LA JOLLA PHARMACEUTICAL CO
Form 10-K
March 16, 2015

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

x ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2014

OR

o TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission file number: 0-24274

LA JOLLA PHARMACEUTICAL COMPANY
(Exact name of registrant as specified in its charter)

California	33-0361285
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

4660 La Jolla Village Drive, Suite 1070, San Diego, California, 92122
(Address of principal executive offices, including Zip Code)

Registrant's telephone number, including area code: (858) 207-4264

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

Title of each class	Name of each exchange on which registered
Common Stock, Par Value $0.0001 per share	The NASDAQ Capital Market

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

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

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

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

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

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

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

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

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

The aggregate market value of voting and non-voting common stock held by non-affiliates of the registrant as of June 30, 2014 totaled approximately $76,360,000. As of February 27, 2015, there were 15,243,340 shares of the Company's common stock, $0.0001 par value, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

None.

Table of Contents

EXPLANATORY NOTE

The registrant meets the accelerated filer requirements as of the end of its 2014 fiscal year pursuant to Rule 12b-2 of the Securities Exchange Act of 1934, as amended, or Exchange Act. However, pursuant to Rule 12b-2 and SEC Release No. 33-8876, the Registrant (as a smaller reporting company transitioning to the larger reporting company system) is not required to satisfy the larger reporting company disclosure requirements until its first quarterly report on Form 10-Q for the 2015 fiscal year.

TABLE OF CONTENTS

Table of Contents

FORWARD-LOOKING STATEMENTS

This report contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as “intends,” “believes,” “anticipates,” “indicates,” “plans,” “intends,” “expects,” “suggests,” “may,” “should,” “potential,” “designed to,” “will” and similar references. Such statements in but are not limited to, statements about: our ability to successfully develop LJPC-501, GCS-100, LJPC-1010, LJPC-401 and our other product candidates (collectively our "product candidates"); the future success of our clinical trials with our product candidates; the timing for the commencement and completion of clinical trials; and our ability to obtain orphan status, break-through status or other regulatory designations with respect to any of our product candidates. Forward-looking statements are neither historical facts nor assurances of future performance. These statements are based only on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, projections, anticipated events and trends, the economy and other future conditions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict and many of which are outside of our control. Our actual results and financial condition may differ materially from those indicated in the forward-looking statements. Therefore, you should not rely on any of these forward-looking statements.

Important factors that could cause our actual results and financial condition to differ materially from those indicated in the forward-looking statements include, among others:

- the risk that our clinical trials with our product candidates may not be successful in evaluating their safety and tolerability or providing evidence of efficacy;
- the successful and timely completion of clinical trials;
- our plans and timing with respect to seeking regulatory approvals and uncertainties regarding the regulatory process;
- the availability of funds and resources to pursue our research and development projects, including clinical trials with our product candidates;
- uncertainties associated with obtaining and enforcing patents;
- the potential commercialization of any of our drug candidates that receive regulatory approval;
- our estimates for future performance;
- our estimates regarding our capital requirements and our needs for, and ability to obtain, additional financing; and
- those risk factors identified in this Annual Report on Form 10-K under the heading “Risk Factors” and in other filings the Company periodically makes with the Securities and Exchange Commission.

Forward-looking statements contained in this Annual Report on Form 10-K speak as of the date hereof and the Company does not undertake to update any of these forward-looking statements to reflect a change in its views or events or circumstances that occur after the date of this Annual Report on Form 10-K. In Addition Please see the "Risk Factors" section of this Annual Report on Form 10-K. These risk factors may be updated from time to time by our future filings under the Exchange Act.

Table of Contents

PART I

In this report, all references to "we," "our," "us," "La Jolla" and "the Company" refer to La Jolla Pharmaceutical Company, a California corporation.

Item 1. Business

Overview

La Jolla Pharmaceutical Company is a biopharmaceutical company focused on the discovery, development and commercialization of innovative therapies intended to significantly improve outcomes in patients suffering from life-threatening diseases. We have four product candidates in development. LJPC-501 is our proprietary formulation of angiotensin II for the potential treatment of catecholamine-resistant hypotension and hepatorenal syndrome. GCS-100 is our first-in-class galectin-3 inhibitor for the potential treatment of chronic kidney disease. LJPC-1010, our second-generation galectin-3 inhibitor, is a more potent and purified derivative of GCS-100 that can be delivered orally for the potential treatment of nonalcoholic steatohepatitis and other diseases characterized by tissue fibrosis. LJPC-401 is our novel formulation of hepcidin for the potential treatment of conditions characterized by iron overload, such as hemochromatosis and beta thalassemia.

