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Registration No. 333-197008

Prospectus

2,017,349 shares

Common Stock

Ultragenyx Pharmaceutical Inc. is offering 1,311,277 shares of its common stock and the selling stockholders identified in this prospectus are offering 706,072 shares of our common stock. We will not receive any proceeds from the sale of any shares by the selling stockholders.

Our common stock is listed on The NASDAQ Global Select Market under the symbol "RARE". The last reported sale price of our common stock on The NASDAQ Global Select Market on July 8, 2014 was $41.40 per share.

We are an "emerging growth company," as that term is used in the Jumpstart Our Business Startups Act of 2012, and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 15.

	Per share	Total
Public offering price	$ 40.00	$80,693,960
Underwriting discounts and commissions(1)	$ 2.40	$ 4,841,638
Proceeds to Ultragenyx Pharmaceutical Inc., before expenses	$ 37.60	$49,304,015
Proceeds to selling stockholders	$ 37.60	$26,548,307

(1) See "Underwriting" for additional disclosure regarding underwriting discounts, commissions and estimated offering expenses.

We have granted the underwriters an option for a period of 30 days to purchase up to an additional 302,602 shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to purchasers on or about July 14, 2014.

J.P. Morgan **Morgan Stanley**

Cowen and Company

Baird

July 8, 2014

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Neither we nor the selling stockholders have authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We and the selling stockholders take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We and the selling stockholders are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock. Our business, financial condition, results of operations, and prospects may have changed since that date.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

PROSPECTUS SUMMARY

The items in the following summary are described in more detail later in this prospectus. This summary provides an overview of selected information and does not contain all of the information you should consider before buying our common stock. Therefore, you should read the entire prospectus carefully, including the information in our filings with the Securities and Exchange Commission, or SEC, incorporated by reference in this prospectus, before deciding to invest in our common stock. Investors should carefully consider the information set forth under "Risk Factors" beginning on page 15 of this prospectus and those identified in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014. In this prospectus, unless the context otherwise requires, references to "the Company," "we," "us," "our," or "Ultragenyx" refer to Ultragenyx Pharmaceutical Inc.

Overview

We are a development-stage biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating genetic diseases. We focus on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies. Since our inception in 2010, we have in-licensed potential treatments for five different diseases that are currently in or have completed Phase 1/2 or Phase 2 clinical studies. Our strategy, which is predicated upon time- and cost-efficient drug development, allows us to pursue multiple programs in parallel with the goal of delivering safe and effective therapies to patients with the utmost urgency.

Our current product candidate pipeline has been either in-licensed from academic institutions or derived from partnerships with other pharmaceutical companies. Our strategy is to acquire and retain global commercialization rights to our products to maximize long-term value, where possible. Over time, we intend to build our own commercial organization, which we believe will be of modest size due to the relatively small number of specialists who treat patients with rare and ultra-rare diseases.

The patients we seek to treat have diseases with limited or no treatment options, and we recognize that their lives and well-being are highly dependent upon our efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care. We strive to build a company that is faster, better, and smarter about advancing multiple product candidates through approval.

We were founded in April 2010 by our current President and Chief Executive Officer, Dr. Emil Kakkis, M.D., Ph.D. We have assembled an experienced team with extensive rare disease drug development and commercialization capabilities. Dr. Kakkis and the team at Ultragenyx have been previously involved at other companies in the development and/or commercialization of many therapies approved or in development for rare metabolic genetic diseases, including Aldurazyme, Naglazyme, Kuvan, and Vimizim (BioMarin); Lumizyme/Myozyme (Sanofi-Genzyme); and asfotase alpha (Enobia; now Alexion).

Our Strategy

Our strategy is to identify, acquire, develop, and commercialize novel products for the treatment of rare and ultra-rare diseases in the United States, the European Union, and select international markets, with the goal of becoming a leading rare disease company. The critical components of our business strategy include the following:

- Focus on rare and ultra-rare diseases with significant unmet medical need;
- Focus on diseases and therapies with clear mechanisms of action;
- Leverage our experience and relationships to in-license promising product candidates;
- Develop and commercialize multiple product candidates in parallel;

- Focus on excellent and rapid clinical and regulatory execution; and
- Seek to retain global commercialization rights to product candidates.

Product Candidates

Our current pipeline consists of two product categories: biologics, including a monoclonal antibody and enzyme replacement therapies; and small-molecule substrate replacement therapies. Enzymes are proteins that the body uses to process materials needed for normal cellular function, and substrates are the materials upon which enzymes act. When enzymes or substrates are missing, the body is unable to perform its normal cellular functions, often leading to significant clinical disease. Several of our therapies are intended to replace deficient enzymes or substrates and one product candidate is an antibody that decreases the activity of an elevated hormone.

The following table summarizes our product candidate pipeline:

Candidate	Description	Indication	Pre-clinical	Phase 1	Phase 1/2 or Phase 2	Phase 3 or pivotal	Status / Anticipated milestones	Ultragenyx commercial rights
Biologics								
KRN23 (UX023)	Anti-FGF23 monoclonal antibody	XLH					▪ Expect interim data from pediatric Phase 2 clinical study in 2015	▪ U.S. and Canada: Joint with KHK (profit share) ▪ Mexico, Central and South America
rhGUS (UX003)	Enzyme replacement	MPS 7					▪ Expect interim data from Phase 1/2 clinical study in the second half of 2014	▪ Worldwide
rhPPCA (UX004)	Enzyme replacement	Galactosialidosis					▪ Expect to continue preclinical development during 2014	▪ Worldwide
Substrate replacement therapies								
Triheptanoin (UX007)	Substrate replacement	LC-FAOD					▪ Expect interim data from Phase 2 clinical study in 2015	▪ Worldwide
Triheptanoin (UX007)	Substrate replacement	Glut1 DS					▪ Expect interim data from Phase 2 clinical study in 2015	▪ Worldwide
SA-ER (UX001)	Substrate replacement	HIBM					▪ Expect data from ongoing Phase 2 extension study in late 2014	▪ Worldwide (excluding Japan and certain other Asian territories)

KRN23 (UX023) for the treatment of XLH

KRN23 is a fully human monoclonal antibody administered via subcutaneous injection that is designed to bind and reduce the biological activity of FGF23 to increase abnormally low phosphate levels in patients with X-linked hypophosphatemia, or XLH. Patients with XLH have low serum phosphate levels due to excessive phosphate loss into the urine, which is directly caused by the effect on kidney function of excess FGF23 production in bone cells. Low phosphate levels lead to poor bone mineralization and a variety of clinical manifestations, including rickets leading to bowing and other skeletal deformities, short stature, bone pain and fractures, poor quality bone, and muscle weakness. There is no approved drug therapy or treatment for the underlying cause of XLH. Most patients are managed using frequently divided doses of oral phosphate and vitamin D therapy, which has significant side effects. Oral phosphate/vitamin D replacement therapy requires extremely close monitoring due to the potential for excessive phosphate levels and secondary increases in calcium, which can result in severe damage to the kidneys from excess calcium phosphate deposits and other complications. Additionally, some patients are unable to tolerate the regimen due to the chalky stool that results from taking large amounts of oral phosphate or the high frequency of dosing required.

In August 2013, we formed a collaboration with Kyowa Hakko Kirin Co., Ltd., or KHK, to jointly develop and commercialize KRN23 for the treatment of XLH. KHK has conducted one Phase 1 study, one Phase 1/2 study, and one longer-term Phase 1/2 study of KRN23 in adults with XLH. We reviewed safety and efficacy data from the Phase 1 and Phase 1/2 studies prior to entering into our collaboration with KHK, and we entered into the collaboration based in part upon our conclusion that these data were supportive of further development (serum phosphate, renal tubular reabsorption of phosphate, and vitamin D levels were increased, and the product appeared well tolerated).

Results from the Phase 1 single dose study in 38 adult XLH patients were presented at the American Society for Bone and Mineral Research Annual Meeting in October 2013 and published in the Journal of Clinical Investigation in February 2014. The data demonstrated that KRN23 was well tolerated and increased serum phosphate, or phosphorus, as well as vitamin D levels. Of the 38 adult XLH patients, 12 received a single subcutaneous injection of KRN23 (at doses of 0.1, 0.3, 0.6, or 1.0 mg/kg), 17 received a single intravenous injection of KRN23 (at doses of 0.003, 0.01, 0.03, 0.1, or 0.3 mg/kg) and 9 received placebo. The effect of KRN23 on the increase in serum phosphate levels was comparable between intravenous and subcutaneous administration; however, time to reach peak effect was slower and duration of effect was greater with subcutaneous administration compared with intravenous administration. The demonstrated improvement in serum phosphate levels suggests that significant benefit could be expected. Corresponding changes were observed in renal tubular reabsorption of phosphate. Increases in vitamin D were also observed, suggesting improved intestinal absorption of both phosphate and calcium. Changes were not observed in serum calcium.

No serious adverse events were reported in the Phase 1 study. Approximately 83% of the subjects experienced at least one non-serious treatment-emergent adverse event, the most common of which were nausea and headache; no patients in the placebo or subcutaneous treatment arms reported these events. In the subcutaneous arm, two patients (approximately 17%) experienced elevated levels of the enzyme amylase in the blood, and two other patients (approximately 17%) experienced back pain. The elevations of the enzyme amylase were modest and not clinically significant. There did not appear to be a relationship between the incidence and types of adverse events and the dose administered following a single dose of study drug.

Results from a multiple-dose Phase 1/2 study with up to four escalating doses from 0.05 mg/kg to 0.6 mg/kg that was completed in 28 adult XLH patients were presented at the 2014 ICE/ENDO joint meeting of The Endocrine Society and the International Congress on Endocrinology in June 2014. The data demonstrated that repeat doses of KRN23 over four months led to an increase in serum phosphate in 100% of patients, with approximately 89% of patients reaching the low end of the normal range. Peak mean serum phosphorus increased to 3.03 ± 0.42 mg/dL after the fourth dose, which is an approximately 60% increase from the mean of 1.89 ± 0.33 mg/dL at baseline. Comparable increases were observed in mean reabsorption of phosphate from the urine (TmP/GFR) and mean serum 1,25 dihydroxy vitamin D levels.

Increases in bone remodeling markers of bone formation and bone resorption were also observed in this Phase 1/2 study. The increase in P1NP (procollagen type I N propeptide) from baseline was statistically significant ($p<0.05$) after all doses and the increase in osteocalcin was statistically significant ($p<0.05$) after the fourth dose. These data support the concept that KRN23's impact on improving phosphate metabolism will improve bone remodeling, a critical part of creating strong, properly-formed bones. Increases in quality of life and disability measures were also observed and we intend to objectively evaluate these in a future randomized controlled study.

There were no significant changes in parathyroid hormone, serum calcium, or urinary calcium excretion in this Phase 1/2 study, consistent with the Phase 1 data showing that KRN23 can specifically improve phosphate control without interfering with calcium control. The most common adverse events were nasopharyngitis, joint pain, diarrhea, back pain, and restless leg syndrome. Joint pain and back pain are both known symptoms of XLH in adults. There were no serious adverse events related to treatment or renal or cardiac tissue calcification. One patient discontinued treatment due to an injection site reaction. No anti-KRN23 antibodies were observed.

Data from the long-term Phase 1/2 study to evaluate KRN23 treatment for an additional 12 doses is expected to be presented at the American Society for Bone and Mineral Research (ASBMR) Annual Meeting in September 2014.

