and we may not be able to retain or recruit qualified personnel in the future in this competitive environment to adequately support our new organization. To manage any future growth effectively, we may be required to continue to manage and expand the sales and marketing efforts for our existing products while continuing to identify and acquire attractive additions to our product portfolio, enhance our operational, financial and management controls, reporting systems and procedures and establish and increase our access to commercial supplies of our products, which will be challenging and for which we might not be successful, especially given our newly-expanded organization. We will be required to expand and maintain our facilities and equipment and manage our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors, collaborators, distributors and other third parties. In addition, management may have to divert a disproportionate amount of its attention away from day-to-day activities and towards managing these growth activities, which could be disruptive to our business. Our future financial performance and our ability to execute on our business plan will depend, in part, on our ability to effectively manage our recent and any future growth. If we experience difficulties or are unsuccessful in managing our expansion, our results of operations and business prospects will be negatively impacted.

Our level of indebtedness and the terms of the Term Loan Facility (including the financial covenants) and Convertible Notes could adversely affect our operations and limit our ability to plan for or respond to changes in our business or acquire additional products for our portfolio. If we are unable to comply with restrictions in the Term Loan Facility or cannot repay or refinance the Convertible Notes, the indebtedness under the Term Loan Facility could be accelerated.

We entered into the Term Loan Facility, which provided us with $340.0 million to finance our acquisition of Lumara Health. We also incurred significant indebtedness in the amount of $200.0 million in aggregate principal with additional accrued interest under our Convertible Notes (as defined below). Our level of indebtedness could adversely affect our business by, among other things:

- requiring us to dedicate a substantial portion of our cash flow from operations to payments on our indebtedness, thereby reducing the availability of our cash flow for other purposes, including further diversification of our product portfolio and expansion of sales of *Feraheme* in the current or broader indications;

- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we operate, thereby placing us at a competitive disadvantage compared to our competitors that may have less debt; and

- increasing our vulnerability to adverse economic and industry conditions.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance our indebtedness, including the Term Loan Facility and the Convertible Notes, depends on our future

Table of Contents

performance, which is subject to economic, financial, competitive and other factors that may be beyond our control. Our business may not generate cash flow from operations in the future sufficient to service our debt and support our growth strategies. If we are unable to generate such cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, including under the Term Loan Facility or the Convertible Notes.

The Term Loan Facility requires us to make certain payments of principal and interest over time and contains a number of other restrictive covenants, including a financial covenant based on the total amount of debt we have as a multiple of our cash flow, as defined in the Facility, and a requirement that we reduce our indebtedness over time. The Term Loan Facility also contains covenants and terms limiting our ability to enter into new acquisitions, licenses, mergers, foreign investments, to take on new debt and sell assets, and requiring us to pay penalties in the event we want to prepay the Term Loan Facility early. The maturity date of the Term Loan Facility could also be accelerated in certain circumstances, including if we are not able to repay or refinance our Convertible Notes or in the event of an uncured event of default as outlined in the Term Loan Facility. The Term Loan Facility has a floating interest rate based on the prevailing London Interbank Offered Rate ("LIBOR") rate, making interest payments subject to adjustment depending on the interest rate environment. These and other terms in the Term Loan Facility have to be monitored closely for compliance and could restrict our ability to grow our business or enter into transactions that we believe will be beneficial to our business.

Further, holders of the Convertible Notes have the right to require us to repurchase their notes upon the occurrence of a fundamental change at a repurchase price equal to 100% of the principal amount of the notes to be repurchased, plus accrued and unpaid interest. Upon conversion of the Convertible Notes (which are currently convertible), unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional share), we will be required to make cash payments in respect of the notes being converted. We may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of Convertible Notes surrendered therefor or at the time Convertible Notes are being converted. Our failure to repurchase Convertible Notes at a time when the repurchase is required by the indenture or to pay any cash payable on future conversions of the Convertible Notes would constitute an event of default. If the repayment of any indebtedness were to be accelerated because of such event of default (whether under the Convertible Notes, our Term Loan Facility or otherwise), we may not have sufficient funds to repay the indebtedness and repurchase the Convertible Notes or make cash payments upon conversions thereof. Moreover, if our stock price increases, the parties with whom we entered into warrant transactions in connection with the pricing of the Convertible Notes (the "Warrants") could exercise such warrants, thereby causing substantial dilution to our stockholders.

We cannot make any assurances that our future operating results will be sufficient to ensure compliance with the covenants in these arrangements or to remedy any such default. In the event of an acceleration of this indebtedness, we may not have or be able to obtain sufficient funds to make any accelerated payments. Any of the factors discussed above could materially and adversely affect our business, financial condition and results of operations. In addition, if we incur additional indebtedness, the risks related to our business and our ability to service or repay our indebtedness would increase.

