

Ultragenyx Pharmaceutical Inc.  
Form DEF 14A  
April 18, 2016  
Table of Contents

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
**Washington, D.C. 20549**

**SCHEDULE 14A INFORMATION**  
**Proxy Statement Pursuant to Section 14(a) of the**  
**Securities Exchange Act of 1934**

Filed by the Registrant

Filed by a party other than the Registrant

Check the appropriate box:

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- Confidential, for Use of the Commission Only (as permitted by Rule 14a-6(e)(2))**
- Definitive Proxy Statement
- Definitive Additional Materials
- Soliciting Material under §240.14a-12

**ULTRAGENYX PHARMACEUTICAL INC.**

**(Name of Registrant as Specified In Its Charter)**

**N/A**

**(Name of Person(s) Filing Proxy Statement, if Other Than The Registrant)**

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Table of Contents

**60 Leveroni Court**

**Novato, California 94949**

**NOTICE OF ANNUAL MEETING OF STOCKHOLDERS**

**To Be Held on June 9, 2016**

**at 4:00 p.m. Pacific time**

Dear Stockholder:

You are cordially invited to attend the Annual Meeting of Stockholders of Ultragenyx Pharmaceutical Inc., a Delaware corporation (the **Company**), which will be held on June 9, 2016, at 4:00 p.m. Pacific Time virtually via the Internet at [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16) (the **Annual Meeting**). Instructions on how to participate in the Annual Meeting and demonstrate proof of stock ownership are posted at [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16). The webcast of the Annual Meeting will be archived for one year after the date of the Annual Meeting at [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16). Only stockholders who held stock at the close of business on the record date, April 12, 2016, may vote at the Annual Meeting, including any adjournment or postponement thereof.

At the Annual Meeting, you will be asked to consider and vote upon: (1) the election of the three directors named in the Proxy Statement as Class III directors; (2) the ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2016; (3) an advisory (non-binding) vote to approve the Company's executive compensation, (4) an advisory (non-binding) vote on the frequency of future advisory votes to approve executive compensation, and (5) any other business that may properly come before the Annual Meeting or any adjournment or postponement thereof. No other items of business are expected to be considered, and no other director nominees will be entertained, at the Annual Meeting.

The accompanying Proxy Statement more fully describes the details of the business to be conducted at the Annual Meeting. Proposal 1 relates solely to the election of the three directors nominated by the Board of Directors and does not include any other matters relating to the election of directors, including, without limitation, the election of directors nominated by any stockholder of the Company. After careful consideration, our Board of Directors has unanimously approved the proposals and recommends that you vote FOR each of the three director nominees and FOR each proposal described in the Proxy Statement, including FOR holding an advisory vote to approve the Company's executive compensation every year. In accordance with Delaware law, a list of stockholders entitled to vote at the meeting will be available in electronic form during the annual meeting at the following URL: [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16), and will be accessible during normal business hours for ten days prior to the meeting at our principal place of business, 60 Leveroni Court, Novato, California 94949.

We are pleased to make use of the Securities and Exchange Commission rules that allow companies to furnish proxy materials to their stockholders via the Internet. We believe the ability to deliver proxy materials electronically allows us to provide our stockholders with the information they need, while lowering the costs of delivery and reducing the environmental impact from the distribution of our Annual Meeting materials.

**Table of Contents**

We look forward to speaking with you at the Annual Meeting.

Sincerely,

Emil D. Kakkis, M.D., Ph.D.

President and Chief Executive Officer

April 18, 2016

**WHETHER OR NOT YOU EXPECT TO ATTEND THE ANNUAL MEETING, PLEASE VOTE VIA THE INTERNET OR OVER THE TELEPHONE AS INSTRUCTED IN THE *NOTICE REGARDING THE AVAILABILITY OF PROXY MATERIALS* OR PROXY CARD OR, IF YOU REQUESTED AND RECEIVED A PRINTED COPY OF THE PROXY STATEMENT, COMPLETE, DATE, SIGN AND RETURN THE ENCLOSED PROXY CARD USING THE ENCLOSED RETURN ENVELOPE, AS PROMPTLY AS POSSIBLE SO THAT YOUR SHARES MAY BE REPRESENTED AT THE ANNUAL MEETING. YOU MAY ALSO VOTE THROUGH OUR VIRTUAL WEB CONFERENCE IF YOU ATTEND THE ANNUAL MEETING.**

**IMPORTANT NOTICE REGARDING THE AVAILABILITY OF PROXY MATERIALS  
FOR THE ANNUAL MEETING OF STOCKHOLDERS TO BE HELD ON JUNE 9, 2016:**

**The Proxy Statement and 2015 Annual Report are  
available at [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16).**

**Table of Contents**

**TABLE OF CONTENTS**

|   |    |
|---|----|
| <u>GENERAL INFORMATION</u>  | 1  |
| <u>Shares Outstanding and Voting Rights</u>   | 1  |
| <u>PROPOSAL NO. 1 ELECTION OF DIRECTORS</u>   | 4  |
| <u>Nomination of Directors</u>  | 4  |
| <u>Nominees and Incumbent Directors</u>   | 5  |
| <u>Class III Directors Nominated for Election</u>   | 5  |
| <u>Class I Directors Continuing in Office Until 2017</u>  | 6  |
| <u>Class II Directors Continuing in Office Until 2018</u>   | 7  |
| <u>Vote Required</u>  | 7  |
| <u>PROPOSAL NO. 2 RATIFICATION OF SELECTION OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM</u>            | 8  |
| <u>Fees for Independent Registered Public Accounting Firm</u>   | 9  |
| <u>Vote Required</u>  | 9  |
| <u>REPORT OF THE AUDIT COMMITTEE</u>  | 10 |
| <u>PROPOSAL NO. 3 ADVISORY VOTE TO APPROVE EXECUTIVE COMPENSATION</u>                                       | 11 |
| <u>Background</u>   | 11 |
| <u>Recommendation</u>   | 11 |
| <u>Vote Required</u>  | 11 |
| <u>PROPOSAL NO. 4 ADVISORY VOTE ON FREQUENCY OF FUTURE ADVISORY VOTES TO APPROVE EXECUTIVE COMPENSATION</u> | 12 |
| <u>Background</u>   | 12 |
| <u>Recommendation</u>   | 12 |
| <u>Vote Required</u>  | 12 |
| <u>CORPORATE GOVERNANCE</u>   | 13 |
| <u>Director Independence</u>  | 13 |
| <u>Code of Business Conduct and Ethics</u>  | 13 |
| <u>Stockholder Communications</u>   | 13 |
| <u>BOARD OF DIRECTORS AND COMMITTEES</u>  | 13 |
| <u>Board Leadership Structure and Risk Oversight</u>  | 14 |
| <u>Board Committees</u>   | 14 |
| <u>Compensation Committee Interlocks and Insider Participation</u>  | 15 |
| <u>EXECUTIVE OFFICERS</u>   | 16 |
| <u>CERTAIN RELATIONSHIPS AND RELATED-PERSON TRANSACTIONS</u>  | 17 |
| <u>Related-Person Transactions</u>  | 17 |
| <u>Procedures for Related-Person Transactions</u>   | 17 |
| <u>SECTION 16(A) BENEFICIAL OWNERSHIP REPORTING COMPLIANCE</u>  | 18 |
| <u>SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT</u>                                       | 18 |
| <u>EXECUTIVE COMPENSATION</u>   | 21 |
| <u>Compensation Discussion and Analysis</u>   | 21 |
| <u>Compensation Philosophy and Objectives</u>   | 21 |
| <u>Roles in Determining Compensation</u>  | 22 |
| <u>Defining and Comparing Compensation to Market Benchmarks</u>   | 23 |

**Table of Contents**

|  |    |
|--|----|
| <i><u>Annual Performance Reviews</u></i>   | 25 |
| <i><u>Base Salary</u></i>  | 26 |
| <i><u>Fiscal 2015 and 2016 base salary levels for each named executive officer</u></i> | 27 |
| <i><u>Annual Bonus Incentive</u></i>   | 28 |
| <i><u>Equity Compensation</u></i>  | 32 |
| <i><u>Equity Grants Awarded in Fiscal 2015</u></i>                                     | 33 |
| <i><u>Employee Benefit Program</u></i>   | 34 |
| <i><u>Tax and Accounting Considerations</u></i>  | 34 |
| <i><u>Allocation of Compensation</u></i>   | 34 |
| <i><u>Timing of Compensation Actions</u></i>   | 35 |
| <i><u>Minimum Stock Ownership Requirements</u></i>                                     | 35 |
| <i><u>Conclusion</u></i>   | 35 |
| <i><u>Risk Management and Mitigation</u></i>   | 35 |
| <i><u>Compensation Committee Report</u></i>  | 35 |
| <i><u>Summary Compensation Table</u></i>   | 36 |
| <i><u>Narrative Disclosure to Summary Compensation Table</u></i>                       | 37 |
| <i><u>Employment Arrangements with Our Named Executive Officers</u></i>                | 37 |
| <i><u>Involuntary Termination of Employment</u></i>                                    | 38 |
| <i><u>Covered Transaction</u></i>  | 39 |
| <i><u>Definitions</u></i>  | 39 |
| <i><u>Grants of Plan-Based Awards</u></i>  | 41 |
| <i><u>Outstanding Equity Awards at December 31, 2015</u></i>                           | 41 |
| <i><u>Option Exercises and Stock Vested</u></i>  | 44 |
| <i><u>Pension Benefits</u></i>   | 44 |
| <i><u>Nonqualified Deferred Compensation</u></i>                                       | 44 |
| <i><u>Potential Payments Upon Termination or Change of Control</u></i>                 | 45 |
| <b><u>EQUITY COMPENSATION PLAN INFORMATION</u></b>                                     | 47 |
| <b><u>DIRECTOR COMPENSATION</u></b>  | 47 |
| <b><u>OTHER BUSINESS</u></b>   | 48 |
| <b><u>STOCKHOLDER PROPOSALS</u></b>  | 49 |
| <b><u>DELIVERY OF PROXY MATERIALS</u></b>  | 50 |

**Table of Contents**

**60 Leveroni Court**

**Novato, California 94949**

**PROXY STATEMENT FOR**

**2016 ANNUAL MEETING OF STOCKHOLDERS**

**TO BE HELD ON JUNE 9, 2016**

**at 4:00 p.m. Pacific time**

**GENERAL INFORMATION**

This Proxy Statement is furnished in connection with the solicitation of proxies by the Board of Directors (the *Board*) of Ultragenyx Pharmaceutical Inc. (*Ultragenyx* or the *Company*) for use at the Company's 2016 Annual Meeting of Stockholders, to be held virtually via the Internet at [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16) on June 9, 2016, at 4:00 p.m. Pacific Time. The Notice Regarding the Availability of Proxy Materials (the *Notice*) containing instructions on how to access this Proxy Statement and our annual report is first being mailed on or about April 28, 2016 to all stockholders entitled to vote at the Annual Meeting. Pursuant to the rules promulgated by the Securities and Exchange Commission (the *SEC*), we have elected to provide access to our proxy materials primarily by notifying you of the availability of our proxy materials on the Internet, instead of mailing printed copies of those materials to stockholders. The Proxy Statement and 2015 Annual Report are available at [www.ultragenyx.com](http://www.ultragenyx.com) in the SEC Filings subsection of the Investors tab.

The Notice instructs you as to how you may access and review important information contained in the proxy materials. The Notice also instructs you on how you may submit your proxy via the Internet. If you receive a Notice by mail and would like to receive a printed copy of our proxy materials, you should follow the instructions for requesting such materials included in the Notice.

For a proxy to be effective, it must be properly executed and received prior to the Annual Meeting. Each proxy properly tendered will, unless otherwise directed by the stockholder, be voted for each of the proposals set forth in this Proxy Statement (including voting for holding an advisory vote to approve the Company's executive compensation every year) and each of the three director nominees named in this Proxy Statement and at the discretion of the proxy holder(s) with regard to all other matters that may properly come before the Annual Meeting.

The Company will pay all of the costs of soliciting proxies. We will provide copies of our proxy materials to brokerage firms, fiduciaries and custodians for forwarding to beneficial owners who request printed copies of these materials and will reimburse these persons for their costs of forwarding these materials. Our directors, officers and employees may also solicit proxies by telephone, facsimile, or personal solicitation; however, we will not pay these individuals additional compensation for any of these services.

**Shares Outstanding and Voting Rights**

Only holders of record of our common stock at the close of business on April 12, 2016 (the *Record Date*), are entitled to notice of and to vote at the Annual Meeting. On the Record Date, 39,009,156 shares of





**Table of Contents**

common stock were issued and outstanding. Each share of common stock is entitled to one vote on all matters to be voted upon at the Annual Meeting. Holders of common stock do not have the right to cumulative voting in the election of directors. The presence, in person or by proxy, of the holders of a majority of the outstanding shares on the Record Date will constitute a quorum for the transaction of business at the Annual Meeting and any postponement or adjournment thereof, though the Board may fix a new record date for purposes of a postponed or adjourned meeting.

Persons who hold shares of Ultragenyx common stock directly on the Record Date and not through a broker, bank or other financial institution ( **registered holders** ) may vote before the Annual Meeting over the telephone (by calling 1-800-690-6903 until 11:59 p.m. Eastern Time the day before the Annual Meeting), through the Internet (you may vote before the meeting by going to [www.proxyvote.com](http://www.proxyvote.com) until 11:59 p.m. Eastern Time the day before the Annual Meeting), or return an executed proxy card (that we must receive before the Annual Meeting). Registered holders who attend the Annual Meeting may also vote during the Annual Meeting by going to [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16) and following the instructions regarding voting. Persons who hold shares of Ultragenyx common stock indirectly on the Record Date through a brokerage firm, bank or other financial institution ( **beneficial holders** ) may (i) attend the Annual Meeting and vote during the Annual Meeting by going to [www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16) and following the instructions regarding voting or (ii) return a voting instruction form to have their shares voted on their behalf. Beneficial holders also may be able to vote over the telephone or through the Internet in accordance with the voting instructions provided by the institution that holds their shares. Brokerage firms, banks or other financial institutions that do not receive voting instructions from beneficial holders may vote these shares on behalf of the beneficial holders only with respect to routine matters, or else return a proxy leaving these shares un-voted (a **broker non-vote** ). The ratification of the selection of the independent registered public accounting firm (Proposal No. 2) is the only item on the agenda for the Annual Meeting that is considered routine. Abstentions and broker non-votes will be counted for the purpose of determining the presence or absence of a quorum. The required vote for each of the proposals expected to be acted upon at the Annual Meeting and the treatment of abstentions and broker non-votes under each proposal are described below:

*Proposal No. 1 Election of directors.* Directors are elected by a plurality of the votes cast, with the three nominees obtaining the greatest number of affirmative votes being elected as directors. Broker non-votes and shares as to which a stockholder withholds voting authority are not considered votes cast and therefore will have no effect on the vote outcome.

*Proposal No. 2 Ratification of selection of independent registered public accounting firm.* This proposal must be approved by a majority of the votes cast on the proposal (meaning the number of shares voted for this proposal must exceed the number of shares voted against such proposal). As a result, abstentions and broker non-votes will have no effect on the vote outcome.

*Proposal No. 3 Advisory (non-binding) vote to approve executive compensation.* This advisory proposal must be approved by a majority of the votes cast on the proposal (meaning the number of shares voted for this proposal must exceed the number of shares voted against such proposal). As a result, abstentions and broker non-votes will have no effect on the vote outcome.

*Proposal No. 4 Advisory (non-binding) vote on frequency of future advisory votes to approve executive compensation.* This advisory vote provides a choice among three frequency periods for future advisory votes to approve executive compensation (so-called say-on-pay votes). The frequency period that receives the most votes (every one, two or three years) will be deemed to be the recommendation of the stockholders. As a result, any shares that are not voted, whether by abstention, broker non-votes or otherwise, will not affect the outcome of this proposal, except to the extent that the failure to vote for a particular frequency period may result in another frequency period receiving a larger proportion of the votes cast.



**Table of Contents**

We encourage you to vote by proxy, whether via telephone, through the Internet or by mailing an executed proxy card or voting instruction form, as discussed above. Voting in advance of the Annual Meeting reduces the likelihood that the Company will be forced to incur additional expenses soliciting proxies for the Annual Meeting.

Any registered holder of our common stock may attend the Annual Meeting and may change or revoke a delivered proxy by:

executing and returning a new, later-dated proxy card by mail, or submitting a new vote via telephone or through the Internet, as instructed above in advance of the applicable deadline;

delivering a written revocation to the corporate secretary before the Annual Meeting; or

voting at the Annual Meeting.

Beneficial holders of our common stock who wish to change or revoke their voting instructions should contact their brokerage firm, bank or other financial institution for information on how to do so.

**Table of Contents**

**PROPOSAL NO. 1 ELECTION OF DIRECTORS**

The Amended and Restated Certificate of Incorporation of the Company provides that the Board is to be divided into three classes as nearly equal in number as reasonably possible, with directors in each class generally serving three-year terms. The total Board size is currently fixed at seven directors. Currently, the Class III directors (whose terms expire at the Annual Meeting) are William Aliski, Lars Ekman, M.D., Ph.D. and Matthew K. Fust. The Class I directors (whose terms expire at the 2017 annual meeting of stockholders) are Emil D. Kakkis, M.D., Ph.D. and Daniel G. Welch. The Class II directors (whose terms expire at the 2018 annual meeting of stockholders) are Michael Narachi and Clay B. Siegall, Ph.D. The Class III directors elected at the Annual Meeting will hold office until the 2019 annual meeting of stockholders and until their successors are elected and qualified, unless they resign or their seats become vacant due to death, removal, or other cause in accordance with the Amended and Restated Bylaws of the Company.

As described below, the Board has nominated Messrs. Aliski and Fust and Dr. Ekman for election as Class III directors at the Annual Meeting. Messrs. Aliski and Fust and Dr. Ekman have indicated their willingness to serve if elected. Should Mr. Aliski, Dr. Ekman or Mr. Fust become unavailable for election at the Annual Meeting, the persons named on the enclosed proxy as proxy holders may vote all proxies given in response to this solicitation for the election of a substitute nominee(s) chosen by the Board or the Board may reduce the size of the Board.

**Nomination of Directors**

The Nominating and Corporate Governance Committee reviews and recommends to the Board potential nominees for election to the Board. In reviewing potential nominees, the Nominating and Corporate Governance Committee considers the qualifications of each potential nominee in light of the Board's existing and desired mix of experience and expertise. Specifically, the Nominating and Corporate Governance Committee considers each potential nominee's personal and professional ethics, integrity and values, experience and interest in the Company, and commitment to the representation of the long-term interests of the stockholders. The Board membership criteria are set forth in our Corporate Governance Guidelines, a copy of which is available on our website at [www.ultragenyx.com](http://www.ultragenyx.com) in the Corporate Governance subsection of the Investors tab.

After reviewing the qualifications of potential Board candidates, the Nominating and Corporate Governance Committee presents its recommendations to the Board, which selects the final director nominees. Upon the recommendation of the Nominating and Corporate Governance Committee, the Board nominated Messrs. Aliski and Fust and Dr. Ekman for election as Class III directors. The Company did not pay any fees to any third parties to identify or assist in identifying or evaluating nominees for the Annual Meeting.

The Nominating and Corporate Governance Committee considers stockholder-recommended director nominees using the same criteria set forth above. Stockholders who wish to recommend a potential nominee to the Nominating and Corporate Governance Committee for consideration for election at a future annual meeting of stockholders must provide the Nominating and Corporate Governance Committee with the same information that would be required to nominate a director candidate in accordance with the process and within the time periods for nominating director candidates set forth on page 49 under the caption Stockholder Proposals.

Although the Nominating and Corporate Governance Committee may consider whether nominees assist in achieving a mix of Board members that represents a diversity of background and experience, which is not only limited to race, gender or national origin, we have no formal policy regarding board diversity. The Nominating and Corporate Governance Committee assesses the effectiveness of its Board membership criteria as part of the director selection and nomination process.