In July 2014, we announced the first patient screened and enrolled in the Phase 2 pediatric study of KRN23 in patients with XLH. We expect to enroll approximately 30 prepubertal patients in this study. The primary objectives of the study are to identify a dose and dosing regimen and to establish the safety profile of treatment with KRN23 in pediatric XLH patients. We will also assess preliminary clinical effects of KRN23 treatment on bone health and deformity as measured by radiographic assessments, growth, muscle strength, and motor function, as well as markers of bone health and patient-reported outcomes of pain, disability, and quality of life. The study has been evaluated and accepted for conduct by the United States Food and Drug Administration, or FDA, the United Kingdom Medicinal and Health Regulatory Authority, and the Dutch Medicines Evaluation Board.

The study will consist of a 16-week individual dose-titration period followed by a 48-week treatment period. The goal of the dose-titration period is to identify the individualized dose of KRN23 required to achieve stable serum phosphorus levels in the target range. Patients will be divided into three cohorts of escalating starting dose levels of KRN23 with either monthly or biweekly dosing regimens. At the end of the 16-week dose-titration period, patients will receive their individually-optimized dose of KRN23 on a monthly or biweekly basis for the 48-week treatment period. An interim analysis of safety and pharmacodynamic data will be conducted after 24 weeks of the treatment period.

Depending on the results of our Phase 2 pediatric study, we intend to conduct a Phase 3 pediatric trial. In our recent meeting with the FDA, the FDA agreed that blinded radiographic assessments of changes in bone abnormalities, i.e. rickets and bowing, and changes in growth may be used as primary endpoint measures in the pediatric development program and a potential Phase 3 study. The FDA also indicated that a Phase 3 study in pediatric patients could be open-label, but recommended inclusion of a standard of care control arm for comparison on a non-inferiority basis. We expect that the final design of a pediatric Phase 3 study would be determined once sufficient safety and efficacy data are available and after further consultation with the FDA.

Given the high turnover and growth of bone during childhood and the critical role phosphate plays in bone growth, pediatric XLH patients have the highest morbidity and potential for benefit in a shorter timeframe. As a result, pediatric XLH patients may also have the greatest potential for improvement based on third-party data regarding enzyme replacement therapy in hypophosphatasia, which is another genetic bone disease with poor bone mineralization related to phosphate metabolism caused by a different, unrelated mechanism. We also expect to continue to develop KRN23 in adults with XLH and plan to conduct an adult Phase 2b study in parallel with our Phase 3 pediatric trial.

Potential market opportunity

Based on incidence and prevalence rates published in a Danish epidemiologic study and surveys of physicians in the United States, we estimate that there are approximately 3,000 cases of XLH in pediatric patients in the United States. Further, there are an estimated 9,000 cases of XLH in adult patients in the United States. However, we expect that many of these adult patients may not seek treatment if their bone disease is not too severe.

rhGUS (UX003) for the treatment of MPS 7

Recombinant human beta-glucuronidase, or rhGUS, is an intravenous, or IV, enzyme replacement therapy for the treatment of mucopolysaccharidosis 7, or MPS 7, also known as Sly Syndrome. Patients with MPS 7 suffer from severe cellular and organ dysfunction that typically leads to death in the teens or early adulthood. MPS 7 is caused by a deficiency of the lysosomal enzyme beta-glucuronidase, which is required for the breakdown of certain complex carbohydrates known as glycosaminoglycans, or GAGs. The inability to properly

break down GAGs leads to their accumulation in many tissues, resulting in a serious multi-system disease. Patients with MPS 7 may have abnormal coarsened facial features, enlargement of the liver and spleen, airway obstruction, lung disease, cardiovascular complications, joint stiffness, short stature, and a skeletal disease known as dysostosis multiplex. In addition, many patients experience progressive lung problems as a result of airway obstruction and mucous production, often leading to sleep apnea and pulmonary insufficiency, and eventually requiring tracheostomy. There are currently no approved drug therapies for MPS 7.

We licensed exclusive worldwide rights to rhGUS-related know-how and cell lines from Saint Louis University in November 2010 and initiated development in 2012. We have conducted preclinical studies to support the chronic IV administration of rhGUS. Administration of rhGUS resulted in substantial distribution of enzyme, as well as reduction in tissue pathology in a wide variety of tissues, including the liver, spleen, lung, heart, kidney, muscle, bone, and brain. No adverse toxicology related to rhGUS was noted in these studies.

In December 2013, we initiated an open-label, Phase 1/2 study in the United Kingdom to evaluate the safety, tolerability, efficacy, and dose of IV administration every other week of rhGUS in up to five patients with MPS 7 who are between five and 30 years of age. The initial 12-week treatment period will be followed by a dose-titration period and a long-term extension study. We expect to release interim data from this study during 2014.

Preliminary data from the Phase 1/2 study were presented at the American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting in March 2014. Results from three patients who had been administered 2 mg/kg of rhGUS every other week for two, six, and 12 weeks showed evidence of clearance of lysosomal storage as indicated by the decrease in urinary GAG excretion beginning at two weeks of treatment of approximately 30-50%. At the 12 week assessment of the first patient, absolute liver size was reduced by approximately 11%. This represents a 46% decrease in the excess liver size above normal for age and gender. The remaining patients have not yet reached the 12 week time point for liver size assessment. No serious adverse events were observed during up to 12 weeks of treatment, and no infusion-associated reactions were observed after a total of 13 infusions to date in these three subjects. The Phase 1/2 study will continue, and additional 12-week interim data are expected in the second half of 2014. If these results are supportive, we plan to initiate a pivotal Phase 3 study in 2014.

We are also supplying rhGUS to an investigator who is treating a single U.S. patient under an emergency investigational new drug, or eIND, application. Preliminary results from the treatment of this patient were presented at the Lysosomal Disease Network's 10th Annual World Symposium in February 2014. Preliminary data showed evidence of a reduction in lysosomal storage based on reduced excretion of urinary GAG of 50% and a reduction in the size of the enlarged liver and spleen. The patient showed an improvement of pulmonary function and no infusion-associated reactions during the first 14 weeks of treatment. The patient's caregivers also reported the patient's improved stamina and increased time spent in school.

The European Medicines Agency, or EMA, has agreed that approval under exceptional circumstances could be possible for a proposed 12-patient placebo-controlled pivotal study in this disease with urinary GAG levels as a surrogate primary endpoint provided the data was strongly supportive of a favorable benefit/risk ratio. The EMA requested that some evidence of trend in improvement in clinical endpoints be observed to support the primary endpoint, but recognized that a statistically significant result on clinical endpoints was unlikely given the small number of patients expected to be enrolled in the study. The FDA has not yet agreed to the pivotal study plan or study design and primary endpoint and would like to see additional data correlating urinary GAG levels with other clinical endpoints, which we are collecting and will submit to the FDA. We expect to meet with the FDA to discuss the Phase 3 plan, although our plan is currently based on our agreement with the EMA.

In addition to the above development plan, we intend to study MPS 7 patients under the age of five years, including potentially young infants born with hydrops fetalis. Currently, these infants often die within a few months to one year, but enzyme replacement therapy might be able to reduce GAG storage and improve health

and survival in these patients. This program would not start until we had obtained sufficient information from the Phase 1/2 study to support the initiation of a trial in younger patients.

rhPPCA (UX004) for the treatment of galactosialidosis

Recombinant human protective protein cathepsin-A, or rhPPCA, which was in-licensed from St. Jude Children's Research Hospital in September 2012, is in preclinical development as an enzyme replacement therapy for galactosialidosis, a rare lysosomal storage disease for which there are currently no approved drug therapies. Similar to MPS patients, patients with galactosialidosis present with both soft tissue storage in the liver, spleen, and other tissues, as well as connective tissue (bone and cartilage) related disease. As with MPS 7, an enzyme deficiency results in accumulation of substrates in the lysosomes, causing skeletal and organ dysfunction, and death. We plan to continue preclinical development of rhPPCA during 2014.

Triheptanoin (UX007) for the treatment of LC-FAOD

We are developing triheptanoin for oral administration intended as a substrate replacement therapy for patients with long-chain fatty acid oxidation disorders, or LC-FAOD. Triheptanoin is a medium odd-chain triglyceride of three seven-carbon fatty acids designed to provide substrate replacement for fatty acid metabolism and restore production of energy. Patients with LC-FAOD have a deficiency that impairs the ability to produce energy from fat, which can lead to depletion of glucose in the body, and severe liver, muscle, and heart disease, as well as death. There are currently no approved drugs or treatments specifically for LC-FAOD. The current standard of care for LC-FAOD includes diligent prevention of fasting combined with the use of low-fat/high-carbohydrate diets, carnitine supplementation in some cases, and medium even-chain triglyceride oil supplementation. Despite treatment with the current standard of care, many patients continue to suffer significant morbidity and mortality.

We licensed certain intellectual property rights for triheptanoin from Baylor Research Institute in August 2012. Triheptanoin has been studied clinically for 13 years in approximately 130 human subjects affected by a variety of diseases, including more than 60 patients with LC-FAOD. Multiple investigator-sponsored open-label studies suggest clinical improvements with triheptanoin treatment, even for patients who were on standard of care. We recently presented a retrospective medical record review study assessing the clinical outcome of triheptanoin treatment on LC-FAOD subjects who have been participating in a compassionate use program at the University of Pittsburgh Medical Center. The data showed that treatment with triheptanoin appeared to reduce the frequency and severity of hospitalizations previously experienced by these patients for disease-related causes, including muscle rupture, hypoglycemia, and cardiomyopathy. Among a number of results suggesting significant improvement after crossing over onto triheptanoin treatment, a reduction in mean total hospital days per year from 17.55 to 5.40 (69%; $p = 0.0242$) was observed after transitioning from standard of care to triheptanoin therapy. These results are clinically important but are derived from a retrospective medical review, and not from a randomized controlled study. The preliminary results of our retrospective medical review are as follows:

Description	Pre-treatment	Post-treatment	% decrease	n	p-value
Mean total hospitalizations/year[1]	1.94	1.26	36%	16	0.1126
Mean total hospital days/year[1],[2]	17.55	5.4	69%	15	0.0242
Mean infant total hospitalizations/year[3]	13.01	1.37	89%	4	0.0892
Mean hypoglycemia events/year[1],[4]	0.92	0.04	96%	9	0.0091
Mean hypoglycemia total hospital days/year[1],[2],[4]	8.42	0.18	98%	9	0.0257
Mean rhabdomyolysis events/year[1],[5]	1.05	0.68	35%	11	0.4604
Mean rhabdomyolysis total hospital days/year[1],[5]	5.94	2.16	64%	9	0.1224
Mean peak creatine kinase (units) for rhabdomyolysis events[1],[5]	85,855	25,797	68%	7	0.1279

(1) Excludes data for four infants dosed within first six months of life.

(2) Excludes hospitalizations with unknown discharge dates.

(3) Four infants were dosed within the first six months of life.

(4) Includes only those patients with hypoglycemia events prior to treatment.

(5) Includes only those patients with rhabdomyolysis events prior to treatment.

Triheptanoin is currently being evaluated in a prospective international open-label Phase 2 study in approximately 30 severely affected LC-FAOD patients. The principal goals of the study are to determine the appropriate clinical endpoints and patient population for testing in potential later-stage pivotal studies. The study will evaluate patients, ages six months to 35 years, exhibiting significant clinical manifestations of LC-FAOD despite current therapy. Prior to initiating treatment with triheptanoin, subjects will continue current therapy for four weeks to establish their baseline condition. Triheptanoin will then be titrated to an expected target dose of 25-35% of total daily caloric intake via oral administration, while ensuring tolerability. The study will assess the impact of triheptanoin on several endpoints, including cycle ergometer performance, 12-minute walk test, muscle strength, creatine kinase levels, hypoglycemia, liver size, cardiac disease, and major medical events. The patients will be followed to evaluate the effects of triheptanoin treatment on acute clinical pathophysiology associated with LC-FAOD over 24 weeks, then may continue treatment for an additional 54 weeks for observation of major medical events. We expect data from this study to be available in 2015.

