

LA JOLLA PHARMACEUTICAL CO  
Form 10-Q  
July 27, 2017

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549

FORM 10-Q

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

For the quarterly period ended June 30, 2017

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

For the transition period from to  
Commission file number: 1-36282

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 No.)

10182 Telesis Court, 6th Floor, San Diego, CA 92121  
(Address of principal executive offices) (Zip Code)

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

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

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

Large accelerated filer  Accelerated filer   
Non-accelerated filer  Smaller reporting company   
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act.

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

As of July 21, 2017, La Jolla Pharmaceutical Company had 22,123,456 shares of common stock, \$0.0001 par value per share, outstanding.

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LA JOLLA PHARMACEUTICAL COMPANY  
FORM 10-Q  
QUARTERLY REPORT

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## PART I. FINANCIAL INFORMATION

## ITEM 1. CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

## LA JOLLA PHARMACEUTICAL COMPANY

## Condensed Consolidated Balance Sheets

(in thousands, except share and par value amounts)

	June 30, 2017	December 31, 2016
	(Unaudited)	
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 141,317	\$ 65,726
Restricted cash	910	200
Prepaid expenses and other current assets	1,775	1,505
Total current assets	144,002	67,431
Property and equipment, net	4,585	3,145
Other assets	—	219
Total assets	\$ 148,587	\$ 70,795
<b>LIABILITIES AND SHAREHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 5,920	\$ 6,652
Accrued clinical and other expenses	488	1,029
Accrued payroll and related expenses	1,863	2,077
Total current liabilities	8,271	9,758
Shareholders' equity:		
Common Stock, \$0.0001 par value; 100,000,000 shares authorized, 22,123,456 and 18,261,557 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	2	2
Series C-1 <sup>2</sup> Convertible Preferred Stock, \$0.0001 par value; 11,000 shares authorized, 3,906 shares issued and outstanding at June 30, 2017 and December 31, 2016, and liquidation preference of \$3,906 at June 30, 2017 and December 31, 2016	3,906	3,906
Series F Convertible Preferred Stock, \$0.0001 par value; 10,000 shares authorized, 2,737 shares issued and outstanding at June 30, 2017 and December 31, 2016, and liquidation preference of \$2,737 at June 30, 2017 and December 31, 2016	2,737	2,737
Additional paid-in capital	790,351	661,103
Accumulated deficit	(656,680)	(606,711)
Total shareholders' equity	140,316	61,037
Total liabilities and shareholders' equity	\$ 148,587	\$ 70,795

See accompanying notes to the condensed consolidated financial statements.

LA JOLLA PHARMACEUTICAL COMPANY  
 Unaudited Condensed Consolidated Statements of Operations  
 (in thousands, except per share amounts)

	Three Months Ended		Six Months Ended	
	June 30,		June 30	
	2017	2016	2017	2016
Revenue				
Contract revenue - related party	\$—	\$253	\$—	\$487
Total revenue	—	253	—	487
Expenses				
Research and development	20,808	12,404	38,573	25,119
General and administrative	6,022	3,466	11,525	7,519
Total expenses	26,830	15,870	50,098	32,638
Loss from operations	(26,830 )	(15,617 )	(50,098 )	(32,151 )
Other income, net	101	51	129	104
Net loss	\$(26,729)	\$(15,566)	\$(49,969)	\$(32,047)
Basic and diluted net loss per share	\$(1.21 )	\$(0.90 )	\$(2.46 )	\$(1.86 )
Weighted average common shares outstanding - basic and diluted	22,123	17,211	20,277	17,211

See accompanying notes to the condensed consolidated financial statements.

LA JOLLA PHARMACEUTICAL COMPANY  
 Unaudited Condensed Consolidated Statements of Cash Flows  
 (in thousands)