**Table of Contents****Nominees and Incumbent Directors**

The Nominating and Corporate Governance Committee has recommended, and the Board has nominated, Messrs. Aliski and Fust and Dr. Ekman to be elected as Class III directors at the Annual Meeting. Dr. Ekman was recommended to the Nominating and Corporate Governance Committee as a potential director candidate by the Chairman of our Board, Daniel G. Welch. The following table sets forth the following information for Messrs. Aliski and Fust and Dr. Ekman and the Company's continuing directors: the year each was first elected a director of the Company; their respective ages as of the Record Date; the positions currently held with the Company; the year their current term will expire; and their current class.

| <b>Nominee/Director Name</b>                  | <b>Age</b> | <b>Position</b>                       | <b>Director Since</b> | <b>Year Current Term Expires</b> | <b>Current Director Class</b> |
|---|------------|---------------------------------------|-----------------------|----------------------------------|-------------------------------|
| <b><i>Nominees for Class III Director</i></b> |            |                                       |                       |                                  |                               |
| William Aliski                                | 68         | Director                              | 2011                  | 2016                             | III                           |
| Lars Ekman, M.D., Ph.D.                       | 66         | Director                              | 2016                  | 2016                             | III                           |
| Matthew K. Fust                               | 51         | Director                              | 2014                  | 2016                             | III                           |
| <b><i>Continuing Directors</i></b>            |            |                                       |                       |                                  |                               |
| Emil D. Kakkis, M.D., Ph.D.                   | 55         | President and Chief Executive Officer | 2010                  | 2017                             | I                             |
| Daniel G. Welch                               | 58         | Chairman of the Board                 | 2015                  | 2017                             | I                             |
| Michael Narachi                               | 56         | Director                              | 2015                  | 2018                             | II                            |
| Clay B. Siegall, Ph.D.                        | 55         | Director                              | 2014                  | 2018                             | II                            |

**Class III Directors Nominated for Election**

The following three people have been nominated by the Board to be elected as Class III directors at the 2016 Annual Meeting.

**William Aliski** has served as a member of our Board since January 2011. Mr. Aliski currently serves as a commercial consultant for early-stage orphan disease companies, including Ra Pharmaceuticals, Inc. and Clementia Pharmaceuticals, Inc. Mr. Aliski previously served as a commercial consultant to other early-stage orphan disease companies including OxThera from January 2015 through April 2015, Prosensa during 2014, Adimab LLC from November 2013 until December 2013, NPS Pharmaceuticals from April 2013 through December 2014, Fidelity Biosciences from August 2012 until December 2012 and Enobia Pharma from September 2011 until March 2012. Before that, Mr. Aliski served as Senior Vice President and Chief Commercial Officer of FoldRx Pharmaceuticals, a rare disease company that is now a wholly-owned subsidiary of Pfizer Inc., from June 2009 until March 2011, as Director of Simon Kucher Partners, a global consulting firm, from January 2008 until June 2009, and as General Manager of BioMarin Europe at BioMarin Pharmaceuticals Inc. from December 2005 until January 2008. Mr. Aliski received a B.S. in Economics and a Master of Social Planning from Boston College and an M.P.A. from the Kennedy School of Government at Harvard University. We believe that Mr. Aliski is qualified to serve on our Board due to his extensive experience in the life sciences industry, membership on various boards of directors, and his leadership and management experience.

**Lars Ekman, M.D., Ph.D.** has served as a member of our Board since March 2016. He has more than 30 years of experience in the pharmaceutical industry and is currently an executive partner at Sofinnova Ventures, a venture capital firm, where Lars has served as executive partner since March 2008. He currently serves as Chairman of the

Board of Amarin Corporation plc, Chairman of the Board of Sophiris Bio, Inc., Chairman of the Board of Prothena Corporation plc and also serves as a director of Spark Therapeutics, Inc. Dr. Ekman previously served on the boards of directors of Elan Corporation plc from 2007 through 2013, InterMune Inc. from 2006 through 2013 and Ocera Therapeutics, Inc. from 2013 through 2015. From October 2008 to 2011 he served as Co-Founder and Chief Executive



## Table of Contents

Officer of Cebix Inc., a C-peptide replacement therapy company. He was Executive Vice President and President of Global Research and Development at Elan Corporation plc, from January 2001 to December 2007. Prior to joining Elan, he was Executive Vice President, Research and Development at Schwarz Pharma AG from February 1997 to December 2000, and prior to that was employed in a variety of senior scientific and clinical functions at Pharmacia, now Pfizer. Dr. Ekman is a board-certified surgeon with a Ph.D. in experimental biology and has held several clinical and academic positions in both the United States and Europe. He obtained his Ph.D. and M.D. from the University of Gothenburg, Sweden. We believe that Dr. Ekman is qualified to serve on our Board due to his extensive experience in the life sciences industry, and particularly his research and development expertise, as well as his membership on various boards of directors in the biotechnology industry.

**Matthew K. Fust** has served as a member of our Board since January 2014. He is a board member and advisor to life sciences companies. Mr. Fust retired as Executive Vice President of Onyx Pharmaceuticals, Inc., a biopharmaceutical company, where he served from January 2009 until January 2014. From May 2003 to December 2008, Mr. Fust served as Chief Financial Officer at Jazz Pharmaceuticals, Inc., a specialty pharmaceutical company. From 2002 to 2003, Mr. Fust served as Chief Financial Officer at Perlegen Sciences, a biopharmaceutical company. Previously, he was Senior Vice President and Chief Financial Officer at ALZA Corporation, a pharmaceutical company, where he was an executive from 1996 until 2002. From 1991 until 1996, Mr. Fust was a manager in the healthcare strategy practice at Andersen Consulting. Mr. Fust serves on the board of directors of Sunesis Pharmaceuticals, Inc., Atara Biotherapeutics, Inc., Dermira, Inc., and MacroGenics, Inc., which are publicly traded biopharmaceutical companies. Mr. Fust received a B.A. in accounting from the University of Minnesota and an M.B.A. from the Stanford Graduate School of Business. We believe that Mr. Fust is qualified to serve on our Board due to his extensive experience in the life sciences industry, his financial experience and ability to be our audit committee financial expert, and his service as a director of other public biopharmaceutical companies.

## **Class I Directors Continuing in Office Until 2017**

**Emil D. Kakkis, M.D., Ph.D.** is our founder and has served as our President and Chief Executive Officer and as a member of our Board since the Company's inception in April 2010. Prior to Ultragenyx, Dr. Kakkis served from September 1998 to February 2009 in various executive capacities, and ultimately as Chief Medical Officer, at BioMarin Pharmaceutical Inc., a biopharmaceutical company. Dr. Kakkis then served as a development consultant to BioMarin from 2009 through 2010. Dr. Kakkis also serves as President and Founder of EveryLife Foundation for Rare Diseases, a non-profit organization he started in 2009 to accelerate biotechnology innovation for rare diseases. Dr. Kakkis is board certified in both Pediatrics and Medical Genetics. He holds a B.A. in Biology from Pomona College and received combined M.D. and Ph.D. degrees from the UCLA School of Medicine's Medical Scientist Training Program where he received the Bogen prize for his research. We believe that Dr. Kakkis possesses specific expert knowledge of genetics and rare diseases and operational experience in the life sciences sector that qualify him to serve on our Board.

**Daniel G. Welch** has served as a member of our Board since April 2015 and Chairman of the Board since June 2015. Mr. Welch is currently an Executive Partner at Sofinnova Ventures, a venture capital firm. Prior to Sofinnova, Mr. Welch served as Chairman, Chief Executive Officer and President of InterMune, a biotechnology company, from May 2008 to October 2014 and served as President and Chief Executive Officer of InterMune and a member of its board of directors from September 2003 to May 2008. From August 2002 to January 2003, Mr. Welch served as Chairman and Chief Executive Officer of Triangle Pharmaceuticals, Inc., a pharmaceutical company. From October 2000 to June 2002, Mr. Welch served as president of the pharmaceutical division of Elan Corporation, PLC. Mr. Welch currently serves as chairman of the board of AveXis, Inc., a publicly traded biotechnology company, and also serves on the boards of directors of Seattle Genetics, Inc. and Intercept Pharmaceuticals, Inc., both of which are publicly traded biotechnology companies. Mr. Welch previously served on the boards of directors of Hyperion

Therapeutics, Inc., a publicly traded biotechnology company, from 2012 through its acquisition in 2015, as well as Corium International, Inc., a publicly traded biopharmaceutical company, from 2007 through 2014. Mr. Welch holds a B.S. from the University of Miami and

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**Table of Contents**

an M.B.A. from the University of North Carolina. We believe that Mr. Welch is a strong operating executive with operational and strategic expertise in the global pharmaceutical market, whose experience contributes valuable insight to the Board.

**Class II Directors Continuing in Office Until 2018**

**Michael Narachi** has served as a member of our Board since February 2015. Mr. Narachi currently serves as President and Chief Executive Officer and a director of biotechnology company Orexigen Therapeutics, Inc., a position he has held since March 2009. Previously, Mr. Narachi served as Chairman, Chief Executive Officer and President of Ren Pharmaceuticals, Inc., a private biotechnology company, from November 2006 to March 2009. In 2004, Mr. Narachi retired as an officer and Vice President of Amgen Inc., a leading therapeutics company, where he served as General Manager of Amgen's Anemia Business from 1999 to 2003. Mr. Narachi joined Amgen in 1984 and held various senior positions throughout the organization over a 20-year career including: Vice President of Development and Representative Director for Amgen Japan; Head of Corporate Strategic Planning; Chief Operations Officer of Amgen BioPharma; and Vice President, Licensing and Business Development. He served as General Manager of Amgen's Anemia Business from 1999 to 2003 until his retirement in 2004. He currently serves as the chairman of the board of directors of Celladon Corporation, a publicly traded biotechnology company. Mr. Narachi also currently serves as a member of the Board of Directors of PhRMA, the Pharmaceutical Research and Manufacturers of America, and BIO, the Biotechnology Industry Organization. Mr. Narachi received a B.S. in Biology and an M.A. degree in Biology and Genetics from the University of California at Davis. He received an M.B.A. from the Anderson Graduate School of Management at University of California, Los Angeles. We believe that Mr. Narachi is qualified to serve on our Board due to his extensive experience in the life sciences industry, his role as Chief Executive Officer of a publicly traded biotechnology company and his membership on various boards of directors in the biotechnology and pharmaceutical sectors.

**Clay B. Siegall, Ph.D.** has served as a member of our Board since January 2014. Dr. Siegall currently serves as President and Chief Executive Officer and Chairman of the Board of Seattle Genetics, Inc., a biotechnology company. Dr. Siegall co-founded Seattle Genetics in 1998. Prior to Seattle Genetics, Dr. Siegall worked for the Bristol-Myers Squibb Pharmaceutical Research Institute from 1991 to 1997 and the National Cancer Institute, National Institutes of Health from 1988 to 1991. In addition to Seattle Genetics, Dr. Siegall serves as a director of Alder BioPharmaceuticals, Inc., a publicly traded biotechnology company, and Mirna Therapeutics, Inc., a publicly traded biotechnology company. Dr. Siegall received a B.S. in Zoology from the University of Maryland and a Ph.D. in Genetics from George Washington University. We believe that Dr. Siegall is qualified to serve on our Board due to his extensive experience in the life sciences industry and his role as Chief Executive Officer of a publicly traded biotechnology company, both of which give him the skills to provide strategic leadership to our Company.

**Vote Required**

The three nominees who receive the greatest number of affirmative votes will be elected as Class III directors. Broker non-votes and shares as to which a stockholder withholds voting authority are not considered votes cast and therefore will have no effect on the vote outcome.

Holders of proxies solicited by this Proxy Statement will vote the proxies received by them as directed on the proxy card or, if no direction is given, then FOR the election of each of the nominees named in this Proxy Statement.

**THE BOARD OF DIRECTORS RECOMMENDS A VOTE FOR  
EACH OF THE THREE DIRECTOR NOMINEES IDENTIFIED ABOVE.**



Table of Contents

**PROPOSAL NO. 2 RATIFICATION OF SELECTION OF INDEPENDENT REGISTERED  
PUBLIC ACCOUNTING FIRM**

Our Audit Committee has selected Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2016, and has further directed that we submit the selection of Ernst & Young LLP for ratification by our stockholders at the Annual Meeting.

The Company is not required to submit the selection of our independent registered public accounting firm for stockholder approval, but is submitting its selection of Ernst & Young LLP for stockholder ratification as a matter of good corporate governance. If the stockholders do not ratify this selection, the Audit Committee will reconsider its selection of Ernst & Young LLP. Even if the selection is ratified, our Audit Committee may direct the appointment of a different independent registered public accounting firm at any time during the year if the Audit Committee determines that the change would be in the best interests of the Company.

The Audit Committee reviews and pre-approves all audit and non-audit services performed by its independent registered public accounting firm. The Audit Committee may delegate its pre-approval authority to one or more of its members and has delegated such authority to the Chairman of the committee; any pre-approval decisions made by the Chairman are reported by the Chairman to the Audit Committee at the next scheduled committee meeting. The Audit Committee may pre-approve specified audit-related services (assurance and related services that are reasonably related to the performance of the audit or review of the Company's financial statements and that are traditionally performed by the independent auditor) as well as specified tax services that the Audit Committee believes would not impair the independence of the independent auditor, and that are consistent with rules on auditor independence established by the SEC and the Public Company Accounting Oversight Board ( *PCAOB* ). The Audit Committee may also pre-approve those permissible non-audit services classified as all other services that it believes are routine and recurring services and would not impair the independence of the independent auditor and are consistent with SEC and PCAOB rules on auditor independence. All requests or applications for services to be provided by the independent auditor will be submitted to the Chief Financial Officer and must include a detailed description of the services to be rendered. The Chief Financial Officer or the Principal Accounting Officer, as the case may be, will authorize those services that have been pre-approved by the Audit Committee. If there is any question as to whether a proposed service fits within the pre-approved categories of services, the Chairman of the Audit Committee is to be consulted for a determination. For services that have not been pre-approved by the Audit Committee, requests or applications to provide services will be submitted to the Audit Committee by both the independent auditor and the Chief Financial Officer, and must include a joint oral or written statement as to whether, in their view, the request or application is consistent with the SEC's and PCAOB's rules on auditor independence.

All services rendered by Ernst & Young LLP in fiscal 2015 were approved in accordance with these policies. In its review of non-audit services, the Audit Committee considers, among other things, the possible impact of the performance of such services on the independent registered public accounting firm's independence. The Audit Committee has determined that the non-audit services performed by Ernst & Young LLP in the fiscal year ended December 31, 2015 were compatible with maintaining the independent registered public accounting firm's independence. Additional information concerning the Audit Committee and its activities can be found in the following sections of this Proxy Statement: Board Committees and Report of the Audit Committee.

Ernst & Young LLP has audited our financial statements since inception. Representatives of Ernst & Young LLP are expected to be present at the Annual Meeting, will have the opportunity to make a statement if they desire to do so and will be available to respond to appropriate stockholder questions.



**Table of Contents****Fees for Independent Registered Public Accounting Firm**

The following is a summary of the aggregate fees billed to the Company for the audit and other services rendered by Ernst & Young LLP, our independent registered public accounting firm, for the fiscal years ended December 31, 2015 and 2014.

|                               | <b>2015</b>         | <b>2014</b>       |
|-------------------------------|---------------------|-------------------|
| Audit fees <sup>(1)</sup>     | \$ 1,409,000        | \$ 974,000        |
| Audit-related fees            |                     |                   |
| Tax fees <sup>(2)</sup>       | 91,000              | 9,000             |
| All other fees <sup>(3)</sup> | 2,000               | 3,000             |
| <b>Total</b>                  | <b>\$ 1,502,000</b> | <b>\$ 986,000</b> |

- (1) Audit fees consist of the aggregate fees billed for professional services rendered for the audit of our annual financial statements included in our Annual Report on Form 10-K, including for the first time in 2015 an opinion expressed on the internal control over financial reporting for the year ended December 31, 2015; the review of our interim financial statements included in the Quarterly Reports on Form 10-Q; assistance with registration statements filed with the SEC; and the issuance of comfort letters and consents.
- (2) Tax fees principally include fees for tax compliance and tax advice, including \$52,000 in fees for a transaction cost analysis study that was performed in 2015 related to the deductibility of costs incurred in connection with the Company's initial public offering in January 2014.
- (3) All other fees consist of fees for accessing Ernst & Young LLP's online research database.

**Vote Required**

Ratification of the selection of the independent registered public accounting firm requires the affirmative vote of a majority of the votes cast. Because abstentions and broker non-votes are not counted as votes for or against this proposal, they will have no effect on the outcome of the vote.

**THE BOARD OF DIRECTORS RECOMMENDS A VOTE FOR PROPOSAL NO. 2.**

**Table of Contents**

**REPORT OF THE AUDIT COMMITTEE**

The Audit Committee evaluates auditor performance, manages relations with the Company's independent registered public accounting firm, and evaluates policies and procedures relating to internal control systems. The Audit Committee operates under a written Audit Committee Charter that has been adopted by the Board, a copy of which is available on the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com). All members of the Audit Committee currently meet the independence and qualification standards for Audit Committee membership set forth in the listing standards and rules of NASDAQ and the SEC.

No member of the Audit Committee is a professional accountant or auditor. The members' functions are not intended to duplicate or to certify the activities of management and the independent registered public accounting firm. The Audit Committee serves a board-level oversight role in which it provides advice, counsel and direction to management and the auditors on the basis of the information it receives, discussions with management and the auditors, and the experience of the Audit Committee's members in business, financial and accounting matters.

The Audit Committee oversees the Company's financial reporting process on behalf of the Board. The Company's management has the primary responsibility for the financial statements and reporting process, including the Company's system of internal controls over financial reporting. In fulfilling its oversight responsibilities, the Audit Committee reviewed and discussed with management the audited financial statements included in the Annual Report on Form 10-K for the fiscal year ended December 31, 2015. This review included a discussion of the quality and the acceptability of the Company's financial reporting, including the nature and extent of disclosures in the financial statements and the accompanying notes.

The Audit Committee also reviewed with the Company's independent registered public accounting firm, which is responsible for expressing an opinion on the conformity of the audited financial statements with accounting principles generally accepted in the United States of America, their judgments as to the quality and the acceptability of the Company's financial reporting and such other matters as are required to be discussed with the Committee pursuant to Auditing Standard No. 16, *Communication with Audit Committees* issued by the Public Company Accounting Oversight Board. The Audit Committee has received the written disclosures and the letter from the independent registered public accounting firm required by the Public Company Accounting Oversight Board regarding the independent accountant's communications with the Audit Committee concerning independence. The Audit Committee discussed with the independent registered public accounting firm their independence from management and the Company, including the matters required by the applicable rules of the Public Company Accounting Oversight Board.

In addition to the matters specified above, the Audit Committee discussed with the Company's independent registered public accounting firm the overall scope, plans and estimated costs of their audit. The Audit Committee met with the independent registered public accounting firm periodically, with and without management present, to discuss the results of the independent registered public accounting firm's examinations, the overall quality of the Company's financial reporting and the independent registered public accounting firm's reviews of the quarterly financial statements, and drafts of the quarterly and annual reports.

In reliance on the reviews and discussions referred to above, the Audit Committee recommended to the Board that the Company's audited financial statements should be included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2015.

Submitted by the Audit Committee of the Board of Directors

Matthew Fust, Chairperson



William Aliski

Michael Narachi

**Table of Contents**

**PROPOSAL NO. 3 ADVISORY VOTE TO APPROVE EXECUTIVE COMPENSATION**

**Background**

The Dodd-Frank Wall Street Reform and Consumer Protection Act (the *Dodd-Frank Act*) requires that stockholders have the opportunity to cast an advisory (non-binding) vote to approve executive compensation (a *say-on-pay vote*), as well as an advisory vote with respect to whether future say-on-pay votes will be held every one, two or three years, which is the subject of Proposal No. 4.