LJPC-501

Catecholamine-Resistant Hypotension

LJPC-501 is our proprietary formulation of angiotensin II. Angiotensin II, the major bioactive component of the renin-angiotensin system, serves as one of the body’s central regulators of blood pressure. We are developing LJPC-501 for the treatment of catecholamine-resistant hypotension, or CRH, which is an acute, life-threatening condition in which blood pressure drops to dangerously low levels and is poorly responsive to current treatments. Angiotensin II has been shown to raise blood pressure in a randomized, placebo-controlled clinical trial in CRH, as well as in animal models of hypotension. In October 2014, we presented positive data from a preclinical study of LJPC-501 for the treatment of CRH.

We plan to initiate a Phase 3 clinical trial with LJPC-501 for the treatment of CRH, called the Athos3 trial, in the first quarter of 2015. In February 2015, we reached agreement with the FDA on a Special Protocol Assessment, or SPA, for this multicenter, randomized, double-blind, placebo-controlled, Phase 3 clinical trial. In accordance with the SPA, the primary efficacy endpoint for the Athos3 registration trial will be increase in blood pressure. The Athos3 trial is designed to enroll approximately 315 patients. Patients will be randomized in a 1:1 fashion to receive either: (i) LJPC-501 plus standard-of-care vasopressors; or (ii) placebo plus standard-of-care vasopressors. Randomized patients will receive their assigned treatment via continuous IV infusion for up to 7 days. The primary efficacy endpoint in the study is to compare the change in mean arterial pressure in patients with CRH who receive an IV infusion of LJPC-501 plus standard-of-care vasopressors to those that receive placebo plus standard-of-care vasopressors. Secondary endpoints include comparison of changes in Sequential Organ Failure Assessment, or SOFA scores, and the safety and tolerability LJPC-501 in patients with CRH.

Hepatorenal Syndrome

We are also developing LJPC-501 for hepatorenal syndrome, or HRS. HRS is a life-threatening form of progressive renal failure in patients with liver cirrhosis or fulminant liver failure. In these patients, the diseased liver secretes vasodilator substances (e.g., nitric oxide and prostaglandins) into the bloodstream that cause under-filling of blood

vessels. This low blood pressure state causes a reduction in blood flow to the kidneys. As a means to restore systemic blood pressure, the kidneys induce both sodium and water retention, which contribute to ascites, a major complication associated with HRS. Studies have shown that LJPC-501 may improve renal function in patients with conditions similar to HRS. We are currently enrolling patients in a Phase 1/2 clinical trial of LJPC-501 in HRS.

Table of Contents

GCS-100

GCS-100 is our first-in-class galectin-3 inhibitor. GCS-100 is a complex polysaccharide derived from pectin that binds to, and blocks the activity of, the pro-fibrotic mediator galectin-3. Over-expression of galectin-3 has been implicated in a number of human diseases characterized by progressive tissue fibrosis, such as chronic kidney disease, or CKD. In 2010, the United States Renal Data System estimated that 49 million adults in the United States suffered from CKD. As described in more detail below, we have recently completed a multicenter, randomized, placebo-controlled, Phase 2 clinical trial in advanced CKD patients, in which treatment with GCS-100 resulted in a statistically significant improvement in kidney function compared to placebo. We plan to initiate a large, multicenter, randomized, placebo-controlled, Phase 2b clinical trial of GCS-100 in CKD in the first quarter of 2015.

Phase 2 Clinical Trial of GCS-100 in Advanced CKD

In November 2014, we presented positive results from our randomized, placebo-controlled, Phase 2 trial of GCS-100 in CKD at the American Society of Nephrology's Annual Kidney Week. The trial met its primary efficacy endpoint of a statistically significant improvement in kidney function. Specifically, a dose of 1.5 mg/m^2 led to a statistically significant ($p=0.045$) increase in estimated glomerular filtration rate, or eGFR, compared to placebo between baseline and end of treatment. This improvement, on a placebo-corrected basis, was maintained at 5 weeks following the completion of dosing ($p=0.07$). At the 30 mg/m^2 dose, there was no statistically significant difference. The lack of consistent response in the 30 mg/m^2 group may be due to off-target drug effects, as this dose is 1,400-fold in excess, on a molar basis, versus known circulating galectin-3 levels. Off-target effects may include antagonizing other galectins like galectin-9, which has opposing biological effects to galectin-3.