	Six Months Ended June 30,	
	2017	2016
Operating activities		
Net loss	\$(49,969)	\$(32,047)
Adjustments to reconcile net loss to net cash used for operating activities:		
Share-based compensation expense	9,330	6,960
Third party share-based compensation expense	364	86
Depreciation expense	581	310
Loss on disposal of equipment	—	75
Changes in operating assets and liabilities:		
Restricted cash	(710	) 37
Prepaid expenses and other current assets	(270	) (702
Other assets	219	(147
Accounts payable	(732	) (250
Accrued clinical and other expenses	(541	) 888
Accrued payroll and related expenses	(214	) (295
Net cash used for operating activities	(41,942	) (25,085
Investing activities		
Purchase of property and equipment	(2,021	) (910
Net cash used for investing activities	(2,021	) (910
Financing activities		
Net proceeds from the issuance of common stock	117,480	—
Proceeds from the exercise of stock options for common stock	2,074	85
Net cash provided by financing activities	119,554	85
Net increase (decrease) in cash and cash equivalents	75,591	(25,910
Cash and cash equivalents at beginning of period	65,726	126,467
Cash and cash equivalents at end of period	\$ 141,317	\$ 100,557

See accompanying notes to the condensed consolidated financial statements.

LA JOLLA PHARMACEUTICAL COMPANY

Notes to Condensed Consolidated Financial Statements  
(Unaudited)

June 30, 2017

1. Business

La Jolla Pharmaceutical Company (collectively with its subsidiaries, the 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. The Company has several product candidates in development. LJPC-501 is the Company's proprietary formulation of synthetic human angiotensin II for the potential treatment of catecholamine resistant hypotension. LJPC-401 is the Company's proprietary formulation of synthetic human hepcidin for the potential treatment of conditions characterized by iron overload, such as hereditary hemochromatosis, beta thalassemia, sickle cell disease and myelodysplastic syndrome. LJPC-30S is the Company's next-generation gentamicin derivative program that is focused on the potential treatment of serious bacterial infections as well as rare genetic disorders, such as cystic fibrosis and Duchenne muscular dystrophy. The Company was incorporated in 1989 as a Delaware corporation and reincorporated in California in 2012.

As of June 30, 2017, the Company had \$141.3 million in cash and cash equivalents. Based on our current operating plans and projections, management believes that available cash and cash equivalents are sufficient to fund operations for at least one year from the date this Quarterly Report on Form 10-Q is filed with the U.S. Securities and Exchange Commission (SEC).

2. Summary of Significant Accounting Policies

During the three and six months ended June 30, 2017, there have been no changes to the Company's significant accounting policies as described in the Annual Report on Form 10-K for the fiscal year ended December 31, 2016.

Basis of Presentation and Use of Estimates

The accompanying unaudited condensed consolidated financial statements of the Company have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and with the instructions to Form 10-Q and Article 10 of the SEC Regulation S-X. Accordingly, they should be read in conjunction with the audited financial statements and notes thereto for the year ended December 31, 2016, included in the Company's Annual Report on Form 10-K filed with the SEC on February 23, 2017. The accompanying unaudited condensed consolidated financial statements include the accounts of La Jolla Pharmaceutical Company and its wholly-owned subsidiaries. All significant inter-company transactions and balances have been eliminated in consolidation. The unaudited condensed consolidated financial statements contain all normal recurring accruals and adjustments that, in the opinion of management, are necessary to present fairly the condensed consolidated balance sheet of the Company at June 30, 2017, the condensed consolidated statement of operations for the three and six months ended June 30, 2017, and the condensed consolidated statement of cash flows for the six months ended June 30, 2017. Estimates were made relating to useful lives of fixed assets, valuation allowances, impairment of assets, share-based compensation expense and accruals for clinical studies and research and development expenses. Actual results could differ materially from those estimates. Certain amounts previously reported in the financial statements have been reclassified to conform to the current presentation. Such reclassifications did not affect net loss, shareholders' equity or cash flows. The results of operations for the three and six months ended June 30, 2017 are not

necessarily indicative of the results to be expected for the full year or any future interim periods. The accompanying condensed consolidated balance sheet at December 31, 2016 has been derived from the audited consolidated balance sheet at December 31, 2016, contained in the above referenced Form 10-K.

#### Comprehensive Loss

Comprehensive loss for the periods reported was comprised solely of the Company's net loss. The comprehensive loss for the three and six months ended June 30, 2017 was \$26.7 million and \$50.0 million, respectively, and for the three and six months ended June 30, 2016 was \$15.6 million and \$32.0 million, respectively. There were no other changes in equity that were excluded from net loss for all periods presented.