The advisory vote to approve executive compensation is a non-binding vote on the compensation of the Company's named executive officers, as described in the Compensation Discussion and Analysis section, the tabular disclosure regarding such compensation, and the accompanying narrative disclosure, set forth in this proxy statement. The advisory vote to approve executive compensation is not a vote on the Company's general compensation policies, compensation of the Company's Board of Directors, or the Company's compensation policies as they relate to risk management. The Dodd-Frank Act requires the Company to hold the advisory vote to approve executive compensation at least once every three years.

Our philosophy in setting compensation policies for executive officers has two fundamental objectives: (1) to attract and retain a highly-skilled team of executives and (2) to align our executives' interests with those of our stockholders by rewarding short-term and long-term performance and tying compensation to increases in stockholder value. The Compensation Committee believes that executive compensation should be directly linked both to continuous improvements in corporate performance (so-called *pay for performance*) and accomplishments that are expected to increase stockholder value. The Compensation Discussion and Analysis section starting on page 21 provides a more detailed discussion of the executive compensation program and compensation philosophy.

The vote under this Proposal No. 3 is advisory, and therefore not binding on the Company, the Board or our Compensation Committee. However, our Board, including our Compensation Committee, values the opinions of our stockholders and, to the extent there is any significant vote against the executive officer compensation as disclosed in this proxy statement, we will consider our stockholders' concerns and evaluate what actions may be appropriate to address those concerns.

Stockholders will be asked at the Annual Meeting to approve the following resolution pursuant to this Proposal No. 3:

RESOLVED, that the stockholders of Ultragenyx Pharmaceutical Inc. approve, on an advisory basis, the compensation of the Company's named executive officers (as defined in the Proxy Statement), as such compensation is described in the Compensation Discussion and Analysis section, the tabular disclosure regarding such compensation, and the accompanying narrative disclosure, set forth in the Company's definitive proxy statement for the 2016 Annual Meeting of Stockholders (the *Proxy Statement*).

**Recommendation**

The Board of Directors recommends a vote **FOR** approval of the foregoing resolution. Proxies will be so voted unless stockholders specify otherwise in their proxies.

**Vote Required**

Approval of this resolution requires the affirmative vote of a majority of the votes cast on this proposal. Because abstentions and broker non-votes are not counted as votes cast for or against this resolution, they will have no effect

on the outcome of the vote.

**THE BOARD OF DIRECTORS RECOMMENDS A VOTE FOR PROPOSAL NO. 3.**

**Table of Contents**

**PROPOSAL NO. 4 ADVISORY VOTE ON FREQUENCY OF FUTURE ADVISORY VOTES TO APPROVE EXECUTIVE COMPENSATION**

**Background**

We are also required by the Dodd-Frank Act to provide stockholders with a separate advisory (non-binding) vote for the purpose of asking stockholders to express their preference for the frequency of future say-on-pay votes. Stockholders may indicate whether they would prefer an advisory vote to approve executive compensation once every one, two or three years. We are required to solicit stockholder votes on the frequency of future say-on-pay proposals at least once every six years, although we may seek stockholder input more frequently.

The frequency period that receives the most votes (every one, two or three years) will be deemed to be the recommendation of the stockholders. However, because this vote is advisory and not binding on the Board of Directors or the Company, the Board may decide that it is in the best interests of our stockholders and the Company to hold an advisory vote to approve executive compensation more or less frequently than the option selected by a plurality of our stockholders. The Board of Directors will, however, carefully consider the outcome of this vote when considering the frequency of future advisory votes on executive compensation.

**Recommendation**

The Board believes there is a reasonable basis for holding the say-on-frequency vote every year, every two years and every three years; less frequent votes encourage a more long-term analysis of the Company's executive compensation programs and would avoid the burden that annual votes would impose on stockholders required to evaluate the executive compensation program each year, but more frequent votes provide stockholders with the opportunity to react to emerging trends in compensation and gives the Board and the Compensation Committee the opportunity to evaluate the compensation program each year in light of timely input from stockholders. The Board also believes that it is best practice to hold the advisory vote on executive compensation annually.

Therefore, the Board recommends that stockholders vote to hold the advisory vote on executive compensation every year. Stockholders are not voting, however, to approve or disapprove of this particular recommendation. The proxy card provides for four choices and stockholders are entitled to vote on whether the advisory vote on executive compensation should be held every one, two, or three years, or to abstain from voting.

**Vote Required**

The frequency period that receives the most votes (every one, two or three years) will be deemed to be the recommendation of the stockholders. Abstentions and broker non-votes will have no effect on the outcome of the vote, except to the extent that the failure to vote for a particular frequency period may result in another frequency period receiving a larger proportion of the votes cast.

**Table of Contents**

**CORPORATE GOVERNANCE**

**Director Independence**

Our Board currently consists of seven members. Our Board has determined that Mr. Aliski, Dr. Ekman, Mr. Fust, Mr. Narachi, Dr. Siegall and Mr. Welch qualify as independent directors in accordance with the NASDAQ listing requirements and rules. In addition, the Board previously determined that Eran Nadav, Ph.D., whose term expired at the 2015 annual meeting of stockholders, was an independent director. Dr. Kakkis is not considered independent because he is an employee of Ultragenyx. Under NASDAQ rules, the Board's determination of a director's independence considers objective tests, such as whether the director is, or has been within the last three years, an employee of the Company and whether the director or any of his family members has engaged in certain types of business dealings with the Company. Under NASDAQ rules, our Board also evaluates whether any relationships exist that, in the opinion of our Board, would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In making these independence determinations, our Board reviewed and discussed information provided by the directors and us with regard to each director's business and personal activities and relationships as they may relate to us and our management, including any significant stock holdings by funds affiliated with the directors. There are no family relationships among any of our directors or executive officers.

**Code of Business Conduct and Ethics**

We have adopted a Code of Business Conduct and Ethics that applies to all of our employees, officers, and directors, including those officers responsible for financial reporting. Our Code of Business Conduct and Ethics is available on our website, [www.ultragenyx.com](http://www.ultragenyx.com), under the Corporate Governance subsection of the Investors tab. We will promptly disclose on our website any future changes or amendments to the Code of Business Conduct and Ethics, and any waivers of our Code of Business Conduct and Ethics that applies to our Chairman of the Board, any of our executive officers, or any member of our Board.

**Stockholder Communications**

Generally, stockholders who have questions or concerns regarding the Company should contact our Investor Relations department at (844) 758-7273. However, any stockholders who wish to address questions regarding the business or affairs of the Company directly with the Board, or any individual director, should direct his or her questions in writing to the Chairman of the Board, c/o Ultragenyx Pharmaceutical Inc., 60 Leveroni Court, Novato, California 94949. Upon receipt of any such communications, the correspondence will be directed to the appropriate person, including individual directors.

**BOARD OF DIRECTORS AND COMMITTEES**

During fiscal 2015, our Board met six times and also acted by written consent five times. Each director attended at least 75% of the aggregate of the meetings of the Board and meetings of the committees of which he was a member in our last fiscal year.

The Board has a standing Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee. All members of the Audit, Compensation and Nominating and Corporate Governance Committees are non-employee directors whom the Board has determined are independent under the applicable independence standards.

Four of the six directors serving at the time of the 2015 Annual Meeting of Stockholders attended such annual meeting. Each director who is up for election at an annual meeting of stockholders or who has a term that continues after such annual meeting is expected to attend the annual meeting of stockholders.

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**Table of Contents****Board Leadership Structure and Risk Oversight**

The Company currently separates the positions of Chairman of the Board and Chief Executive Officer, which allows our Chief Executive Officer, Dr. Kakkis, to focus on our day-to-day business, while allowing the Chairman of the Board, Mr. Welch, to lead the Board in its fundamental role of providing advice to and independent oversight of management. Independent oversight of management is an important goal of the Board, which is why our Corporate Governance Guidelines provide that a lead independent director will be appointed by the Board if the Chairman is not independent. Additionally, our Board recognizes the time, effort, and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as our Chairman. Our Board also believes that the separation of the Chairman and Chief Executive Officer positions fosters a greater role for the independent directors in the oversight of our Company and active participation of the independent directors in setting agendas and establishing priorities and procedures for the work of our Board. The benefits of the separated Chairman and Chief Executive Officer positions are augmented by the independence of six of our seven current directors, including our Chairman, and our independent Board committees that provide appropriate oversight in the areas described below. At executive sessions of independent directors, these directors can speak candidly on any matter of interest. The independent directors of the Board regularly meet in executive sessions, having done so three times in 2015, and the Chairman presides at these sessions. We believe this structure provides effective oversight of our management and the Company.

The Board has overall responsibility for the oversight of the Company's risk management process, which is designed to support the achievement of organizational objectives, including strategic objectives, to improve long-term organizational performance, and enhance stockholder value. Risk management includes not only understanding company-specific risks and the steps management implements to manage those risks, but also what level of risk is acceptable and appropriate for the Company. Management is responsible for establishing our business strategy, identifying, and assessing the related risks and implementing appropriate risk management practices. The Board periodically reviews our business strategy and management's assessment of the related risk and discusses with management the appropriate level of risk for the Company. The Board also delegates to Board committees oversight of selected elements of risk as set forth below.

**Board Committees**

*Audit Committee.* The members of the Audit Committee are Mr. Fust (Chairperson), Mr. Aliski and Mr. Narachi. The Audit Committee appoints, approves the compensation of, reviews the performance of and assesses the independence of our independent registered public accounting firm; approves audit and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm; reviews the audit plan with the independent registered public accounting firm and members of management responsible for preparing our financial statements; reviews and discusses with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures and critical accounting policies; reviews the adequacy of our internal control over financial reporting; establishes policies and procedures for the receipt and retention of accounting-related complaints and concerns; recommends whether our audited financial statements shall be included in our Annual Report on Form 10-K; prepares the audit committee report to be included in our annual proxy statements; reviews all related-person transactions; reviews policies related to risk assessment and risk management; establishes, maintains and oversees our Code of Business Conduct and Ethics; and performs other duties, as specified in the Audit Committee Charter, a copy of which is available on the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com) under the Corporate Governance subsection of the Investors tab. The Audit Committee has been delegated the task of overseeing all financial risks facing the Company and reporting back to the Board regarding same. The Audit Committee met six times in fiscal 2015. All members of the Audit Committee satisfy the current independence and financial literacy standards promulgated by NASDAQ and the SEC, and the Board has determined that Mr. Fust

qualifies as an audit committee financial expert, as the SEC has defined that term in Item 407 of Regulation S-K.



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**Table of Contents**

*Compensation Committee.* The members of the Compensation Committee are Mr. Aliski (Chairperson), Dr. Siegall and Mr. Welch. The Compensation Committee annually reviews and approves individual and corporate goals and objectives relevant to the compensation of our executive officers; evaluates the performance of our executive officers in light of such individual and corporate goals and objectives and determines the compensation of our executive officers; appoints, compensates and oversees the work of any compensation consultant, legal counsel or other advisor retained by the Compensation Committee; conducts the independence assessment outlined in NASDAQ rules with respect to any compensation consultant, legal counsel, or other advisor retained by the Compensation Committee; annually reviews and reassesses the adequacy of the Compensation Committee charter; oversees, and has the authority to administer, our compensation and similar plans; reviews and approves our policies and procedures for the grant of equity-based awards; reviews and makes recommendations to the Board with respect to director compensation; reviews and discusses with management the compensation discussion and analysis, if any, to be included in our annual proxy statements or Annual Reports on Form 10-K; reviews and discusses with the Board corporate succession plans for the Chief Executive Officer and other senior management positions; and performs other duties, as specified in the Compensation Committee Charter, a copy of which is available on the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com) under the Corporate Governance subsection of the Investors tab. The Compensation Committee may form and delegate authority to subcommittees, each consisting of one or more members of the Compensation Committee, with such powers as the Compensation Committee shall from time to time confer. Our Chief Executive Officer may recommend the amount and form of compensation of other executive officers to the Compensation Committee. The Compensation Committee met five times in fiscal 2015 and also acted by written consent seven times. All members of the Compensation Committee satisfy the current NASDAQ and SEC independence standards.

*Nominating and Corporate Governance Committee.* The members of the Nominating and Corporate Governance Committee are Mr. Fust (Chairperson), Mr. Narachi and Mr. Welch. The Nominating and Corporate Governance Committee develops and recommends to the Board criteria for Board and committee membership; establishes procedures for identifying and evaluating Board candidates, including nominees recommended by stockholders; identifies individuals qualified to become members of the Board; recommends to the Board the persons to be nominated for election as directors and to each of the Board's committees; develops and recommends to the Board a set of corporate governance guidelines; and performs other duties, as specified in the Nominating and Corporate Governance Committee Charter, a copy of which is available on the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com) under the Corporate Governance subsection of the Investors tab. The Nominating and Corporate Governance Committee has been delegated the task of overseeing all risks, other than financial risks (which are overseen by the Audit Committee), facing the Company and reporting back to the Board regarding same. The Nominating and Corporate Governance Committee met four times in fiscal 2015. All members of the Nominating and Corporate Governance Committee satisfy the current NASDAQ independence standards.

**Compensation Committee Interlocks and Insider Participation**

During fiscal 2015, the Compensation Committee consisted of Mr. Aliski, Dr. Siegall, Mr. Welch (from June 18, 2015) and Dr. Nadav (through June 18, 2015). None of the members of our Compensation Committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our Board or Compensation Committee.

Table of Contents**EXECUTIVE OFFICERS**

Our current executive officers and their respective ages and positions are set forth in the following table. Biographical information regarding each executive officer (other than Dr. Kakkis) is set forth following the table. Biographical information for Dr. Kakkis is set forth above under Proposal No. 1 (Election of Directors).

| <b>Name</b>                 | <b>Age</b> | <b>Position</b>   |
|-----------------------------|------------|---|
| Emil D. Kakkis, M.D., Ph.D. | 55         | President and Chief Executive Officer, Director (principal executive officer)               |
| Sunil Agarwal, M.D.         | 46         | Chief Medical Officer and Senior Vice President   |
| Jayson Dallas, M.D.         | 48         | Chief Commercial Officer and Senior Vice President  |
| Dennis Huang                | 51         | Chief Technical Operations Officer and Senior Vice President                                |
| Thomas Kassberg             | 55         | Chief Business Officer and Senior Vice President  |
| John R. Pinion II           | 50         | Chief Quality Operations Officer and Senior Vice President Analytical Sciences and Research |
| Shalini Sharp               | 41         | Chief Financial Officer and Senior Vice President (principal financial officer)             |

**Sunil Agarwal, M.D.** has served as our Chief Medical Officer and Senior Vice President since August 2014. Prior to Ultragenyx, Dr. Agarwal served in various leadership capacities at Genentech, a pharmaceutical company, for 11 years. Most recently, he held the position of Senior Vice President and Global Head of Clinical Development for OMNI (Ophthalmology, Metabolism, Neurosciences, Immunology and Infectious Diseases) since January 2013. Prior to that, Dr. Agarwal held the positions of Senior Vice President for Immunology and Infectious Diseases, and Vice President of Rheumatology from July 2009 to December 2012. He also held the position of Vice President of Genentech Drug Safety from January 2009 to July 2009. From September 2003 to January 2009, Dr. Agarwal held positions of increasing responsibility in Genentech's Immunology clinical organization, and was involved in the development oversight of multiple molecules including Raptiva, Rituxan, and Ocrelizumab. Dr. Agarwal has served as a board member of Calithera Biosciences, Inc. since September 2015 and MyoKardia, Inc. since March 2016. Dr. Agarwal obtained his Bachelor of Science in Neuro-Biology at Cornell University and then earned his medical degree from Tufts University School of Medicine. He completed his residency at Children's National Medical Center (CNMC), Washington, D.C. and subsequently joined the facility at George Washington University School of Medicine as an Assistant Clinical Professor of Pediatrics. He practiced in the Pediatric Emergency Department at CNMC.

**Jayson Dallas, M.D.** has served as our Chief Commercial Officer and Senior Vice President since August 2015. Prior to Ultragenyx, Dr. Dallas served as General Manager of Roche, a healthcare company, in the United Kingdom from July 2012 to July 2015. Prior to this, he held two different positions at Genentech, a pharmaceutical company, as Head of Global Oncology Launch Excellence and Biosimilar Strategy and Head of Global Product Strategy for Immunology and Ophthalmology from May 2010 to June 2012. Prior to joining Genentech, Dr. Dallas worked at Novartis and Pharmacia in the U.S. and previously at Roche in Switzerland. Dr. Dallas holds an M.D. from the University of the Witwatersrand, Johannesburg, South Africa and an M.B.A. from Ashridge Business School in the United Kingdom.

**Dennis Huang** has served as our Chief Technical Operations Officer and Senior Vice President since May 2015. Prior to Ultragenyx, Mr. Huang served as Senior Vice President of Manufacturing and Supply Chain at InterMune, Inc., a biotechnology company, from August 2013 to March 2015. Prior to InterMune, Mr. Huang served as Vice President of Biologic Manufacturing and Development at Allergan, Inc., a global pharmaceutical company, from May 2006 to August 2013. Mr. Huang holds a B.A. in Chemistry from Knox College in Galesburg, Illinois.

**Thomas Kassberg** has served as our Chief Business Officer and Senior Vice President since November 2011. Prior to Ultragenyx, Mr. Kassberg worked as Vice President of Business Development at Corium International, Inc., a biotechnology company, from July 2010 until October 2011. Prior to his work at Corium

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## **Table of Contents**

International, Inc., Mr. Kassberg worked as an independent consultant in corporate development and business strategy and consulted with a number of companies from March 2009 to June 2010, including Corium International, Inc. and Rib-X Pharmaceuticals, Inc., a pharmaceutical company focused on the development of novel antibiotics. Before becoming a consultant, Mr. Kassberg worked at Proteolix, Inc., a biotechnology company subsequently acquired by Onyx Pharmaceuticals, from January 2008 until February 2009, where he served as Senior Vice President of Corporate Development. Mr. Kassberg holds a B.A. in Business Administration from Gustavus Adolphus College and an M.B.A. from Northwestern University.

**John R. Pinion II** has served as our Chief Quality Operations Officer and Senior Vice President of Analytical Sciences and Research since July 2015. Prior to Ultragenyx, Mr. Pinion joined Genentech, a pharmaceutical company, in 2005 as the Vice President of External Quality and held roles with increasing responsibilities including his most recent position as the Senior Vice President and Global Head of Quality and Compliance for Roche/Genentech Pharma Technical Operations from October 2009 through July 2015. Mr. Pinion holds a Bachelor of Science in Mechanical Engineering from the University of West Virginia.

**Shalini Sharp** has served as our Chief Financial Officer and Senior Vice President since May 2012. Prior to Ultragenyx, Ms. Sharp served in various executive capacities, and ultimately as Chief Financial Officer, of Agenus Inc., a biotechnology company, from August 2003 until May 2012. Prior to Agenus, Ms. Sharp held strategic planning and corporate finance roles and ultimately served as chief of staff to the chairman of the board at Elan Pharmaceuticals, a biotechnology company, from August 1998 to August 1999 and September 2001 to August 2003. Prior to Elan, Ms. Sharp was a management consultant at McKinsey & Company and an investment banker at Goldman Sachs, specializing in pharmaceuticals and medical devices. Ms. Sharp has also served as a board member of Agenus since May 2012. Ms. Sharp holds a B.A. and an M.B.A. from Harvard University.

## **CERTAIN RELATIONSHIPS AND RELATED-PERSON TRANSACTIONS**

### **Related-Person Transactions**

Since January 1, 2015, we have not become, and are not currently proposed to be, a party to any transactions required to be disclosed under SEC rules with any related persons, which are generally considered to be our directors and executive officers, nominees for director, holders of more than 5% of our outstanding common stock and members of their immediate families.