Table of Contents

We may need additional capital to achieve our business objectives and to service our debt obligations, including the Term Loan Facility, our Convertible Notes and contingent payments that may become due under the Lumara Agreement, which could cause significant dilution to our stockholders.

We estimate that our cash resources as of December 31, 2014, combined with cash we currently expect to receive from product sales and earnings on our investments will be sufficient to finance our currently planned operations for at least the next 12 months. We may require additional funds or need to establish additional alternative strategic arrangements to execute a business development transaction. We may at any time seek funding through additional arrangements with collaborators through public or private equity or debt financings, subject to the covenants in our Term Loan Facility. We may not be able to obtain financing or to secure alternative strategic arrangements on acceptable terms or within an acceptable timeframe, if at all, which would limit our ability to execute on our strategic plan. Moreover, the condition of the credit markets can be unpredictable and we may experience a reduction in value or loss of liquidity with respect to our investments, which would put further strain on our cash resources.

Further, in November 2014, we completed the acquisition of Lumara Health, which required us to pay $600.0 in upfront cash consideration and approximately 3.2 million shares of newly issued common stock. We used a combination of cash on hand and the $340.0 million Term Loan Facility to pay the upfront cash consideration. In addition to the consideration paid at closing, our definitive merger agreement with Lumara Health (the "Lumara Agreement") provides for contingent consideration of up to an additional $350.0 million based on the achievement of various sales milestones for *Makena,* which could be paid in all cash. We also incurred substantial costs and expenses associated with the transaction. As a result, our current level of cash on hand may limit our ability to take advantage of attractive business development opportunities and execute on our strategic plan. In addition, our cash on hand may not be sufficient to service the principal and interest payments under the Term Loan Facility, our existing Convertible Notes or any cash milestone payments to the former Lumara Health security holders upon the achievement of sales milestones. Our ability to make these required payments could be adversely affected if we do not achieve expected revenue and cash flow forecasts or if we are unable to find other sources of cash in the future. If we therefore need to pay the former Lumara Health security holders in stock upon the achievement of sales milestones, it will result in dilution to our stockholders.

Our long-term capital requirements will depend on many other factors, including, but not limited to the commercial success of our products and efforts we make in connection with commercialization and development, our ability to realize synergies and opportunities in connection with our acquisitions and portfolio expansion, the outcome of and costs associated with any material litigation or patent challenges to which we are or may become a party, the timing and magnitude of costs associated with qualifying additional manufacturing capacities and alternative suppliers, and our ability to raise additional capital on terms and within a timeframe acceptable to us, if necessary.

The $200.0 million of 2.5% convertible senior notes due February 15, 2019 (the "Convertible Notes") are, the Warrants may be, and any additional equity or equity-linked financings or alternative strategic arrangements would be, dilutive to our stockholders. In addition, the terms of our current debt instruments or any additional debt financing could greatly restrict our ability to raise additional capital and may provide rights and preferences to the investors in any such financing which are senior to those of, and not available to, current stockholders, impose restrictions on our day-to-day operations or place limitations on our ability to enter into combination transactions with other entities. Our inability to raise additional capital on terms and within a timeframe acceptable to us when needed could force us to dramatically reduce our expenses and delay, scale back or eliminate certain of our activities and operations, including our commercialization and development activities, any of which would have a material adverse effect on our business, financial condition and future business prospects.

Table of Contents

Our ability to use net operating loss carryforwards and tax credit carryforwards is dependent on generating future taxable income and may be limited, including as a result of future transactions involving our common stock.

In general, under Section 382 of the Internal Revenue Code of 1986, as amended, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such "ownership change" by allowing us to utilize only a portion of the net operating losses and tax credits that would otherwise be available but for such ownership change. Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income or the failure to generate sufficient taxable income could require us to pay more U.S. federal income taxes than we have estimated and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position, including our after-tax net income. Similar rules and limitations may apply for state income tax purposes.

In September 2014, we adopted an amendment to our shareholder rights plan to help preserve our tax assets by deterring certain stockholders from increasing their percentage ownership in our stock; however, such amendment is merely a deterrent that does not actually prevent Section 382 ownership limitations and there can be no assurance that we will not undergo an ownership change. Even minor accumulations by certain of our stockholders could result in triggering an ownership change under Section 382. If such an ownership change were to occur, we expect that our net operating losses could become limited; however, the amount of the limitation would depend on a number of factors including our market value at the time of the ownership change. For a discussion of the amendment to our shareholder rights plan, see the discussion in Note O, "*Stockholders' Equity*," to our consolidated financial statements included in this Annual Report on Form 10-K.