## Net Loss per Share

Basic net loss per share is calculated based on the weighted-average number of common shares outstanding, excluding unvested restricted stock awards. Diluted net loss per share is calculated using the weighted-average number of common shares outstanding plus common stock equivalents. Convertible preferred stock, stock options, warrants and unvested restricted stock awards are considered common stock equivalents and are included in the calculation of diluted net loss per share using the treasury stock method when their effect is dilutive. Common stock equivalents are excluded from the calculation of diluted net loss per share when their effect is anti-dilutive. As of June 30, 2017 and 2016, there were common stock equivalents of 11.5 million shares and 11.1 million shares, respectively. Common stock equivalents were excluded from the calculation of diluted net loss per share because they were anti-dilutive.

## Recent Accounting Pronouncements

In May 2017, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2017-09, Compensation - Stock Compensation (Topic 718), Scope of Modification Accounting. The new standard clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. ASU 2017-09 will be effective for the Company in the first quarter of 2018. Early adoption is permitted, including adoption in an interim period for which financial statements have not yet been issued. The Company plans to adopt the ASU in the first quarter of 2018 and expects the standard to have no material impact on the Company's financial position or results of operations.

In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash. The new standard update clarifies the presentation of restricted cash and requires companies to include restricted cash and cash equivalents in the beginning and ending cash on the statement of cash flows. Additional disclosures will be required to describe the amount and detail of the restriction by balance sheet line item. ASU 2016-18 will be effective for the Company in the first quarter of 2018. Early adoption is permitted, including adoption in an interim period using a retrospective transition method to each period presented. The Company plans to adopt the ASU in the first quarter of 2018.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The new standard requires lessees to recognize most leases on their balance sheets as lease liabilities with corresponding right-of-use assets and eliminates certain real estate-specific provisions. ASU 2016-02 will be effective for the Company in the first quarter of 2019 and will be adopted on a modified retrospective transition basis for leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements. The Company currently plans to implement ASU 2016-02 in the first quarter of 2019. By 2019, all of the Company's active existing leases will have ended. Those leases will not have an impact on the consolidated financial statements upon adoption in 2019, and there will be no requirement for modified retrospective application to the years prior to adoption. In December 2016, the Company entered into a 10-year lease agreement for corporate headquarters with an option to terminate the lease early, subject to certain contingencies. The early termination option expired unexercised in March 2017. The expected lease commencement date is November 1, 2017. Upon adoption of ASU 2016-02, this lease will be recognized on the balance sheet as a lease liability with a corresponding right-of-use asset, which would require modified retrospective application upon adoption in 2019 back to the fourth quarter of 2017.

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606). The new standard is based on the principle that revenue should be recognized to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. ASU 2014-09 will be effective for the Company in the first quarter of 2018 and allows for full retrospective or a modified retrospective adoption approach. We currently do not have any products or revenues from customers, and the adoption of this standard will not have a material impact on the Company's financial

position or results of operations. The Company plans to adopt the ASU prospectively upon recognition of the first revenue from customers, and there will not be any impact retrospectively or modified retrospectively to our financial statements. The Contract Revenue - Related Party reported in our results of operations for 2015 and 2016, which represents expense reimbursements from a related party, would not be impacted by the adoption of the new guidance.

### 3. Contract Revenue - Related Party

During the year ended December 31, 2015, the Company entered into a services agreement with a related party. Pursuant to the services agreement, the Company provides certain services to this related party, including, but not limited to, research and development and clinical study design and management for projects undertaken. In exchange for providing such services, the Company receives payments at a negotiated, arms-length rate. As a result, the consideration received by the Company for its services is considered to be no less favorable to the Company than comparable terms that the Company could obtain from an unaffiliated third party in an arms-length transaction. The services agreement may be canceled by either party

upon 60-days' written notice to the other party. In addition, the Company has a non-voting profit interest in the related party, which provides the Company with the potential to receive a portion of the future distributions of profits, if any.

No contract revenue was recognized for the three and six months ended June 30, 2017. For the three and six months ended June 30, 2016, the Company recognized approximately \$253,000 and \$487,000, respectively, in contract revenue for services provided and reimbursement of costs incurred under the services agreement.

#### 4. Shareholders' Equity

##### 2017 Common Stock Offering

In March 2017, the Company offered and sold an aggregate of 3,731,344 shares of common stock in an underwritten public offering at a price of \$33.50 per share, with gross proceeds of approximately \$125.0 million. The Company received proceeds of approximately \$117.5 million, net of approximately \$7.5 million in underwriting commissions, discounts and other issuance costs.