### **Procedures for Related-Person Transactions**

We have adopted a written related-person transaction approval policy that governs the review, approval and/or ratification of transactions with a related person where the amount involved exceeds \$100,000 and in which any related person has or will have a direct or indirect interest. Under the policy, related person is defined as a director, nominee for director or executive officer of the Company; a beneficial owner of five percent or more of any class of the Company's voting securities; and a person who is an immediate family member of any such director, executive officer or five percent beneficial owner (the term immediate family member shall include any child, stepchild, parent, stepparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law or sister-in-law and any person (other than a tenant or employee) sharing the household of any such director, executive officer or five percent beneficial owner). Pursuant to this policy, if we want to enter into a transaction with a related person, our Chief Financial Officer will review the proposed transaction to determine, based on applicable NASDAQ and SEC rules, if such transaction qualifies as a related-person transaction. If the Chief Financial Officer determines that the proposed transaction is a related-person transaction, then the proposed transaction shall be submitted to the Audit Committee for pre-approval at the next regular or special Audit Committee meeting; if the Chief Financial Officer, in

consultation with the Chief Executive Officer, determines that it is not practicable to wait until the next meeting of the Audit Committee, then the Chief Financial Officer shall submit the proposed transaction to the chairperson of the Audit Committee.

## **Table of Contents**

In the event that our Chief Executive Officer or Chief Financial Officer becomes aware of a related-person transaction that has not been previously approved or previously ratified under our related-person transaction approval policy, the transaction, if pending or ongoing, will be promptly submitted to the Audit Committee or the chairperson of the Audit Committee for consideration. If the transaction is already completed, the Audit Committee or the chairperson of the Audit Committee shall evaluate the transaction to determine if rescission of the transaction and/or any disciplinary action is appropriate. In evaluating these transactions, the Audit Committee considers all of the relevant facts and circumstances available, including (if applicable) but not limited to: the benefits to the Company; the impact on a director's independence in the event the related person is a director, an immediate family member of a director or an entity in which a director has a position or relationship; the availability of other sources for comparable products or services; the terms of the transaction; and the terms available to unrelated third parties or to employees generally. Additionally, no member of the Audit Committee shall participate in any review, consideration or approval of any related-person transaction with respect to which such member or any of his or her immediate family members is the related person.

### **SECTION 16(A) BENEFICIAL OWNERSHIP REPORTING COMPLIANCE**

Under Section 16(a) of the Exchange Act and SEC rules, our directors, executive officers and beneficial owners of more than 10% of any class of equity security are required to file periodic reports of their ownership, and changes in that ownership, with the SEC. To our knowledge, based solely on the review of copies of the reports filed with the SEC and any written representations that no other reports were required, all reports required to be filed by our executive officers, directors and beneficial owners of more than 10% of our common stock were timely filed in fiscal 2015, except that Dr. Kakkis, Dr. Agarwal, Mr. Kassberg, Ms. Sharp and Mr. Huizenga each filed one Form 4 late, which Forms 4 each reported one award of annual equity grants, each of which includes a grant of stock options and restricted stock units.

### **SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT**

The following table sets forth information relating to the beneficial ownership of our common stock as of April 11, 2016 (unless otherwise indicated), by:

each person, or group of affiliated persons, known by us to beneficially own more than 5% of our outstanding shares of common stock;

each of our directors;

each of our named executive officers; and

all current directors and executive officers as a group.

The number of shares beneficially owned by each entity, person, director or executive officer is determined in accordance with the rules of the SEC, and the information is not necessarily indicative of beneficial ownership for any other purpose. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days through the exercise of any stock option, warrants or other rights. We believe, based on the information furnished to

us, that except as otherwise indicated, and subject to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of common stock held by that person.

The percentage of shares beneficially owned is computed on the basis of 39,005,470 shares of our common stock outstanding as of April 11, 2016. Shares of our common stock that a person has the right to acquire within 60 days are deemed outstanding for purposes of computing the percentage ownership of the person holding such rights, but are not deemed outstanding for purposes of computing the percentage ownership

**Table of Contents**

of any other person, except with respect to the percentage ownership of all directors and executive officers as a group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o Ultragenyx Pharmaceutical Inc., at 60 Leveroni Court, Novato, California 94949.

| Name and Address of Beneficial Owner   | Beneficial Ownership |            |
|--|----------------------|------------|
|  | Number of Shares     | % of Total |
| <b>Stockholders Owning Greater than 5%:</b>                                  |                      |            |
| FMR LLC <sup>(1)</sup>   | 5,825,205            | 14.9%      |
| Capital Research Global Investors <sup>(2)</sup>                             | 4,756,473            | 12.2%      |
| Wellington Management Group LLP and affiliates <sup>(3)</sup>                | 4,050,477            | 10.4%      |
| BlackRock, Inc. <sup>(4)</sup>   | 3,294,747            | 8.4%       |
| The Vanguard Group <sup>(5)</sup>  | 2,393,587            | 6.1%       |
| <b>Directors and Named Executive Officers:</b>                               |                      |            |
| William Aliski <sup>(6)</sup>  | 86,721               | *          |
| Lars Ekman, M.D., Ph.D. <sup>(7)</sup>                                       | 833                  | *          |
| Matthew K. Fust <sup>(8)</sup>   | 33,611               | *          |
| Michael Narachi <sup>(9)</sup>   | 14,792               | *          |
| Clay B. Siegall, Ph.D. <sup>(10)</sup>                                       | 28,611               | *          |
| Daniel G. Welch <sup>(11)</sup>  | 14,306               | *          |
| Emil D. Kakkis, M.D., Ph.D. <sup>(12)</sup>                                  | 3,298,679            | 8.5%       |
| Shalini Sharp <sup>(13)</sup>  | 169,313              | *          |
| Dennis Huang <sup>(14)</sup>   | 18,125               | *          |
| John R. Pinion II  |                      |            |
| Jayson Dallas, M.D.  |                      |            |
| All executive officers and directors as a group <sup>(15)</sup> (13 persons) | 3,937,870            | 10.1%      |

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

(1) Based on information set forth in a Schedule 13G/A filed with the SEC on February 12, 2016 by FMR LLC. The Schedule 13G/A reported that, as of December 31, 2015, FMR LLC has sole voting power with respect to 281,247 of shares and sole dispositive power with respect to 5,825,205 shares. The principal business address for FMR LLC is listed in such filing as 245 Summer Street, Boston, MA 02210.

(2) Based on information set forth in a Schedule 13G/A filed with the SEC on February 12, 2016 by Capital Research Global Investors. The Schedule 13G/A reported that, as of December 31, 2015, Capital Research Global Investors has sole voting and sole dispositive power with respect to all shares beneficially owned as a result of acting as investment advisor to various investment companies. The principal business address for Capital Research Global Investors is listed in such filing as 333 South Hope Street, Los Angeles, CA 90071.



- (3) Based on information set forth in a Schedule 13G/A filed with the SEC on March 10, 2016 by Wellington Management Group LLP, Wellington Group Holdings LLP, Wellington Investment Advisors Holdings LLP and Wellington Management Company LLP (the Wellington entities ). The Schedule 13G/A reported that, as of February 29, 2016, Wellington Management Group LLP, Wellington Group Holdings LLP, and Wellington Investment Advisors Holdings LLP have shared voting power with respect to 2,989,614 of these shares and shared dispositive power with respect to 4,050,477 of these shares; and Wellington Management Company LLP has shared voting power with respect to 2,840,421 of these shares and shared dispositive power with respect to 3,759,725 of these shares. The Wellington entities have no sole voting or sole dispositive power with respect to any of these shares. The principal business address for the Wellington entities is listed in such filing as c/o Wellington Management Company LLP, 280 Congress Street, Boston, MA 02210.

**Table of Contents**

- (4) Based on information set forth in a Schedule 13G filed with the SEC on January 28, 2016 by BlackRock, Inc. The Schedule 13G reported that, as of December 31, 2015, BlackRock, Inc. has sole voting power with respect to 3,219,531 shares and sole dispositive power with respect to 3,294,747 shares. The principal business address for BlackRock, Inc. is listed in such filing as 55 East 52nd Street, New York, NY 10055.
- (5) Based on information set forth in a Schedule 13G filed with the SEC on February 11, 2016 by The Vanguard Group. The Schedule 13G reported that, as of December 31, 2015, The Vanguard Group has sole voting power with respect to 82,307 shares, shared voting power with respect to 1,500 shares, sole dispositive power with respect to 2,311,380 shares, and shared dispositive power with respect to 82,207 shares. The principal business address for The Vanguard Group is listed in such filing as 100 Vanguard Blvd., Malvern, PA 19355.
- (6) Consists of (a) 58,110 shares of common stock and (b) 28,611 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (7) Consists of 833 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (8) Consists of (a) 11,319 shares of common stock and (b) 22,292 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (9) Consists of 14,792 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (10) Consists of 28,611 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (11) Consists of 14,306 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016.
- (12) Consists of (a) 2,552,241 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009, (b) 546,119 shares of common stock held by Dr. Kakkis, (c) 149,700 shares of common stock that may be acquired pursuant to the exercise of warrants held by Dr. Kakkis, (d) 47,769 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016 and (e) 2,850 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 11, 2016. Dr. Kakkis shares voting and dispositive power over the 2,552,241 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009; each of Dr. Kakkis and Dr. Soriano is a trustee of such trust. Dr. Kakkis has sole voting and dispositive power over the 546,119 shares of common stock held by him, the 149,700 shares of common stock that may be acquired pursuant to the exercise of warrants held by Dr. Kakkis, the 47,769 shares of common stock issuable pursuant to stock options held by Dr. Kakkis and the 2,850 shares of common stock issuable pursuant to the vesting of restricted stock units.

- (13) Consists of (a) 37,401 shares of common stock, (b) 130,812 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016 and (c) 1,100 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 11, 2016.
- (14) Consists of (a) 16,250 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 11, 2016 and (b) 1,875 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 11, 2016.
- (15) Consists of (a) 3,270,994 shares held by our directors and executive officers, (b) 149,700 shares of common stock that may be acquired pursuant to the exercise of warrants by Dr. Kakkis, (c) 508,168 shares of common stock issuable pursuant to stock options held by our directors and officers that are exercisable within 60 days of April 11, 2016 and (d) 9,008 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 11, 2016.

**Table of Contents**

**EXECUTIVE COMPENSATION**

**Compensation Discussion and Analysis**

The following compensation discussion and analysis describes the material elements of compensation earned in fiscal 2015 by each of the executive officers identified below in the Summary Compensation Table, who are referred to collectively as our named executive officers. Our named executive officers with respect to the fiscal year that ended on December 31, 2015 are:

Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer;

Shalini Sharp, Chief Financial Officer and Senior Vice President;

Dennis Huang, Chief Technical Operations Officer and Senior Vice President;

John R. Pinion II, Chief Quality Operations Officer and Senior Vice President Analytical Sciences and Research; and

Jayson Dallas, M.D., Chief Commercial Officer and Senior Vice President.

These persons constitute our principal executive officer, our principal financial officer and our three other most highly paid executive officers serving during fiscal 2015. This disclosure includes payments that were made and compensation-related actions that were taken in the first quarter of fiscal 2016, where these payments and decisions related to performance in fiscal 2015.

***Compensation Philosophy and Objectives***

Our philosophy in setting compensation policies for executive officers has two fundamental objectives: (1) to attract and retain a highly-skilled team of executives and (2) to align our executives' interests with those of our stockholders by rewarding short-term and long-term performance and tying compensation to increases in stockholder value. The Compensation Committee believes that executive compensation should be directly linked both to continuous improvements in corporate performance ( pay for performance ) and accomplishments that are expected to increase stockholder value. In furtherance of this goal, the Compensation Committee has adhered to the following guidelines as a foundation for decisions that affect the levels of compensation:

provide a competitive total compensation package that enables the Company to attract and retain highly qualified executives with the skills and experience required for the achievement of business goals;

align compensation elements with the Company's annual goals and long-term business strategies and objectives;

promote the achievement of key strategic and financial performance measures by linking short-term and long-term cash and equity incentives to the achievement of measurable corporate and individual performance goals and objectives; and

align executives' incentives with the creation of stockholder value.

The Compensation Committee has historically compensated executive officers with three compensation components: base salary, annual incentive bonus and equity-based compensation. The Compensation Committee believes that cash compensation in the form of base salary and an annual incentive bonus provides our executives with short-term rewards for success in operations, and that long-term compensation through the award of stock options, restricted stock and other equity awards aligns the objectives of management with those of our stockholders with respect to long-term performance and success.

**Table of Contents**

We target total cash compensation and total equity incentive compensation for our named executive officers at 50% of the market median, in each case compared to compensation paid to similarly situated executives of the companies in our peer group. However, because we have a pay-for-performance philosophy, actual compensation levels are correlated to the achievement of corporate and individual goals and objectives.

In addition to our direct compensation elements, the following compensation program features are designed to align our executive team with stockholder interests and market best practices:

we do not offer any tax gross-up payments to our executive team for any change-of-control payments;

we prohibit our employees, including our executive team, from engaging in hedging transactions that transfer, with respect to equity compensation received by an employee, all or a portion of the risk of a decline in the market price of shares of our stock as well as prohibiting our employees, including our executive team, from pledging our securities; and

we do not offer our executive team any substantially enhanced benefits or perquisites when compared to our overall employee population.

As described below, during 2015, we made significant progress on our clinical development and business goals, including:

initiating our Phase 3 study in adult XLH patients and initiating our Phase 3 study in GNE Myopathy patients;

completing enrollment in our Phase 3 study in MPS 7 patients;

filing a conditional marketing authorization application in Europe for Ace-ER;

completing two equity financings for approximately \$461 million in net proceeds to us;

hiring over 140 new employees and adding two new experienced Board members; and

implementing an enterprise resource planning system.

In addition, as a result of positive clinical results and strong business progress, our stock price appreciated over 142% during 2015. Based on our performance during 2015 relative to pre-established corporate goals, our Compensation Committee determined that we achieved 105% of our corporate goals for 2015 under our annual bonus plan, as discussed below.

***Roles in Determining Compensation***

*Compensation Committee*

The Board has delegated to the Compensation Committee the responsibility to ensure that total compensation paid to our executive officers, including named executive officers, is consistent with our compensation policy and objectives. The Compensation Committee oversees and approves all compensation arrangements and actions for our executive officers, including the named executive officers. While the Compensation Committee draws on a number of resources, including input from the Board, the Chief Executive Officer and independent compensation consultants, to make decisions regarding the Company's executive compensation program, ultimate decision-making authority rests with the Compensation Committee. The Compensation Committee retains discretion over base salary, annual incentive bonus, equity compensation and

## **Table of Contents**

other compensation considerations. The Compensation Committee relies upon the judgment of its members in making compensation decisions, after reviewing the performance of the Company and carefully evaluating an executive's performance during the year against established goals, operational performance and business responsibilities. In addition, the Compensation Committee incorporates judgment in the assessment process to respond to and adjust for the evolving business environment.

### *Compensation Consultant*

The Compensation Committee retains the services of an external compensation consultant, Radford, a division of AON ( **Radford** ). The mandate of the consultant is to assist the Compensation Committee in its review of executive and director compensation practices, including the competitiveness of pay levels, executive compensation design, benchmarking with the Company's peers in the industry and other technical considerations, including tax- and accounting-related matters. The Compensation Committee regularly evaluates Radford's performance, considers alternative compensation consultants and has the final authority to engage and terminate Radford's services. Our Compensation Committee has assessed the independence of Radford consistent with NASDAQ listing standards and has concluded that the engagement of Radford does not raise any conflict of interest.

### *Chief Executive Officer*

The Chief Executive Officer attends Compensation Committee meetings and works with the Compensation Committee Chairman and Radford to develop compensation recommendations for the executive officers (excluding the Chief Executive Officer), based upon individual experience and breadth of knowledge, internal considerations, individual performance during the fiscal year and other factors deemed relevant by the Compensation Committee. The recommendations are then submitted to the Compensation Committee for review and consideration. The Compensation Committee works directly with Radford and the non-employee directors of the Board to determine compensation actions for the Chief Executive Officer.

### ***Defining and Comparing Compensation to Market Benchmarks***

In evaluating the total compensation of our named executive officers, our Compensation Committee, using information provided by Radford, establishes a peer group of publicly traded, national and regional companies in the biopharmaceutical and biotechnology industries that is selected based on a balance of the following criteria:

primary factors of our industry (publicly-traded, pre-commercial therapeutics/biotechnology companies), stage of development (companies predominantly in Phase 2 and Phase 3 clinical studies) and market capitalization (companies with market capitalization generally between \$500 million and \$5 billion, which was a range of 0.3x to 3.0x of our market capitalization at the time the peer group was approved); and

a secondary factor of organizational complexity (companies with generally fewer than 200 employees).

While we do not establish compensation levels based solely on benchmarking, pay practices at other companies are an important factor that the Compensation Committee considers in assessing the reasonableness of compensation and ensuring that our compensation practices are competitive in the marketplace. Our peer group for 2015, referred to as our 2015 peer group, used in determining compensation actions in the 2015 fiscal year, were selected by the



Compensation Committee with Radford's support in May 2014 on the basis of the criteria

**Table of Contents**

set forth above. The 2015 peer group consisted of the following 25 publicly-traded companies in the pharmaceutical and biotechnology industries:

|                               |                                  |                               |
|-------------------------------|----------------------------------|-------------------------------|
| ACADIA Pharmaceuticals Inc.   | Insmed Incorporated              | Portola Pharmaceuticals, Inc. |
| Acceleron Pharma Inc.         | Intercept Pharmaceuticals, Inc.  | PTC Therapeutics, Inc.        |
| Alnylam Pharmaceuticals, Inc. | Keryx Biopharmaceuticals, Inc.   | Puma Biotechnology, Inc.      |
| Anacor Pharmaceuticals, Inc.  | KYTHERA Biopharmaceuticals, Inc. | Relypsa, Inc.                 |
| Auspex Pharmaceuticals, Inc.  | MacroGenics, Inc.                | Sangamo BioSciences, Inc.     |
| bluebird bio, Inc.            | Merrimack Pharmaceuticals, Inc.  | Sarepta Therapeutics, Inc.    |
| Celldex Therapeutics, Inc.    | Neurocrine Biosciences, Inc.     | Synageva BioPharma Corp.      |
| Clovis Oncology, Inc.         | OncoMed Pharmaceuticals, Inc.    | TESARO, Inc.                  |
| Enanta Pharmaceuticals, Inc.  |                                  |                               |

We believe that the compensation practices of our 2015 peer group provided us with appropriate compensation benchmarks for evaluating the compensation of our named executive officers during 2015. Notwithstanding the similarities of the 2015 peer group to Ultragenyx, due to the nature of our business, we compete for executive talent with many public companies that are larger and more established than we are or that possess greater resources than we do, or with smaller private companies that may be able to offer greater equity compensation potential. Accordingly, in 2015, our Compensation Committee generally targeted compensation for our executive officers as follows:

base salaries at approximately the 50<sup>th</sup> percentile of the salaries in our 2015 peer group;

annual cash incentive award opportunities at approximately the 50<sup>th</sup> percentile of our 2015 peer group;

total annual equity incentive awards, provided in the form of a mix of stock options and restricted stock units, at approximately the 50<sup>th</sup> percentile of our 2015 peer group; and

total compensation for our named executive officers is targeted at the 50<sup>th</sup> percentile of compensation paid to similarly situated executive officers of the companies in our 2015 peer group.

Our Compensation Committee may consider other criteria, including market factors, the experience level of the executive and the executive's performance against established corporate goals, as well as the need to attract and recruit executives from larger biotechnology companies, in determining variations to this general target range.

The Compensation Committee reviews the Company's list of peer companies periodically to reflect changes in market capitalization, developments at the Company relative to its peer companies and other factors. During fiscal 2015, the Compensation Committee noted that the above list of peer companies, selected in fiscal 2014, was in need of updating

in light of the large increase in the Company's market capitalization, stage of development and organizational complexity. Accordingly, the Compensation Committee engaged Radford to assist in determining a new list of peer companies to include late-stage development as well as newly commercial peers, peers with comparable market capitalizations (i.e., in the range of 0.3x to 2.5x the Company's approximately \$4 billion market capitalization in September 2015) and commercial peers with headcounts above 200 where necessary.