Potential market opportunity

Based upon data from the National Newborn Screening Information System, we estimate that there are approximately 2,000 to 3,500 LC-FAOD patients in the United States, depending on the assumed mortality rate.

It is unclear how many of these patients are currently diagnosed because the availability of newborn screening in all 50 states in the United States is a relatively new development. Furthermore, until further clinical development of triheptanoin is conducted, it is not clear which subsets of diagnosed patients would be considered by clinicians to be good candidates for triheptanoin treatment. Outside of the United States, where newborn screening is not consistently done, figures regarding the prevalence of LC-FAOD are more uncertain.

Triheptanoin (UX007) for the treatment of Glut1 DS

We are also developing triheptanoin for patients with glucose transporter type 1 deficiency syndrome, or Glut1 DS. Glut1 DS is caused by a mutation affecting the gene for Glut1, a protein that transports glucose from the blood into the brain. Because glucose is the primary source of energy for the brain, Glut1 DS results in a chronic state of brain energy deficiency and is characterized by seizures, developmental delay, and movement disorder. There are currently no approved drugs specific to Glut1 DS. The current standard of care for Glut1 DS is the ketogenic diet, an extreme high-fat (70-80% of daily calories as fat)/low-carbohydrate diet, which generates ketone bodies as an alternative energy source to glucose, and one or more antiepileptic drugs. The ketogenic diet can be effective in reducing seizures but compliance can be difficult, and the diet has demonstrated limited effectiveness in the treatment of developmental delay and movement disorders. In addition, ketogenic diet can lead to side effects including renal stones. In general, Glut1 DS patients are considered relatively refractory to antiepileptic drugs with only approximately 8% achieving seizure control on antiepileptic drugs alone. There are currently no antiepileptic drugs approved specifically for patients with Glut1 DS.

Triheptanoin is intended as a substrate replacement therapy to provide an alternative source of energy to the brain in Glut1 DS patients. Although there are open-label investigator-sponsored clinical studies ongoing and the results have not yet been reported publicly, there are anecdotal reports of benefit in terms of reduced seizures and improved developmental function in some Glut1 DS subjects taking triheptanoin. In March 2014, we initiated a Phase 2 global, randomized, double-blind, placebo-controlled, parallel-group study that may enroll up to 50 patients who are currently not fully compliant with ketogenic diet and continue to have seizures. The primary efficacy objective is the reduction in frequency of seizures compared to placebo following a six-week baseline period and subsequent eight-week placebo-controlled treatment period. The blinded treatment period will be followed by an open-label extension period in which patients will be treated with triheptanoin through week 52. Patient enrollment may be modified based on an interim analysis. Assuming timely patient enrollment in this study, we expect to release data from this trial in 2015.

We also continue to support investigator-sponsored trials studying triheptanoin across multiple indications.

Potential market opportunity

While a comprehensive genetic analysis of birth incidence has not been conducted, published literature suggests a range of 3,000 to 7,000 Glut1 DS patients in the United States based on evaluations of generalized or absence seizures. The increasing recognition of alternative or variable motor forms of the disease suggests that older patients may be discovered over time. Given that the disease can be inherited as an autosomal dominant disease, the discovery of one patient may be used to identify other affected relatives in some cases, which can be important in marketing of the product.

SA-ER (UX001) for the treatment of HIBM

We are developing an extended-release, oral formulation of sialic acid, or SA-ER, for the treatment of hereditary inclusion body myopathy, or HIBM, which is also known as GNE myopathy. HIBM is characterized by severe progressive muscular myopathy, or disease in which muscle fibers do not function properly, with onset typically in the late teens or twenties. Patients with HIBM have a genetic defect in the gene coding for a particular enzyme that is involved in the first step in the biosynthesis of sialic acid. Therefore, HIBM patients

have a sialic acid deficiency, which interferes with muscle function, leading to myopathy and atrophy. Patients typically lose a substantial amount of muscle function within ten to 20 years of diagnosis. There is no approved drug therapy for HIBM.

We are studying SA-ER as a potential substrate replacement therapy designed to address sialic acid deficiency and restore muscle function in HIBM patients. We have conducted a Phase 2 randomized, double-blind, placebo-controlled study of SA-ER in 47 HIBM patients. Data from this study were presented at the American Academy of Neurology Annual Meeting in April 2014. Patients in the study were initially randomized to receive placebo, three grams, or six grams of SA-ER per day. After 24 weeks, placebo patients crossed over to either three grams or six grams total daily dose, on a blinded basis, for an additional 24 weeks. The final analysis compared change at week 48 from baseline for the combined groups at six grams versus three grams of SA-ER. Assessments included pharmacokinetics, composites of upper extremity and lower extremity muscle strength as measured by dynamometry, other clinical endpoints, patient reported outcomes, and safety.

At 24 weeks, assessments of upper extremity composite of muscle strength showed a statistically significant difference in the six gram group compared to placebo (+2.33 kg; 5.5% relative difference from baseline; p=0.040). At 48 weeks, a statistically significant difference between the combined six gram group and the combined three gram group was observed (+3.44 kg; 8.5% relative difference from baseline; p=0.0033). Patients with less advanced disease (able to walk more than 200 meters at baseline), a predefined subset, showed a more pronounced difference (+4.69 kg; 9.6% relative difference from baseline; p=0.00055). The lower extremity composite showed a similar pattern of response but did not show a statistically significant difference between the dose groups. None of the groups showed a significant decline in the lower extremity composite during the treatment period. A positive trend was seen in patient-reported outcomes of functional activity consistent with the potential clinical meaningfulness of the muscle strength assessment. SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group, and no dose-dependent treatment-emergent adverse events were identified. Most adverse events were mild to moderate and most commonly were gastrointestinal in nature and pain related to muscle biopsy procedures.

We continue to treat these patients in an extension study evaluating an increased daily dosage of 12 grams of sialic acid based on the dose dependence observed at weeks 24 and 48. We anticipate that data from the extension study should be available in late 2014. We also plan to discuss data from this program with regulatory authorities during 2014 with the objective of agreeing on the design and primary endpoint for a pivotal study.

Potential market opportunity

Approximately 400 HIBM cases have been reported in the published literature. HIBM is expected to occur in one in every 1,600 persons of Persian Jewish descent. Patients have also been identified in Asian Indian, European, Chinese, Japanese, Korean, and Middle Eastern populations. To better understand the patient population, we conducted an initial survey of 420 myopathy clinics in the United States, and the extrapolated results suggest a patient population of 300 to 400 in the United States and 1,200 to 2,000 worldwide.

Risks Associated with Our Business

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled “Risk Factors” immediately following this prospectus summary and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014, incorporated by reference herein. These risks include, among others:

- We are a development-stage company and have a limited operating history on which to assess our business, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future;

- We are heavily dependent upon the success of our product candidates, which are in the early stages of clinical development, and we cannot provide any assurance that any of our product candidates will receive regulatory approval;
- Because the target patient populations of our product candidates are small, and the addressable patient populations potentially even smaller, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth;
- Even if this offering is successful, we expect that we will need to raise additional funding before we can expect to become profitable from sales of our products;
- The insurance coverage and reimbursement status of newly-approved products is uncertain and failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue;
- If we are unable to obtain and maintain effective patent rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets; and
- Our future success depends in part upon our ability to retain our Founder, President, and Chief Executive Officer and to attract, retain, and motivate other qualified personnel.

Our Corporate Information

We were founded in April 2010 as a California corporation, and we reincorporated as a Delaware corporation in June 2011. Our principal executive offices are located at 60 Leveroni Court, Novato, CA 94949, and our telephone number is (415) 483-8800. Our web site address is *www.ultragenyx.com*. The information on, or that can be accessed through, our web site is not part of this prospectus. We have included our web site address as an inactive textual reference only.

We have filed trademark applications with the U.S. Patent and Trademark Office for the marks Ultragenyx™ and Ultragenyx Pharmaceutical™, and we are developing commercial names for our product candidates. This prospectus, and the information incorporated by reference herein, also contains trademarks of others, including Aldurazyme®, Naglazyme®, Kuvan®, Vimizim™, Lumizyme®, Myozyme® and asfotase alpha. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

THE OFFERING

Common stock offered by us	1,311,277 shares
Common stock offered by the selling stockholders	706,072 shares
Common stock to be outstanding after this offering	31,360,927 shares (31,663,529 shares if the underwriters exercise their option to purchase additional shares in full)
Underwriters' option to purchase additional shares	We have granted the underwriters a 30-day option to purchase up to an additional 302,602 shares of our common stock.
Use of proceeds	We estimate that the net proceeds to us from this offering will be approximately $48.8 million, or approximately $60.2 million if the underwriters exercise their option to purchase additional shares in full, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, together with our existing cash, cash equivalents, and short-term investments to continue to advance our clinical and preclinical pipeline, including potential new formulations of or indications for our existing product candidates or potential new product candidates, and to increase investment in early-stage research capabilities and general infrastructure, with any remaining proceeds to be used for other ongoing research and development, working capital and other general corporate purposes. We may also use a portion of the net proceeds to us to in-license, acquire, or invest in additional businesses, technologies, products, or assets, though we currently have no specific agreements, commitments, or understandings with respect to any in-licensing or acquisitions. See "Use of Proceeds." We will not receive any proceeds from the sale of any shares by the selling stockholders.
Risk factors	You should read the "Risk Factors" section of this prospectus and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014, incorporated by reference herein, for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
NASDAQ Global Select Market symbol	"RARE"

The number of shares of common stock to be outstanding after this offering is based on 30,049,650 shares of common stock outstanding as of March 31, 2014.

The number of shares of our common stock to be outstanding after this offering excludes the following:

- 2,396,323 shares of common stock issuable upon the exercise of outstanding stock options as of March 31, 2014 having a weighted-average exercise price of $6.35 per share;
- 353,459 shares of common stock issuable upon the exercise of outstanding warrants as of March 31, 2014 having a weighted-average exercise price of $3.01 per share;

- 2,167,000 shares of common stock reserved for issuance pursuant to future equity awards under our 2014 Incentive Plan as of March 31, 2014, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan; and
- 600,000 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or 2014 ESPP, as of March 31, 2014, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2014 ESPP.

Except as otherwise indicated, all information contained in this prospectus:

- assumes that the underwriters do not exercise their option to purchase additional shares; and
- assumes no exercise of outstanding options or warrants after March 31, 2014.

SUMMARY FINANCIAL DATA

The following table summarizes our statements of operations and balance sheet data. We have derived the following statements of operations data for the years ended December 31, 2011, 2012, and 2013 from our audited financial statements incorporated by reference in this prospectus from our Annual Report on Form 10-K for the fiscal year ended December 31, 2013, or our 2013 Annual Report, and we have derived the following statements of operations data for the three months ended March 31, 2013 and March 31, 2014 and the balance sheet data as of March 31, 2014 from our unaudited interim financial statements incorporated by reference in this prospectus from our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014, or our March 2014 Quarterly Report. You should read this data together with our financial statements and related notes, as well as the information under the captions "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" appearing in our 2013 Annual Report and our March 2014 Quarterly Report, which are incorporated by reference herein. Our historical results are not necessarily indicative of our future results, and results of interim periods are not necessarily indicative of results for the entire year.