GCS-100's effect on eGFR in this Phase 2 trial was more pronounced ($p=0.029$) in the prospectively defined subset of patients with diabetic etiology. Analysis of this subset was predefined based on the observation that galectin-3 is elevated in diabetes patients and that galectin-3 levels correlate with proteinuria (a marker of kidney health) in these patients.

Key secondary endpoints were also met, and the effect on circulating galectin-3 levels was consistent with the effect on eGFR. For the 1.5 mg/m^2 dose, there was a statistically significant ($p=0.067$) reduction in circulating levels of galectin-3, while there was no significant difference at the 30 mg/m^2 dose level. Potassium, uric acid and blood urea nitrogen, or BUN, all improved at the 1.5 mg/m^2 dose level.

GCS-100 was well-tolerated. Out of 121 patients enrolled, 117 completed treatment, including all 41 patients treated at the 1.5 mg/m^2 dose. There were no serious adverse events, or SAEs, in the 1.5 mg/m^2 dose group compared to two in the placebo group and two in the 30 mg/m^2 group. All SAEs were deemed by the investigators as not drug-related.

Phase 2b Clinical Trial of GCS-100 in Advanced CKD with Diabetes

We plan to initiate a Phase 2b clinical trial in advanced CKD patients with diabetes in the first quarter of 2015. The Phase 2b clinical trial will be a double-blind, multicenter, placebo-controlled, randomized trial of GCS-100 in diabetic patients with Stage 3b or 4 CKD. The clinical trial is designed to enroll approximately 375 patients. Patients will be randomized 1:1:1:1 to receive fixed doses of GCS-100 (1, 3 or 9 mg) or placebo. Randomized patients will receive their assigned treatment via IV injection once a week for 8 weeks and then once every other week for an additional 16 weeks.

The primary endpoint of this Phase 2b clinical trial is to compare the change in kidney function, as measured by eGFR, from baseline to week 26, which is 2 weeks after the last injection, between patients receiving GCS-100 or

placebo. Secondary efficacy endpoints include a responder analysis based on pre-specified percentage changes in eGFR and an analysis on progression to renal replacement therapy. Other secondary endpoints are focused on the long-term safety and tolerability of GCS-100, including an evaluation of the incidence of major cardiac events.

LJPC-1010

LJPC-1010 is our second-generation galectin-3 inhibitor. LJPC-1010 is a more potent and purified derivative of GCS-100 that can be delivered orally. We are developing LJPC-1010 for the treatment of nonalcoholic steatohepatitis, or NASH, and other diseases characterized by tissue fibrosis. NASH is the more serious form of nonalcoholic fatty liver disease, or NAFLD, which can lead to liver failure. In July 2014, we announced positive preclinical data of LJPC-1010 in NASH. We plan to file an Investigational New Drug Application, or IND, with the FDA and initiate a Phase 1 clinical trial of LJPC-1010 in the second quarter of 2015.

Table of Contents

LJPC-401

LJPC-401 is our novel formulation of hepcidin. Hepcidin is a naturally occurring peptide hormone that controls and regulates iron metabolism. By suppressing iron release, hepcidin prevents iron accumulation in tissues, such as the heart, where it can cause significant damage and even result in death. We are developing LJPC-401 for the treatment of conditions characterized by iron overload, such as hemochromatosis and beta thalassemia. We expect to file an IND and commence a Phase 1 clinical trial of LJPC-401 in the second half of 2015.

Manufacturing

We do not currently own or operate manufacturing facilities for the production of clinical or commercial quantities of any of our product candidates. We rely on a small number of third-party manufacturers to produce our compounds and expect to continue to do so to meet the preclinical and clinical requirements of our potential product candidates and for all of our commercial needs. We do not have long-term agreements with any of these third parties. We require in our manufacturing and processing agreements that all third-party contract manufacturers and processors produce active pharmaceutical ingredients, or API, and finished products in accordance with the FDA's current Good Manufacturing Practices, or cGMP, and all other applicable laws and regulations. We maintain confidentiality agreements with potential and existing manufacturers in order to protect our proprietary rights related to our drug candidates.