**Table of Contents**

Based on Radford's assessment and recommendations and the Compensation Committee's criteria in determining the Company's new list of peer companies in September 2015, referred to as our revised 2015 peer group, the Compensation Committee selected 23 publicly-traded companies in the pharmaceutical and biotechnology industries to serve as the Company's new list of peer companies for the remainder of 2015 and beyond. The revised 2015 peer group is comprised of the following 23 companies in the pharmaceutical and biotechnology industries:

|                               |                                 |                               |
|-------------------------------|---------------------------------|-------------------------------|
| ACADIA Pharmaceuticals Inc.   | Dyax Corp.                      | Novavax, Inc.                 |
| Agios Pharmaceuticals, Inc.   | Halozyme Therapeutics, Inc.     | OPKO Health, Inc.             |
| Alnylam Pharmaceuticals, Inc. | Insmed Incorporated             | Portola Pharmaceuticals, Inc. |
| Anacor Pharmaceuticals, Inc.  | Intercept Pharmaceuticals, Inc. | PTC Therapeutics, Inc.        |
| bluebird bio, Inc.            | Juno Therapeutics, Inc.         | Puma Biotechnology, Inc.      |
| Celldex Therapeutics, Inc.    | Kite Pharma, Inc.               | Sarepta Therapeutics, Inc.    |
| Chimerix, Inc.                | Merrimack Pharmaceuticals, Inc. | TESARO, Inc.                  |
| Clovis Oncology, Inc.         | Neurocrine Biosciences, Inc.    |                               |

In addition to the selection of the revised 2015 peer group, the Compensation Committee engaged Radford to conduct a comprehensive benchmarking study reporting on compensation levels and practices, including equity, relative to the Company's revised 2015 peer group. An Executive Compensation Assessment report was prepared by Radford in January 2016 that provided a competitive assessment of the Company's executive compensation program as compared to the market data for base salaries, target total cash compensation and equity compensation of the revised 2015 peer group. Based on the benchmarking data for the revised 2015 peer group, adjustments to compensation were made in the first quarter of fiscal 2016, as described below.

***Annual Performance Reviews***

Our Compensation Committee conducts an annual performance review of our named executive officers and approves the compensation of each member of our senior management team. By the first quarter of each year, annual corporate goals and individual performance objectives are determined and set forth in writing. After the end of each year, our Compensation Committee determines executive compensation levels after carefully reviewing overall corporate performance and performing an evaluation of each named executive officer's annual performance against established corporate goals, as well as each individual executive officer's contributions to achievement of the corporate goals and, in the case of executive officers other than our Chief Executive Officer, the achievement of individual performance objectives. In addition, our Compensation Committee may apply its discretion, as it deems appropriate, in determining executive compensation.

Annual corporate goals for fiscal 2015 were proposed by our senior management team and approved by our Board. For 2015, our Chief Executive Officer's bonus was based entirely on performance relative to corporate goals and the bonus of our other named executive officers was based 75% on corporate goals and 25% on individual objectives. Individual objectives for 2015 for our senior management team focused on individual contributions that were intended to drive achievement of the corporate goals as well as building and managing their functions, and were proposed by each member of senior management, with review and input from our Chief Executive Officer. Any merit increases in base salary and any cash awards made under our 2015 annual incentive program were based on the achievement of these corporate and individual performance goals and objectives. Our Compensation Committee

established the cash bonus opportunity for each member of our senior management team, where the cash bonus opportunity represents a percentage of each individual's base salary.

During the last quarter of fiscal 2015, our senior management team evaluated our corporate performance and, in the first quarter of fiscal 2016, our Chief Executive Officer evaluated each executive officer's individual

## **Table of Contents**

performance, as compared to the corporate goals and, as applicable, the individual objectives for fiscal 2015. Based on this evaluation, our Chief Executive Officer recommended to our Compensation Committee increases in base salary and cash awards under our annual incentive program. Our Compensation Committee, with input from the Board, evaluates our Chief Executive Officer's individual performance and determines whether to change his base salary and/or change his cash award under our annual incentive program. Our Compensation Committee typically determines changes in base salary and the amount of any cash incentive payments for our Chief Executive Officer at its first regularly scheduled meeting of the new year. Our Compensation Committee may also review the compensation of our executive officers throughout the course of the year. With respect to year-end reviews, any changes in base salary are effective at the beginning of the following year. The cash incentive payments awarded under our 2015 annual incentive program were paid in March 2016.

### ***Base Salary***

#### *Overview*

Our Compensation Committee aims to set our named executive officers' base salaries near the 50<sup>th</sup> percentile of salaries of similarly situated executive officers in the Company's peer group. The Compensation Committee believes it is important to provide adequate fixed compensation to our executive officers working in a highly volatile and competitive industry. Our Compensation Committee believes that the 50<sup>th</sup> percentile for base salaries is the minimum cash compensation level that will allow us to attract and retain highly-skilled executives. The Compensation Committee's choice of this target percentile reflects consideration of our stockholders' interests in paying what is necessary to achieve our corporate goals, while conserving cash and equity as much as practicable. We believe that, given the industry in which we operate and our compensation philosophy and objectives, base salaries at the 50<sup>th</sup> percentile are generally sufficient to retain our current executives and to hire new executives when and as required, although sometimes we must pay executive officers base salaries at a level higher than the 50<sup>th</sup> percentile to attract and recruit executives from larger commercial-stage biotechnology companies as we build out our organization. For example, in 2015, we recruited three of our named executive officers, Mr. Huang, Mr. Pinion and Dr. Dallas, from larger commercial-stage biotechnology companies and offered a total compensation package in excess of our 50<sup>th</sup> percentile target to successfully recruit them to Ultragenyx. In determining appropriate base salary levels for a given executive officer, the Compensation Committee considers the following factors:

individual performance of the executive, as well as our overall performance, during the prior year;

level of responsibility, including breadth, scope and complexity of the position;

level of experience and expertise of the executive;

internal review of the executive's compensation relative to other executives to ensure internal equity; and

executive officer compensation levels at other similar companies to ensure competitiveness.

#### *Changes in Base Salaries for Fiscal 2016*

The Executive Compensation Assessment report prepared by Radford in January 2016 provided a competitive assessment of the Company's compensation practices as compared to that of the Company's revised 2015 peer group suggested that the 2015 base salary level for our Chief Executive Officer was below the 50<sup>th</sup> percentile as compared to the Company's revised 2015 peer group. In light of the Company's accomplishments since the completion of its initial public offering in January 2014, the Compensation Committee approved an increase to the Chief Executive Officer's base salary for fiscal 2016, with the goal of bringing his base salary to competitive base salary levels at or near the 50<sup>th</sup> percentile. Because all of our other named executive officers

**Table of Contents**

have base salaries at or above the 50<sup>th</sup> percentile of the Company's revised 2015 peer group, the Compensation Committee did not increase the base salaries for these named executive officers beyond merit increases for fiscal 2015 performance. The Compensation Committee deemed the base salary increase for our Chief Executive Officer to be consistent with the Company's stated compensation philosophy and objectives considering the experience level of our Chief Executive Officer and necessary in order to retain him to achieve the Company's goals and business objectives.

The following table shows the base salaries for our named executive officers for fiscal 2016, after giving effect to salary adjustments made in January 2016, which were effective as of January 1, 2016, as well as the average salaries in our 2016 peer group (as disclosed above) at the 25<sup>th</sup>, 50<sup>th</sup> and 75<sup>th</sup> percentiles.

| Name                        | Title   | Fiscal 2016<br>Base<br>Salary <sup>(1)</sup> | Market Data <sup>(2)</sup>                    |   |   |
|-----------------------------|---|--|---|---|---|
|                             |   |  | Base Salary<br>25 <sup>th</sup><br>Percentile | Base Salary<br>50 <sup>th</sup><br>Percentile | Base Salary<br>75 <sup>th</sup><br>Percentile |
| Emil D. Kakkis, M.D., Ph.D. | President and Chief Executive Officer                                     | \$ 567,200                                   | \$ 513,600                                    | \$ 567,200                                    | \$ 605,400                                    |
| Shalini Sharp               | Chief Financial Officer and Senior Vice President                         | \$ 441,000                                   | \$ 340,100                                    | \$ 388,500                                    | \$ 415,100                                    |
| Dennis Huang                | Chief Technical Operations Officer and Senior Vice President              | \$ 349,000                                   | \$ 301,200                                    | \$ 306,100                                    | \$ 346,300                                    |
| John R. Pinion II           | Chief Quality Operations Officer and SVP Analytical Sciences and Research | \$ 399,185                                   | \$ 247,000                                    | \$ 268,000                                    | \$ 293,000                                    |
| Jayson Dallas, M.D.         | Chief Commercial Officer and Senior Vice President                        | \$ 433,000                                   | \$ 347,600                                    | \$ 369,300                                    | \$ 382,700                                    |

(1) Effective as of January 1, 2016.

(2) Source: Radford's Executive Compensation Assessment report of January 2016.

Because Dr. Kakkis's 2015 base salary was below the 50<sup>th</sup> percentile of the revised 2015 peer group, and based on the subjective factors listed above, the Compensation Committee approved an approximate 6.0% increase in Dr. Kakkis's base salary for fiscal 2016, which brought his base salary up to the 50<sup>th</sup> percentile level. Other increases were approximately 3-4% (with such percentages prorated for Mr. Huang, Mr. Pinion and Dr. Dallas as they joined the Company during 2015), as a result of standard annual merit increases, as shown in the following table.

***Fiscal 2015 and 2016 base salary levels for each named executive officer***

| Name | Title |
|------|-------|
|------|-------|



|                             |   | <b>Fiscal<br/>2015 Base<br/>Salary</b> | <b>Fiscal 2016<br/>Base<br/>Salary<sup>(1)</sup></b> | <b>Percentage<br/>(%)<br/>Increase</b> |
|-----------------------------|---|--|--|--|
| Emil D. Kakkis, M.D., Ph.D. | President and Chief Executive Officer                                     | \$ 535,000                             | \$ 567,200   | 6.0%                                   |
| Shalini Sharp               | Chief Financial Officer and Senior Vice President                         | \$ 420,000                             | \$ 441,000   | 5.0%                                   |
| Dennis Huang                | Chief Technical Operations Officer and Senior Vice President              | \$ 340,000                             | \$ 349,000   | 2.6%                                   |
| John R. Pinion II           | Chief Quality Operations Officer and SVP Analytical Sciences and Research | \$ 390,000                             | \$ 399,185   | 2.4%                                   |
| Jayson Dallas, M.D.         | Chief Commercial Officer and Senior Vice President                        | \$ 425,000                             | \$ 433,000   | 1.9%                                   |

(1) Effective as of January 1, 2016, inclusive of merit-based increases and market-based adjustments (if applicable).

## **Table of Contents**

### ***Annual Bonus Incentive***

#### *Overview*

Our Board has adopted a corporate bonus plan, or the bonus plan, that provides for cash bonus payments based upon the attainment of performance targets established by our Compensation Committee. The payment targets relate to corporate, financial, and operational measures or objectives, or corporate performance goals, as well as individual performance objectives for all named executives other than the CEO.

Our Compensation Committee may select corporate performance goals from among the following: sales; revenue; assets; expenses; earnings from operations, earnings before or after deduction for all or any portion of interest, taxes, depreciation, amortization, incentives, service fees or extraordinary or special items, whether or not on a continuing operations or an aggregate or per share basis; net income or net income per common share (basic or diluted); return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow, free cash flow, cash flow return on investment, or net cash provided by operations; stock price, dividends or total stockholder return; development of new technologies or products; sales of particular products or services; economic value created or added; operating margin or profit margin; customer acquisition or retention; raising or refinancing of capital; successful hiring of key individuals; resolution of significant litigation; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings; or strategic business criteria, consisting of one or more objectives based on the following goals: meeting specified market penetration or value added, product development or introduction (including, without limitation, any clinical trial accomplishments, regulatory or other filings or approvals, or other product development milestones), geographic business expansion, cost targets, cost reductions or savings, customer satisfaction, operating efficiency, acquisition or retention, employee satisfaction, information technology, corporate development (including, without limitation, licenses, innovation, research or establishment of third party collaborations), manufacturing or process development, legal compliance or risk reduction, patent application or issuance goals, or goals relating to acquisitions or divestitures (in whole or in part), joint ventures or strategic alliances, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, as compared to results of a peer group, against the market as a whole, compared to applicable market indices and/or measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the bonus plan has a target bonus opportunity set for each performance period. The bonus formulas are adopted in each performance period by the Compensation Committee and communicated to each executive. The corporate performance goals are measured at the end of each performance period after our financial reports have been published or such other appropriate time as the Compensation Committee determines. If the corporate performance goals and individual performance objectives are met, payments are made following the end of each performance period. Subject to the rights contained in any agreement between the executive officer and the Company, an executive officer must be employed by the Company on the bonus payment date to be eligible to receive a bonus payment. The bonus plan also permits the Compensation Committee to approve additional bonuses in its sole discretion, though such discretion was not exercised with respect to payments for fiscal 2015.

For fiscal 2015, corporate goals were established at the end of fiscal 2014 with input from senior management and with final approval by the independent members of the Board of Directors. The target annual incentive bonus amounts vary depending on each executive's accountability, scope of responsibilities and potential impact on the Company's performance. Accordingly, the higher the level of control and accountability that is exercisable by an executive officer over our overall performance, the greater the percentage of the executive officer's target total cash compensation that is dependent on annual performance-based cash bonus award.



**Table of Contents**

Our Compensation Committee sets annual incentive bonus amounts for executive officers as a percent of base salary generally targeting the 50<sup>th</sup> percentile in our 2015 peer group. Although the Compensation Committee has selected a target bonus in some cases above or below the 50<sup>th</sup> percentile in percentage terms, the actual total cash compensation is generally expected to be at or near the 50<sup>th</sup> percentile measured in dollar terms with respect to our named executive officers. The target bonuses, as a percentage of base salary, for the named executive officers for fiscal 2015 are set forth in the following table.

| Name                        | Title   | Target Bonus for Fiscal 2015 (% of Base Salary) | Target Annual Incentive Bonus Market Data <sup>(1)</sup> |                             |                             |
|-----------------------------|---|---|--|-----------------------------|-----------------------------|
|                             |   |   | 25 <sup>th</sup> Percentile                              | 50 <sup>th</sup> Percentile | 75 <sup>th</sup> Percentile |
| Emil D. Kakkis, M.D., Ph.D. | President and Chief Executive Officer                                     | 50%   | 50%  | 55%                         | 60%                         |
| Shalini Sharp               | Chief Financial Officer and Senior Vice President                         | 40%   | 35%  | 40%                         | 45%                         |
| Dennis Huang                | Chief Technical Operations Officer and Senior Vice President              | 40%   | 25%  | 30%                         | 35%                         |
| John R. Pinion II           | Chief Quality Operations Officer and SVP Analytical Sciences and Research | 40%   | 30%  | 30%                         | 30%                         |
| Jayson Dallas, M.D.         | Chief Commercial Officer and Senior Vice President                        | 40%   | 40%  | 40%                         | 45%                         |

For fiscal 2016, the bonus target for our Chief Executive Officer was increased from 50% to 60% of base salary to bring his bonus target up from the 25<sup>th</sup> percentile of the revised 2015 peer group. Because the bonus targets for all other executive officers are already at or above the 50<sup>th</sup> percentile of the revised 2015 peer group, their bonus targets remained the same at 40% of base salary.

The Compensation Committee considers the individual performance of each executive officer (other than our Chief Executive Officer, whose bonus is based 100% on corporate performance) and the Company's overall performance for the preceding fiscal year in deciding whether to award a bonus and, if one is to be awarded, the amount of the bonus. For fiscal 2015, the maximum bonus for each executive was 150% of his or her respective target and the minimum bonus, or threshold, for each executive was zero. All executive officers, except for the Chief Executive Officer, are assigned annual incentive bonus targets with 75% of the bonus attributed to corporate performance and 25% based on individual performance. The annual incentive bonus for the Chief Executive Officer is based 100% on overall corporate performance. In addition, the Compensation Committee has, in its sole discretion, the ability to modify the final calculation of an award, not to exceed 150% of target for any bonus award, based on additional considerations of performance and to account for the evolving business environment during the performance year, though this discretion

was not utilized with respect to bonus payments for fiscal 2015.

By the end of the first quarter of each fiscal year, individual and corporate performance are measured versus plan and a percentage of target is fixed, which then determines the size of the total bonus pool from which annual bonus incentives are to be paid to executive officers. All cash bonuses are awarded retrospectively. Payout dates for all annual incentive bonuses to executive officers are targeted during the first quarter of each fiscal year.

**Table of Contents***Fiscal 2015 Annual Bonus Incentive*

In December 2015, the Compensation Committee assessed the Company's overall performance against the achievement of corporate performance goals established in December 2014. Performance against each goal is assessed by the Compensation Committee and the Compensation Committee determines a total percentage of achievement of the corporate goals between 50% and 150%. Once this percentage of achievement is determined, the funding of the bonus pool is determined from the scale shown below, with the Compensation Committee retaining the discretion as set forth above to adjust individual awards, within certain limits.

| <b>Goal Achievement</b> | <b>Percentage Payout</b> |
|-------------------------|--------------------------|
| 150%                    | 150%                     |
| 140%                    | 140%                     |
| 130%                    | 130%                     |
| 120%                    | 120%                     |
| 110%                    | 110%                     |
| 100%                    | 100%                     |
| 90%                     | 90%                      |
| 80%                     | 80%                      |
| 70%                     | 70%                      |
| 60%                     | 60%                      |
| 50%                     | 0%                       |

In fixing the targets for each fiscal year, the Compensation Committee selects performance goals that are considered aggressive, meaning that they are goals that are considered achievable, but only with a high degree of diligence and success in execution. In accruing for bonuses during each fiscal year, management assumes that the target goals will be achieved (i.e., a percentage achievement of 100%), absent a set of circumstances arising during the course of the year that would suggest otherwise.

Set forth below are the general performance goals that were considered by the Compensation Committee in assessing overall performance for the 2015 fiscal year, as well as the relative weighting of these goals and the Compensation Committee's assessment of achievement for each goal.

***Preclinical and Clinical Development Goals (79%):*** These goals established target performance for the Company in its preclinical pipeline as well as in each of its clinical programs. The specific goals were as follows:

*Preclinical (4%):* This goal measured demonstrating of proof of concept in one of the Company's preclinical programs and identification of lead prodrug candidate(s) on another preclinical program.

*UX001 (20%):* This goal measured achievement of enrollment of the first patient in our Phase 3 study, the availability of drug product in advance of enrollment of the first patient in our Phase 3 study, enrollment of the first patient in our non-ambulatory study, filing of a conditional marketing authorization application with the European Medicines Agency (EMA) and obtaining first reimbursement approval under our named patient sales program.

*UX003 (20%):* This goal measured completion of enrollment of our Phase 3 study, approval of early access program reimbursement for the first patient, finalization of the commercial manufacturing process and completion of a successful commercial scale run and generation of supportive data on treatment of hydrops for a planned biologics license application.

*UX007 (5%):* This goal measured availability of interim topline data from our Phase 2 study, reaching agreement with the U.S. Food and Drug Administration ( *FDA* ) on the Phase 3 plan and completion of a Phase 1 pharmacokinetic study.

**Table of Contents**

*UX007G (10%):* This goal measured enrollment of the first patient in our adult movement disorder study and completion of Phase 2 interim data analysis.

*UX023 (20%):* This goal measured, with respect to the x-linked hypophosphatemia indication, agreement with the FDA and EMA on the design of the adult Phase 3 study, interim data from the pediatric Phase 2 study demonstrating safety and phosphate homeostasis to enable dose, availability of data from the pediatric Phase 2 study of at least 30 patients at week 24 and at least 10 patients at week 40, and reaching agreement with EMA on design of pediatric Phase 3 study. With respect to the tumor-induced osteomalacia indication, this goal measured completion of enrollment for our Phase 2 study, generation of data to support proof of concept and submission of breakthrough therapy designation.

*Corporate Development Goals (21%):* These goals set targets for enhancing corporate infrastructure and talent, increasing the Company's preclinical pipeline, demonstrating fiscal responsibility and building stockholder value.