##### Share-Based Compensation Expense

Total share-based compensation expense related to all share-based awards for the three and six months ended June 30, 2017 and 2016 was comprised of the following (in thousands):

	Three Months Ended June 30, 2017		Six Months Ended June 30, 2016	
Research and development:				
Stock options	\$2,563	\$1,215	\$5,016	\$2,476
Restricted stock	—	—	—	30
Warrants	16	4	33	15
Research and development share-based compensation expense	2,579	1,219	5,049	2,521
General and administrative:				
Stock options	1,993	1,559	3,948	3,269
Restricted stock	—	510	409	1,129
Warrants	139	53	288	127
General and administrative share-based compensation expense	2,132	2,122	4,645	4,525
Total share-based compensation expense included in expenses	\$4,711	\$3,341	\$9,694	\$7,046

Share-based compensation expense recognized for the three and six months ended June 30, 2017 and 2016, was reduced by actual forfeitures in the period that the forfeiture occurred.

As of June 30, 2017, there was \$47.8 million of total unrecognized share-based compensation expense related to non-vested stock options. The Company expects to recognize this expense over a weighted-average period of 2.9 years.

##### Stock Option Valuation

The fair value of each stock option award is estimated on the grant date using a Black-Scholes option pricing model (Black-Scholes model), which uses the assumptions noted in the following table. Expected volatility is based on historical volatility of the Company's common stock. In determining the expected life of employee stock options, the Company uses the "simplified" method. The expected life assumptions for non-employee options are based upon the

contractual term of the stock options. The risk-free interest rate is based on the U.S. Treasury yield for a period consistent with the expected term of the stock options in effect at the time of the grants. The dividend yield assumption is based on the expectation of no future dividend payments by the Company.

The Company estimated the fair value of each stock option grant on the grant date using the Black-Scholes model with the following weighted-average assumptions:

	Six Months Ended			
	June 30,			
	2017		2016	
Expected volatility	137	%	143	%
Expected life	6.23 years		5.27 years	
Risk-free interest rate	2.0	%	1.3	%
Dividend yield	—		—	

### Stock Option Activity

The Company's 2013 Equity Plan stock option activity for the six months ended June 30, 2017 was comprised of the following:

	Outstanding Stock Options and 2013 Equity Plan	
	Shares	Weighted- Underlying Average Stock Exercise Price Options per Share
Outstanding at December 31, 2016	2,627,462	\$ 21.07
Granted	1,475,425	\$ 20.54
Exercised	(130,555 )	\$ 15.89
Forfeited	(74,217 )	\$ 21.97
Outstanding at June 30, 2017	3,898,115	\$ 21.03

As of June 30, 2017, there were 558,992 shares of common stock available for future grants under the 2013 Equity Plan, and the Company has reserved an additional 3,838,115 shares of common stock for future issuance upon exercise of all outstanding stock options granted under the 2013 Equity Plan.

During the six months ended June 30, 2017, stock options to purchase 130,555 shares of common stock were exercised with an intrinsic value of \$2.4 million.

### Restricted Stock Award Activity

The Company's restricted stock award activity for the six months ended June 30, 2017 was comprised of the following:

	Number of Shares	Weighted-
		Average Grant Date Fair Market Value
Unvested at December 31, 2016	542,680	\$ 13.22
Vested	(542,680)	\$ 13.22
Unvested at June 30, 2017	—	\$ —

### Warrants

At June 30, 2017, the Company had 93,013 warrants outstanding. In January 2017, the Company issued a warrant to purchase up to 25,013 shares of the Company's common stock to an outside third party at an exercise price equal to the fair market value of the Company's common stock on the grant date.

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## ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

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

### Forward-Looking Statements

The forward-looking statements in this report involve significant risks, assumptions and uncertainties, and a number of factors, both foreseen and unforeseen, which could cause actual results to differ materially from our current expectations. Forward-looking statements include those that express a plan, belief, expectation, estimation, anticipation, intent, contingency, future development or similar expression. Accordingly, you should not rely upon forward-looking statements as predictions of future events. Forward-looking statements include, but are not limited to, statements regarding risks relating to: the timing of the New Drug Application (NDA) submission for LJPC-501 and prospects for approval of the LJPC-501 NDA; risks relating to the scope of product label(s) (if approved) and potential market sizes, as well as the broader commercial opportunity; the anticipated timing for regulatory actions; the success of future development activities; potential indications for which the Company's product candidates may be developed; and the expected duration over which the Company's cash balances will fund our operations. The outcomes of the events described in these forward-looking statements are subject to the risks, uncertainties and other factors described in this "Management's Discussion and Analysis of Financial Condition and Results of Operations," in the "Risk Factors" section contained in our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the U.S. Securities and Exchange Commission (SEC) on February 23, 2017, and in other reports and registration statements that we file with the SEC from time to time. We expressly disclaim any intent to update forward-looking statements.