In addition, we have recorded deferred tax assets based on our assessment that we will be able to realize the benefits of our net operating losses and other favorable tax attributes. Realization of deferred tax assets involve significant judgments and estimates which are subject to change and ultimately depends on generating sufficient taxable income of the appropriate character during the appropriate periods. Changes in circumstances may affect the likelihood of such realization, which in turn may trigger a write-down of our deferred tax assets, the amount of which would depend on a number of factors. A write-down would reduce our reported net income, which may adversely impact our financial condition or results of operations or cash flows. In addition, we are potentially subject to ongoing and periodic tax examinations and audits in various jurisdictions, including with respect to the amount of our net operating losses and any limitation thereon. An adjustment to such net operating loss carryforwards, including an adjustment from a taxing authority, could result in higher tax costs, penalties and interest, thereby adversely impacting our financial condition, results of operations or cash flows.

An adverse determination in any current or future lawsuits in which we are a defendant could have a material adverse effect on us.

The administration of our products to, or the use of our products by, humans may expose us to liability claims, whether or not our products are actually at fault for causing an injury. As *Feraheme* is used over longer periods of time by a wider group of patients taking numerous other medicines or by patients with additional underlying health problems, the likelihood of adverse drug reactions or unintended side effects, including death, may increase. While these adverse events are rare, all

Table of Contents

IV irons, including *Feraheme*, can cause patients to experience serious hypersensitivity reactions, including anaphylactic-type reactions, some of which have been life-threatening and/or fatal. *Makena* is a prescription hormone medicine (progestin) used to lower the risk of preterm birth in women who are pregnant and who have previously delivered preterm in the past. It is not known if *Makena* is safe and effective in women who have other risk factors for preterm birth. In one clinical study, certain complications or events associated with pregnancy occurred more often in women who received *Makena*. These included miscarriage (pregnancy loss before 20 weeks of pregnancy), hospital admission for preterm labor, preeclampsia, gestational hypertension and gestational diabetes. In addition, other hormones administered during pregnancy have in the past been shown to cross the placenta and have negative effects on the offspring. Although we maintain product liability insurance coverage for claims arising from the use of our products in clinical trials and commercial use, coverage is expensive, and we may not be able to maintain sufficient insurance at a reasonable cost, if at all. Product liability claims, whether or not they have merit, could also decrease demand for our products, subject us to product recalls or harm our reputation, cause us to incur substantial costs, and divert management's time and attention.

As discussed below in Item 3. Legal Proceedings, we were the target of a purported class action complaint filed in March 2010 entitled *Silverstrand Investments et. al. v. AMAG Pharm., Inc., et. al.*, which alleged certain securities laws violations. Although we settled the *Silverstrand* case in January 2015, we may also be the target of claims asserting violations of securities and fraud and abuse laws and derivative actions or other litigation in the future. Any future litigation could result in substantial costs and divert our management's attention and resources, which could cause serious harm to our business, operating results and financial condition. Further, we may not be successful in defending ourselves in the litigation and, as a result, our business could be materially harmed. These lawsuits may result in large judgments or settlements against us, any of which could have a negative effect on our financial condition and business if in excess of our insurance coverage. Though we maintain liability insurance, if any costs or expenses associated with litigation exceed our insurance coverage, we may be forced to bear some or all of these costs and expenses directly, which could be substantial.

Our success depends on our ability to attract and retain key employees, and any failure to do so may be disruptive to our operations.

We are a pharmaceutical company focused on marketing commercial products and we plan to expand our product portfolio with additional commercial-stage products through acquisitions and in-licensing; thus, the range of skills of our executive officers needs to be broad and deep. If we are not able to hire and retain talent to drive commercialization and expansion of our product portfolio, we will be unlikely to be profitable. For example, in October 2014, our Senior Vice President and Chief Development and Regulatory Officer resigned from the Company to pursue other opportunities, which may be disruptive to our regulatory discussions with the FDA or other regulators. Further, because of the specialized nature of our business, our success depends to a significant extent on our ability to continue to attract, retain and motivate qualified sales, technical operations, managerial, scientific, regulatory compliance and medical personnel of all levels. The loss of key personnel or our inability to hire and retain personnel who have such sales, technical operations, managerial, scientific, regulatory compliance and medical backgrounds could materially adversely affect our research and development efforts and our business.

Our operating results will likely fluctuate, including as a result of wholesaler, distributor and customer buying patterns, so you should not rely on the results of any single quarter to predict how we will perform over time.

Our future operating results will likely vary from quarter to quarter depending on a number of factors, including the factors described in these Risk Factors, many of which we cannot control, as well as the timing and magnitude of:

The timing and magnitude of product revenues;

The loss of a key customer or GPO;

Table of Contents

Costs and liabilities incurred in connection with business development activities or business development transactions into which we may enter;

Costs associated with the commercialization of our products in the U.S., including costs associated with pursuing a broader indication of *Feraheme* or the *Makena* lifecycle management program;

Makena milestone payments we may be required to pay to the former shareholders of Lumara Health pursuant to the Lumara Agreement;

The timing and magnitude of tax payments and of principal and interest payments in connection with the Term Loan Facility and our Convertible Notes;

