Ultragenyx Pharmaceutical Inc. Form 424B5 January 23, 2018 Table of Contents

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The information in this preliminary prospectus supplement is not complete and may be changed. This preliminary prospectus supplement and the accompanying prospectus are not an offer to sell these securities, and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated January 23, 2018

Prospectus Supplement

(To Prospectus dated February 3, 2015)

\$175,000,000

Common Stock

We are offering \$175,000,000 of shares of our common stock.

Our common stock trades on The NASDAQ Global Select Market under the symbol RARE. On January 19, 2018, the last reported sale price for our common stock on The NASDAQ Global Select Market was \$49.16 per share.

Investing in our common stock involves a high degree of risk. See <u>Risk Factors</u> beginning on page S-6 of this prospectus supplement and page 4 of the accompanying prospectus, as well as the section captioned Risk Factors in our most recently filed quarterly report on Form 10-Q and any subsequent periodic report we file with the SEC, which are incorporated by reference in this prospectus supplement.

	Per		
	share	Total	
Public offering price	\$	\$	
Underwriting discounts and commissions ⁽¹⁾	\$	\$	
Proceeds to Ultragenyx Pharmaceutical Inc., before expenses	\$	\$	

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

We have granted the underwriters an option for a period of 30 days to purchase up to \$26,250,000 of additional shares of our common stock.

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

The underwriters expect to deliver the shares of common stock to purchasers on or about January , 2018.

J.P. Morgan

BofA Merrill Lynch Goldman Sachs & Co. LLC Cowen

The date of this prospectus supplement is January , 2018

TABLE OF CONTENTS

Prospectus Supplement

	Page
Prospectus Supplement Summary	S-1
The Offering	S-2
Summary Financial Data	S-4
Risk Factors	S-6
Cautionary Note Regarding Forward-Looking Statements	S-8
<u>Use of Proceeds</u>	S-10
Price Range of Common Stock	S-11
<u>Dividend Policy</u>	S-12
Capitalization	S-13
<u>Dilution</u>	S-15
<u>Description of Capital Stock</u>	S-17
Underwriting	S-21
<u>Legal Matters</u>	S-30
<u>Experts</u>	S-30
Where You Can Find More Information	S-30
Incorporation of Certain Information By Reference	S-30
Prospectus	
	Page
About This Prospectus	1
About Ultragenyx Pharmaceutical Inc.	2
Risk Factors	4
Cautionary Note Regarding Forward-Looking Statements	4
<u>Use of Proceeds</u>	5
Ratio of Earnings to Fixed Charges	5
<u>Description of Securities</u>	5
Selling Stockholders	13
<u>Plan of Distribution</u>	13
<u>Legal Matters</u>	15
<u>Experts</u>	15
<u>Incorporation of Certain Information By Reference</u>	15
Where You Can Find More Information	16

This document is in two parts. The first part is this prospectus supplement, which describes the specific terms of this offering. The second part, the accompanying prospectus, gives more general information, some of which may not apply to this offering. In the event that the description of this offering varies between this prospectus supplement and the accompanying prospectus, you should rely on the information contained in this prospectus supplement. Generally, when we refer to the prospectus, we are referring to this prospectus supplement and the accompanying prospectus together.

We have not authorized anyone to provide you with information other than that contained in this prospectus supplement, the accompanying prospectus or in any free writing prospectus prepared by or on behalf of us or to which

we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus supplement is accurate only as of the date of this prospectus supplement, regardless of the time of delivery of this prospectus supplement or any sale of our common stock. Our business, financial condition, results of operations, and prospects may have changed since that date.

i

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

ii

PROSPECTUS SUPPLEMENT SUMMARY

The items in the following summary are described in more detail later in this prospectus supplement and the accompanying prospectus. This summary provides an overview of selected information and does not contain all of the information you should consider before buying our common stock. Therefore, you should read the entire prospectus supplement and the accompanying prospectus carefully, including the information in our filings with the Securities and Exchange Commission, or SEC, incorporated by reference herein, before deciding to invest in our common stock. Investors should carefully consider the information set forth under Risk Factors beginning on page S-6 of this prospectus supplement and page 4 of the accompanying prospectus and those identified in our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q. In this prospectus supplement, unless the context otherwise requires, references to the Company, we, us, our, or Ultragenyx refer to Ultragenyx Pharmaceutical Inc. and its consolidated subsidiaries.

Overview

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

Our Corporate Information

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

THE OFFERING

Common stock offered by us

shares

Underwriters option to purchase additional We have granted the underwriters a 30-day option to purchase up to an shares additional shares of our common stock.