*Finance/Legal (7%):* This goal measured the raising of sufficient capital to fund operations through 2017, completion of Sarbanes-Oxley implementation and building stockholder value in excess of the NASDAQ Biotechnology Index.

*Budget (7%):* This goal measured the Company's aggregate cash expenditures on operations for fiscal 2015 and the management of such expenditures within 10% of budget for fiscal 2015.

*Corporate (7%):* This goal measured the Company's efforts to continue to grow as an organization in terms of infrastructure (by entering into a lease agreement for the building at 52 Leveroni Court in Novato, California and completing the buildout of such space) and employee talent (by hiring key talent in a timely manner to execute corporate deliverables and maintain the voluntary attrition rate of high performers to less than 5%). This goal also measured the Company's successful implementation of enterprise resource planning finance and supply chain modules.

In assessing the Preclinical and Clinical development and Corporate Development goals, the Compensation Committee reviewed each goal and determined whether or not it was achieved and, if a goal was achieved, if there was overachievement for such goal. The Compensation Committee also evaluated the relative importance of the weightings of each goal. After such review and evaluation was complete, the Compensation Committee determined that achievement of corporate goals was approximately 100% and that there were additional corporate objectives achieved that, although not formal goals, were achieved above expectations. Accordingly, the Compensation Committee determined that overall achievement against corporate goals for fiscal 2015 was 105%.

The individual performance objectives for Emil Kakkis, the Company's Chief Executive Officer, were the same as the overall corporate performance goals for the Company, as the primary responsibility of the Chief Executive Officer is to help ensure the overall success of the Company by executing the Company's business strategies. All other executive officers are assigned annual incentive bonus targets with 75% of the bonus attributed to corporate performance and 25% based on individual performance. In January 2016, the Compensation Committee assessed our Chief Executive Officer's individual accomplishments as well as the individual accomplishments of other executive officers. The individual objectives for Ms. Sharp, Mr. Huang, Mr. Pinion and Dr. Dallas are discussed below. In addition, as described above, the Compensation Committee has, in its sole discretion, the ability to modify the final calculation of



an award, not to exceed 150% of target for any bonus award, based on additional considerations of performance and to account for the evolving business environment during the performance year.

The individual performance achievements for Shalini Sharp, the Company's Chief Financial Officer and Senior Vice President, for fiscal 2015 included: creating an organizational structure in the Finance department to

**Table of Contents**

support significant growth; implementing Phase 1 of our enterprise resource planning system on time and on budget; assisting in raising approximately \$461 million in net proceeds in two equity offerings; leading initiatives on international tax structure; and leading a global operations task force resulting in the foundation for global launch planning.

The individual performance achievements for Dennis Huang, the Company's Chief Technical Operations Officer and Senior Vice President, for fiscal 2015 included: growing the Technical Operations department without experiencing any turnover; initiating the process for implementation of Phase 2 of the Company's enterprise resource planning system; developing decision-making framework charters for Chemistry, Manufacturing and Controls teams and review forums; assisting with a timely filing of the UX001 marketing authorization application; and strengthening relationships with key contract manufacturing organizations.

The individual performance achievements for John Pinion, the Company's Chief Quality Operations Officer and Senior Vice President of Analytical Sciences and Research, for fiscal 2015 included: growing the Quality organization; developing an overall quality plan in terms of organizational design, resource needs, organizational purpose and operating norms; and preparing for the Company's first health authority inspection by initiating a risk-based inspection readiness effort with a cross-functional team.

The individual performance achievements for Jayson Dallas, the Company's Chief Commercial Officer and Senior Vice President, for fiscal 2015 included: completing the design of the Commercial function, with a focus on building a team tailored to rare disease commercialization; growing the Commercial function by making hires in key roles, including initiating establishment of the Commercial function in Europe; achieving alignment and approval for pricing for named patient sales of UX001; and strengthening the relationship with Kyowa Hakko Kirin regarding the Company's UX023 program.

Dr. Kakkis evaluated the performance of Ms. Sharp, Mr. Huang, Mr. Pinion and Dr. Dallas after considering the above achievements and provided a proposed annual bonus incentive amount for each such officer to the Compensation Committee in light of such officer's achievements during 2015.

*Achievement of Goals and Relationship to Compensation Awarded*

For fiscal 2015, the Compensation Committee determined that the Company's performance against the corporate performance goals established in December 2014 merited a percentage achievement of 105%, resulting in a bonus pool equal to 105% of the target amount. This percentage achievement, combined with Dr. Kakkis's proposed annual bonus incentive amount for each of Ms. Sharp, Mr. Huang, Mr. Pinion and Dr. Dallas in light of their achievements during fiscal 2015 resulted in the Compensation Committee approving bonus awards for performance in 2015 as set forth in the following table:

| <b>Name</b>                 | <b>Title</b>   | <b>Fiscal 2015</b> |
|-----------------------------|--|--------------------|
| Emil D. Kakkis, M.D., Ph.D. | President and Chief Executive Officer  | \$ 280,875         |
| Shalini Sharp               | Chief Financial Officer and Senior Vice President  | \$ 182,700         |
| Dennis Huang                | Chief Technical Operations Officer and Senior Vice President                                   | \$ 95,805          |
| John R. Pinion II           | Chief Quality Operations Officer and Senior Vice President<br>Analytical Sciences and Research | \$ 79,945          |
| Jayson Dallas, M.D.         | Chief Commercial Officer and Senior Vice President   | \$ 74,724          |

*Equity Compensation*

*Overview*

*Stock Options and Restricted Stock Units.* As an additional component of our compensation program, executive officers are eligible to receive equity compensation in the form of stock options and/or restricted stock

## **Table of Contents**

units. The Compensation Committee grants stock options and restricted stock units to executive officers to aid in their retention, to motivate them to assist with the achievement of corporate objectives and to align their interests with those of our stockholders by creating a return tied to the performance of our stock price. In determining the form, date of issuance and value of a grant, the Compensation Committee considers the contributions and responsibilities of each executive officer, appropriate incentives for the achievement of our long-term growth, the size and value of grants made to other executives at peer companies holding comparable positions, individual achievement of designated performance goals, and the Company's overall performance relative to corporate objectives.

Under the terms of our 2014 Incentive Plan, pursuant to which all new equity grants are currently made, the exercise price of any stock options awarded under the Plan must be equal to at least 100% of the fair market value of our common stock (the closing sales price on The NASDAQ Global Select Market) on the date of grant. We do not have any program, plan or obligation that requires us to grant equity awards on specified dates, although historically we have made annual grants to existing officers and employees in May of each year, to new hires on a fixed schedule within one month of the commencement of their employment, and periodically in connection with broader compensation surveys. We also do not have any program, plan or practice to time stock option grants to our executive officers in coordination with the release of material nonpublic information. Equity awards may occasionally be granted following a significant change in job responsibilities or to meet other special retention or performance objectives.

Authority to make equity grants to employees rests with the Compensation Committee. With respect to executive officers, recommendations for equity grants are made by our external compensation consultant, Radford, and then reviewed by the Chief Executive Officer before being sent to the Compensation Committee for review and consideration. Our Chief Executive Officer and the Compensation Committee Chairman have been delegated the authority to review and approve awards to employees at the level of Executive Director and below, within limits set by the Compensation Committee.

We believe that periodic equity awards serve as useful performance recognition mechanisms with respect to key employees, as all awards are subject to time-based vesting provisions. Our typical option awards to executive officers (including the named executive officers) have a term of 10 years and vest and become exercisable over a period of four years, with 25% of the underlying shares vesting on the first anniversary of the grant date and the remainder monthly over the next three years. Our typical restricted stock unit awards to executive officers (including the named executive officers) vest and become exercisable over a period of four years, with 25% of the underlying shares vesting on each anniversary of the grant date. We believe that such periodic equity awards encourage executive officers to remain with the Company and also focus on our long-term performance as well as the achievement of specific performance goals.

### ***Equity Grants Awarded in Fiscal 2015***

In May 2015, the Compensation Committee approved its first annual equity awards for the Company's executive officers and employees. These annual equity awards, awarded as a mix of stock options and restricted stock units at a 75%/25% ratio, are planned to be awarded to existing officers and employees in May of each year. For the 2015 fiscal year, the Compensation Committee approved granting annual equity awards to the Company's executive officers targeting the 50<sup>th</sup> percentile of our 2015 peer group. The Company's equity guidelines are based on recommendations by Radford using market data regarding long-term incentives and equity compensation from the Company's 2015 peer group. All annual option awards granted in fiscal 2015 to executive officers, including our Chief Executive Officer, have a term of 10 years and vest and become exercisable over a period of four years, with 25% of the underlying shares vesting on the first anniversary of the grant date and the remainder monthly over the next three years. All annual restricted stock unit awards to executive officers, including our Chief Executive Officer, vest and become

exercisable over a period of four years, with 25% of the underlying shares vesting on each anniversary of the grant date. Three of our executive officers hired in fiscal 2015 (Mr. Huang, Mr. Pinion and Dr. Dallas) were either not eligible for annual equity awards or were hired after such annual equity awards were granted. The table below sets forth all equity awards granted in fiscal 2015; for Dr. Kakkis and Ms. Sharp these awards represent annual equity awards and for

**Table of Contents**

Mr. Huang, Mr. Pinion and Dr. Dallas, these represent new hire equity awards. With respect to annual equity awards, Radford recommended a target award level (based on 100% options and then the application of a 75%/25% split between options and restricted stock units) at the 50<sup>th</sup> percentile using a stock price of \$61.08, which represents a 30-day average between March 23, 2015 and April 22, 2015.

| <b>Name</b>                 | <b>Date of Grant</b> | <b>Number of Options</b> | <b>Number of Restricted Stock Units</b> |
|-----------------------------|----------------------|--------------------------|---|
| Emil D. Kakkis, M.D., Ph.D. | May 21, 2015         | 68,300                   | 11,400                                  |
| Shalini Sharp               | May 21, 2015         | 26,300                   | 4,400                                   |
| Dennis Huang                | May 5, 2015          | 60,000                   | 7,500                                   |
|                             | July 16, 2015        | 15,000                   |   |
| John R. Pinion II           | July 16, 2015        | 90,000                   | 7,500                                   |
| Jayson Dallas, M.D.         | August 4, 2015       | 90,000                   | 10,000                                  |

The values of the equity grants awarded to executive officers for the 2015 fiscal year, as well as all compensation actions taken with respect to the named executive officers in fiscal 2015, are reflected in the Summary Compensation Table.

***Employee Benefit Program***

Executive officers are eligible to participate in all of our employee benefit plans, including medical, dental, vision, group life, disability and accidental death and dismemberment insurance, in each case on the same basis as other employees, subject to applicable law. We also provide vacation and other paid holidays to all employees, including executive officers, all of which we believe to be comparable to those provided at peer companies. These benefit programs are designed to enable us to attract and retain our workforce in a competitive marketplace. Health, welfare and vacation benefits ensure that we have a productive and focused workforce through reliable and competitive health and other benefits.

Our retirement savings plan (401(k) Plan) is a tax-qualified retirement savings plan, pursuant to which all employees, including the named executive officers, are able to contribute certain amounts of their annual compensation, subject to limits prescribed by the Internal Revenue Service. We make matching contributions of up to 100% of the first 3% of salary contributed to the plan. The value of these benefits for each of our named executive officers is reflected in the All Other Compensation column of the Summary Compensation Table.

***Tax and Accounting Considerations***

*Deductibility of Executive Compensation.* In making compensation decisions affecting our executive officers, the Compensation Committee considers our ability to deduct under applicable federal corporate income tax law compensation payments made to executives. Specifically, the Compensation Committee considers the requirements and impact of Section 162(m) of the Internal Revenue Code, which limits the tax deductibility to us of compensation in excess of \$1.0 million in any year for certain executive officers, except for qualified performance-based compensation under the Section 162(m) rules. The Compensation Committee considers the Section 162(m) rules as a factor in determining compensation, but will not necessarily limit compensation to amounts deductible under Section 162(m).

*Accounting for Stock-Based Compensation.* Under the Accounting Standards Codification, or ASC, Topic 718, we are required to estimate and record an expense at the measurement date for each award of equity compensation over the vesting period of the award. Accounting rules also require us to record cash compensation as an expense at the time the obligation is incurred.

***Allocation of Compensation***

There is no pre-established policy or target for the allocation of compensation. The factors described above, as well as the overall compensation philosophy, are reviewed to determine the appropriate level and mix of compensation. In fiscal 2015, the largest portion of compensation to each of our named executive officers was in the form of equity compensation.

## **Table of Contents**

### ***Timing of Compensation Actions***

Cash compensation, including base salary adjustments, for our named executive officers is reviewed annually, usually in the first quarter of the fiscal year and upon promotion or other change in job responsibilities. Equity compensation is granted upon the hiring of the named executive officer as well as annually each May (provided that the named executive officer must have been employed for at least six months to receive an annual equity grant).

### ***Minimum Stock Ownership Requirements***

There are no minimum stock ownership guidelines for our executives or employees, although senior members of our management team are encouraged and expected to have a significant direct interest in the value of our common stock through open market purchases and/or receipt of equity awards.

### ***Conclusion***

Our compensation policies are designed and are continually being developed to retain and motivate our executive officers and to reward them for outstanding individual and corporate performance.

### **Risk Management and Mitigation**

In reviewing the compensation structure in fiscal 2015, the Compensation Committee also considered how the Company's compensation policies may affect the Company's risk profile and how compensation policies may be used to mitigate risks facing the Company. More specifically, the Compensation Committee considered the general design philosophy of the Company's policies for employees whose conduct would be most affected by incentives established by compensation policies. In considering these issues, the Compensation Committee concluded that the use of performance-based bonuses and long-term equity awards did not appear to create undue risks for the Company or encourage excessive risk-taking behavior on the part of employees.

With respect to bonus awards, the amount of an individual's award depends principally (exclusively, in the case of our Chief Executive Officer) on overall Company performance, which reduces the ability and incentive for an individual to take undue risks in an effort to increase the amount of his or her bonus award for a particular year. For fiscal 2015, the Company's performance goals were reviewed and approved by the independent members of the Board at the end of fiscal 2014 and are considered to be generally of the nature that would not encourage or reward excessive risk taking. Additionally, the Compensation Committee monitors Company performance throughout the year and has the ability to intervene in instances where actions by the Company vis-à-vis Company performance goal attainment would be considered unduly risky to prevent or penalize such actions.

With respect to equity awards, these awards typically vest over several years, meaning that long-term value creation, contrasted with short-term gain, presents the best opportunity for employees to profit from these awards.

### **Compensation Committee Report**

The Compensation Committee of the Company has reviewed and discussed with management the Compensation Discussion and Analysis required by Item 402(b) of Regulation S-K. Based on this review and discussion, the Compensation Committee recommended to the Board of Directors that the foregoing Compensation Discussion and Analysis be included in this proxy statement.

Submitted by the Compensation Committee of the Board of Directors



William Aliski, Chairman

Clay B. Siegall, Ph.D.

Daniel G. Welch

**Table of Contents****Summary Compensation Table**

The following table sets forth the compensation earned during the years ended December 31, 2015, 2014 and 2013 by our Chief Executive Officer, Chief Financial Officer and our next three highest-paid executive officers. We refer to these officers as our named executive officers.

| Name and Principal Position  | Year | Salary     | Bonus      | Stock Awards <sup>(1)</sup> | Option Awards <sup>(2)</sup> | Non-Equity                                 | All                               | Total        |
|--|------|------------|------------|-----------------------------|------------------------------|--|-----------------------------------|--------------|
|  |      |            |            |                             |                              | Incentive Plan Compensation <sup>(3)</sup> | Other Compensation <sup>(4)</sup> |              |
| Emil D. Kakkis, M.D.,<br>Ph.D.<br><i>President and Chief Executive Officer</i>   | 2015 | \$ 535,000 | \$         | \$ 967,746                  | \$ 3,578,620                 | \$ 280,875                                 | \$ 38,419                         | \$ 5,400,660 |
|  | 2014 | \$ 496,867 | \$         | \$                          | \$                           | \$ 175,000                                 | \$ 27,163                         | \$ 699,030   |
|  | 2013 | \$ 306,034 | \$         | \$                          | \$ 218,873                   | \$ 140,910                                 | \$ 19,721                         | \$ 685,538   |
| Shalini Sharp,<br><i>Chief Financial Officer and Senior Vice President</i>   | 2015 | \$ 420,000 | \$         | \$ 373,516                  | \$ 1,378,004                 | \$ 182,700                                 | \$ 28,082                         | \$ 2,382,302 |
|  | 2014 | \$ 350,000 | \$ 30,000  | \$                          | \$                           | \$ 117,563                                 | \$ 14,500                         | \$ 512,063   |
|  | 2013 | \$ 278,440 | \$ 30,000  | \$                          | \$ 182,394                   | \$ 110,206                                 | \$ 24,193                         | \$ 625,233   |
| Dennis Huang <sup>(5)</sup><br><i>Chief Technical Operations Officer and Senior Vice President</i>                                     | 2015 | \$ 215,135 | \$         | \$ 469,725                  | \$ 3,441,486                 | \$ 95,805                                  | \$ 20,390                         | \$ 4,242,541 |
|  | 2014 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |
|  | 2013 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |
| John R. Pinion II <sup>(6)</sup><br><i>Chief Quality Operations Officer and Senior Vice President Analytical Sciences and Research</i> | 2015 | \$ 171,432 | \$         | \$ 936,525                  | \$ 6,731,608                 | \$ 79,945                                  | \$ 10,681                         | \$ 7,930,191 |
|  | 2014 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |
|  | 2013 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |
| Jayson Dallas, M.D. <sup>(7)</sup><br><i>Chief Commercial Officer and Senior Vice President</i>  | 2015 | \$ 164,279 | \$ 100,000 | \$ 1,164,500                | \$ 6,266,173                 | \$ 74,724                                  | \$ 40,603                         | \$ 7,810,279 |
|  | 2014 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |
|  | 2013 | \$         | \$         | \$                          | \$                           | \$   | \$                                | \$           |

(1) The amounts reported in this column represent the grant date fair value of the restricted stock units granted to our named executive officers during 2015, as computed in accordance with Accounting Standards Codification, or ASC, Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the restricted stock units reported in this column are set forth in the notes to our financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015. Note that the amounts reported in this column reflect the accounting cost for these restricted stock units, and do not correspond to the actual economic value that may be received by the named executive officers from the restricted stock units.

(2)

The amounts reported in this column represent the grant date fair value of the stock options granted to our named executive officers during 2015 and 2013, respectively, as computed in accordance with ASC Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in the notes to our financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the named executive officers from the options.

- (3) Amounts for fiscal 2015 represent cash bonuses earned in fiscal 2015, and paid during 2016, based on achievement of performance goals and other factors deemed relevant by our Compensation Committee. Amounts for fiscal 2014 represent cash bonuses earned in fiscal 2014, and paid during 2015, based on achievement of performance goals and other factors deemed relevant by our Compensation Committee. Amounts for fiscal 2013 represent cash bonuses earned in 2013, and paid during 2014, based on achievement of performance goals and other factors deemed relevant by our Compensation Committee.
  
- (4) For fiscal 2015, amounts reported in this column for Dr. Kakkis consist of \$7,950 for 401(k) matching contributions, \$3,750 for health savings account contributions, \$20,169 for medical, dental, vision and life/accidental death & dismemberment premiums and \$6,550 for key person life insurance premiums paid by us. Amounts reported in this column for Ms. Sharp consist of \$7,950 for 401(k) matching contributions, \$3,750 for health savings account contributions and \$16,382 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us.

**Table of Contents**

Amounts reported in this column for Mr. Huang consist of \$5,643 for 401(k) matching contributions and \$14,747 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us. Amounts reported in this column for Mr. Pinion consist of \$10,681 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us. Amounts reported in this column for Dr. Dallas consist of \$32,257 for relocation reimbursements and \$8,346 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us. For fiscal 2014, amounts reported in this column for Dr. Kakkis consist of health savings account contributions and medical, dental, vision and life/accidental death & dismemberment and key person life insurance premiums paid by us and amounts reported for Ms. Sharp consist of health savings account contributions and medical, dental, vision and life/accidental death & dismemberment premiums. For fiscal 2013, amounts reported in this column for Dr. Kakkis consist of medical, dental, vision and life/accidental death & dismemberment and key person life insurance premiums paid by us and amounts reported for Ms. Sharp consist of medical, dental, vision and life/accidental death & dismemberment premiums paid by us.