	Year Ended December 31,			Three Months Ended March 31,	
	2011	2012	2013	2013	2014
	(in thousands, except share and per share amounts)			(unaudited)	
Statements of Operations Data:					
Operating expenses:					
Research and development	$ 4,717	$ 12,641	$ 27,829	$ 5,664	$ 8,353
General and administrative	1,844	3,344	4,451	1,083	1,986
Total operating expenses	6,561	15,985	32,280	6,747	10,339
Loss from operations	(6,561)	(15,985)	(32,280)	(6,747)	(10,339)
Interest income	4	1	216	26	93
Interest expense	(270)	—	—	—	—
Other expense, net	(22)	(350)	(3,006)	(14)	(3,384)
Net loss	$ (6,849)	$ (16,334)	$ (35,070)	$ (6,735)	$ (13,630)
Net loss attributable to common stockholders[1]	$ (7,466)	$ (19,561)	$ (50,289)	$ (8,205)	$ (18,438)
Net loss per share attributable to common stockholders, basic and diluted	$ (4.62)	$ (14.20)	$ (14.87)	$ (2.84)	$ (0.85)
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	1,617,384	1,377,207	3,382,489	2,893,997	21,582,435

	As of March 31, 2014	
	Actual	As Adjusted[2]
	(in thousands) (unaudited)	
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$165,397	$ 214,241
Working capital	162,825	211,669
Total assets	172,707	221,551
Deficit accumulated during the development stage	(92,869)	(92,869)
Total stockholders' equity	165,714	214,558

(1) See (a) Notes 2 and 14 to our audited financial statements included in our 2013 Annual Report incorporated by reference herein and (b) Notes 2 and 11 to our interim financial statements included in our March 2014 Quarterly Report incorporated herein by reference for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders.

(2) The as-adjusted balance sheet data reflects the sale of 1,311,277 shares of common stock offered by us in this offering at the public offering price of $40.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We will not receive any proceeds from any sale of shares of our common stock in this offering by the selling stockholders; accordingly, there is no impact upon the adjusted balance sheet for these sales.

Quarterly Financial Data (unaudited)

The following tables present certain unaudited quarterly financial information. This information has been prepared on the same basis as the audited financial statements and includes all adjustments (consisting only of normal recurring adjustments) necessary to present fairly the unaudited quarterly results of operations set forth herein. The operating results for any quarter are not necessarily indicative of results for any future period. Net loss per share for all periods presented has been retroactively adjusted to reflect the 1-for-3.1345 reverse stock-split effected on January 17, 2014. All data is in thousands except per share data.

	2014
	Q1
Operating expenses	$ 10,339
Net loss	(13,630)
Net loss attributable to common stockholders	(18,438)
Net loss per share attributable to common stockholders, basic and diluted	(0.85)

	2013			
	Q1	Q2	Q3	Q4
Operating expenses	$ 6,747	$ 8,247	$ 7,761	$ 9,525
Net loss	(6,735)	(8,591)	(8,427)	(11,317)
Net loss attributable to common stockholders	(8,205)	(10,830)	(12,589)	(18,665)
Net loss per share attributable to common stockholders, basic and diluted	(2.84)	(3.32)	(3.48)	(4.98)

	2012			
	Q1	Q2	Q3	Q4
Operating expenses	$ 3,092	$ 3,571	$ 4,644	$ 4,678
Net loss	(3,103)	(3,672)	(4,629)	(4,930)
Net loss attributable to common stockholders	(3,384)	(4,063)	(5,302)	(6,812)
Net loss per share attributable to common stockholders, basic and diluted	(6.96)	(5.72)	(2.81)	(2.83)

RISK FACTORS

Investing in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus or incorporated by reference in this prospectus, including the risks and uncertainties discussed under "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014, which are incorporated by reference herein in their entirety. If any of the risks incorporated by reference herein or set forth below occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to this Offering

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the as-adjusted book value per share of our tangible assets after subtracting our liabilities. As a result, investors purchasing shares of common stock in this offering will incur immediate dilution of $33.16 per share, based on the public offering price of $40.00 per share, and our as-adjusted net tangible book value as of March 31, 2014 after giving effect to this offering. For information on how the foregoing amounts were calculated, see "Dilution."

This dilution is due to the substantially lower price paid by our investors who purchased shares prior to this offering as compared to the price offered to the public in this offering, and the exercise of stock options granted to our employees. In addition, as of March 31, 2014, we had outstanding options and warrants to purchase 2,749,782 shares of our common stock; the exercise of any of these options or warrants would result in additional dilution. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the market price of our common stock could decline. Based upon the number of shares of common stock outstanding as of March 31, 2014, upon the closing of this offering we will have outstanding a total of approximately 31,360,927 shares of common stock. Of these shares, the shares of our common stock sold in our initial public offering (other than any shares purchased by our then-existing investors and directors and officers), which was completed in February 2014, are currently freely tradable, and the shares to be sold in this offering, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable, without restriction, in the public market immediately following this offering. J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, however, may, in their sole discretion, permit our officers, directors and stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

As of March 31, 2014, we had a total of 30,049,650 shares of common stock issued and outstanding. The lock-up agreements pertaining to our initial public offering will expire on July 29, 2014, following which at least approximately 23.4 million shares of common stock will be eligible for sale in the public market, subject to any additional restrictions, including a lock-up in connection with this offering. The lock-up agreements pertaining to this offering will expire 90 days from the date of this prospectus, following which approximately 7.2 million shares of common stock will be eligible for sale in the public market, all of which shares are held by current directors, executive officers and the selling stockholders and may be subject to Rule 144 under the Securities Act.

In addition, as of March 31, 2014, approximately 5.5 million shares of common stock that are either subject to outstanding options, reserved for future issuance under our equity incentive plans, or subject to outstanding warrants will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements, and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

After this offering, the holders of approximately 19.4 million shares of our common stock, or 19.7 million including the shares underlying outstanding warrants, will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the vesting schedules and lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these stockholders could have a material adverse effect on the market price of our common stock.

We will receive only limited proceeds from this offering and will have broad discretion in the use of the net proceeds to us from this offering; we may not use the offering proceeds that we receive effectively.

We will receive only limited proceeds from this offering, as a portion of the proceeds will be paid to the selling stockholders. We will receive net proceeds of only $48.8 million from this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

Our management will have broad discretion in the application of the net proceeds to us from this offering, including for any of the purposes described in the section entitled “Use of Proceeds,” and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds to us from this offering, their ultimate use may vary from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds to us from this offering in investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains 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. All statements other than statements of historical facts contained in this prospectus are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our expectations regarding the timing of commencing our clinical studies and reporting results from same;
- the timing and likelihood of regulatory approvals for our product candidates;
- the potential market opportunities for commercializing our product candidates;
- our expectations regarding the potential market size and the size of the patient populations for our product candidates, if approved for commercial use;
- estimates of our expenses, future revenue, capital requirements, and our needs for additional financing;
- our ability to develop, acquire, and advance product candidates into, and successfully complete, clinical studies;
- the implementation of our business model and strategic plans for our business and product candidates;
- the initiation, timing, progress, and results of future preclinical studies and clinical studies, and our research and development programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- our ability to maintain and establish collaborations or obtain additional funding;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act;
- our use of proceeds from this offering;
- our financial performance and expansion of our organization;
- our ability to obtain supply of our product candidates; and
- developments and projections relating to our competitors and our industry.

Any forward-looking statements in this prospectus reflect our current views with respect to future events or our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk Factors” and elsewhere and incorporated by reference in this prospectus. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This prospectus also contains estimates, projections, and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources.

USE OF PROCEEDS

We estimate that the net proceeds to us from the sale of 1,311,277 shares of common stock offered by us in this offering will be approximately $48.8 million, based on the public offering price of $40.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that our net proceeds will be approximately $60.2 million, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We will not receive any of the proceeds from any sale of shares in this offering by the selling stockholders.

We intend to use the net proceeds to us of this offering, together with our existing cash, cash equivalents and short-term investments, for the following purposes:

- Continued advancement of our clinical and preclinical pipeline, including potential new formulations of or indications for our existing product candidates or potential new product candidates;
- Increased investment in early-stage research capabilities and general infrastructure; and
- The remainder for other ongoing research and development, working capital, and other general corporate purposes.

Our expected use of net proceeds to us from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with complete certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We may also use a portion of the net proceeds to us to in-license, acquire, or invest in additional businesses, technologies, products, or assets. Although we have no specific agreements, commitments, or understandings with respect to any in-license or acquisition, we evaluate such opportunities and engage in related discussions with other companies from time to time. Due to the many variables inherent to the development of our product candidates, we cannot currently predict the stage of development we expect the net proceeds to us of this offering to achieve for our clinical studies and product candidates.

The amount and timing of our actual expenditures will depend upon numerous factors, including the results of our research and development efforts, the timing and success of preclinical studies, our ongoing clinical studies or clinical studies we may commence in the future and the timing of regulatory submissions. As a result, our management will have broad discretion over the use of the net proceeds to us from this offering.

Pending the use of the proceeds to us from this offering, we intend to invest these proceeds in interest-bearing, investment-grade securities, certificates of deposit, or government securities.

We estimate that, with our current operating plan, the proceeds from this offering, together with our existing cash, cash equivalents and short-term investments, will fund our activities through 2016.

PRICE RANGE OF COMMON STOCK

Our common stock has been publicly traded on The NASDAQ Global Select Market under the symbol “RARE” since January 31, 2014. Prior to that time, there was no public market for our common stock. The following table sets forth the high and low sale prices per share for our common stock on The NASDAQ Global Select Market for the periods indicated:

	High	Low
2014		
First Quarter (beginning January 31, 2014)	$69.77	$35.15
Second Quarter	$60.00	$32.02

On July 8, 2014, the last reported sale price of our common stock on The NASDAQ Global Select Market was $41.40 per share. As of July 2, 2014, we had approximately 67 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

DIVIDEND POLICY

We have never declared or paid cash dividends on our common stock. Although we have paid dividends to our holders of preferred stock in the past, including a $4.3 million cash dividend paid in February 2014 in connection with our initial public offering, all dividends were agreed to at the time of the private placement financings. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors or any authorized committee thereof.

CAPITALIZATION

The following table sets forth our cash, cash equivalents and short-term investments and capitalization as of March 31, 2014:

- on an actual basis; and
- on an as-adjusted basis to reflect the issuance and sale by us of 1,311,277 shares of our common stock in this offering at the public offering price of $40.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this information together with our financial statements and related notes and the information under the captions "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," appearing in our 2013 Annual Report and March 2014 Quarterly Report, incorporated by reference in this prospectus. For more details on how you can obtain our Commission reports and other information, you should read the section of the prospectus entitled "Where You Can Find More Information."

	As of March 31, 2014	
	Actual	As Adjusted
	(in thousands, except share and per share data) (unaudited)	
Cash, cash equivalents and short-term investments	$165,397	$ 214,241
Stockholders' equity:		
Preferred stock, par value $0.001 per share—25,000,000 shares authorized; no shares issued or outstanding, actual and as adjusted	—	—
Common stock, par value $0.001 per share—250,000,000 shares authorized; 30,049,650 shares issued and outstanding, actual; 250,000,000 shares authorized, 31,360,927 shares issued and outstanding, as adjusted	30	31
Additional paid-in capital	258,599	307,442
Accumulated other comprehensive loss	(46)	(46)
Deficit accumulated during the development stage	(92,869)	(92,869)
Total stockholders' equity	165,714	214,558
Total capitalization	$165,714	$ 214,558

The number of shares of common stock issued and outstanding in the table above excludes the following shares as of March 31, 2014:

- 2,396,323 shares of common stock issuable upon the exercise of outstanding stock options as of March 31, 2014 having a weighted-average exercise price of $6.35 per share;
- 353,459 shares of common stock issuable upon the exercise of outstanding warrants as of March 31, 2014 having a weighted-average exercise price of $3.01 per share;
- 2,167,000 shares of common stock reserved for issuance pursuant to future equity awards under our 2014 Incentive Plan as of March 31, 2014, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan; and
- 600,000 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or 2014 ESPP, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2014 ESPP.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted to the extent of the difference between the public offering price per share of our common stock and the as-adjusted net tangible book value per share of our common stock immediately after this offering.