Costs associated with the manufacture of our products, including costs of raw and other materials and costs associated with maintaining commercial inventory and qualifying additional manufacturing capacities and alternative suppliers;

The recognition of deferred tax assets during periods in which we generate taxable income and our ability to preserve our net operating loss carryforwards and other tax assets;

Costs associated with our ongoing and planned clinical studies of *Feraheme* in connection with our pediatric program, our current or future post-marketing commitments for the EMA and other regulatory agencies, our pursuit, if any, of additional indications and our development of *Feraheme* in countries outside of the U.S;

Costs associated with the ongoing and planned clinical studies of *Makena* in connection with current or future post-marketing commitments, and our pursuit, if any, of additional indications or lifecycle management program;

Any changes to the mix of our business;

Costs associated with manufacturing batch failures or inventory write-offs due to out-of-specification release testing or ongoing stability testing that results in a batch no longer meeting specifications;

Changes in accounting estimates related to reserves on revenue, returns, contingent consideration, impairment of long-lived or intangible assets or other accruals or changes in the timing and availability of government or customer discounts, rebates and incentives;

The implementation of new or revised accounting or tax rules or policies; and

Costs associated with the implementation of new or revised regulations of the Public Company Accounting Oversight Board, NASDAQ Global Select Market ("NASDAQ"), the U.S. Securities and Exchange Commission ("SEC") and similar entities.

Our results of operations, including, in particular, product sales, may also vary from period to period due to the buying patterns of our wholesalers, distributors, pharmacies, clinics or hospitals and specialty pharmacies. Further, our contracts with GPOs often require certain performance from the members of the GPOs on an individual account level or group level such as growth over prior periods or certain market share attainment goals in order to qualify for discounts off the list price of our products, and a GPO may be able to influence the demand for our products from its members in a particular quarter through communications they make to their members. In the event wholesalers, distributors,

pharmacies, clinics or hospitals with whom we do business determine to limit their purchases of our products, our product sales could be adversely affected. Also, in the event wholesalers, distributors, pharmacies, clinics or hospitals purchase increased quantities of our products to take advantage of volume discounts or similar benefits, our quarterly results will fluctuate as re-orders become less frequent, and our overall net pricing may decrease as a result of such discounts and similar

Table of Contents

benefits. In addition, these contracts are often cancellable at any time by our customers, often without notice, and are non-exclusive agreements within the *Feraheme* or *Makena* markets. While these contracts are intended to support the use of our products, our competitors could offer better pricing, incentives, higher rebates or exclusive relationships. Because *Feraheme* is not indicated for the broad IDA population, the incentives in our contracts for a particular site of care are capped based on our estimate of their patients covered by our current CKD label. Because some of our competitors' products have the broad IDA label, they may provide additional incentives for all of a customer's IV iron usage, essentially becoming an exclusive provider to that particular customer.

Our contracting strategy can also have an impact on the timing of certain purchases causing product sales to vary from quarter to quarter. For example, in advance of an anticipated price increase, following the publication of our quarterly ASP, which affects the rate at which *Feraheme* is reimbursed, or a reduction in expected rebates or discounts for *Feraheme*, customers may order *Feraheme* in larger than normal quantities which could cause *Feraheme* sales to be lower in subsequent quarters than they would have been otherwise. Further, any changes in purchasing patterns or inventory levels, changes to our contracting strategy, increases in product returns, delays in purchasing products or delays in payment for products by one of our distributors or GPOs could also have a negative impact on our revenue and results of operations.

As a result of these and other factors, our quarterly operating results could fluctuate, and this fluctuation could cause the market price of our common stock to decline. Results from one quarter should not be used as an indication of future performance.

If the estimates we make, or the assumptions on which we rely, in preparing our consolidated financial statements and/or our projected guidance prove inaccurate, our actual results may vary from those reflected in our projections and accruals.

Our consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us, and the related disclosure of contingent assets and liabilities. On an ongoing basis, our management evaluates our critical and other significant estimates and judgments, including among others those associated with revenue recognition related to product sales and collaboration agreements, product sales allowances and accruals, assessing investments for potential other-than-temporary impairment and determining the fair values of our investments, the fair value of our debt obligations, the fair value of assets acquired in a business combination, contingent consideration, the impairment of long-lived assets, including intangible assets, accrued expenses, income tax and equity-based compensation expense. We base our estimates on market data, our observance of trends in our industry, and various other assumptions that we believe to be reasonable under the circumstances. If actual results differ from these estimates, there could be a material adverse effect on our financial results and the performance of our stock.

Further, in January 2015, we issued financial guidance, including expected 2015 total revenues and *Feraheme* and *Makena* net sales, which is likewise based on estimates and the judgment of management. If, for any reason, we are unable to realize our projected 2015 revenue, we may not realize our publicly announced financial guidance. If we fail to realize or if we change or update any element of our publicly disclosed financial guidance or other expectations about our business, our stock price could decline in value.