With regard to our lead product candidate, LJPC-501, we use third parties to supply API and to formulate, fill and finish our final product. After sourcing the API for LJPC-501 from independent suppliers, we use different third parties to formulate the bulk drug product and complete the process by filling bulk drug product into vials. To date, LJPC-501 has been manufactured in small quantities for preclinical studies and clinical trials. If LJPC-501 is approved for commercial sale, we will need to manufacture the product in larger quantities. Significant scale-up of manufacturing requires additional process development and validation studies, which the FDA must review and approve. We are currently starting the process of completing this scale-up and validation work. If approved, the commercial success of LJPC-501, in the near-term, will be dependent upon the ability of our contract manufacturers to produce product in commercial quantities at competitive costs of manufacture. If LJPC-501 receives regulatory approval, we plan to scale-up manufacturing through our third-party manufacturers with the objective of realizing important economies of scale. These scale-up activities will take time to implement, require additional capital investment, process development, validation studies and FDA approval. We cannot guarantee that we will be successful in achieving competitive manufacturing costs through such scale-up activities.

Patents and Proprietary Technologies

Patents and other proprietary rights are important to our business. As part of our strategy to protect our current product candidates and to provide a foundation for future products, we have filed a number of patent applications and have licensed rights from third parties in other patent applications related to our product candidates.

We own two U.S. patent applications and one international application covering methods of use for LJPC-501. Our license with the George Washington University provides rights in a U.S. application and an international application directed to methods of using LJPC-501. These applications, if issued as patents, will have expiration dates in 2034 or 2035. Please refer to Note 3 to the accompanying financial statements included in Item 15 of this Annual Report on Form 10-K.

We own: (i) eight issued patents and three pending patent applications in the United States; (ii) one pending patent application in Canada; and (iii) one pending patent application in Europe, related to GCS-100. The issued patents

protect GCS-100 and will expire between March 2025 and March 2028, not taking into account any potential patent term extensions that may be available in the future. The pending applications include U.S. applications directed to compositions and methods of use of GCS-100 that, if issued as patents, will expire between April 2024 and 2025, and a provisional application directed to methods of use of GCS-100 that will support U.S. and foreign applications that, if issued as patents, will expire in March 2035.

Our license from Inserm in France provides rights in a portfolio of patents and applications covering methods of use of LJPC-401. This portfolio includes one issued U.S. patent, one pending U.S. application, issued patents in Canada, China, Europe, and Japan, and pending applications in Europe, China, and Japan. The issued U.S. patent will expire in May 2022.

In addition to the above, we plan to file additional patent applications that, if issued, would provide further protection for LJPC-501, GCS-100, LJPC-1010 and LJPC-401.

Table of Contents

Although we believe the bases for these patents and patent applications are sound, they are untested, and there is no assurance that they will not be successfully challenged. There can be no assurance that any patent previously issued will be of commercial value, that any patent applications will result in issued patents of commercial value, or that our technology will not be held to infringe patents held by others.

Competition

The biotechnology and pharmaceutical industries are subject to rapid technological change. Competition from domestic and foreign biotechnology companies, large pharmaceutical companies and other institutions is intense and expected to increase. A number of companies are pursuing the development of pharmaceuticals in our targeted areas. These include companies that are conducting preclinical studies and clinical trials in the field of galectin mediation, including Galectin Therapeutics Inc. and Galecto Biotech AB. These also include companies that are conducting preclinical studies in the field of treating iron overload with hepcidin-derived compounds, including Merganser Biotech, Inc.

Government Regulation

United States

Our research and development activities and the future manufacturing and marketing of any products we develop are subject to significant regulation by numerous government authorities in the United States and other countries. In the United States, the Federal Food, Drug and Cosmetic Act and the Public Health Service Act govern the testing, manufacture, safety, efficacy, labeling, storage, record keeping, approval, advertising and promotion, and distribution of our drug candidates and any products we may develop. In addition, this regulatory framework is subject to changes that may adversely affect approval, delay an application or require additional expenditures.