### Introduction

Management's discussion and analysis of financial condition and results of operations is provided as a supplement to the accompanying unaudited condensed consolidated financial statements and notes, which are included in Item 1 of this Quarterly Report on Form 10-Q, to help provide an understanding of our financial condition, the changes in our financial condition and our results of operations. Our discussion is organized as follows:

- **Business Overview.** This section provides a general description of our business and significant events and transactions that we believe are important in understanding our financial condition and results of operations.
- **Program Overview.** This section provides a current status overview for each of our product candidates in development.
- **Critical Accounting Policies and Estimates.** This section provides a description of our significant accounting policies, including the critical accounting policies and estimates, which are summarized in Note 2 to the accompanying unaudited condensed consolidated financial statements included in Item 1 of this Quarterly Report on Form 10-Q.
- **Results of Operations.** This section provides an analysis of our results of operations presented in the accompanying unaudited condensed consolidated statements of operations by comparing the results for the three and six months ended June 30, 2017 to the results for the three and six months ended June 30, 2016.
- **Liquidity and Capital Resources.** This section provides an analysis of our historical cash flows, as well as our future capital requirements.

### 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 several product candidates in development. LJPC-501 is our proprietary formulation of synthetic human angiotensin II for the potential treatment of catecholamine resistant hypotension. LJPC-401 is our proprietary formulation of synthetic human hepcidin for the potential treatment of conditions characterized by iron overload, such as hereditary hemochromatosis, beta thalassemia, sickle cell disease and myelodysplastic syndrome. LJPC-30S is our next-generation gentamicin derivative program that is focused on the potential treatment of serious bacterial infections as well as rare genetic disorders, such as cystic fibrosis and Duchenne muscular dystrophy.



## Program Overview

### LJPC-501

LJPC-501 is our proprietary formulation of synthetic human 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. LJPC-501 is being developed for the treatment of patients with catecholamine resistant hypotension (CRH), which is an acute, life-threatening condition in which blood pressure drops to dangerously low levels in patients who respond poorly to current treatments. LJPC-501 is the first synthetic human angiotensin II product candidate to be tested in a Phase 3 study.

We initiated a Phase 3 study of LJPC-501 for the treatment of CRH, called the ATHOS-3 (Angiotensin II for the Treatment of High-Output Shock) Phase 3 study, in March 2015. Prior to commencing ATHOS-3, we reached agreement with the U.S. Food and Drug Administration (FDA) on a Special Protocol Assessment (SPA) for this multicenter, randomized, double-blind, placebo-controlled, Phase 3 study. ATHOS-3 was conducted without any amendment to any part of the clinical protocol subject to the SPA agreement, including the primary endpoint and all other endpoints. In ATHOS-3, patients were 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 received their assigned treatment via continuous IV infusion for up to seven days. The primary efficacy endpoint was the percentage of patients with a mean arterial pressure (MAP)  $\geq$  75 mmHg or a 10 mmHg increase from baseline MAP at 3 hours following the initiation of study treatment without an increase in standard-of-care vasopressors. Secondary endpoints include comparison of changes in cardiovascular Sequential Organ Failure Assessment (SOFA) scores and the safety and tolerability of LJPC-501 in patients with CRH.

The ATHOS-3 Phase 3 study completed enrollment of 344 patients in the fourth quarter of 2016. In February 2017, we reported positive top-line results from ATHOS-3. In May 2017, the results of ATHOS-3 were published by The New England Journal of Medicine.

The analysis of the primary efficacy endpoint, defined as the percentage of patients achieving a pre-specified target blood pressure response, was highly statistically significant: 23% of the 158 placebo-treated patients had a blood pressure response compared to 70% of the 163 LJPC-501-treated patients ( $p < 0.00001$ ). In addition, a trend toward longer survival was observed: 22% reduction in mortality risk through day 28 [hazard ratio=0.78 (0.57-1.07),  $p=0.12$ ] for LJPC-501-treated patients.