As part of our revenue recognition policy, our estimates of product returns, rebates and chargebacks, fees and other discounts require subjective and complex judgments due to the need to make estimates about matters that are inherently uncertain. The same estimates will need to be made for *Makena* sales since much of Lumara Health's business provides for discounts, fees, rebates and

Table of Contents

chargebacks, in particular, since a significant amount of *Makena* sales are to Medicaid patients. Any significant differences between our actual results and our estimates could materially affect our financial position and results of operations.

In addition, to determine the required quantities of *Feraheme* and *Makena* and their related manufacturing schedules, we also need to make significant judgments and estimates based on inventory levels, current market trends, anticipated sales, forecasts and other factors. Because of the inherent nature of estimates, there could be significant differences between our estimates and the actual amount of product need. For example, the level of our access to wholesaler and distributor inventory levels and sales data in the U.S., which varies based on the wholesaler, distributor, clinic or hospital, affects our ability to accurately estimate certain reserves included in our financial statements. Any difference between our estimates and the actual amount of product demand could result in unmet demand or excess inventory, each of which would adversely impact our financial results and results of operations.

Our stock price has been and may continue to be volatile, and your investment in our stock could decline in value or fluctuate significantly, including as a result of analysts' activities.

The market price of our common stock has been, and may continue to be, volatile, and your investment in our stock could decline in value or fluctuate significantly. Our stock price has ranged between $16.49 and $48.50 in the fifty-two week period through February 4, 2015. The stock market has from time to time experienced extreme price and volume fluctuations, particularly in the biotechnology and pharmaceuticals sectors, which have often been unrelated to the operating performance of particular companies. Various factors and events, including the factors and events described in these Risk Factors, many of which are beyond our control, may have a significant impact on the market price of our common stock. Our stock price could also be subject to fluctuations as a result of general market conditions, shareholder activism and attempts to disrupt our strategy by activist investors or sales of large blocks of our common stock or the dilutive effect of our Convertible Notes or any other equity or equity-linked financings or alternative strategic arrangements.

In addition, the trading market for our common stock relies in part on the research and reports that industry or financial analysts publish about us and our business. As of February 4, 2015, five financial analysts publish reports about us and our business. We do not control these or any other analysts. Furthermore, there are many large, well-established, publicly traded companies active in our industry and market, which may mean that it is less likely that we will receive widespread analyst coverage. In addition, our future operating results are subject to substantial uncertainty, and our stock price could decline significantly if we fail to meet or exceed analysts' forecasts and expectations. If any of the analysts who cover us downgrade our stock, lower their price target or issue commentary or observations about us or our stock that are perceived by the market as negative, our stock price would likely decline rapidly. In addition, if these analysts cease coverage of our company, we could lose visibility in the market, which in turn could also cause our stock price to decline.

There is a potential market overhang that could depress the value of our common stock, and future sales of our common stock could put a downward pressure on the price of our shares and could have a material adverse effect on the price of our shares.

In connection with the Lumara Agreement, we issued approximately 3.2 million shares of newly issued common stock to former Lumara Health security holders. Upon the demand of a certain number or percentage of such former Lumara Health security holders, we have agreed to file a registration statement to register the disposition of the shares of our common stock issued to such shareholders. In accordance with the Lumara Agreement, on February 10, 2015, we filed a registration statement on Form S-3 to register the resale of approximately 1.6 million shares of such common stock. Furthermore, upon the expiration of a contractual lock-up period, the remainder (approximately 1.6 million shares) of the common stock issued to former Lumara Health security holders can be sold.

Table of Contents

If these shares are sold, or if other existing stockholders or our officers or directors sell, or indicate an intention to sell (which may include sales pursuant to written plans for trading shares in reliance on Rule 10b5-1 under the Securities Act of 1933), substantial amounts of our common stock in the public market, the market price of our common stock could decline.

If we identify a material weakness in our internal controls over financial reporting, our ability to meet our reporting obligations and the trading price of our stock could be negatively affected.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Accordingly, a material weakness increases the risk that the financial information we report contains material errors.

We regularly review and update our internal controls, disclosure controls and procedures, and corporate governance policies. In addition, we are required under the Sarbanes-Oxley Act of 2002 to report annually on our internal control over financial reporting. Any system of internal controls, however well designed and operated, is based in part on certain assumptions and can provide only reasonable, not absolute, assurances that the objectives of the system are met. If we, or our independent registered public accounting firm, determine that our internal controls over our financial reporting are not effective, or we discover areas that need improvement in the future, or we experience high turnover of our personnel in our financial reporting functions, these shortcomings could have an adverse effect on our business and financial results, and the price of our common stock could be negatively affected.