Net tangible book value per share is determined by dividing our total tangible assets less our total liabilities by the number of shares of common stock outstanding. Our historical net tangible book value as of March 31, 2014 was approximately $165.7 million, or $5.51 per share.

Dilution per share to new investors represents the difference between the amount per share paid by purchasers of shares of common stock in this offering and the as-adjusted net tangible book value per share of common stock immediately after completion of this offering. After giving effect to the sale of 1,311,277 shares of common stock in this offering by us at the public offering price of $40.00 per share, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our as-adjusted net tangible book value as of March 31, 2014 would have been $214.6 million, or $6.84 per share. This represents an immediate increase in net tangible book value of $1.33 per share to existing stockholders and an immediate dilution of $33.16 per share to investors participating in this offering, as illustrated in the following table:

Public offering price per share		$40.00
Historical net tangible book value per share as of March 31, 2014	$5.51	
Increase in as-adjusted net tangible book value per share attributable to new investors	1.33	
As-adjusted net tangible book value per share after this offering		6.84
Dilution per share to investors participating in this offering		$33.16

The foregoing calculations exclude the following shares as of March 31, 2014:

- 2,396,323 shares of common stock issuable upon the exercise of outstanding stock options as of March 31, 2014 having a weighted-average exercise price of $6.35 per share;
- 353,459 shares of common stock issuable upon the exercise of outstanding warrants as of March 31, 2014 having a weighted-average exercise price of $3.01 per share;
- 2,167,000 shares of common stock reserved for issuance pursuant to future equity awards under our 2014 Incentive Plan as of March 31, 2014, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan; and
- 600,000 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or 2014 ESPP, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2014 ESPP.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. New investors will experience further dilution if any of our outstanding options or warrants are exercised, new options are issued and exercised under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future.

CERTAIN RELATIONSHIPS AND RELATED-PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2010 to which we have been a party, in which the amount involved exceeds $120,000, and in which any of our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unaffiliated third parties.

Sales and Purchases of Securities

Series A Convertible Preferred Stock Financing

On June 16, 2011, we sold an aggregate of 18,052,464 shares of our Series A convertible preferred stock to eight investors at a purchase price of $1.034 per share, for an aggregate purchase price of approximately $15.0 million in cash and $3.7 million in converted bridge notes. On July 16, 2012, we sold, pursuant to a second tranche closing, an aggregate of 14,604,895 shares of our Series A convertible preferred stock to six investors at a purchase price of $1.034 per share, for an aggregate purchase price of $15.1 million in cash. The table below sets forth the aggregate number of shares of Series A convertible preferred stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an immediate family member thereof, as applicable:

Name of Stockholder	Number of Shares of Series A Convertible Preferred Stock	Total Purchase Price
Emil D. Kakkis, M.D., Ph.D.	1,814,944	$ 1,876,471
William Aliski	247,049	$ 255,424

Convertible Notes and Series A Convertible Preferred Stock Warrants

On June 30, 2010, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $1.0 million to Dr. Kakkis and also issued him a warrant to purchase up to 241,803 shares of our Series A convertible preferred stock. Emil D. Kakkis, M.D., Ph.D. is our President and Chief Executive Officer and one of our directors.

On February 23, 2011, we entered into a Note and Warrant Purchase Agreement with William Aliski. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $250,000 to Mr. Aliski and also issued him a warrant to purchase up to 84,631 shares of our Series A convertible preferred stock. William Aliski is one of our directors.

On June 14, 2011, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $300,000 to Dr. Kakkis and also issued him a warrant to purchase up to 72,541 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a second Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $500,000 to Dr. Kakkis and also issued him a warrant to purchase up to 120,901 shares of our Series A convertible preferred stock.

Series B Convertible Preferred Stock Financing

On December 18, 2012, we sold an aggregate of 27,081,680 shares of our Series B convertible preferred stock to 34 investors at a purchase price of $2.769 per share, for an aggregate purchase price of approximately $75 million in cash. The table below sets forth the aggregate number of shares of Series B convertible preferred stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an immediate family member thereof, as applicable:

Name of Stockholder	Number of Shares of Series B Convertible Preferred Stock	Total Purchase Price
TPG Biotechnology Partners III, L.P.	541,634	$ 1,500,001
Beacon Bioventures Fund II Limited Partnership	541,634	$ 1,500,001
HealthCap VI L.P.	481,452	$ 1,333,333
Entities affiliated with A.M. Pappas Life Science Ventures IV, L.P.	240,727	$ 666,669

Participation in our Initial Public Offering

In connection with our initial public offering, or IPO, the underwriters allocated shares of our common stock in the offering to certain of our greater than 5% holders on the same terms as the other shares that were offered and sold in our IPO. These allocations included allocations of 240,000 shares to Adage Capital Partners, L.P. and 175,000 shares to Beacon Bioventures Fund II Limited Partnership. All of these shares were sold at $21.00, which was the price at which our common stock was sold in the IPO.

Indemnification Agreements and Directors' and Officers' Liability Insurance

We have entered into indemnification agreements with each of our directors and executive officers. These agreements, among other things, require us to indemnify each director and executive officer to the fullest extent permitted by Delaware law, including indemnification of expenses such as attorneys' fees, judgments, penalties, fines and settlement amounts incurred by the director or executive officer in any action or proceeding, including any action or proceeding by or in right of us, arising out of the person's services as a director or executive officer.

Registration Rights Agreement

We and certain of our stockholders have entered into an investor rights agreement pursuant to which these stockholders have, among other things, registration rights under the Securities Act of 1933, as amended, with respect to common stock that they hold. See "Description of Capital Stock — Registration Rights" for a further description of the terms of this agreement.

Procedures for Related Party Transactions

We have adopted a related person transaction approval policy that governs the review, approval and/or ratification of related person transactions. Pursuant to this policy, if we want to enter into a transaction with a related person or an affiliate or immediate family member of a related person, our Chief Financial Officer will review the proposed transaction to determine, based on applicable NASDAQ and SEC rules, if such transaction qualifies as a related person transaction. If the Chief Financial Officer determines that the proposed transaction is a related person transaction, then the proposed transaction shall be submitted to the Audit Committee for pre-approval at the next regular or special Audit Committee meeting; if the Chief Financial Officer, in consultation with the Chief Executive Officer, determines that it is not practicable to wait until the next meeting of the Audit Committee, then the Chief Financial Officer shall submit the proposed transaction to the chairperson of the Audit Committee. In the event that our Chief Executive Officer or Chief Financial Officer becomes aware of a related person transaction that has not been previously approved or previously ratified under our related person transaction approval policy, the transaction, if pending or ongoing, will be promptly submitted to the Audit Committee or the chairperson of the Audit Committee for consideration. If the transaction is already completed, the Audit Committee or the chairperson of the Audit Committee shall evaluate the transaction to determine if rescission of the transaction and/or any disciplinary action is appropriate.

PRINCIPAL AND SELLING STOCKHOLDERS

The following table sets forth information relating to the beneficial ownership of our common stock as of June 20, 2014 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our outstanding shares of common stock;
- each of our directors;
- each of our named executive officers;
- all directors and executive officers as a group; and
- each of the selling stockholders.

The number of shares beneficially owned by each entity, person, director, executive officer or selling stockholder is determined in accordance with the rules of the SEC and the information is not necessarily indicative of beneficial ownership for any other purpose. Under such rules, beneficial ownership includes any shares over which the individual or entity has sole or shared voting power or investment power as well as any shares that the individual or entity has the right to acquire within 60 days of June 20, 2014 through the exercise of any stock option, warrants or other rights. Except as otherwise indicated, and subject to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of common stock held by that person or entity.

The percentage of shares beneficially owned is computed on the basis of 30,059,288 shares of our common stock outstanding as of June 20, 2014. Shares of our common stock that a person or entity has the right to acquire within 60 days of June 20, 2014 are deemed outstanding for purposes of computing the percentage ownership of the person or entity holding such rights, but are not deemed outstanding for purposes of computing the percentage ownership of any other person or entity, except with respect to the percentage ownership of all directors and executive officers as a group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o Ultragenyx Pharmaceutical Inc., at 60 Leveroni Court, Novato, California 94949.

The information in the table below with respect to each selling stockholder has been obtained from that selling stockholder. When we refer to the “selling stockholder” in this prospectus, we mean those persons listed in the table below as offering shares, as well as the pledgees, donees, assignees, transferees, successors and others who may hold any of the selling stockholders’ interest.

Name of Beneficial Owner	Shares Beneficially Owned Before Offering		Shares to be Sold in Offering	Shares Beneficially Owned after Offering		Shares Beneficially Owned after Offering Assuming Underwriters Exercise Option in Full	
	Number	%		Number	%	Number	%
Stockholders Owning Greater than 5%:							
TPG Biotechnology Partners III, L.P.(1)	3,085,240	10.3%	488,820	2,596,420	8.3%	2,596,420	8.2%
Beacon Bioventures Fund II Limited Partnership(2)	3,260,240	10.9%	—	3,260,240	10.4%	3,260,240	10.3%
HealthCap VI, L.P.(3)	2,742,436	9.1%	—	2,742,436	8.7%	2,742,436	8.7%
Adage Capital Partners, L.P.(4)	1,867,713	6.2%	—	1,867,713	6.0%	1,867,713	5.9%
Additional Selling Stockholder:							
Entities affiliated with A.M. Pappas Life Science Ventures IV, L.P.(5)	1,371,213	4.6%	217,252	1,153,961	3.7%	1,153,961	3.6%
Directors and Named Executive Officers:							
Eran Nadav, Ph.D.(6)	2,917	*	—	2,917	*	2,917	*
Mårten Steen, M.D., Ph.D.(7)	2,917	*	—	2,917	*	2,917	*
William Aliski(8)	68,928	*	—	68,928	*	68,928	*
Matthew K. Fust(9)	7,917	*	—	7,917	*	7,917	*
Clay B. Siegall, Ph.D.(10)	2,917	*	—	2,917	*	2,917	*
Emil D. Kakkis, M.D., Ph.D(11)	3,326,181	11.0%	—	3,326,181	10.6%	3,326,181	10.5%
Thomas Kassberg(12)	172,230	*	—	172,230	*	172,230	*
Shalini Sharp(13)	108,184	*	—	108,184	*	108,184	*
All executive officers and directors as a group(14) (8 persons)	3,692,191	12.2%	—	3,692,191	11.8%	3,692,191	11.7%

* Indicates beneficial ownership of less than 1% of the total outstanding common stock.

(1) Based on information set forth in a Form 4 filed with the SEC by TPG Group Holdings (SBS) Advisors, Inc. on February 7, 2014. TPG Biotechnology Partners III, L.P. is a Delaware limited partnership, whose general partner is TPG Biotechnology GenPar III, L.P., a Delaware limited partnership, whose general partner is TPG Biotechnology GenPar III Advisors, LLC, a Delaware limited liability company, whose sole member is TPG Holdings I, L.P., a Delaware limited partnership, whose general partner is TPG Holdings I-A, LLC, a Delaware limited liability company, whose sole member is TPG Group Holdings (SBS), L.P., a Delaware limited partnership, whose general partner is TPG Group Holdings (SBS) Advisors, Inc., or Group Advisors, a Delaware corporation. Messrs. David Bonderman and James G. Coulter are officers and sole shareholders of Group Advisors and may therefore be deemed to be the beneficial owners of the shares held by TPG Biotechnology Partners III, L.P. Each of Messrs. Bonderman and Coulter and TPG Biotechnology Partners III, L.P. expressly disclaims beneficial ownership of the securities listed above except to the extent of any pecuniary interest therein. The address for Messrs. Bonderman and Coulter and TPG Biotechnology Partners III, L.P. is c/o TPG Global, LLC, 301 Commerce Street, Suite 3300, Fort Worth, TX 76102.