Common stock to be outstanding after this offering

shares (shares if the underwriters exercise their option to purchase additional shares in full)

Use of proceeds

We estimate that the net proceeds to us from this offering will be approximately \$164.5 million, or approximately \$189.2 million if the underwriters exercise their option to purchase additional shares in full, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us at the assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAO Global Select Market on January 19, 2018. We intend to use the net proceeds of the offering to fund the potential commercialization of Mepsevii and burosumab in multiple territories, development of clinical-stage assets such as UX007 for Glut1 DS and LC-FAOD, and gene therapies for OTC and GSD1a, as well as advancement of our early-stage translational research programs. We may also use a portion of the net proceeds to in-license, acquire, or invest in additional businesses, technologies, products, or assets, though we currently have no specific agreements, commitments, or understandings with respect to any in-licensing or acquisitions. Finally, we may use any remaining proceeds for working capital and other general corporate purposes.

Risk factors

You should read the Risk Factors section of this prospectus and our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q, incorporated by reference herein, for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.

NASDAQ Global Select Market symbol RARE

The number of shares of common stock to be outstanding after this offering is based on 42,488,312 shares of common stock outstanding as of September 30, 2017.

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

4,834,159 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2017 having a weighted-average exercise price of \$65.95 per share;

756,083 shares of common stock issuable upon the vesting of restricted stock units outstanding as of September 30, 2017;

149,700 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2017 having a weighted-average exercise price of \$3.01 per share;

1,881,670 shares of common stock reserved for issuance pursuant to future equity awards under our 2014 Incentive Plan as of September 30, 2017, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan; and

S-2

1,816,052 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or 2014 ESPP, as September 30, 2017, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2014 ESPP.

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

assumes that the underwriters do not exercise their option to purchase additional shares; and

assumes no exercise of outstanding options or warrants or vesting of restricted stock units after September 30, 2017.

S-3

SUMMARY FINANCIAL DATA

The following table summarizes our statements of operations and balance sheet data. We have derived the following statements of operations data for the years ended December 31, 2014, 2015, and 2016 from our audited financial statements incorporated by reference in this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2016, or our 2016 Annual Report, and we have derived the following statements of operations data for the nine months ended September 30, 2017 and September 30, 2016 and the balance sheet data as of September 30, 2017 from our unaudited interim financial statements incorporated by reference in this prospectus from our Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, or our September 2017 Quarterly Report. You should read this data together with our financial statements and related notes, as well as the information under the captions Selected Financial Data and Management s Discussion and Analysis of Financial Condition and Results of Operations appearing in our 2016 Annual Report and our September 2017 Quarterly Report, which are incorporated by reference herein. Our historical results are not necessarily indicative of our future results, and results of interim periods are not necessarily indicative of results for the entire year.

Year Ended December 31,

Nine Months Ended

September 30,

		Teal Ended December 31,				September 30,			<i>5</i> 0,	
		2014		2015		2016		2016		2017
	(in thousands, except share and per share amounts)									
				,	-	-		(unau		l)
Statements of Operations										
Data:										
Revenue	\$		\$		\$	133	\$	128	\$	198
Operating expenses:										
Research and development		45,967		114,737		183,204		132,458		170,117
General and administrative		10,811		33,001		64,936		45,128		62,189
Total operating expenses		56,778		147,738		248,140		177,586		232,306
Loss from operations		(56,778)		(147,738)		(248,007)		(177,458)		(232,108)
Interest income		608		2,320		3,789		2,877		3,350
Other income (expense), net		(3,632)		(200)		(1,621)		(6)		8,368
Total other income (expense),										
net		(3,024)		2,120		2,168		2,871		11,718
		, , ,								
Loss before income taxes		(59,802)		(145,618)		(245,839)		(174,587)		(220,390)
Income tax provision						(35)				(18)
•										
Net loss	\$	(59,802)	\$	(145,618)	\$	(245,874)	\$	(174,587)	\$	(220,408)
										, , ,
Net loss attributable to										
common stockholders ⁽¹⁾	\$	(64,610)	\$	(145,618)	\$	(245,874)	\$	(174,587)	\$	(220,408)
		, , ,				, , ,				
Net loss per share attributable	\$	(2.25)	\$	(3.96)	\$	(6.21)	\$	(4.46)	\$	(5.22)
to common stockholders, basic		. ,				,				, ,

and diluted

Shares used in computing net loss per share attributable to common stockholders, basic and diluted

28,755,758 36,782,603

39,586,908

39,184,994

42,222,413

S-4

As of September 30, 2017 Actual As-Adjusted⁽²⁾⁽³⁾ (in thousands)