If we cannot conclude that we have effective internal control over our financial reporting, or if our independent registered public accounting firm is unable to provide an unqualified opinion regarding the effectiveness of our internal control over financial reporting, investors could lose confidence in the reliability of our financial statements, which could lead to a decline in our stock price. Because Lumara Health had been a private company, it has not been required to comply with the Sarbanes-Oxley Act of 2002. As such, we are in the process of integrating Lumara Health related controls into our current control environment. However, our 2014 assessment did not include evaluating the effectiveness of internal control over financial reporting of Lumara Health its subsidiaries, the consolidated results of which are included in our fiscal year 2014 consolidated financial statements. Failure to comply with reporting requirements could subject us to sanctions and/or investigations by the SEC, NASDAQ or other regulatory authorities.

Our shareholder rights plan, certain provisions in our charter and by-laws, certain provisions of our Convertible Notes, certain contractual relationships and certain Delaware law provisions could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove the current members of our Board.

We have a shareholder rights plan, the provisions of which are intended to protect our net operating loss and tax credit carryforwards and could function to deter a hostile takeover by making any proposed hostile acquisition of us more expensive and less desirable to a potential acquirer by enabling our stockholders (other than the potential hostile acquiror) to purchase significant amounts of additional shares of our common stock at dilutive prices. The rights issued pursuant to our shareholder rights plan, as amended in September 2014, become exercisable generally upon the earlier of 10 days after a person or group acquires 4.99% or more of our outstanding common stock or 10 business days after the announcement by a person or group of an intention to acquire 4.99% of our outstanding common stock via tender offer or similar transaction. The shareholder rights plan could delay or discourage transactions involving an actual or potential change in control of us or our management, including transactions in which stockholders might otherwise receive a premium for their shares over then-current prices.

Table of Contents

In addition, certain provisions in our certificate of incorporation and our by-laws may discourage, delay or prevent a change of control or takeover attempt of our company by a third-party as well as substantially impede the ability of our stockholders to benefit from a change of control or effect a change in management and our Board. These provisions include:

- the ability of our Board to increase or decrease the size of the Board without stockholder approval;
- advance notice requirements for the nomination of candidates for election to our Board and for proposals to be brought before our annual meeting of stockholders;
- the authority of our Board to designate the terms of and issue new series of preferred stock without stockholder approval;
- non-cumulative voting for directors; and
- limitations on the ability of our stockholders to call special meetings of stockholders.

As a Delaware corporation, we are subject to the provisions of Section 203 of the Delaware General Corporation Law ("Section 203"), which prevents us from engaging in any business combination with any "interested stockholder," which is defined generally as a person that acquires 15% or more of a corporation's outstanding voting stock, for a period of three years after the date of the transaction in which the person became an interested stockholder, unless the business combination is approved in the manner prescribed in Section 203. These provisions could have the effect of delaying or preventing a change of control, whether or not it is desired by, or beneficial to, our stockholders.

In addition to the above factors, an acquisition of our company could be made more difficult by employment agreements we have in place with our executive officers, as well as a company-wide change of control policy, which provide for severance benefits as well as the full acceleration of vesting of any outstanding options or restricted stock units in the event of a change of control and subsequent termination of employment. Further, our Third Amended and Restated 2007 Equity Incentive Plan generally permits our Board to provide for the acceleration of vesting of options granted under that plan in the event of certain transactions that result in a change of control.

Furthermore, holders of the Convertible Notes have the right to require us to repurchase their notes at a price equal to 100% of the principal amount thereof and the conversion rate for the Convertible Notes may be increased as described in the indenture, in each case, upon the occurrence of certain change of control transactions. Additionally, upon certain change of control transactions, the offsetting convertible bond hedge and warrant transactions that we entered into at the time we issued the Convertible Notes may be exercised and/or terminated early. Upon any such exercise and/or early termination, the proceeds we receive upon the exercise of the convertible bond hedge transactions may prove to be significantly lower than the amounts we would be required to pay upon termination of the warrant transactions. These features of the Convertible Notes and the convertible bond hedge and warrants, including the financial implications of any renegotiation of the above-mentioned provisions, could have the effect of preventing a change of control, whether or not it is desired by, or beneficial to, our stockholders, or may result in the acquisition of us being on terms less favorable to our stockholders than it would otherwise be.

ITEM 1B. UNRESOLVED STAFF COMMENTS:

None.

ITEM 2. PROPERTIES:

In June 2013, we entered into a lease agreement with BP Bay Colony LLC (the "Waltham Landlord") for the lease of certain real property located at 1100 Winter Street, Waltham,

Table of Contents

Massachusetts (the "Waltham Premises") for use as our principal executive offices. Beginning in September 2013, the initial term of the lease is five years and two months with one five-year extension term at our option. During the extension period, the base rent will be an amount agreed upon by us and the Waltham Landlord. In addition to base rent, we are also required to pay a proportionate share of the Waltham Landlord's operating costs.