Throughout ATHOS-3, safety outcomes were followed by an independent Data Safety Monitoring Board (DSMB). The DSMB recommended that the study continue as originally planned. In this critically ill patient population: 92% of placebo-treated patients compared to 87% of LJPC-501-treated patients experienced at least one adverse event, and 22% of placebo-treated patients compared to 14% of LJPC-501-treated patients discontinued treatment due to an adverse event.

We are working towards an FDA-accepted New Drug Application filing for LJPC-501 in the second half of 2017.

### LJPC-401

LJPC-401 is our proprietary formulation of synthetic human hepcidin. Hepcidin, an endogenous peptide hormone, is the body's naturally occurring regulator of iron absorption and distribution. In healthy individuals, hepcidin prevents excessive iron accumulation in vital organs, such as the liver and heart, where it can cause significant damage and even result in death.

We are developing LJPC-401 for the potential treatment of iron overload, which occurs as a result of diseases such as hereditary hemochromatosis (HH), beta thalassemia, sickle cell disease (SCD) and myelodysplastic syndrome (MDS). HH is a disease characterized by a genetic deficiency in hepcidin. HH is the most common genetic disease in Caucasians and causes liver cirrhosis, liver cancer, heart disease and/or failure, diabetes, arthritis and joint pain. Beta thalassemia, SCD and MDS are genetic diseases of the blood that can cause life-threatening anemia and usually require frequent and life-long blood transfusions. These blood transfusions cause excessive iron accumulation in the body, which is toxic to vital organs, such as the liver and heart. In addition, the underlying anemia causes excessive iron accumulation independent of blood transfusions.

In 2015, the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) designated LJPC-401 as an orphan medicinal product for the treatment of beta thalassemia intermedia and major. In 2016, the EMA COMP designated LJPC-401 as an orphan medicinal product for the treatment of SCD.

In September 2016, we reported positive results from a Phase 1 study of LJPC-401 in patients at risk of iron overload suffering from HH, thalassemia and SCD. Single, escalating doses of LJPC-401 were associated with a dose-dependent,

statistically significant reduction in serum iron. LJPC-401 was well-tolerated with no dose-limiting toxicities. Injection-site reactions were the most commonly reported adverse event and were all mild or moderate in severity, self-limiting, and fully resolved.

Also in September 2016, we announced that we reached agreement with the EMA on the design of a pivotal study of LJPC-401. The pivotal study will be a randomized, controlled, multicenter study in beta thalassemia patients suffering from iron overload, a major unmet need in an orphan patient population. The primary endpoint will be a clinically relevant measurement directly related to iron overload. We plan to initiate this study in the second half of 2017.

#### LJPC-30S

LJPC-30S is our next-generation gentamicin derivative program. Despite kidney toxicity, gentamicin has become one of the most commonly prescribed hospital antibiotics due to its broad spectrum of antimicrobial efficacy. Gentamicin consists primarily of a mixture of four distinct but closely related chemical entities that may contribute differentially to the product's toxicity profile.

Our LJPC-30S program is focused on therapeutics derived from purified components of the currently marketed gentamicin product that retain the biologic activity of gentamicin, yet appear to lack the traditional kidney toxicity associated with its use. This program includes the development of potential treatments for serious bacterial infections as well as rare genetic disorders, such as cystic fibrosis and Duchenne muscular dystrophy.

Gentamicin's ability to induce a lack of fidelity in gene transcription, intrinsic to its antimicrobial mechanism of action, can also be leveraged in the correction of certain human genetic mutations that are the hallmark of diseases such as cystic fibrosis and Duchenne muscular dystrophy. In spite of favorable short-term clinical proof-of-efficacy data in cystic fibrosis, development of gentamicin as a chronic treatment for these genetic diseases has been limited by its toxicity profile.

Following a pre-investigational new drug (IND) meeting with the FDA, we have received guidance that we may proceed with a proposed Phase 1 study following the submission of an IND application.

#### Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are based on our unaudited condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these unaudited condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses, and related disclosure of contingent assets and liabilities. We evaluate our estimates on an ongoing basis. We base our estimates on historical experience and on other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

There have been no material changes to the critical accounting policies as previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed on February 23, 2017.