	(unaudited)		
Balance Sheet Data:			
Cash, cash equivalents and short-term investments	\$ 371,066	\$	535,565
Working capital	326,642		491,141
Total assets	435,047		599,546
Accumulated deficit	(750,941)		(750,941)
Total stockholders equity	365,558		530,057

- (1) See (a) Notes 2 and 11 to our audited financial statements included in our 2016 Annual Report incorporated by reference herein and (b) Note 7 to our interim financial statements included in our September 2017 Quarterly Report incorporated herein by reference for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders.
- (2) The as-adjusted balance sheet data reflects the sale of an assumed 3,559,804 shares of common stock offered by us in this offering at an assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.
- (3) A \$1.00 increase (decrease) in the assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, would increase (decrease) the as-adjusted amount of each of cash, cash equivalents and short-term investments, working capital, total assets and total stockholders—equity by approximately \$3.4 million, assuming that the number of shares offered by us remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A 100,000 share increase (decrease) in the number of shares offered by us at the assumed public offering price of \$49.16 per share would increase (decrease) the as-adjusted amount of each of cash, cash equivalents and short-term investments, working capital, total assets and total stockholders equity by approximately \$4.6 million after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

RISK FACTORS

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

Risks Related to this Offering

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

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the as-adjusted book value per share of our tangible assets after subtracting our liabilities. As a result, investors purchasing shares of common stock in this offering will incur immediate dilution of \$37.65 per share, based on an assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, and our as-adjusted net tangible book value as of September 30, 2017 after giving effect to this offering. For information on how the foregoing amounts were calculated, see Dilution.

This dilution is due to the substantially lower price paid by our investors who purchased shares prior to this offering as compared to the price offered to the public in this offering, and the exercise of stock options granted to our employees. In addition, as of September 30, 2017, we had outstanding 5,590,242 restricted stock units and options, and warrants to purchase 149,700 shares of our common stock; the vesting of the restricted stock units and the exercise of any of these options or warrants would result in additional dilution. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

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

If our existing stockholders, particularly our directors, executive officers and significant stockholders, sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the market price of our common stock could decline. Our directors and executive officers have entered into lock-up agreements with the underwriters in connection with this offering pursuant to which they have agreed with the underwriters not to offer, sell, dispose of or hedge any shares of common stock or securities convertible into or exchangeable for shares of common stock, subject to specified limited exceptions and extensions described elsewhere in this prospectus, for a period of 60 days after the date of this prospectus without the prior written consent of J.P. Morgan Securities LLC, Merrill Lynch, Pierce, Fenner & Smith Incorporated, Goldman Sachs & Co. LLC and Cowen and Company, LLC. Based upon the number of shares of common stock outstanding as of September 30, 2017, and an assumed offering price per share of \$49.16 as of January 19, 2018, upon the closing of this offering we will have outstanding a total of approximately 46,048,116 shares of common stock (assuming no exercise of the underwriters—option to purchase additional shares). Other than any shares held by our directors and executive officers subject to lock-up agreements with the underwriters, all of these outstanding shares of common stock are currently freely tradable, and the shares to be sold in this offering, plus

any shares sold upon exercise of the underwriters option to purchase additional shares, will be freely tradable, without restriction, in the public market following this offering. J.P. Morgan Securities LLC, Merrill Lynch, Pierce, Fenner & Smith Incorporated,

Goldman Sachs & Co. LLC and Cowen and Company, LLC may, in their sole discretion, permit our executive officers and directors who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements. Sales of a substantial number of shares upon expiration of the lock-up agreements, the perception that such sales may occur, or early release of these agreements, could cause our market price to fall or make it more difficult for you to sell your common stock at a time and price that you deem appropriate.

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

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

S-7

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus supplement contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this prospectus are forward-looking statements. In some cases, you can identify forward-looking statements by words such believe, contemplate, as anticipate, continue, could, estimate, expect, intend, potentia would, or the negative of these words or other comparable terminology. These seek, should, target, will. forward-looking statements include, but are not limited to, statements about:

our commercialization, marketing, and manufacturing capabilities and strategy;

our expectations regarding the timing of clinical study commencements and reporting results from same;

the timing and likelihood of regulatory approvals for our product candidates;

the anticipated indications for our product candidates, if approved;

the potential market opportunities for commercializing our product and product candidates;

our expectations regarding the potential market size and the size of the patient populations for our product and product candidates, if approved for commercial use;

estimates of our expenses, future revenue, capital requirements, and our needs for additional financing;