The steps required before a pharmaceutical compound may be marketed in the United States include: preclinical laboratory and animal testing; submission of an IND to the FDA, which must become effective before clinical trials may commence; conducting adequate and well-controlled clinical trials to establish the safety and efficacy of the drug; submission of a New Drug Application, or NDA, or Biologics License Application, or BLA, to the FDA; satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities to assess compliance with cGMP; and FDA approval of the NDA or BLA prior to any commercial sale or shipment of the drug. In addition to obtaining FDA approval for each product, each drug-manufacturing establishment used must be registered with the FDA and be operated in conformity with cGMP. Drug product manufacturing facilities may also be subject to state and local regulatory requirements.

Preclinical testing includes laboratory evaluation of product chemistry and animal studies to assess the safety and efficacy of the product and its formulation. The results of preclinical testing are submitted to the FDA as part of an IND, and, unless the FDA objects, the IND becomes effective 30 days following its receipt by the FDA.

Clinical trials involve administration of the study drug to healthy volunteers and to patients diagnosed with the condition for which the study drug is being tested under the supervision of qualified clinical investigators. Clinical trials are conducted in accordance with protocols that detail the objectives of the study, the parameters to be used to monitor safety and the efficacy criteria to be evaluated. Each protocol is submitted to the FDA as part of the IND. Each clinical trial is conducted under the auspices of an independent Institutional Review Board, or IRB, in the United States, or Ethics Committee, or EC, outside the United States, for each trial site. The IRB or EC considers, among other matters, ethical factors and the safety of human clinical trial subjects.

Clinical trials are typically conducted in three sequential phases, but the phases may overlap or be repeated. In Phase 1 clinical trials, the drug is initially introduced into healthy human subjects or patients and is tested for adverse effects, dosage tolerance, pharmacokinetics, and clinical pharmacology. Phase 2 clinical trials involve the testing of a limited patient population in order to characterize the actions of the drug in targeted indications, in order to determine drug tolerance and optimal dosage and to identify possible adverse side effects and safety risks. When a compound appears to be effective at a specific dosage and have an acceptable safety profile in Phase 2 clinical trials, Phase 3 clinical trials are undertaken to further evaluate and confirm clinical efficacy and safety within an expanded patient population at multiple clinical trial sites. The FDA reviews the clinical plans and monitors the results of the trials and may discontinue the trials at any time if significant safety issues arise. Similarly, an IRB or EC may suspend or terminate a trial at a study site that is not being conducted in accordance with the IRB or EC's requirements or that has been associated with unexpected serious harm to subjects.

Table of Contents

The results of preclinical testing and clinical trials are submitted to the FDA for marketing approval in the form of an NDA or BLA. The submission of an NDA or BLA also requires the payment of user fees, but a waiver of the fees may be obtained under specified circumstances. The testing and approval process is likely to require substantial time, effort and resources and there can be no assurance that any approval will be granted on a timely basis, if at all, or that conditions of any approval, such as warnings, contraindications, or scope of indications will not materially impact the potential market acceptance and profitability of the drug product. Data obtained from clinical trials are not always conclusive, and the FDA may interpret data differently than we interpret the same data. The FDA may refer the application to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it generally follows such recommendations. The approval process is affected by a number of factors, including the severity of the disease, the availability of alternative treatments and the risks and benefits of the product demonstrated in clinical trials.

Additional preclinical testing or clinical trials may be requested during the FDA review period and may delay any marketing approval. After FDA approval for the initial indications, further clinical trials may be necessary to gain approval for the use of the product for additional indications. In addition, after approval, certain types of changes to the approved product, such as manufacturing changes, are subject to further FDA review and approval. The FDA mandates that adverse effects be reported to the FDA, and the regulatory agency may also require post-marketing testing to continue monitoring for expected and unexpected adverse effects, which can involve significant expense. Adverse effects observed during the commercial use of a drug product or which arise in the course of post-marketing studies can result in the need for labeling revisions, including additional warnings and contraindications; and if the findings significantly alter the risk/benefit assessment, the potential withdrawal of the drug from the market.

Among the conditions for FDA approval is the requirement that the prospective manufacturer’s quality control and manufacturing procedures conform to the FDA’s cGMP requirements. Domestic manufacturing facilities are subject to biannual FDA inspections and foreign manufacturing facilities are subject to periodic inspections by the FDA or foreign regulatory authorities. If the FDA finds that a company is not operating in compliance with cGMPs, the continued availability of the product can be interrupted until compliance is achieved; and if the deficiencies are not corrected within a reasonable time frame, the drug could be withdrawn from the market. In addition, the FDA strictly regulates labeling, advertising and promotion of drugs. Failure to conform to requirements relating to licensing, manufacturing and promoting drug products can result in informal or formal sanctions, including warning letters, injunctions, seizures, civil and criminal penalties, adverse publicity and withdrawal of approval.