The Waltham Landlord agreed to pay for certain agreed-upon improvements and we agreed to pay for any increased costs due to changes by us to the agreed-upon plans. We record all tenant improvements paid by us as leasehold improvements and amortize these improvements over the shorter of the estimated useful life of the improvement or the remaining life of the initial lease term. Amortization of leasehold improvements is included in depreciation expense.

In addition, in connection with our lease for the Waltham Premises, in June 2013 we delivered to the Waltham Landlord a security deposit of $0.4 million in the form of an irrevocable letter of credit. This security deposit will be reduced to $0.3 million on the second anniversary of the date the lease commenced. The cash securing this letter of credit is classified on our balance sheet as of December 31, 2014 as a long-term asset and is restricted in its use.

In June 2013, we also entered into an Assignment and Assumption of Lease (the "Assignment Agreement") with Shire Human Genetic Therapies, Inc. ("Shire") effecting the assignment to Shire of the right to occupy our former office space located at 100 Hayden Avenue, Lexington, Massachusetts (the "Prior Space"). Under the Assignment Agreement, the assignment to Shire became effective on September 21, 2013, the date of our departure from the Prior Space, and Shire assumed all of our obligations as the tenant of the Prior Space. The Assignment Agreement also provided for the conveyance of furniture and other personal property by us to Shire.

In connection with our acquisition of Lumara Health, we have assumed the lease of certain real property located at 16640 Chesterfield Grove Road, Chesterfield, Missouri (the "St. Louis Premises"), which we are currently using as temporary office space for Lumara Health employees as they relocate to the Waltham Premises. Beginning in September 2013, the initial term of the lease is five years and two months. In addition to base rent, we are also required to pay a proportionate share of the Chesterfield Landlord's operating costs. We are attempting to sublease the St. Louis Premises and if successful, future operating lease commitments will be partially offset by proceeds received from the sublease.

The above leases for the Waltham, Massachusetts and Chesterfield, Missouri properties requires us to pay base rent during the initial term as follows (in thousands):

Period	Minimum Lease Payments
Year Ended December 31, 2015	$ 1,451
Year Ended December 31, 2016	1,456
Year Ended December 31, 2017	1,462
Year Ended December 31, 2018	1,174
Total	$ 5,543

ITEM 3. LEGAL PROCEEDINGS:

We accrue a liability for legal contingencies when we believe that it is both probable that a liability has been incurred and that we can reasonably estimate the amount of the loss. We review these accruals and adjust them to reflect ongoing negotiations, settlements, rulings, advice of legal counsel and other relevant information. To the extent new information is obtained and our views on the probable outcomes of claims, suits, assessments, investigations or legal proceedings change, changes in

Table of Contents

our accrued liabilities would be recorded in the period in which such determination is made. For the matters referenced below, the liability is not probable or the amount cannot be reasonably estimated and, therefore, accruals have not been made. In addition, in accordance with the relevant authoritative guidance, for any matters in which the likelihood of material loss is at least reasonably possible, we will provide disclosure of the possible loss or range of loss. If a reasonable estimate cannot be made, however, we will provide disclosure to that effect.

Silverstrand Class Action

A purported class action complaint was originally filed on March 18, 2010 in the U.S. District Court for the District of Massachusetts, entitled Silverstrand Investments et. al. v. AMAG Pharm., Inc., et. al., Civil Action No. 1:10-CV-10470-NMG, and was amended on September 15, 2010 and on December 17, 2010. The second amended complaint, filed on December 17, 2010 alleged that we and our former President and Chief Executive Officer, former Chief Financial Officer, the then-members of our Board, and certain underwriters in our January 2010 offering of common stock violated certain federal securities laws, specifically Sections 11 and 12(a)(2) of the Securities Act of 1933, as amended, and that our former President and Chief Executive Officer and former Chief Financial Officer violated Section 15 of such Act, respectively, by making certain alleged omissions in a registration statement filed in January 2010. The plaintiffs sought unspecified damages on behalf of a purported class of purchasers of our common stock pursuant to our common stock offering on or about January 21, 2010. After litigating the class action lawsuit for several years, on September 12, 2014, we and the other defendants entered into a stipulation of settlement with the lead plaintiffs (on behalf of themselves and each of the class members) to resolve the class action securities lawsuit. Pursuant to the stipulation of settlement, and in exchange for a release of all claims by the class members and certain other persons, and dismissal of the lawsuit with prejudice, we agreed to cause our insurer to pay eligible class members and their attorneys a total of $3.75 million. On October 2, 2014, the U.S. District Court preliminarily approved the settlement, and potential class members were notified of the proposed settlement and the procedures by which they could seek to recover from the settlement fund, object to the settlement or request to be excluded from the settlement class and on January 30, 2015, the stipulation of settlement was approved by the U.S. District Court. The U.S. District Court entered final judgment on February 2, 2015. Any appeals of the settlement are due by March 4, 2015. We have recorded the $3.75 million settlement amount in prepaid and other current assets and a corresponding amount in accrued expenses on our consolidated balance sheet as of December 31, 2014, as the settlement amount will be fully covered by our insurance carrier. There was no impact to our consolidated statement of operations for the year ended December 31, 2014.