our ability to develop, acquire, and advance product candidates into, and successfully complete, clinical studies;

the implementation of our business model and strategic plans for our business, product and product candidates and the integration and performance of any acquired businesses;

the initiation, timing, progress, and results of ongoing and future preclinical and clinical studies, and our research and development programs;

the scope of protection we are able to establish and maintain for intellectual property rights covering our product and product candidates;

our ability to maintain and establish collaborations or strategic relationships or obtain additional funding;

our ability to maintain and establish relationships with third parties, such as contact research organizations, suppliers and distributors;

our use of proceeds from this offering;

our financial performance and expansion of our organization;

our ability to obtain supply of our product and product candidates;

the scalability and commercial viability of our manufacturing methods and processes;

developments and projections relating to our competitors and our industry; and

other risks and uncertainties, including those listed or incorporated by reference in Risk Factors. Any forward-looking statements in this prospectus reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Factors that may cause

S-8

actual results to differ materially from current expectations include, among other things, those discussed under Risk Factors and elsewhere and incorporated by reference in this prospectus. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

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

S-9

USE OF PROCEEDS

We estimate that the net proceeds to us from the assumed sale of 3,559,804 shares of common stock offered by us in this offering will be approximately \$164.5 million, based on an assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that our net proceeds will be approximately \$189.2 million, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each \$1.00 increase (decrease) in the assumed public offering price of \$49.16 per share, which is the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, would increase (decrease) the net proceeds to us by approximately \$3.4 million, assuming that the number of shares offered by us remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. Each increase (decrease) of 100,000 shares in the number of shares offered by us would increase (decrease) the net proceeds to us from this offering by approximately \$4.6 million, assuming that the assumed public offering price remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

We intend to use the net proceeds of the offering to fund the potential commercialization of Mepsevii and burosumab in multiple territories, development of clinical-stage assets such as UX007 for Glut1 DS and LC-FAOD, and gene therapies for OTC and GSD1a, as well as advancement of our early-stage translational research programs. We may also use a portion of the net proceeds to in-license, acquire, or invest in additional businesses, technologies, products, or assets, though we currently have no specific agreements, commitments, or understandings with respect to any in-licensing or acquisitions. Finally, we may use any remaining proceeds for working capital and other general corporate purposes.

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

S-10

PRICE RANGE OF COMMON STOCK

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

	High	Low
2018		
First Quarter (through January 19, 2018)	\$ 53.39	\$45.69
2017		
First Quarter	\$ 91.35	\$65.55
Second Quarter	\$ 68.46	\$51.67
Third Quarter	\$ 71.99	\$49.57
Fourth Quarter	\$ 58.00	\$43.14
2016		
First Quarter	\$110.06	\$49.00
Second Quarter	\$ 78.13	\$46.52
Third Quarter	\$ 81.40	\$48.33
Fourth Quarter	\$ 86.77	\$52.60

On January 19, 2018, the last reported sale price of our common stock on The NASDAQ Global Select Market was \$49.16 per share. As of January 19, 2018, we had approximately 4 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

S-11

DIVIDEND POLICY

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, for the development, operation and expansion of our business and do not anticipate paying any cash dividends for the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors or any authorized committee thereof.

S-12

CAPITALIZATION

The following table sets forth our cash, cash equivalents and short-term investments and capitalization as of September 30, 2017:

on an actual basis; and

on an as-adjusted basis to reflect the issuance and sale by us of an assumed 3,559,804 shares of our common stock in this offering at an assumed public offering price of \$49.16 per share, which was the last reported sale price of our common stock on The NASDAQ Global Select Market on January 19, 2018, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this information together with our financial statements and related notes and the information under the captions Selected Financial Data and Management's Discussion and Analysis of Financial Condition and Results of Operations, appearing in our 2016 Annual Report and September 2017 Quarterly Report, incorporated by reference in this prospectus. For more details on how you can obtain our Commission reports and other information, you should read the section of the prospectus entitled Where You Can Find More Information.

As of September 30, 2017

As

Actual Adjusted⁽¹⁾
(in thousands, except share and per share data)

(unaudited)

Cash, cash equivalents and short-term investments

\$ 371,066 \$ 535,565

Stockholders equity:

Preferred stock, par value \$0.001 per share 25,000,000 shares authorized; no shares issued or outstanding, actual and as-adjusted

Common stock, par value \$0.001 per share 250,000,000 shares authorized;

42,488,312 shares issued and outstanding, actual; 250,000,000 shares authorized,

46,048,116 shares issued and outstanding, as-adjusted

42