(2) Based on information set forth in a Schedule 13G filed with the SEC by FMR LLC on February 14, 2014. Beacon Bioventures Advisors Fund II Limited Partnership is the general partner of Beacon Bioventures Fund II Limited Partnership. Beacon Bioventures Advisors Fund II Limited Partnership is solely managed by Northern Neck Investors LLC, its general partner and investment manager. Northern Neck Investors LLC is owned by the shareholders and certain employees of FMR LLC, including certain members of the family of Edward C. Johnson 3d. Members of the family of Edward C. Johnson 3d, Chairman of FMR LLC, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in

accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Each of the individuals and entities listed above expressly disclaims beneficial ownership of the securities listed above except to the extent of any pecuniary interest therein. The address for each of the individuals and entities listed above is 82 Devonshire Street, Boston, Massachusetts 02109.

(3) HealthCap VI GP SA, L.L.C. ("HCSA") is the sole general partner of HealthCap VI, L.P. HCSA has voting and dispositive power over the shares held by HealthCap VI, L.P. HCSA disclaims beneficial ownership of such shares, except to the extent of its pecuniary interest therein. Francois Kaiser, Dag Richter, and Daniel Schafer, the members of the board of HCSA, share voting and dispositive power over the shares held by HealthCap VI, L.P. and may be deemed to have indirect beneficial ownership of the shares held by such entities. The members of the board of HCSA disclaim beneficial ownership of shares held by HealthCap VI, L.P. except to the extent of any pecuniary interest therein. The address of HealthCap VI, L.P. is c/o HealthCap VI GP SA, 18, Avenue d Ouchy, 1006 Lausanne, Switzerland.

(4) Adage Capital Partners, GP, LLC ("ACPGP"), serves as the general partner of Adage Capital Partners, L.P., a Delaware limited partnership (the "Fund") and as such has discretion over the portfolio of securities beneficially owned by the Fund. Adage Capital Advisors, LLC, a Delaware limited liability company ("ACA"), is managing member of ACPGP and directs ACPGP's operations. Robert Atchinson and Phillip Gross are the managing members of ACPGP and ACA and general partners of the Fund. Robert Atchinson and Phillip Gross disclaim beneficial ownership of the reported securities except to the extent of their pecuniary interest therein. The address of Adage Capital Partners, L.P. is 200 Clarendon Street, 52nd Floor, Boston, MA 02116.

(5) Consists of (a) 1,308,916 shares of common stock held by A.M. Pappas Life Science Ventures IV, L.P., and (b) 62,297 shares of common stock held by PV IV CEO Fund, L.P. AMP&A Management IV, LLC is the general partner of each of A.M. Pappas Life Science Ventures IV, L.P. and PV IV CEO Fund, L.P. (collectively, the "Pappas Funds"), and AMP&A Management IV, LLC has a management agreement with A.M. Pappas & Associates, LLC whereby A.M. Pappas & Associates, LLC provides management services for the Pappas Funds. As a result, A.M. Pappas & Associates, LLC's investment committee exercises sole dispositive and voting power over the shares owned by the Pappas Funds. The address for each of A.M. Pappas Life Science Ventures IV, L.P. and PV IV CEO Fund, L.P. is c/o Pappas Ventures, 2520 Meridian Parkway, Suite 400, Durham, NC 27713.

(6) Consists of 2,917 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(7) Consists of 2,917 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(8) Consists of (a) 36,903 shares of common stock, (b) 29,108 shares of common stock that may be acquired pursuant to the exercise of a warrant held by Mr. Aliski and (c) 2,917 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(9) Consists of (a) 5,000 shares of common stock and (b) 2,917 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(10) Consists of 2,917 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(11) Consists of (a) 2,552,241 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009, (b) 624,240 shares of common stock held by Dr. Kakkis and (c) 149,700 shares of common stock that may be acquired pursuant to the exercise of warrants held by Dr. Kakkis. Dr. Kakkis shares voting and dispositive power over the 2,552,241 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009; each of Dr. Kakkis and Dr. Soriano is a trustee of such trust. Dr. Kakkis has sole voting and dispositive power over the 624,240 shares of common stock held by him and the 149,700 shares of common stock that may be acquired pursuant to the exercise of warrants held by Dr. Kakkis.

(12) Consists of (a) 125,804 shares of common stock and (b) 46,426 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(13) Consists of (a) 84,255 shares of common stock and (b) 23,929 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014.

(14) Consists of (a) 3,428,443 shares held by our directors and officers and entities affiliated with certain of our directors, (b) 178,808 shares of common stock that may be acquired pursuant to the exercise of warrants by certain of our directors and officers, and (c) 84,940 shares of common stock issuable pursuant to stock options exercisable within 60 days of June 20, 2014 held by our directors and officers.

DESCRIPTION OF CAPITAL STOCK

General

Our authorized capital stock consists of 250,000,000 shares of common stock, par value $0.001 per share, and 25,000,000 shares of preferred stock, par value $0.001 per share. The following description of our capital stock is intended as a summary only and is qualified in its entirety by reference to our amended and restated certificate of incorporation, and amended and restated by-laws, copies of which are incorporated by reference as exhibits to the registration statement, of which this prospectus forms a part, and to the applicable provisions of the Delaware General Corporation Law.

Common Stock

As of March 31, 2014, there were 30,049,650 shares of our common stock outstanding, held of record by approximately 65 stockholders. Based on shares outstanding as of March 31, 2014, upon completion of this offering, there will be 31,360,927 shares of our common stock outstanding.

Holders of our common stock are entitled to one vote for each share of common stock held of record for the election of directors and on all matters submitted to a vote of stockholders. Holders of our common stock are entitled to receive dividends ratably, if any, as may be declared by our board of directors out of legally available funds, subject to any preferential dividend rights of any preferred stock then outstanding. Upon our dissolution, liquidation or winding up, holders of our common stock are entitled to share ratably in our net assets legally available after the payment of all our debts and other liabilities, subject to the preferential rights of any preferred stock then outstanding. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future. Except as described below in “Anti-Takeover Effects of Delaware Law, Our Certificate of Incorporation and Our By-laws,” the affirmative vote of a majority of our outstanding shares of capital stock is generally required to take action under our amended and restated certificate of incorporation and amended and restated by-laws.

Preferred Stock

Our board of directors is authorized, without action by the stockholders, to designate and issue up to an aggregate of 25,000,000 shares of preferred stock in one or more series. The board of directors can fix the rights, preferences and privileges of the shares of each series and any of its qualifications, limitations or restrictions. Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of common stock. The issuance of preferred stock, while providing flexibility in connection with possible future financings and acquisitions and other corporate purposes could, under certain circumstances, have the effect of delaying or preventing a change in control of our company and might harm the market price of our common stock.

Our board of directors will make any determination to issue such shares based on its judgment as to our best interests and the best interests of our stockholders. We have no current plans to issue any shares of preferred stock.

Warrants

As of March 31, 2014, warrants to purchase a total of 353,459 shares of our common stock were outstanding with an exercise price of $3.01 per share. These warrants are exercisable immediately and each expire on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the 10 year anniversary of the date of such warrant which in the case of the outstanding warrants of the Company is June 2020, February 2021 and June 2021, respectively.

Registration Rights

We are party to an amended and restated investors' rights agreement, dated as of December 18, 2012, with certain holders of our common stock. Following this offering, the holders of approximately 19.4 million shares, or 19.7 million including the shares underlying outstanding warrants, will have the right to require us to register their shares under the Securities Act of 1933, as amended. These shares will represent approximately 63% of our outstanding common stock after this offering, or approximately 62% if the underwriters exercise their option to purchase additional shares in full. These shares also may be sold under Rule 144 under the Securities Act of 1933, depending on their holding period and subject to restrictions in the case of shares held by persons deemed to be our affiliates. The registration rights will terminate (i) on February 5, 2019, which is the fifth anniversary of the closing of our initial public offering, or (ii) after this offering, as to any holder of registrable securities, when such holder holds 1% or less of our common stock and all registrable securities held by such holder can be sold in any 90-day period without registration in compliance with Rule 144.

Demand Registration Rights

Under the amended and restated investors' rights agreement, beginning July 29, 2014, which is the date that is 180 days after the effective date of the registration statement for our initial public offering, or 45 days following the effective date of any other Company-initiated registration statement, the holders of at least 50% of the registrable shares (or a lesser percentage if the anticipated aggregate offering price is not less than $10 million) then outstanding can, on not more than two occasions, demand that we file a registration statement or request that their shares be included on a registration statement that we are otherwise filing, in either case, registering the resale of their shares of common stock. We are required to use our best efforts to effect the registration and will pay all registration expenses, other than underwriting discounts and commissions, related to any demand registration. These registration rights are subject to conditions and limitations, including the right, in certain circumstances, of the underwriters of an offering to limit the number of shares included in such registration and our right, in certain circumstances, not to effect a requested registration.

Piggyback Registration Rights

If we propose to register any of our securities under the Securities Act for our own account or the account of any other holder, the "significant holders" (as defined in the amended and restated investors' rights agreement) are entitled to notice of such registration and to request that we include registrable shares for resale on such registration statement, subject to the right of any underwriter to limit the number of shares included in such registration.

We will pay all registration expenses, other than underwriting discounts and commissions, related to any piggyback registration. The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders, in the event of misstatements or omissions in the registration statement attributable to us and they are obligated to indemnify us for misstatements or omissions attributable to them.

Form S-3 Registration Rights

The holders of at least 10% of the registrable securities outstanding can make a written request that we register their shares on Form S-3 if we are eligible to file a registration statement on Form S-3 and if the aggregate price to the public of the shares offered is at least $1 million. These stockholders may make an unlimited number of requests for registration on Form S-3. However, we will not be required to effect a registration on Form S-3 if we determine that such a registration would be seriously detrimental to us and our stockholders or if we have already effected three registration statements on Form S-3 in the 12-month period preceding the date of the request. We will pay all registration expenses, other than underwriting discounts and commissions, related to any S-3 registration.

Anti-Takeover Effects of Delaware Law, our Certificate of Incorporation and Our By-laws

Our certificate of incorporation and by-laws include a number of provisions that may have the effect of encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

In accordance with our certificate of incorporation, our board is divided into three classes serving three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by a resolution of the board.

No Written Consent of Stockholders

Our certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting.

Meetings of Stockholders

Our certificate of incorporation and by-laws provide that, subject to any rights of holders of any series of preferred stock, only the board or the chairman of the board may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our by-laws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our by-laws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days or more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. The notice must contain certain information specified in the by-laws. These provisions may have the effect of precluding the conduct of certain business at a meeting if the proper procedures are not followed. These provisions may also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer’s own slate of directors or otherwise attempting to obtain control of our company.

Amendment to By-laws and Certificate of Incorporation

As required by the Delaware General Corporation Law, any amendment of our certificate of incorporation must first be approved by a majority of our board of directors and, if required by law or our certificate of incorporation, thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment, and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to directors, stockholders, the amendment of our by-laws and certificate of incorporation and exclusive jurisdiction of Delaware Courts must be approved by not less than 75% of the outstanding shares entitled to vote on the amendment, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a class. Our by-laws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the by-laws, and may also be amended by the affirmative vote of at least 75% of the outstanding shares entitled to vote on the amendment.