#### Recent Accounting Pronouncements

Recent accounting pronouncements are disclosed in Note 2 to the accompanying unaudited condensed consolidated financial statements included in Item 1 of this Quarterly Report on Form 10-Q.



## Results of Operations

The following summarizes the results of our operations for the three and six months ended June 30, 2017 and 2016 (in thousands):

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2017	2016	2017	2016
Contract revenue - related party	\$—	\$253	\$—	\$487
Research and development expense	(20,808 )	(12,404 )	(38,573 )	(25,119 )
General and administrative expense	(6,022 )	(3,466 )	(11,525 )	(7,519 )
Other income, net	101	51	129	104
Net loss	\$(26,729)	\$(15,566)	\$(49,969)	\$(32,047)

## Contract Revenue - Related Party

During the year ended December 31, 2015, we entered into a services agreement with a related party. Pursuant to the services agreement, we provide certain services to this related party, including, but not limited to, research and development and clinical study design and management for projects undertaken. Contract revenue is a function of the availability of potential projects identified by our customer and our ability and willingness to take on such projects. As such, this revenue may be significantly reduced in future periods, as has happened for the three and six months ended June 30, 2017. In exchange for providing such services, we receive payments at a negotiated, arms-length rate. As a result, the consideration received by us for our services is considered to be no less favorable to us than comparable terms that we could obtain from an unaffiliated third party in an arms-length transaction. The services agreement may be canceled by either party upon 60-days' written notice to the other party. In addition, we have a non-voting profit interest in the related party, which provides us with the potential to receive a portion of the future distributions of profits, if any.

No contract revenue was recognized for the three and six months ended June 30, 2017. For the three and six months ended June 30, 2016, we recognized approximately \$0.3 million and \$0.5 million, respectively, in contract revenue for services provided and reimbursement of costs incurred under the agreement.

## Research and Development Expense

The following summarizes our research and development expense for the three and six months ended June 30, 2017 and 2016 (in thousands):

	Three Months		Six Months	
	Ended		Ended	
	June 30,		June 30,	
	2017	2016	2017	2016
Clinical development costs	\$10,157	\$6,311	\$18,151	\$13,302
Personnel and related costs	5,433	2,861	10,704	5,377
Share-based compensation expense	2,579	1,219	5,049	2,521
Technology in-licensing costs	—	—	270	185
Other research and development costs	2,639	2,013	4,399	3,734
Total research and development expense	\$20,808	\$12,404	\$38,573	\$25,119

For the three and six months ended June 30, 2017, research and development expense increased to \$20.8 million and \$38.6 million, respectively, from \$12.4 million and \$25.1 million for the same periods in 2016, respectively. The

increase was primarily driven by clinical development costs, personnel costs and share-based compensation, as a result of our increased clinical development activities and increased headcount associated with the development of LJPC-501 and LJPC-401. We anticipate research and development expense to increase during the remainder of 2017 due to planned increases in personnel to support the continuation of our clinical development of LJPC-501 and LJPC-401, the initiation of additional clinical studies and the ongoing development and pre-commercialization activities for our product candidates and additional programs we have acquired.

## General and Administrative Expense

The following summarizes our general and administrative expense for the three and six months ended June 30, 2017 and 2016 (in thousands):

	Three Months		Six Months	
	Ended		Ended	
	June 30,		June 30,	
	2017	2016	2017	2016
Personnel and related costs	\$1,440	\$779	\$2,794	\$1,699
Share-based compensation expense	2,132	2,122	4,645	4,525
Other general and administrative expense	2,450	565	4,086	1,295
Total general and administrative expense	\$6,022	\$3,466	\$11,525	\$7,519

During the three and six months ended June 30, 2017, general and administrative expense increased to \$6.0 million and \$11.5 million, respectively, from \$3.5 million and \$7.5 million for the same periods in 2016, respectively. The increase was primarily due to increased personnel costs and professional and outside services associated with our increased development activities. We anticipate general and administrative expense to increase throughout 2017, due to planned increases in personnel and professional and outside services to support ongoing development and pre-commercialization activities for our product candidates.