(5) Mr. Huang commenced employment with us in May 2015.

(6) Mr. Pinion commenced employment with us in July 2015.

(7) Dr. Dallas commenced employment with us in August 2015.

**Narrative Disclosure to Summary Compensation Table*****Employment Arrangements with Our Named Executive Officers***

***Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer.*** We entered into an executive employment agreement with Dr. Kakkis in June 2011 for the position of President and Chief Executive Officer, which was amended in August 2014. Dr. Kakkis currently receives a base salary of \$567,200, which is subject to adjustment at the discretion of the Board or the Compensation Committee. Dr. Kakkis is also eligible for an annual performance bonus of up to 60% of his base salary, payable based on our overall corporate performance, as determined by the Compensation Committee. Dr. Kakkis is also eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the executive employment agreement, the employment of Dr. Kakkis is at will; we may terminate his employment at any time, without advance notice, for any reason or for no reason at all and Dr. Kakkis may terminate his employment at any time, upon four weeks prior written notice, for any reason or for no reason at all.

***Shalini Sharp, Chief Financial Officer and Senior Vice President.*** We entered into an offer letter in March 2012 with Shalini Sharp for the position of Chief Financial Officer and Senior Vice President, which was amended in August 2014. Ms. Sharp currently receives a base salary of \$441,000, which is subject to adjustment at the discretion of the Board or the Compensation Committee. Ms. Sharp is also eligible for an annual performance bonus of up to 40% of her base salary, payable based on her individual performance evaluated against certain goals mutually agreed upon and our overall performance, as determined by the Chief Executive Officer in consultation with the Compensation Committee. Additionally, pursuant to the terms of the offer letter, Ms. Sharp received an option to purchase up to 191,418 shares of our common stock in connection with her hiring. Ms. Sharp is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Ms. Sharp's employment is at will and may be terminated either by us or by her, with or without advance notice, for any reason or for no reason at all.

***Dennis Huang, Chief Technical Operations Officer and Senior Vice President.*** We entered into an offer letter in February 2015 with Mr. Huang for the position of Senior Vice President, Technical Operations. In May 2015, upon his commencement of employment with the Company, the Compensation Committee approved the title of Chief Technical Operations Officer and Senior Vice President for Mr. Huang. Mr. Huang currently receives a base salary of \$349,000, which is subject to adjustment at the discretion of the Board or the Compensation Committee. Mr. Huang is also eligible for an annual performance bonus of up to 40% of his base salary, payable based on his individual performance evaluated against certain goals mutually agreed upon and

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**Table of Contents**

our overall corporate performance, as determined by the Chief Executive Officer in consultation with the Compensation Committee. Additionally, pursuant to the terms of the offer letter and further input from the Compensation Committee, Mr. Huang received (i) an option to purchase 60,000 shares of our common stock and (ii) 7,500 restricted stock units, in connection with his hiring. In July 2015, Mr. Huang was awarded an additional 15,000 options to align Mr. Huang's equity compensation with other executive officer hires in fiscal 2015. Mr. Huang is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Mr. Huang's employment is at will and may be terminated either by us or by him, with or without advance notice, for any reason or for no reason at all.

***John R. Pinion II, Chief Quality Operations Officer and Senior Vice President of Analytical Sciences and Research.*** We entered into an offer letter in June 2015 with Mr. Pinion for the position of Chief Quality Operations Officer and Senior Vice President of Analytical Sciences and Research. Mr. Pinion currently receives a base salary of \$399,185, which is subject to adjustment at the discretion of the Board or the Compensation Committee. Mr. Pinion is also eligible for an annual performance bonus of up to 40% of his base salary, payable based on his individual performance evaluated against certain goals mutually agreed upon and our overall corporate performance, as determined by the Chief Executive Officer in consultation with the Compensation Committee. Additionally, pursuant to the terms of the offer letter, Mr. Pinion received (i) an option to purchase 90,000 shares of our common stock and (ii) 7,500 restricted stock units, in connection with his hiring. Mr. Pinion is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Mr. Pinion's employment is at will and may be terminated either by us or by him, with or without advance notice, for any reason or for no reason at all.

***Jayson Dallas, M.D., Chief Commercial Officer and Senior Vice President.*** We entered into an offer letter in July 2015, as amended in August 2015, with Dr. Dallas for the position of Chief Commercial Officer and Senior Vice President. Dr. Dallas currently receives a base salary of \$433,000, which is subject to adjustment at the discretion of the Board or the Compensation Committee. Dr. Dallas is also eligible for an annual performance bonus of up to 40% of his base salary, payable based on his individual performance evaluated against certain goals mutually agreed upon and our overall corporate performance, as determined by the Chief Executive Officer in consultation with the Compensation Committee. Additionally, pursuant to the terms of the offer letter, Dr. Dallas received (i) a sign-on bonus in the amount of \$100,000, (ii) a retention bonus of \$100,000 to be paid in two installments (the first installment will be paid on the one-year anniversary of Dr. Dallas's employment and the second installment will be paid on the two-year anniversary of Dr. Dallas's employment), (iii) reimbursement of relocation expenses, (iv) an option to purchase 90,000 shares of our common stock and (v) 10,000 restricted stock units, in connection with his hiring. Dr. Dallas is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Dr. Dallas's employment is at will and may be terminated either by us or by him, with or without advance notice, for any reason or for no reason at all.

Each of these employment arrangements also contain provisions that provide for certain payments and benefits in the event of an involuntary termination of employment. In addition, the named executive officers may be entitled to accelerated vesting of their outstanding and unvested awards in certain circumstances. The information below describes certain compensation that may become due and payable as a result of certain events.

***Involuntary Termination of Employment***

Pursuant to their employment arrangements, each named executive officer is eligible to receive certain payments and benefits in the event of certain qualifying terminations, including termination of his or her employment by us without cause (as defined below) or resignation of his or her employment with good reason or because of a constructive termination (each, as defined below). Upon the timely execution of a general release of claims, each named executive

officer is eligible to receive the following payments and benefits:

if Dr. Kakkis is terminated by us other than for cause or because of death or disability, he shall be entitled to receive 24 months of base salary continuation;

**Table of Contents**

if Dr. Kakkis resigns his employment with us for good reason following a change in control (as defined below) within six months of the event constituting good reason and after providing us with 20 days to cure the good reason, then he shall be entitled to receive 24 months of base salary continuation; and

if Ms. Sharp, Mr. Huang, Mr. Pinion or Dr. Dallas is terminated by us without cause or resigns employment with us due to a constructive termination, each executive will be entitled to: (i) extend the exercise period applicable to any options then held such that the executive has 12 months from termination to exercise any of the vested shares, provided that in no event shall the exercise period be extended beyond the expiration date of any options then held; and (ii) 12 months of base salary continuation.

***Covered Transaction***

Pursuant to the employment agreement with Dr. Kakkis, as amended, the offer letter with Ms. Sharp, as amended, and the offer letters with Mr. Huang, Mr. Pinion and Dr. Dallas, in addition to the severance benefits described above, in the event (i) we consummate a Covered Transaction (as defined in our 2014 Incentive Plan), which includes certain mergers or material asset sales, as well as any dissolution, liquidation, or winding down of the Company, (ii) the executive is employed by us (or our subsidiaries) on the date the Covered Transaction is consummated, and (iii) the executive is terminated by us without cause or resigns employment with us due to a constructive termination (or, in the case of Dr. Kakkis, for good reason) within 12 months after the consummation of the Covered Transaction, the vesting of all of such executive's outstanding equity shall accelerate with respect to 100% of the then-unvested shares.

***Definitions***

For purposes of Dr. Kakkis's employment agreement, cause means his:

commission of a felony or any crime involving dishonesty, breach of trust, or physical harm to any person;

willful engagement in conduct that is in bad faith and materially injurious to us, including but not limited to misappropriation of trade secrets, fraud, or embezzlement;

material breach of his employment agreement that is not cured within 10 days after written notice to him from us; or

willful refusal to implement or follow a lawful policy or directive of ours, which breach is not cured within 10 days after written notice to him from us.

For purposes of each of the offer letters with Ms. Sharp, Mr. Huang, Mr. Pinion and Dr. Dallas, cause means the named executive officer's:



gross negligence in carrying out, or material failure to carry out, his or her duties for us (including, without limitation, failure to cooperate in any Company investigation), after notice from the Board and a reasonable opportunity to cure (if deemed curable);

breach of his or her fiduciary duties to us, after notice from the Board and a reasonable opportunity to cure (if deemed curable);

conviction of, or plea of guilty or no contest to, any felony;

any act of fraud or embezzlement with respect to his or her obligations to us or otherwise relating to our business;

**Table of Contents**

material violation of any of our policies;

material breach of any agreement entered into with us; or

unauthorized use or disclosure of confidential information or trade secrets of ours or of our affiliates.

For purposes of Dr. Kakkis's employment agreement, "good reason" means any of the following events if (i) we effect the event without the consent of Dr. Kakkis and (ii) such event occurs after a change in control:

a change in his position with us that materially reduces his level of responsibility;

a material reduction in his base salary, except for reductions that are comparable to reductions generally applicable to similarly situated executives of ours; or

a relocation of his principal place of employment by more than 50 miles.

For purposes of Dr. Kakkis's employment agreement, "change in control" means a change in ownership or control of us effected through a merger, consolidation, or acquisition by any person or related group of persons (other than an acquisition by us or by an employee benefit plan sponsored by us or by a person or persons that directly or indirectly control, is controlled by, or is under common control with, us) of beneficial ownership of securities possessing more than 50% of the total combined voting power of our outstanding securities.

For purposes of each of the offer letters with Ms. Sharp, Mr. Huang, Mr. Pinion and Dr. Dallas, "constructive termination" means the occurrence of any of the following events without the named executive officer's consent if (i) the executive provides us with written objection (or notice) to the event or condition within 30 days following the occurrence of the event or condition, (ii) we do not reverse or otherwise cure the event within 30 days of receiving such written objection, and (iii) the executive resigns his employment with us within 30 days following the expiration of that cure period:

a material reduction or change in the executive's job duties, responsibilities and requirements from the executive's job duties, responsibilities and requirements immediately prior to such reduction or change, taking into account the differences in job title and duties that are normally occasioned by reason of an acquisition of one company by another;

a material reduction of the executive's base salary (other than an equal, across-the-board reduction in the compensation of all similarly-situated employees of ours or the surviving entity that is approved by the Board); or

a requirement that the executive relocate to a principal office that increases his or her one-way commute by more than 50 miles relative to the executive's immediately preceding principal office.



**Table of Contents****Grants of Plan-Based Awards**

The following table sets forth certain information regarding grants of plan-based awards to the named executive officers during fiscal 2015.

| Name                        | Grant Date | Estimated Future Payouts Under Non-Equity Incentive Plan Awards |                 | Stock Awards: Number of Shares of Stock or Units Granted (#) | Option Awards: Number of Securities Underlying Options Granted (#) | Exercise Price of Option Awards (\$/Share) | Grant Date Fair Value of Stock and Option Awards (\$)(3) |
|-----------------------------|------------|---|-----------------|--|--|--|--|
|                             |            | Target (\$)(1)  | Maximum (\$)(2) |  |  |  |  |
| Emil D. Kakkis, M.D., Ph.D. | 5/21/2015  | \$ 267,500  | \$ 401,250      | 11,400   | 68,300   | \$ 84.89                                   | \$ 967,746   |
|                             | 5/21/2015  |   |                 |  |  |  | \$ 3,578,620   |
| Shalini Sharp               | 5/21/2015  | \$ 168,000  | \$ 252,000      | 4,400  | 26,300   | \$ 84.89                                   | \$ 373,516   |
|                             | 5/21/2015  |   |                 |  |  |  | \$ 1,378,004   |
| Dennis Huang                | 5/5/2015   | \$ 136,000  | \$ 204,000      | 7,500  | 60,000   | \$ 62.63                                   | \$ 469,725   |
|                             | 5/5/2015   |   |                 |  |  |  | \$ 2,319,551   |
|                             | 7/16/2015  |   |                 |  |  |  | \$ 1,121,935   |
| John R. Pinion II           | 7/16/2015  | \$ 156,000  | \$ 234,000      | 7,500  | 90,000   | \$ 124.87                                  | \$ 936,525   |
|                             | 7/16/2015  |   |                 |  |  |  | \$ 6,731,608   |
| Jayson Dallas, M.D.         | 8/4/2015   | \$ 170,000  | \$ 255,000      | 10,000   | 90,000   | \$ 116.45                                  | \$ 1,164,500   |
|                             | 8/4/2015   |   |                 |  |  |  | \$ 6,266,173   |

(1) The amount represents the target amount of each named executive officer's cash payments under our 2015 annual incentive program as established by the Board and described in "Compensation Discussion and Analysis" above. Actual payments made for fiscal 2015 are provided in the Summary Compensation Table.

(2) The amount represents the maximum amount of each named executive officer's cash payments under our 2015 annual incentive program as established by the Board and described in "Compensation Discussion and Analysis" above. Actual payments made for fiscal 2015 are provided in the Summary Compensation Table.

- (3) This column reflects the aggregate grant date fair value of equity awards granted in 2015 as computed in accordance with Accounting Standards Codification, or ASC, Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in the notes to our financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015.

***Outstanding Equity Awards at December 31, 2015***

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2015.

**Table of Contents**

| Name                        | Option Awards   |   |                            |                           | Stock Awards  |  |
|-----------------------------|---|---|----------------------------|---------------------------|---|--|
|                             | Number of Securities Underlying Unexercised Options (#) Exercisable | Number of Securities Underlying Unexercised Options (#) Unexercisable | Option Exercise Price (\$) | Option Expiration Date    | Number of Shares or Units of Stock That Have Not Vested (#) | Market Value of Shares or Units of Stock That Have Not Vested (\$) |
| Emil D. Kakkis, M.D., Ph.D. | 24,923  | 22,930  | \$ 6.86                    | 10/31/2023 <sup>(1)</sup> |   |  |
|                             |   | 68,300  | \$ 84.89                   | 5/20/2025 <sup>(2)</sup>  | 11,400 <sup>(3)</sup>                                       | \$ 1,278,852   |
| Shalini Sharp               | 78,724  | 19,939  | \$ 0.81                    | 8/1/2022 <sup>(4)</sup>   |   |  |
|                             | 20,769  | 19,109  | \$ 6.86                    | 10/31/2023 <sup>(5)</sup> |   |  |
|                             |   | 26,300  | \$ 84.89                   | 5/20/2025 <sup>(6)</sup>  | 4,400 <sup>(7)</sup>  | \$ 493,592   |
| Dennis Huang                |   | 60,000  | \$ 62.63                   | 5/4/2025 <sup>(8)</sup>   | 7,500 <sup>(9)</sup>  | \$ 841,350   |
|                             |   | 15,000  | \$ 124.87                  | 7/15/2025 <sup>(10)</sup> |   |  |
| John R. Pinion II           |   | 90,000  | \$ 124.87                  | 7/15/2025 <sup>(11)</sup> | 7,500 <sup>(12)</sup>                                       | \$ 841,350   |
| Jayson Dallas, M.D.         |   | 90,000  | \$ 116.45                  | 8/3/2025 <sup>(13)</sup>  | 10,000 <sup>(14)</sup>                                      | \$ 1,121,800   |

- (1) Represents options to purchase shares of our common stock granted on November 1, 2013. The shares underlying this option vest as follows: 25% vested on November 1, 2014, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Dr. Kakkis's employment agreement dated June 15, 2011, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (2) Represents options to purchase shares of our common stock granted on May 21, 2015. The shares underlying this option vest as follows: 25% will vest on May 21, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Dr. Kakkis's employment agreement dated June 15, 2011, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (3) Represents restricted stock units granted on May 21, 2015. The restricted stock units vest with respect to 1/4 of the underlying shares on each anniversary of the grant date over a four-year period. Vesting of 100% of the unvested units shall accelerate in connection with a Covered Transaction pursuant to the terms of Dr. Kakkis's employment agreement dated June 15, 2011, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

(4)

Represents options to purchase shares of our common stock granted on August 2, 2012. The shares underlying this option vest as follows: 25% vested on May 21, 2013, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Ms. Sharp's offer letter dated March 12, 2012, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

- (5) Represents options to purchase shares of our common stock granted on November 1, 2013. The shares underlying this option vest as follows: 25% vested on November 1, 2014, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Ms. Sharp's offer letter dated March 12, 2012, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

**Table of Contents**

- (6) Represents options to purchase shares of our common stock granted on May 21, 2015. The shares underlying this option vest as follows: 25% will vest on May 21, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Ms. Sharp's offer letter dated March 12, 2012, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (7) Represents restricted stock units granted on May 21, 2015. The restricted stock units vest with respect to 1/4 of the underlying shares on each anniversary of the grant date over a four-year period. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Ms. Sharp's offer letter dated March 12, 2012, as amended on August 8, 2014, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (8) Represents options to purchase shares of our common stock granted on May 5, 2015. The shares underlying this option vest as follows: 25% will vest on May 5, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Mr. Huang's offer letter dated February 20, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (9) Represents restricted stock units granted on May 5, 2015. The restricted stock units vest with respect to 1/4 of the underlying shares on each anniversary of the grant date over a four-year period. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Mr. Huang's offer letter dated February 20, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (10) Represents options to purchase shares of our common stock granted on July 16, 2015. The shares underlying this option vest as follows: 25% will vest on July 16, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Mr. Huang's offer letter dated February 20, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.
- (11) Represents options to purchase shares of our common stock granted on July 16, 2015. The shares underlying this option vest as follows: 25% will vest on July 16, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Mr. Pinion's offer letter dated June 11, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

(12)



Represents restricted stock units granted on July 16, 2015. The restricted stock units vest with respect to 1/4 of the underlying shares on each anniversary of the grant date over a four-year period. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Mr. Pinion's offer letter dated June 11, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

**Table of Contents**

(13) Represents options to purchase shares of our common stock granted on August 4, 2015. The shares underlying this option vest as follows: 25% will vest on August 4, 2016, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the holder's continued service to us through each such vesting date. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Dr. Dallas's offer letter dated April 27, 2015, as amended on August 3, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

(14) Represents restricted stock units granted on August 4, 2015. The restricted stock units vest with respect to 1/4 of the underlying shares on each anniversary of the grant date over a four-year period. Vesting of 100% of the unvested shares shall accelerate in connection with a Covered Transaction pursuant to the terms of Dr. Dallas's offer letter dated April 27, 2015, as amended on August 3, 2015, as more fully described above under the section entitled Narrative Disclosure to Summary Compensation Table Covered Transaction.

**Option Exercises and Stock Vested**

The following table sets forth the options exercised by our named executive officers during fiscal 2015. There was no vesting of restricted stock units held by the named executive officers during fiscal 2015.

| Name and Principal Position   | Option Awards                             |                                    |
|---|---|------------------------------------|
|   | Number of Shares Acquired on Exercise (#) | Value Realized on Exercise (\$)(1) |
| Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer  |   |                                    |
| Shalini Sharp, Chief Financial Officer and Senior Vice President  | 13,000                                    | \$ 1,258,770                       |
| Dennis Huang, Chief Technical Operations Officer and Senior Vice President                                      |   |                                    |
| John R. Pinion II, Chief Quality Operations Office and Senior Vice President of Analytical Science and Research |   |                                    |
| Jayson Dallas, M.D., Chief Commercial Officer and Senior Vice President   |   |                                    |

(1) Value realized on exercise of stock option awards does not represent proceeds from any sale of any common stock acquired upon exercise, but is determined by multiplying the number of shares acquired upon exercise by the difference between the exercise price of the option and the closing price of our common stock on The NASDAQ Global Select Market at each time of exercise.