Foreign

We are also subject to numerous and varying foreign regulatory requirements governing the design and conduct of clinical trials and marketing approval for pharmaceutical products to be marketed outside of the United States. The approval process varies among countries and regions and can involve additional testing; and the time required to obtain approval may differ from that required to obtain FDA approval.

The steps to obtain approval to market a pharmaceutical compound in the European Union include: preclinical laboratory and animal testing; conducting adequate and well-controlled clinical trials to establish safety and efficacy; submission of a Marketing Authorization Application, or MAA; and the issuance of a product marketing license by the European Commission prior to any commercial sale or shipment of drug. In addition to obtaining a product marketing license for each product, each drug manufacturing establishment must be registered with the European Medicines Agency, or EMA, must operate in conformity with European good manufacturing practice and must pass inspections by the European health authorities.

Upon receiving the MAA, the Committee for Human Medicinal Products, or CHMP, a division of the EMA, will review the MAA and may respond with a list of questions or objections. Answers to questions posed by the CHMP may require additional tests to be conducted. Responses to the list of questions or objections must be provided to and deemed sufficient by the CHMP within a defined time frame. Ultimately, a representative from each of the European Member States will vote whether to approve the MAA.

Foreign regulatory approval processes include all of the risks associated with obtaining FDA approval, and approval by the FDA does not ensure approval by the health authorities of any other country.

Table of Contents

Employees

As of February 27, 2015, we employed 25 regular full-time employees, 18 of whom are engaged in research and clinical development activities, and 7 of whom are in finance, information technology, human resources and administration.

None of our employees are covered by a collective bargaining agreement.

Company Information

La Jolla was incorporated in Delaware in 1989 and reincorporated in California in 2012.

On January 29, 2014, our common stock was approved for listing and began trading on The NASDAQ Capital Market under the symbol LJPC.

Our principal offices are located at 4660 La Jolla Village Drive, Suite 1070, San Diego, CA 92122. Our telephone number is (858) 207-4264. Our website address is www.ljpc.com.

Available Information

You are advised to read this Annual Report on Form 10-K in conjunction with other reports and documents that we file from time to time with the Securities and Exchange Commission, or SEC. In particular, please read our definitive proxy statements, our Quarterly Reports on Form 10-Q and any Current Reports on Form 8-K that we may file from time to time. You may obtain copies of these reports after the date of this annual report directly from us or from the SEC at the SEC's Public Reference Room at 100 F Street, N.E. Washington, D.C. 20549. In addition, the SEC maintains information for electronic filers (including us) at its website at www.sec.gov. The public may obtain information regarding the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. We make our periodic and current reports available on our internet website at www.ljpc.com, free of charge, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

Item 1A. Risk Factors

An investment in our common stock involves a high degree of risk. You should carefully consider the risks described below and the other information before deciding to invest in our common stock. The risks described below are not the only ones facing our Company. Additional risks not presently known to us or that we currently consider immaterial may also adversely affect our business. We have attempted to identify below the major factors that could cause differences between actual and planned or expected results, but we cannot assure you that we have identified all of those factors.

If any of the following risks actually happen, our business, financial condition and operating results could be materially adversely affected. In this case, the trading price of our common stock could decline, and you could lose all or part of your investment.

I. RISK FACTORS RELATING TO THE COMPANY AND THE INDUSTRY IN WHICH WE OPERATE.

We have only limited assets and will need to raise additional capital before we can expect to become profitable.

As of December 31, 2014, we had no revenue sources, an accumulated deficit of $486.6 million and available cash and cash equivalents of approximately $48.6 million. However, to fund future operations to the point where we are

able to generate positive cash flow from the sales or out-licensing of our drug candidates, we will need to raise significant additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support, as well as the overall condition of capital markets, including capital markets for development-stage biopharmaceutical companies. We anticipate that we will seek to fund our operations through public and private equity and debt financings or other sources, such as potential collaboration agreements. We cannot assure you that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through equity securities offerings, there can be no assurance that we will be able to do so in the future. If we are unable to raise additional capital to fund our clinical development and other business activities, we could be forced to abandon one or more programs and curtail or cease our operations.