Blank Check Preferred Stock

Our certificate of incorporation provides for 25,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of us or our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Section 203 of the Delaware General Corporation Law

We are subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation's voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, the board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or
- at or after the time the stockholder became interested, the business combination was approved by the board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

A Delaware corporation may "opt out" of these provisions with an express provision in its original certificate of incorporation or an express provision in its certificate of incorporation or by-laws resulting from a stockholders' amendment approved by at least a majority of the outstanding voting shares. We have not opted out of these provisions. As a result, mergers or other takeover or change in control attempts of us may be discouraged or prevented.

Exclusive Jurisdiction of Certain Actions

Our certificate of incorporation requires, to the fullest extent permitted by law, that derivative actions brought in our name, actions against our directors, officers and employees for breach of fiduciary duty and other similar actions may be brought only in the Court of Chancery in the State of Delaware, unless we otherwise consent. Although we believe this provision benefits us by providing increased consistency in the application of

Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers. Although our certificate of incorporation contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

NASDAQ Global Select Market listing

Our common stock is listed on The NASDAQ Global Select Market under the symbol “RARE”.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar’s address is 620 15th Avenue, Brooklyn, New York 11219.

SHARES ELIGIBLE FOR FUTURE SALE

Future sales of our common stock, including shares issued upon the exercise of outstanding options or warrants, in the public market after this offering, or the perception that those sales may occur, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after completion of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before (to the extent permitted) or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate.

Sale of Restricted Shares

As of March 31, 2014, based on the number of shares of our common stock then outstanding, upon the closing of this offering, and assuming no exercise of outstanding options or warrants and no exercise of the underwriters' option to purchase additional shares, we would have had outstanding an aggregate of approximately 31,360,927 shares of common stock. Of these shares, the 6,624,423 shares sold in our initial public offering (other than any shares purchased by our then-existing investors and directors and officers), the 2,017,349 shares of common stock to be sold in this offering, including both the shares sold by us and any shares sold by the selling stockholders, as well as any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable in the public market without restriction or further registration under the Securities Act of 1933, as amended, or the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 of the Securities Act. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

Lock-Up Agreements

In connection with this offering, we, our directors, our executive officers, and each of the selling stockholders have agreed, subject to certain exceptions, with the underwriters not to dispose of or hedge any shares of our common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of the lock-up agreement continuing through the date 90 days after the date of this prospectus, except with the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, together the representatives of the underwriters. The representatives of the underwriters have advised us that they have no current intent or arrangement to release any of the shares subject to the lock-up agreements prior to the expiration of the lock-up period.

Rule 144

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, for at least 90 days, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the three months preceding a sale, and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to above, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell

such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to above, if applicable).

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- one percent of the number of common shares then outstanding, which will equal approximately 313,609 shares of common stock immediately after this offering (calculated on the basis of the number of shares of our common stock outstanding as of March 31, 2014, and assuming no exercise of the underwriter's option to purchase additional shares and no exercise of outstanding options or warrants); or
- the average weekly trading volume of our common stock on The NASDAQ Global Select Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us.

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, directors, officers, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement in compliance with Rule 701 before the effective date of a registration statement under the Securities Act is entitled to rely on Rule 701 to resell such shares in reliance on Rule 144. An affiliate of the issuer can resell shares in reliance on Rule 144 without having to comply with the holding period requirements of Rule 144, and a non-affiliate of the issuer can resell shares in reliance on Rule 144 without having to comply with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about the issuer.

The SEC has indicated that Rule 701 will apply to typical stock options granted by an issuer before it becomes subject to the reporting requirements of the Exchange Act, along with the shares acquired upon exercise of such options, including exercises after an issuer becomes subject to the reporting requirements of the Exchange Act.

Equity Incentive Plans and Employee Stock Purchase Plan

We have filed with the SEC a registration statement under the Securities Act covering the shares of common stock that we may issue (i) upon exercise of outstanding options under the 2011 Equity Incentive Plan, as amended, exercise of outstanding options under the 2014 Incentive Plan, and options reserved for issuance under the 2014 Incentive Plan, and (ii) pursuant to the 2014 Employee Stock Purchase Plan. Such registration statement was filed and became effective in March 2014. Accordingly, shares registered under such registration statement are available for sale in the open market, subject to Rule 144 volume limitations for affiliates and the lock-up agreements described above, if applicable.

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering, but does not purport to be a complete analysis of all potential tax effects. The effects of other U.S. federal tax laws, such as estate and gift tax laws, and any applicable state, local or foreign tax laws are not discussed. This discussion is based on the Internal Revenue Code of 1986, as amended, or the Code, Treasury Regulations promulgated under the Code, judicial decisions, and published rulings and administrative pronouncements of the U.S. Internal Revenue Service, or IRS, in effect as of the date of this offering. These authorities may change or be subject to differing interpretations. Any such change may be applied retroactively in a manner that could adversely affect a non-U.S. holder of our common stock. We have not sought and will not seek any rulings from the IRS regarding the matters discussed below. There can be no assurance the IRS or a court will not take a contrary position regarding the tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is limited to non-U.S. holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (property held for investment). This discussion does not address all U.S. federal income tax consequences relevant to a non-U.S. holder's particular circumstances, including the impact of the Medicare contribution tax on net investment income. In addition, it does not address consequences relevant to non-U.S. holders subject to particular rules, including, without limitation:

- U.S. expatriates and certain former citizens or long-term residents of the United States;
- persons subject to the alternative minimum tax;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies and other financial institutions;
- real estate investment trusts or regulated investment companies;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies" and corporations that accumulate earnings to avoid U.S. federal income tax;
- S corporations, partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes;
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- tax-qualified retirement plans.

If a partnership (or other entity treated as a partnership for U.S. federal income tax purposes) holds our common stock, the tax treatment of a partner in the partnership will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS FOR INFORMATION PURPOSES ONLY AND IS NOT INTENDED AS TAX ADVICE. INVESTORS SHOULD CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX LAWS OR UNDER THE LAWS OF ANY STATE, LOCAL OR NON-U.S. TAXING JURISDICTION OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of a Non-U.S. Holder

For purposes of this discussion, a "non-U.S. holder" is any beneficial owner of our common stock that is neither a "U.S. person" nor a partnership for United States federal income tax purposes. A U.S. person is any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or other entity taxable as a corporation for U.S. federal income tax purposes) created or organized under the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust that (1) is subject to the primary supervision of a U.S. court and the control of one or more United States persons (within the meaning of Section 7701(a)(30) of the Code), or (2) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

Distributions

As described in the section entitled "Dividend Policy," we do not anticipate declaring or paying dividends to holders of our common stock in the foreseeable future. However, if we do make distributions on our common stock, such distributions of cash or property on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce a non-U.S. holder's adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below in the section titled "— Sale or Other Taxable Disposition."

Dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate specified by an applicable income tax treaty). Even if a non-U.S. holder is eligible for a lower treaty rate, dividend payments will generally be subject to withholding at a 30% rate (rather than the lower treaty rate) unless the non-U.S. holder provides a valid IRS Form W-8BEN or W-8BEN-E (or applicable successor form) certifying such holder's qualification for the reduced rate.

Non-U.S. holders who do not timely provide us or our paying agent with the required certification, but who qualify for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. holders should consult their tax advisors regarding their entitlement to benefits under a tax treaty.

Subject to the discussions below regarding backup withholding and foreign accounts, if dividends paid to a non-U.S. holder are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such dividends are attributable), the non-U.S. holder will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must furnish to us or our paying agent a valid IRS Form W-8ECI (or applicable successor form), certifying that the dividends are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States.

Any dividends paid on our common stock that are effectively connected with a non-U.S. holder's U.S. trade or business (and, if required by an applicable tax treaty, attributable to a permanent establishment maintained by the non-U.S. holder in the United States) generally will be subject to U.S. federal income tax on a net income basis in the same manner as if such holder were a U.S. person. A non-U.S. holder that is a corporation also may, under certain circumstances, be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable tax treaty) on any effectively connected dividends that it receives. Non-U.S. holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

Sale or Other Taxable Disposition

Subject to the discussions below regarding backup withholding and foreign accounts, a non-U.S. holder will not be subject to U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such gain is attributable);
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the disposition and certain other requirements are met; or
- our common stock constitutes a U.S. real property interest, or USRPI, by reason of our status as a U.S. real property holding corporation, or a USRPHC, for U.S. federal income tax purposes.

Gain described in the first bullet point above will generally be subject to U.S. federal income tax on a net income basis at the regular graduated U.S. federal income tax rates. If a non-U.S. holder is eligible for the benefits of a tax treaty between the United States and its country of residence, any such gain will be subject to U.S. federal income tax in the manner specified by the treaty. To claim the benefit of a treaty, a non-U.S. holder must properly submit an IRS Form W-8BEN (or suitable successor or substitute form). A non-U.S. holder that is a foreign corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected gain, as adjusted for certain items.

A non-U.S. holder described in the second bullet point above will be subject to U.S. federal income tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on any gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder (even though the individual is not considered a resident of the United States) provided the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses.

With respect to the third bullet point above, we believe we are not currently and do not anticipate becoming a USRPHC. However, because the determination of whether we are a USRPHC depends on the fair market value of our USRPIs relative to the fair market value of our other business assets and our non-U.S. real property interests, there can be no assurance we are not a USRPHC or will not become one in the future. Even if we are or were to become a USRPHC, gain arising from the sale or other taxable disposition by a non-U.S. holder of our common stock will not be subject to U.S. federal income tax if our common stock is "regularly traded," as defined by applicable Treasury Regulations, on an established securities market, and such non-U.S. holder owned, actually or constructively, 5% or less of our common stock throughout the shorter of the five-year period ending on the date of the sale or other disposition or the non-U.S. holder's holding period.

Non-U.S. holders should consult their tax advisors regarding potentially applicable income tax treaties that may provide for different rules.

Information Reporting and Backup Withholding

A non-U.S. holder will not be subject to backup withholding with respect to payments of dividends on our common stock we make to the non-U.S. holder, provided the holder certifies its non-U.S. status, such as by providing a valid IRS Form W-8BEN, W-8ECI, or other applicable IRS Form, or otherwise establishes an exemption. However, information returns will be filed with the IRS in connection with any dividends on our common stock paid to the non-U.S. holder, regardless of whether any tax was actually withheld. Copies of these information returns may also be made available under the provisions of a specific treaty or agreement to the tax authorities of the country in which the non-U.S. holder resides or is established.

Information reporting and, depending on the circumstances, backup withholding will apply to the proceeds of a sale of our common stock within the United States or conducted through certain U.S.-related financial intermediaries, unless the beneficial owner certifies under penalty of perjury that it is a non-U.S. holder on Form W-8BEN or other applicable form or such owner otherwise establishes an exemption.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a non-U.S. holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

Additional Withholding Tax on Payments Made to Foreign Accounts

Legislation incorporating provisions referred to as the Foreign Account Tax Compliance Act, or "FATCA," was enacted March 18, 2010. A 30% withholding tax may be imposed on dividends paid on, and the gross proceeds from the sale or other disposition of, our common stock paid to a "foreign financial institution" (as defined in the Code) or to a "non-financial foreign entity" (as defined in the Code) (whether such foreign financial institution or non-financial foreign entity is the beneficial owner or an intermediary), unless (1) in the case of a foreign financial institution, the entity undertakes certain diligence and reporting obligations, (2) in the case of a non-financial foreign entity, the entity either certifies that it does not have any "substantial United States owners" (as defined in the Code) or furnishes identifying information regarding each substantial United States owner, or (3) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (1) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertake to identify accounts held by certain U.S. persons or U.S.-owned foreign entities (as defined in applicable Treasury Regulations), annually report certain information about such accounts, and withhold 30% on payments to non-compliant foreign financial institutions and certain other account holders. Foreign governments may enter into an agreement with the IRS to implement FATCA in a different manner. Under current IRS guidance, FATCA withholding will apply to payments of dividends on our common stock made on or after July 1, 2014, and to payments of gross proceeds from the sale or other disposition of such stock on or after January 1, 2017. Prospective investors should consult their tax advisors regarding these withholding provisions.