Makena Securities Litigation

On October 19, 2011, plaintiff Frank Julianello filed a complaint against Lumara Health (then-named K-V Pharmaceutical Company ("K-V Pharmaceutical")) and certain individual defendants, in the United States District Court for the Eastern District of Missouri (the "Court"), alleging violations of the anti-fraud provisions of the federal securities laws on behalf of all purchasers of the publicly traded securities of Lumara Health between February 14, 2011 and April 4, 2011. The complaint alleges class members were damaged by paying artificially inflated stock prices due to Lumara Health's purportedly misleading statements regarding *Makena* related to access and exclusivity. On October 31, 2011, plaintiff Ramakrishna Mukku filed a complaint against Lumara Health, in the United States District Court for the Eastern District of Missouri, alleging violations of the anti-fraud provisions of the federal securities laws on behalf of all purchasers of the publicly traded securities of Lumara Health between February 14, 2011 and April 4, 2011. The complaint alleges class members were damaged by paying artificially inflated stock prices due to Lumara Health's purportedly misleading statements regarding *Makena* related to access and exclusivity. On November 2, 2011, plaintiff Hoichi Cheong filed a complaint against Lumara Health, in the United States District Court for the Eastern District of

Table of Contents

Missouri, on behalf of purchasers of the securities of Lumara Health, who purchased or otherwise acquired K-V Pharmaceutical securities between February 14, 2011 and April 4, 2011, seeking to pursue remedies under the Exchange Act. The complaint alleges class members were damaged by purchasing artificially inflated stock prices due to Lumara Health's purportedly misleading statements regarding *Makena* related to access and exclusivity. On March 8, 2012, the Julianello, Mukku and Cheong cases were consolidated and the consolidated action is now styled In Re K-V Pharmaceutical Company Securities Litigation, Case No. 4:11-CV-1816. On May 4, 2012, the Court appointed Lori Anderson as Lead Plaintiff in the matter. On April 22, 2013, the individual defendants moved to dismiss the complaint and oral argument was held before the Court on November 26, 2013. Lumara Health joined in the motion to dismiss on February 10, 2014. On March 27, 2014, the Court entered an order granting Lumara Health's motion to dismiss the class action complaint without prejudice to the Plaintiffs' ability to file a second amended complaint with respect to a limited issue of whether Lumara Health's statements about Lumara Health's financial assistance program for *Makena* were materially false or misleading. On April 16, 2014, the Plaintiff's filed a motion to reconsider asking the Court to reconsider its order restricting the scope of Plaintiff's ability to amend its complaint. The Court denied Plaintiff's motion to reconsider and entered a judgment granting Lumara Health's motion to dismiss on June 6, 2014. On July 1, 2014, Plaintiffs filed a Notice of Appeal with the Eighth Circuit Court of Appeals and briefs have been submitted to the Court. The Court of Appeals has set March 12, 2015 as the date for oral argument.

European Patent Organization Appeal

In July 2010, Sandoz GmbH ("Sandoz") filed with the European Patent Office ("EPO") an opposition to a previously issued patent which covers ferumoxytol in EU jurisdictions. In October 2012, at an oral hearing, the Opposition Division of the EPO revoked this patent. In December 2012, our notice of appeal of that decision was recorded with the EPO, which also suspended the revocation of our patent. On May 13, 2013, we filed a statement of grounds of appeal and on September 27, 2013, Sandoz filed a response to that statement. In the event that we withdraw our appeal or we do not experience a successful outcome from the appeals process, under EU regulations ferumoxytol would still be entitled to eight years of data protection and ten years of market exclusivity from the date of approval, which we believe would create barriers to entry for any generic version of ferumoxytol into the EU market until sometime between 2020 and 2022. This decision had no impact on our revenues for the year ended December 31, 2014. However, any future unfavorable outcome in this matter could negatively affect the magnitude and timing of future revenues. We do not expect to incur any related liability regardless of the outcome of the appeal and therefore have not recorded any liability as of December 31, 2014. We continue to believe the patent is valid and intend to vigorously appeal the decision.

We may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which we are focused. Other than the above actions, we are not aware of any material claims against us as of December 31, 2014.

ITEM 4. MINE SAFETY DISCLOSURES

Not Applicable.

Table of Contents

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES:

Market Information

Our common stock trades on the NASDAQ Global Select Market ("NASDAQ") under the trading symbol "AMAG." On February 4, 2015, the closing price of our common stock, as reported on the NASDAQ, was $41.50 per share. The following table sets forth, for the periods indicated, the high and low sale prices per share for our common stock as reported on the NASDAQ.