Table of Contents

We have never generated any revenue from product sales and may never be profitable.

We have no products approved for commercialization and have never generated any revenue from product sales. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and nonclinical and clinical development of our product candidates;
- obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- obtaining market acceptance of our product candidates as viable treatment options;
- addressing any competing technological and market developments;
- identifying, assessing, acquiring or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory agencies, domestic or foreign, to change our manufacturing processes or assays, or to perform clinical, nonclinical or other types of studies in addition to those that we currently anticipate. In cases where we are successful in obtaining regulatory approvals to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price, and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

The technology underlying our compounds is uncertain and unproven.

The development efforts for LJPC-501, GCS-100, LJPC-1010 and LJPC-401 are based on unproven technologies and therapeutic approaches that have not been widely tested or used. To date, no products that use the technology underlying these drug candidates have been approved or commercialized. Application of our technology to treat life-threatening diseases is in early stages. Preclinical studies and future clinical trials of these product candidates may be viewed as a test of our entire approach to developing therapies for patients suffering from life-threatening diseases. If our product candidates do not work as intended, or if the data from our future clinical trials indicate that our product candidates are not safe and effective, the applicability of our technology for successfully treating life-threatening diseases will be highly uncertain. As a result, there is a significant risk that our therapeutic approaches will not prove to be successful, and there can be no guarantee that our drug technologies will result in any commercially successful products.

Results from any future clinical trials we may undertake may not be sufficient to obtain regulatory approvals to market our drug candidates in the United States or other countries on a timely basis, if at all.

Drug candidates are subject to extensive government regulations related to development, clinical trials, manufacturing and commercialization. In order to sell any product that is under development, we must first receive regulatory approval. To obtain regulatory approval, we must conduct clinical trials and toxicology studies that demonstrate that our drug candidates are safe and effective. The process of obtaining FDA and foreign regulatory approvals is costly, time-consuming, uncertain and subject to unanticipated delays.

Table of Contents

The FDA and foreign regulatory authorities have substantial discretion in the approval process and may not agree that we have demonstrated that our drug candidates are safe and effective. If our drug candidates are ultimately not found to be safe and effective, we would be unable to obtain regulatory approval to manufacture, market and sell them. We can provide no assurances that the FDA or foreign regulatory authorities will approve our drug candidates or, if approved, what the scope of the approved indication might be.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies may not be predictive of future study results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of nonclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates that have shown promising results in early-stage clinical trials may still suffer significant setbacks in subsequent clinical trials. For example, the safety or efficacy results generated to date in clinical trials for GCS-100 do not ensure that later clinical trials will demonstrate similar results. There is a high failure rate for drugs proceeding through clinical trials, and product candidates in later stages of clinical trials may fail to show the desired safety and efficacy, despite having progressed through nonclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses. We do not know whether any clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain regulatory approval to market our drug candidates.

Future clinical trials that we may undertake may be delayed or halted.

Any clinical trials of our drug candidates that we may conduct in the future may be delayed or halted for various reasons, including:

- we do not have sufficient financial resources;
- supplies of drug product are not sufficient to treat the patients in the studies;
- patients do not enroll in the studies at the rate we expect;
- the product candidates are not effective;
- patients experience negative side effects or other safety concerns are raised during treatment;
- the trials are not conducted in accordance with applicable clinical practices;
- there is political unrest at foreign clinical sites; or
- there are natural disasters at any of our clinical sites.

If any future trials are delayed or halted, we may incur significant additional expenses, and our potential approval of our drug candidates may be delayed, which could have a severe negative effect on our business.

Table of Contents

We rely on third parties to conduct our preclinical and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have agreements with third-party contract research organizations, or CROs, to monitor and manage data for our preclinical and clinical programs. We rely heavily on these parties for execution of our preclinical and clinical trials, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with current good clinical practice, or cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for products in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fails to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with product produced under current good manufacturing practices, or cGMP regulations, and will require a large number of test subjects. Our or our CROs' failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, we may incur significant additional expenses, and our potential approval of our drug candidates may be delayed, which could have a severe negative effect on our business.

If the third-party manufacturers upon which we rely fail to produce our drug candidates that we require on a timely basis, or to comply with stringent regulations app