## Liquidity and Capital Resources

Since January 2012, when the Company was effectively restarted with new assets and a new management team, through June 30, 2017, our cash used in operating activities was \$145.5 million. From inception through June 30, 2017, we have incurred a cumulative net loss of \$656.7 million and have financed our operations through public and private offerings of securities, revenues from collaborative agreements, equipment financings and interest income on invested cash balances. From inception through June 30, 2017, we have raised \$705.7 million in net proceeds from the sales of equity securities.

In March 2017, the Company completed a common stock offering and received proceeds of approximately \$117.5 million, net of issuance costs.

As of June 30, 2017, we had \$141.3 million in cash and cash equivalents, compared to \$65.7 million of cash and cash equivalents at December 31, 2016. Based on our cash and working capital as of June 30, 2017 and our current operating plans and projections, we believe that the available cash and cash equivalents will be sufficient to fund operations for at least one year from the date this Quarterly Report on Form 10-Q is filed with the SEC.

Cash used in operating activities for the six months ended June 30, 2017 was \$41.9 million, compared to \$25.1 million for the same period in 2016. The increase was primarily due to increased research and development activities. For the six months ended June 30, 2017, we used \$2.0 million of cash for investing activities related to purchases of property and equipment, compared to \$0.9 million for the same period in 2016. Cash provided by financing activities for the six months ended June 30, 2017 was \$119.6 million, compared to \$0.1 million for the same period in 2016. The increase was primarily due to the March 2017 common stock offering. As of June 30, 2017, we had positive working capital of \$135.7 million, compared to positive working capital of \$57.7 million at December 31, 2016. The increase in our cash and cash equivalents and working capital was primarily due to the cash provided by financing activities offset by cash used for operating and investing activities.

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 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 and clinical-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. 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.



#### Off-Balance Sheet Arrangements

We have no off-balance sheet arrangements that have, or are reasonably likely to have, a current or future effect on our financial condition, changes in our financial condition, expenses, results of operations, liquidity, capital expenditures or capital resources.

#### ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We are exposed to financial market risks, including changes in interest rates. There were no material changes to our market risks in the six months ended June 30, 2017, when compared to the disclosures in Item 7A of our Annual Report Form 10-K for the year ended December 31, 2016, filed with the SEC on February 23, 2017.

#### ITEM 4. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports, filed under the Securities Exchange Act of 1934, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our chief executive officer and chief financial officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. In addition, the design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, a control may become inadequate because of changes in conditions or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

As required by the SEC Rule 13a-15(b), we carried out an evaluation under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this report. Based on the foregoing, our chief executive officer and chief financial officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

There has been no change in our internal control over financial reporting during our most recent quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

In the ordinary course of business, we may face various claims brought by third parties. Any of these claims could subject us to costly litigation. However, as of the date of this report, we are not currently a party to any legal proceedings that we believe could have a material adverse effect on our business, financial condition or results of operations. However, litigation is inherently uncertain, and any judgment or injunctive relief entered against us or any adverse settlement could negatively affect our business, financial condition and results of operations.

### ITEM 1A. RISK FACTORS

No material changes to risk factors have occurred as previously disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on February 23, 2017, as supplemented by those risk factors set forth under the caption “Risk Factors” in our Prospectus Supplement, dated March 22, 2017, filed with the SEC on March 24, 2017 pursuant to Rule 424(b), which are incorporated herein by reference.

### ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

### ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

### ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

### ITEM 5. OTHER INFORMATION

None.

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## ITEM 6. EXHIBITS

Exhibit Number	Description	Incorporated by	
		Reference Herein	Date
31.1	Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002	Filed herewith	
31.2	Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002	Filed herewith	
32.1	Certification Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002	Filed herewith	
101.INS	XBRL Instance Document	Filed herewith	
101.SCH	XBRL Taxonomy Extension Schema Document	Filed herewith	
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document	Filed herewith	
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document	Filed herewith	
101.LAB	XBRL Taxonomy Extension Label Linkbase Document	Filed herewith	
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document	Filed herewith	

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

La Jolla Pharmaceutical Company

Date: July 27, 2017 /s/ George F. Tidmarsh  
George F. Tidmarsh, M.D., Ph.D.  
President, Chief Executive Officer and Secretary  
(Principal Executive Officer)

/s/ Dennis M. Mulroy  
Dennis M. Mulroy  
Chief Financial Officer  
(Principal Financial and Accounting Officer)