UNDERWRITING

We and the selling stockholders are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We and the selling stockholders have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we and the selling stockholders have severally agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	806,940
Morgan Stanley & Co. LLC	685,899
Cowen and Company, LLC	423,643
Robert W. Baird & Co. Incorporated	100,867
Total	2,017,349

The underwriters are committed to purchase all the shares of common stock offered by us and the selling stockholders if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated. The shares to be sold by us and the selling stockholders are subject to lock-up restrictions agreed to in connection with our initial public offering, which closed in February 2014. The underwriters for our initial public offering have consented to the release of the lock-up restrictions with respect to the shares to be sold in this offering.

The underwriters propose to offer the shares of common stock directly to the public at the public offering price set forth on the cover of this prospectus and to certain dealers at that price less a concession not in excess of $1.44 per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $1.44 per share from the public offering price. After the public offering of the shares, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

We have granted the underwriters an option to buy up to an additional 302,602 shares of our common stock. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us and the selling stockholders per share of common stock. The underwriting fee is $2.40 per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

Paid by us	Without exercise of option to purchase additional shares	With full exercise of option to purchase additional shares
Per Share	$ 2.40	$ 2.40
Total	$3,147,065	$ 3,873,310

Paid by the selling stockholders	Without exercise of option to purchase additional shares	With full exercise of option to purchase additional shares
Per Share	$ 2.40	$ 2.40
Total	$1,694,573	$ 1,694,573

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be

approximately $460,000. We have agreed to reimburse the underwriters for all expenses relating to the clearance of this offering with the Financial Industry Regulatory Authority (in an amount not to exceed $25,000).

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, or file with the Securities and Exchange Commission a registration statement under the Securities Act of 1933, as amended (the "Securities Act"), relating to, any shares of our common stock or any securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of our common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of our common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC for a period of 90 days after the date of this prospectus, other than (i) the shares of our common stock to be sold hereunder, (ii) any shares of our common stock issued upon the exercise of options granted under our existing management incentive plans or warrants described as outstanding in the registration statement of which this prospectus forms a part, (iii) any options and other awards granted under an equity incentive plan described in the registration statement of which this prospectus forms a part, (iv) our filing of any registration statement on Form S-8 or a successor form thereto relating to an equity incentive plan described in the registration statement of which this prospectus forms a part, and (v) shares of common stock or other securities issued in connection with a transaction with an unaffiliated third party that includes a bona fide commercial relationship (including joint ventures, marketing or distribution arrangements, collaboration agreements or intellectual property license agreements) or any acquisition of assets or acquisition of not less than a majority or controlling portion of the equity of another entity, provided that (x) the aggregate number of shares issued pursuant to this clause (v) shall not exceed five percent (5%) of the total number of outstanding shares of common stock immediately following the issuance and sale of the shares of common stock in this offering and (y) the recipient of any such shares of common stock and securities issued pursuant to this clause (v) during the 90-day restricted period described above shall enter into a lock-up agreement.

Our directors and executive officers as well as the selling stockholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 90 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers and shareholders in accordance with the rules and regulations of the Securities and Exchange Commission and securities which may be issued upon exercise of a stock option or warrant), or publicly disclose the intention to make any offer, sale, pledge or disposition, (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of our common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock. The restrictions described in the immediately preceding paragraph to do not apply to:

- transfers or dispositions of shares of common stock (or any security convertible into or exercisable or exchangeable for common stock):
    - as a bona fide gift;

- to any trust for the direct or indirect benefit of the party subject to the lock-up restrictions or the immediate family of such person;
- to any corporation, partnership, limited liability company, investment fund or other entity controlled or managed, or under common control or management by the party subject to the lock-up restrictions or the immediate family of such person;
- by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the party subject to the lockup restrictions; and
- as distributions to partners, members or stockholders of the party subject to the lock-up restrictions,

provided that in the case of any transfer or distribution pursuant to the above five subclauses, (i) each donee or distributee shall sign and deliver a lock-up letter substantially in the form executed by the party subject to the lock-up restrictions and (ii) no filing under Section 16(a) of the Exchange Act, reporting a reduction in beneficial ownership of shares of common stock, shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);

- sales or transfers of common stock made pursuant to a trading plan pursuant to Rule 10b5-1 under the Exchange Act ("Rule 10b5-1") that has been entered into prior to the date of this prospectus, provided that to the extent a public announcement or filing under the Exchange Act, if any, is required or voluntarily made by or on behalf of the party subject to such lock-up restrictions regarding any such sales or transfers, such announcement or filing shall include a statement to the effect that the sale or transfer was made pursuant to a trading plan pursuant to Rule 10b5-1;
- the establishment of a trading plan pursuant to Rule 10b5-1 for the transfer of shares of common stock, provided that (i) such plan does not provide for the transfer of common stock during the restricted period and (ii) no filing under the Exchange Act or other public announcement shall be required or voluntarily made by or on behalf of the party subject to the lock-up restrictions regarding the establishment of such plan;
- the exercise of options to purchase shares of common stock granted under any stock incentive plan or stock purchase plan of the Company, provided that the underlying shares shall continue to be subject to the restrictions on transfer set forth in this agreement and provided further that no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the exercise (whether for cash, cashless, or net exercise) of warrants to purchase shares of common stock (or any security convertible into or exercisable or exchangeable for common stock), provided that the underlying shares shall continue to be subject to the lock-up restrictions and provided further that, other than in respect of warrants that will expire or automatically exercise by their terms in connection with this offering, no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the transfer of shares of common stock (or any security convertible into common stock) to the Company or sold in connection with a vesting event of the Company's securities or upon the exercise of options to purchase the Company's securities, on a "cashless" or "net exercise" basis or to cover tax withholding obligations of the party subject to the lock-up restrictions in connection with such vesting or exercise provided that no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the transfer or disposition of the shares of common stock (or any security convertible into or exercisable or exchangeable for common stock) held by the party subject to the lock-up restrictions that occurs by operation of law, such as pursuant to a qualified domestic order or in connection with a divorce settlement provided that each transferee shall sign and deliver a lock-up letter substantially in the form executed by the party subject to the lock-up restrictions;

- the transfer of shares of common stock (or any security convertible into or exercisable or exchangeable for common stock) pursuant to a bona fide third party tender offer, merger, consolidation or other similar transaction made to all holders of the common stock involving a change of control of the Company, provided that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the common stock owned by the party subject to the lock-up restrictions shall remain subject to such restrictions;
- transactions by any person other than us relating to shares of common stock or other securities acquired in open market transactions after the completion of the offering of the shares, provided that no filing under Section 16(a) of the Exchange Act is required or voluntarily made in connection with subsequent sales of the common stock or other securities acquired in such open market transactions (other than a filing on Form 5); or
- any securities to be sold pursuant to the underwriting agreement.

We and the selling stockholders have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933, as amended.

Our common stock is listed on The NASDAQ Global Select Market under the symbol “RARE.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be “covered” shorts, which are short positions in an amount not greater than the underwriters’ option to purchase additional shares referred to above, or may be “naked” shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The NASDAQ Global Select Market, in the over-the-counter market or otherwise.

Selling Restrictions

General

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or

any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

United Kingdom

Each underwriter has represented and agreed that:

(1) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the FSMA) received by it in connection with the issue or sale of our common shares in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(2) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to our common shares in, from or otherwise involving the United Kingdom.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State"), an offer to the public of any shares which are the subject of the offering contemplated by this prospectus (the "Shares") may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any Shares may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(1) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(2) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(3) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of Shares shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any Shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any Shares to be offered so as to enable an investor to decide to purchase any Shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or the SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this

prospectus nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or the CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Hong Kong

The shares may not be offered or sold by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), or (ii) to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap.571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA"), (ii) to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 by a relevant person which is: (a) a corporation (which is not an accredited investor) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for 6 months after that corporation or that trust has acquired the shares under Section 275 except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA; (2) where no consideration is given for the transfer; or (3) by operation of law.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (the Financial Instruments and Exchange Law) and each underwriter has agreed that it will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulations and ministerial guidelines of Japan.

LEGAL MATTERS

Gibson, Dunn & Crutcher LLP, San Francisco, California will pass upon the validity of the shares of common stock offered hereby. Certain legal matters in connection with this offering will be passed upon for the underwriters by Latham & Watkins LLP, Costa Mesa, California.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2013, as set forth in their report, which is incorporated by reference in this prospectus and elsewhere in the registration statement. Our financial statements are incorporated by reference in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We are required to file annual, quarterly and special reports, proxy statements and other information with the SEC. You may read and copy any document filed by us at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the public reference room. Our filings with the SEC are also available to the public at the SEC's Internet web site at *http://www.sec.gov*.

We have filed a registration statement, of which this prospectus is a part, covering the securities offered hereby. As allowed by SEC rules, this prospectus does not include all of the information contained in the registration statement and the included exhibits, financial statements and schedules. You are referred to the registration statement, the included exhibits, financial statements and schedules for further information. This prospectus is qualified in its entirety by such other information.

We are subject to the information and periodic reporting requirements of the Exchange Act and, in accordance therewith, file periodic reports, proxy statements and other information with the SEC. Such periodic reports, proxy statements and other information are available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at *www.ultragenyx.com*. The reference to our website address does not constitute incorporation by reference of the information contained on our website, and you should not consider the contents of our website in making an investment decision with respect to our common stock.

INCORPORATION OF CERTAIN INFORMATION BY REFERENCE

The SEC allows us to "incorporate by reference" information from other documents that we file with it, which means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be part of this prospectus. Information in this prospectus supersedes information incorporated by reference that we filed with the SEC prior to the date of this prospectus.

We incorporate by reference into this prospectus and the registration statement of which this prospectus is a part the information or documents listed below that we have filed with the SEC (Commission File No. 001-36276).

- our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 24, 2014;
- our Quarterly Report on Form 10-Q for the quarter ended March 31, 2014, filed with the SEC on May 12, 2014;
- our Definitive Proxy Statement on Schedule 14A, filed with the SEC on May 23, 2014; and
- our Current Reports on Form 8-K filed with the SEC on February 5, 2014 and February 25, 2014 (other than the portions of those reports not deemed to be filed).

We will furnish without charge to you, on written or oral request, a copy of any or all of the documents incorporated by reference, including exhibits to these documents. You should direct any requests for documents to Ultragenyx Pharmaceutical Inc., 60 Leveroni Court, Novato, California, 94949. Copies of the above reports may also be accessed from our web site at *http://www.ultragenyx.com*.

Any statement contained in a document incorporated or deemed to be incorporated by reference in this prospectus will be deemed modified, superseded or replaced for purposes of this prospectus to the extent that a statement contained in this prospectus modifies, supersedes or replaces such statement.

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2,017,349 shares

Common Stock

Prospectus

J.P. Morgan **Morgan Stanley**

Cowen and Company

Baird

July 8, 2014