**Pension Benefits**

We do not have a defined benefit plan. Our named executive officers did not participate in, or otherwise receive any special benefits under, any pension or defined benefit retirement plan sponsored by us during fiscal 2015.

**Nonqualified Deferred Compensation**

During fiscal 2015, our named executive officers did not contribute to, or earn any amount with respect to, any defined contribution or other plan sponsored by us that provides for the deferral of compensation on a basis that is not tax-qualified.

**Table of Contents****Potential Payments Upon Termination or Change of Control**

The amount of compensation and benefits payable to each named executive officer in various termination and change in control situations has been estimated in the tables below. The value of the option vesting acceleration and restricted stock unit vesting acceleration was calculated for each of the tables below based on the assumption that the change in control and executive's employment termination occurred on December 31, 2015. The closing price of the Company's stock on The NASDAQ Global Select Market as of December 31, 2015, the last trading day of 2015, was \$112.18, which was used as the value of the Company's stock in the change in control. The value of the option vesting acceleration was calculated by multiplying the number of unvested option shares subject to vesting acceleration as of December 31, 2015 by the difference between the closing price of the Company's stock as of December 31, 2015 and the exercise price for such unvested option shares. No value is attributed to unvested options subject to acceleration which have exercise prices above the closing market price of our common stock as of December 31, 2015. The value of the restricted stock unit vesting acceleration was calculated by multiplying the number of unvested restricted stock units subject to vesting acceleration as of December 31, 2015 by the closing price of the Company's stock as of December 31, 2015.

***Dr. Emil Kakkis***

The following table describes the potential payments upon employment termination for Emil Kakkis, the Company's President and Chief Executive Officer, as if his employment terminated as of December 31, 2015, the last business day of the fiscal year.

| <b>Potential Payments Upon Termination or Change of Control</b> | <b>Termination by Company without Cause</b> | <b>Termination by Company Upon Death or Disability</b> | <b>Termination by Company without Cause or resignation for Good Reason following a Covered Transaction</b> |
|---|---|--|--|
| Base Salary   | \$ 1,070,000                                | \$ 1,070,000   | \$ 1,070,000   |
| Acceleration of equity awards                                   |   |  | \$ 5,557,747   |
| <b>Total</b>  | <b>\$ 1,070,000</b>                         | <b>\$ 1,070,000</b>                                    | <b>\$ 6,627,747</b>  |

***Ms. Shalini Sharp***

The following table describes the potential payments upon employment termination for Shalini Sharp, the Company's Chief Financial Officer and Senior Vice President, as if her employment terminated as of December 31, 2015, the last business day of the fiscal year.

| <b>Potential Payments Upon Termination or Change of</b> | <b>Termination by Company without Cause</b> | <b>Constructive Termination</b> | <b>Termination by Company without Cause or resignation due to a Constructive</b> |
|---|---|---------------------------------|--|
|---|---|---------------------------------|--|

**Control**

|                               |                   |                   | <b>Termination<br/>following a<br/>Covered<br/>Transaction</b> |
|-------------------------------|-------------------|-------------------|--|
| Base Salary                   | \$ 420,000        | \$ 420,000        | \$ 420,000   |
| Acceleration of equity awards |                   |                   | \$ 5,444,299   |
| <b>Total</b>                  | <b>\$ 420,000</b> | <b>\$ 420,000</b> | <b>\$ 5,864,299</b>  |

**Mr. Dennis Huang**

The following table describes the potential payments upon employment termination for Dennis Huang, the Company's Chief Technical Operations Officer and Senior Vice President, as if his employment terminated as of December 31, 2015, the last business day of the fiscal year.

**Table of Contents**

| <b>Potential Payments Upon</b>              |   |                                     | <b>Termination by Company<br/>without Cause or<br/>resignation due to a<br/>Constructive<br/>Termination<br/>following a<br/>Covered<br/>Transaction</b> |
|---|---|-------------------------------------|--|
| <b>Termination or Change of<br/>Control</b> | <b>Termination<br/>by Company<br/>without Cause</b> | <b>Constructive<br/>Termination</b> |  |
| Base Salary                                 | \$ 340,000  | \$ 340,000                          | \$ 340,000   |
| Acceleration of equity awards               |   |                                     | \$ 3,814,350   |
| <b>Total</b>                                | <b>\$ 340,000</b>                                   | <b>\$ 340,000</b>                   | <b>\$ 4,154,350</b>  |

**Mr. John R. Pinion II**

The following table describes the potential payments upon employment termination for John Pinion, the Company's Chief Quality Operations Officer and Senior Vice President of Analytical Sciences and Research, as if his employment terminated as of December 31, 2015, the last business day of the fiscal year.

| <b>Potential Payments Upon</b>              |   |                                     | <b>Termination by Company<br/>without Cause or<br/>resignation due to a<br/>Constructive<br/>Termination<br/>following a<br/>Covered<br/>Transaction</b> |
|---|---|-------------------------------------|--|
| <b>Termination or Change of<br/>Control</b> | <b>Termination<br/>by Company<br/>without Cause</b> | <b>Constructive<br/>Termination</b> |  |
| Base Salary                                 | \$ 390,000  | \$ 390,000                          | \$ 390,000   |
| Acceleration of equity awards               |   |                                     | \$ 841,350   |
| <b>Total</b>                                | <b>\$ 390,000</b>                                   | <b>\$ 390,000</b>                   | <b>\$ 1,231,350</b>  |

**Dr. Jayson Dallas**

The following table describes the potential payments upon employment termination for Jayson Dallas, the Company's Chief Commercial Officer and Senior Vice President, as if his employment terminated as of December 31, 2015, the last business day of the fiscal year.

| <b>Potential Payments Upon</b>              | <b>Termination<br/>by Company<br/>without Cause</b> | <b>Constructive<br/>Termination</b> | <b>Termination by Company<br/>without Cause or<br/>resignation due to a<br/>Constructive<br/>Termination<br/>following a</b> |
|---|---|-------------------------------------|--|
| <b>Termination or Change of<br/>Control</b> |   |                                     |  |
|   |   |                                     |  |

|                               |                   |                   | <b>Covered<br/>Transaction</b> |
|-------------------------------|-------------------|-------------------|--------------------------------|
| Base Salary                   | \$ 425,000        | \$ 425,000        | \$ 425,000                     |
| Acceleration of equity awards |                   |                   | \$ 1,121,800                   |
| <b>Total</b>                  | <b>\$ 425,000</b> | <b>\$ 425,000</b> | <b>\$ 1,546,800</b>            |

**Table of Contents****EQUITY COMPENSATION PLAN INFORMATION**

The table below discloses information as of December 31, 2015 with respect to our equity compensation plans that have been approved by stockholders and equity compensation plans that have not been approved by stockholders.

| <b>Plan Category</b>  | <b>Number of Securities to be Issued upon Exercise of Outstanding Options, Warrants and Rights</b> | <b>Weighted-Average Exercise Price of Outstanding Options, Warrants and Rights</b> | <b>Number of Securities Remaining Available for Future Issuance under Equity Compensation Plans (Excluding Securities Reflected in Column (a))</b> |
|---|--|--|--|
| <b>Equity compensation plans approved by security holders:</b>    |  |  |  |
| 2011 Equity Incentive Plan, as amended                            | 1,212,931  | \$ 5.30  |  |
| 2014 Incentive Plan   | 2,811,183  | 74.44  | 709,966  |
| 2014 Employee Stock Purchase Plan                                 |  |  | 950,295  |
| <b>Equity compensation plans not approved by security holders</b> |  |  |  |
| Total   | 4,024,114  | \$ 53.60   | 1,660,261  |

**DIRECTOR COMPENSATION**

Dr. Kakkis, our President and Chief Executive Officer, receives no compensation for his service as a director.

Our Board has adopted a non-employee director compensation policy that is designed to provide a total compensation package that enables us to attract and retain, on a long-term basis, high caliber non-employee directors. A summary of the non-employee director compensation arrangements for fiscal 2015 is set forth below:

|   | <b>Annual Retainer</b> |
|---|------------------------|
| <b>Board of Directors:</b>                            |                        |
| Chairman  | \$ 52,500              |
| Non-Chairman members                                  | \$ 35,000              |
| <b>Audit Committee:</b>                               |                        |
| Chairman  | \$ 15,000              |
| Non-Chairman members                                  | \$ 7,500               |
| <b>Compensation Committee:</b>                        |                        |
| Chairman  | \$ 10,000              |
| Non-Chairman members                                  | \$ 5,000               |
| <b>Nominating and Corporate Governance Committee:</b> |                        |



|                      |          |
|----------------------|----------|
| Chairman             | \$ 6,000 |
| Non-Chairman members | \$ 3,000 |

**Table of Contents**

Under the non-employee director compensation policy for fiscal 2015, each non-employee director who is initially appointed or elected to the Board will receive an option grant to purchase up to 17,500 shares of our common stock under our stock option plan on the date he or she first becomes a non-employee director, which will vest monthly over a three-year period, subject to the holder's continued service to us through each such vesting date. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director will be eligible to receive an annual option grant to purchase up to 7,500 shares of our common stock, which will vest in full upon the earlier of (a) the Company's subsequent annual meeting of stockholders and (b) the first anniversary of the date of grant, subject to the holder's continued service to us through such vesting date. All of the foregoing options will be granted at fair market value as of the date of grant.

The following table shows the compensation earned in fiscal 2015 by the Company's non-employee directors.

| Name                             | Fees Earned in |                              | Total        |
|----------------------------------|----------------|------------------------------|--------------|
|                                  | Fiscal 2015    | Option Awards <sup>(1)</sup> |              |
| Daniel G. Welch <sup>(2)</sup>   | \$ 39,000      | \$ 1,014,807                 | \$ 1,053,807 |
| William Aliski                   | \$ 50,000      | \$ 410,159                   | \$ 460,159   |
| Mathew K. Fust                   | \$ 56,000      | \$ 410,159                   | \$ 466,159   |
| Eran Nadav, Ph.D. <sup>(3)</sup> | \$ 27,750      | \$                           | \$ 27,750    |
| Michael Narachi <sup>(4)</sup>   | \$ 40,250      | \$ 1,016,416                 | \$ 1,056,666 |
| Clay B. Siegall, Ph.D.           | \$ 40,000      | \$ 410,159                   | \$ 450,159   |

(1) The amounts reported in this column represent the grant date fair value of the stock options granted to our non-employee directors during fiscal 2015, as computed in accordance with Accounting Standards Codification, or ASC, Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in the notes to our financial statements included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the non-employee directors from the options. As of December 31, 2015, Mr. Welch had 3,889 options outstanding, Mr. Aliski had 18,681 options outstanding, Mr. Fust had 12,362 options outstanding, Dr. Nadav had no options outstanding, Mr. Narachi had 4,861 options outstanding and Dr. Siegall had 18,681 options outstanding.

(2) Mr. Welch was appointed to the Board effective April 8, 2015.

(3) Dr. Nadav's term expired at the 2015 annual meeting of stockholders on June 18, 2015. In connection with the expiration of his term, his option to purchase up to 9,722 shares (i.e., the remaining unvested amount from his award of 17,500 options granted in January 2014) was accelerated.

(4) Mr. Narachi was appointed to the Board effective February 20, 2015.

**OTHER BUSINESS**

We know of no other matters to be submitted to a vote of stockholders at the Annual Meeting. If any other matter is properly brought before the Annual Meeting or any adjournment thereof, it is the intention of the persons named in the enclosed proxy to vote the shares they represent in accordance with their judgment. In order for any stockholder to nominate a candidate or to submit a proposal for other business to be acted upon at a given annual meeting, he or she must provide timely written notice to our corporate secretary in the form prescribed by our Bylaws, as described below.

**Table of Contents**

**STOCKHOLDER PROPOSALS**

Stockholder proposals intended to be included in the proxy statement for the 2017 annual meeting of stockholders pursuant to SEC Rule 14a-8 must be received by the Secretary of the Company no later than the close of business on December 29, 2016. In order to be considered for inclusion in our proxy statement, these proposals must satisfy the requirements of SEC Rule 14a-8.

The Company's Amended and Restated Bylaws also establish advance notice procedures with respect to proposals and director nominations submitted by a stockholder for presentation directly at an annual meeting, rather than for inclusion in our proxy statement. To be properly brought before the 2017 annual meeting of stockholders, stockholders who intend to present a stockholder proposal or director nomination at the meeting must deliver written notice of the proposal or nomination to the Secretary of the Company between 90 and 120 days prior to the one-year anniversary date of the 2016 annual meeting (that is, between February 9, 2017 and March 11, 2017); *provided, however*, that if the 2017 annual meeting date is advanced by more than 30 days before or delayed by more than 60 days after the anniversary date of the 2016 annual meeting, then such notice must be received on or before 10 days after the day on which the date of the 2017 annual meeting is first disclosed in a public announcement. Notice of any such stockholder proposals and director nominations must satisfy the requirements set forth in our Amended and Restated Bylaws. Proposals not meeting the requirements set forth in our Amended and Restated Bylaws will not be entertained at the annual meeting. If a stockholder fails to meet these deadlines or fails to satisfy the requirements of SEC Rule 14a-4, the persons named in the enclosed proxy will be allowed to use their discretionary voting authority to vote on any such proposal or nomination as they determine appropriate if and when the matter is raised at the annual meeting.

All notices of proposals or nominations, as applicable, must be addressed to the Secretary of the Company at 60 Leveroni Court, Novato, California 94949.

**Table of Contents**

**DELIVERY OF PROXY MATERIALS**

**Our annual report to stockholders for the fiscal year ended December 31, 2015, including audited financial statements, accompanies this Proxy Statement. Copies of our Annual Report on Form 10-K for fiscal 2015 and the exhibits thereto are available from the Company without charge upon written request of a stockholder to our investor relations department at 60 Leveroni Court, Novato, California 94949.** Copies of these materials are also available online through the SEC at *www.sec.gov*. The Company may satisfy SEC rules regarding delivery of proxy materials, including the proxy statement and annual report or Notice, as applicable, by delivering a single proxy statement and annual report or a single Notice, as applicable, to an address shared by two or more Company stockholders. This delivery method can result in meaningful cost savings for the Company. In order to take advantage of this opportunity, the Company may deliver only one proxy statement and annual report or one Notice, as applicable, to multiple stockholders who share an address, unless contrary instructions are received from one or more stockholders at that address prior to the mailing date. We undertake to deliver promptly upon written or oral request a separate copy of the proxy statement and annual report or Notice, as applicable, to a stockholder at a shared address to which a single copy of these materials was delivered. If you hold stock as a registered holder and prefer to receive separate copies of these materials either now or in the future, please contact the Company's investor relations department at 60 Leveroni Court, Novato, California 94949 or by telephone at (415) 483-8800. Similarly, if you share an address with another stockholder and have received multiple copies of our proxy statement and annual report or Notice, you may write or call us at the address and phone number above to request delivery of a single copy of these materials in the future. If your stock is held through a brokerage firm, bank or other financial institution and you prefer to receive separate copies of our proxy statement and annual report or Notice, as applicable, either now or in the future, please contact your brokerage firm, bank or other financial institution.

**EACH STOCKHOLDER IS URGED TO COMPLETE, DATE, SIGN AND PROMPTLY RETURN  
THE ENCLOSED PROXY.**

**Table of Contents**

**ULTRAGENYX PHARMACEUTICAL INC.**

**60 LEVERONI COURT**

**NOVATO, CA 94949**

**VOTE BY INTERNET**

*Before The Meeting* Go to **[www.proxyvote.com](http://www.proxyvote.com)**

Use the Internet to transmit your voting instructions and for electronic delivery of information up until 11:59 P.M. Eastern Time the day before the cut-off date or meeting date. Have your proxy card in hand when you access the web site and follow the instructions to obtain your records and to create an electronic voting instruction form.

*During The Meeting* Go to **[www.virtualshareholdermeeting.com/RARE16](http://www.virtualshareholdermeeting.com/RARE16)**

You may attend the Meeting via the Internet and vote during the Meeting. Have the information that is printed in the box marked by the arrow available and follow the instructions.

**VOTE BY PHONE 1-800-690-6903**

Use any touch-tone telephone to transmit your voting instructions up until 11:59 P.M. Eastern Time the day before the cut-off date or meeting date. Have your proxy card in hand when you call and then follow the instructions.

**VOTE BY MAIL**

Mark, sign and date your proxy card and return it in the postage-paid envelope we have provided or return it to Vote Processing, c/o Broadridge, 51 Mercedes Way, Edgewood, NY 11717.

TO VOTE, MARK BLOCKS BELOW IN BLUE OR BLACK INK AS FOLLOWS:

KEEP THIS PORTION FOR YOUR RECORDS

DETACH AND RETURN THIS PORTION ONLY

**THIS PROXY CARD IS VALID ONLY WHEN SIGNED AND DATED.**

**ULTRAGENYX PHARMACEUTICAL INC.**

**The Board of Directors recommends that you vote FOR the election of the three nominees for director named below:**

**Nominees:**

1a. William Aliski

**For**

**Withhold**

..

..

1b. Lars Ekman, M.D., Ph.D.

**For**

**Withhold**

..

..

1c. Matthew K. Fust

**For**

**Withhold**

..

..

**Table of Contents**

|   |            |                |                |
|---|------------|----------------|----------------|
| <b>The Board of Directors recommends you vote FOR proposal 2:</b>   | <b>For</b> | <b>Against</b> | <b>Abstain</b> |
| Ratification of the selection of Ernst & Young LLP as independent registered public accounting firm for the fiscal year ending December 31, 2016. | ..         | ..             | ..             |

|   |            |                |                |
|---|------------|----------------|----------------|
| <b>The Board of Directors recommends you vote FOR proposal 3:</b>           | <b>For</b> | <b>Against</b> | <b>Abstain</b> |
| Advisory (non-binding) vote to approve the Company's executive compensation | ..         | ..             | ..             |

|   |               |                |                |                |
|---|---------------|----------------|----------------|----------------|
| <b>The Board of Directors recommends you vote for 1 Year for proposal 4:</b>                            | <b>1 Year</b> | <b>2 Years</b> | <b>3 Years</b> | <b>Abstain</b> |
| Advisory (non-binding) vote on the frequency of future advisory votes to approve executive compensation | ..            | ..             | ..             | ..             |

For address changes and/or comments, please check this box and write them on the back where indicated.

|   |            |           |
|---|------------|-----------|
| Please indicate if you plan to attend this meeting. | ..         | ..        |
|   | <b>Yes</b> | <b>No</b> |

Please sign exactly as your name(s) appear(s) hereon. When signing as attorney, executor, administrator, or other fiduciary, please give full title as such. Joint owners should each sign personally. All holders must sign. If a corporation or partnership, please sign in full corporate or partnership name, by authorized officer.

|                                    |      |                          |      |
|------------------------------------|------|--------------------------|------|
| Signature [PLEASE SIGN WITHIN BOX] | Date | Signature (Joint Owners) | Date |
|------------------------------------|------|--------------------------|------|



**Table of Contents**

**ULTRAGENYX PHARMACEUTICAL INC.**

**Annual Meeting of Stockholders**

**June 9, 2016 4:00 p.m., Pacific Time**

**This proxy is solicited by the Board of Directors**

The stockholder(s) hereby appoint(s) Emil D. Kakkis, M.D., Ph.D. and Shalini Sharp, or either of them, as proxies, each with the power to appoint his or her substitute, and hereby authorize(s) them to represent and to vote, as designated on the reverse side of this ballot, all of the shares of Common Stock of Ultragenyx Pharmaceutical Inc. that the stockholder(s) is/are entitled to vote at the 2016 Annual Meeting of Stockholders to be held at 4:00 p.m., Pacific Time, on June 9, 2016, and at any adjournment or postponement thereof.

**This proxy, when properly executed, will be voted in the manner directed herein. If no such direction is made, this proxy will be voted in accordance with the Board of Directors' recommendations and in the discretion of the proxy holders on all other matters that may come before the meeting or any adjournment or postponement thereof.**

**Address Changes/Comments:**

(If you noted any Address Changes/Comments above, please mark corresponding box on the reverse side.)

**Continued and to be signed on reverse side**