

Ultragenyx Pharmaceutical Inc.
 Form 424B5
 January 24, 2018
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Filed Pursuant to Rule 424(b)(5)
Registration No. 333-201838

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities to be Registered	Amount to be Registered(1)	Proposed Maximum Offering Price Per Share	Proposed Maximum Aggregate Offering Price	Amount of Registration Fee(2)
Common Stock, par value \$0.001 per share	5,043,860	\$57.00	\$287,500,020	\$35,794

(1) Includes 657,895 shares of common stock, par value \$0.001 per share, which may be purchased by the underwriters upon exercise of the underwriters' option to purchase additional shares.

(2) Calculated in accordance with Rules 456(b) and 457(r) of the Securities Act of 1933, as amended.

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Prospectus Supplement

(To Prospectus dated February 3, 2015)

4,385,965 shares

Common Stock

We are offering 4,385,965 shares of our common stock.

Our common stock trades on The NASDAQ Global Select Market under the symbol RARE. On January 23, 2018, the last reported sale price for our common stock on The NASDAQ Global Select Market was \$58.14 per share.

Investing in our common stock involves a high degree of risk. See Risk Factors 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	\$ 57.00	\$ 250,000,005
Underwriting discounts and commissions ⁽¹⁾	\$ 3.28	\$ 14,375,000
Proceeds to Ultragenyx Pharmaceutical Inc., before expenses	\$ 53.72	\$ 235,625,005

(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 657,895 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 26, 2018.

J.P. Morgan

BofA Merrill Lynch Goldman Sachs & Co. LLC
The date of this prospectus supplement is January 23, 2018

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Prospectus

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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.

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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.

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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.

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THE OFFERING

Common stock offered by us	4,385,965 shares
Underwriters' option to purchase additional shares	We have granted the underwriters a 30-day option to purchase up to an additional 657,895 shares of our common stock.
Common stock to be outstanding after this offering	46,874,277 shares (47,532,172 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 \$235.0 million, or approximately \$270.3 million if the underwriters exercise their option to purchase additional shares in full, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us at the public offering price of \$57.00 per share. 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:	

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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

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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.

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Table of Contents**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	
	2014	2015	2016	2016	2017
	(in thousands, except share and per share amounts)				
	(unaudited)				
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 to common stockholders, basic	\$ (2.25)	\$ (3.96)	\$ (6.21)	\$ (4.46)	\$ (5.22)

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
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	As of September 30, 2017	
	Actual	As-Adjusted⁽²⁾
	(in thousands)	
	(unaudited)	
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	\$ 371,066	\$ 606,066
Working capital	326,642	561,642
Total assets	435,047	670,047
Accumulated deficit	(750,941)	(750,941)
Total stockholders' equity	365,558	600,558

- (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 4,385,965 shares of common stock offered by us in this offering at the public offering price of \$57.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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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 \$44.19 per share, based on the public offering price of \$57.00 per share, 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 the sale of 4,385,965 shares of our common stock in this offering, upon the closing of this offering we will have outstanding a total of approximately 46,874,277 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

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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.

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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 as anticipate, believe, contemplate, continue, could, estimate, expect, intend, may, plan, potential, seek, should, target, will, would, or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

our 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 contract 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

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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.

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USE OF PROCEEDS

The net proceeds to us from the sale of 4,385,965 shares of common stock offered by us in this offering will be approximately \$235.0 million, based on the public offering price of \$57.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, our net proceeds will be approximately \$270.3 million, after deducting the 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.

Table of Contents**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 23, 2018)	\$ 58.62	\$ 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 23, 2018, the last reported sale price of our common stock on The NASDAQ Global Select Market was \$58.14 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.

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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.

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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 4,385,965 shares of our common stock in this offering at the public offering price of \$57.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this information together with our financial statements and related notes and the information under the captions "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," appearing in our 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	
	Actual	As Adjusted
	(in thousands, except share and per share data)	
	(unaudited)	
Cash, cash equivalents and short-term investments	\$ 371,066	\$ 606,066
Stockholders' equity:		
Preferred stock, par value \$0.001 per share 25,000,000 shares authorized; no shares issued or outstanding, actual and as-adjusted		