

Ultragenyx Pharmaceutical Inc.  
Form DEF 14A  
April 27, 2018  
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**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:

Preliminary Proxy Statement

**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)**

Payment of Filing Fee (Check the appropriate box):

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No fee required.

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**60 Leveroni Court**

**Novato, California 94949**

**NOTICE OF ANNUAL MEETING OF STOCKHOLDERS**

**To Be Held on June 19, 2018**

**at 2:00 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 19, 2018, at 2:00 p.m. Pacific Time virtually via the Internet at [www.virtualshareholdermeeting.com/RARE18](http://www.virtualshareholdermeeting.com/RARE18) (the **Annual Meeting**). Instructions on how to participate in the Annual Meeting and demonstrate proof of stock ownership are posted at [www.virtualshareholdermeeting.com/RARE18](http://www.virtualshareholdermeeting.com/RARE18). The webcast of the Annual Meeting will be archived for one year after the date of the Annual Meeting at [www.virtualshareholdermeeting.com/RARE18](http://www.virtualshareholdermeeting.com/RARE18). Only stockholders who held stock at the close of business on the record date, April 23, 2018, 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 II 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, 2018; (3) an advisory (non-binding) resolution to approve the compensation of our named executive officers, and (4) 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 No. 1 relates solely to the election of the three directors nominated by the Board of Directors. 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. In accordance with Delaware law, a list of stockholders entitled to vote at the Annual Meeting will be available in electronic form during the Annual Meeting at the following URL: [www.virtualshareholdermeeting.com/RARE18](http://www.virtualshareholdermeeting.com/RARE18), 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.

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 27, 2018

**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 19, 2018:**

**The Proxy Statement and Annual Report on Form 10-K for the year ended December 31, 2017 are available at [www.proxyvote.com](http://www.proxyvote.com).**

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**60 Leveroni Court**

**Novato, California 94949**

**PROXY STATEMENT FOR**

**2018 ANNUAL MEETING OF STOCKHOLDERS**

**TO BE HELD ON JUNE 19, 2018**

**at 2:00 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 2018 Annual Meeting of Stockholders, to be held virtually via the Internet at [www.virtualshareholdermeeting.com/RARE18](http://www.virtualshareholdermeeting.com/RARE18) on June 19, 2018, at 2:00 p.m. Pacific Time (the *Annual Meeting*). The Notice Regarding the Availability of Proxy Materials (the *Notice*) containing instructions on how to access this Proxy Statement and our Annual Report on Form 10-K for the fiscal year ended December 31, 2017 (the *Annual Report*) is first being mailed on or about April 27, 2018 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 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 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.

We 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 23, 2018 (the *Record Date*), are entitled to notice of and to vote at the Annual Meeting. On the Record Date, 49,705,310 shares of





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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 brokerage firm, 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/RARE18](http://www.virtualshareholdermeeting.com/RARE18) 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/RARE18](http://www.virtualshareholdermeeting.com/RARE18) and following the instructions regarding voting or (ii) return a voting instruction form provided to them by the institution that holds their shares in order 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 Class II 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. As further described in Proposal No. 1 below, any nominee for director who receives a greater number of withhold votes for his or her election than votes for his or her election will promptly tender his or her resignation to the Board following certification of the election results.

*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 the compensation of our named executive officers.* 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.

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 we will be forced to incur additional expenses soliciting proxies for the Annual Meeting.



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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.

**Virtual Meeting**

We conduct our annual meeting of stockholders virtually via the Internet to facilitate stockholder attendance and participation. Taking advantage of this virtual approach reduces our expenses and eliminates the time we would otherwise spend managing the various aspects of holding a physical meeting. The virtual format enhances stockholder access by allowing our stockholders to participate in our annual meetings and ask questions of our management during the Q&A sessions from any location. Our annual meetings are only one aspect of our stockholder outreach program, which is a year-long effort by our management to engage with our stockholders in a continuous and meaningful way.

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**PROPOSAL NO. 1 ELECTION OF CLASS II DIRECTORS**

Our Amended and Restated Certificate of Incorporation 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 eight directors. Currently, the Class II directors (whose terms expire at the Annual Meeting) are Deborah Dunsire, M.D., Michael Narachi, and Clay B. Siegall, Ph.D. The Class III directors (whose terms expire at the 2019 annual meeting of stockholders) are William Aliski, Lars Ekman, M.D., Ph.D., and Matthew K. Fust. The Class I directors (whose terms expire at the 2020 annual meeting of stockholders) are Emil D. Kakkis, M.D., Ph.D. and Daniel G. Welch. The Class II directors elected at the Annual Meeting will hold office until the 2021 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 our Amended and Restated Bylaws (the *bylaws* ).

As described below, the Board has nominated Dr. Dunsire, Mr. Narachi, and Dr. Siegall for election as Class II directors at the Annual Meeting. Dr. Dunsire, Mr. Narachi, and Dr. Siegall have indicated their willingness to serve if elected. Should Dr. Dunsire, Mr. Narachi, or Dr. Siegall 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, values, experience, interest in the Company, and commitment to the representation of the long-term interests of the stockholders. The Nominating and Corporate Governance Committee also considers each potential nominee's contribution to the Board's diversity of talents, skill, backgrounds, including with respect to age, gender, national origin, sexual orientation and identification, race, ethnicity and culture, and expertise. Additionally, the Nominating and Corporate Governance Committee considers whether a nominee will be able to dedicate sufficient time to, and focus on, his or her duties as a member of the Board. 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 Dr. Dunsire, Mr. Narachi, and Dr. Siegall for election as Class II directors. We did not pay any fees to any third party 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 50 under the caption Stockholder Proposals.

Although, as noted above, the Nominating and Corporate Governance Committee considers whether nominees assist in achieving a mix of Board members that represents a diversity of talents, skills, backgrounds and expertise, we have no formal policy regarding board diversity. The Nominating and Corporate Governance

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Committee assesses the effectiveness of its Board membership criteria on an ongoing basis and as part of the director selection and nomination process.

**Nominees and Incumbent Directors**

The Nominating and Corporate Governance Committee has recommended, and the Board has nominated, Dr. Dunsire, Mr. Narachi, and Dr. Siegall to be elected as Class II directors at the Annual Meeting. The following table sets forth the following information for Dr. Dunsire, Mr. Narachi, Dr. Siegall and our continuing directors: the year each was first elected a director of the Company; their respective ages as of the date of this Proxy Statement; the positions currently held with the Company; the year their current term will expire; and their current class.

Nominee/Director Name	Age	Position	Year		
			Director Since	Current Term Expires	Current Director Class
<b><i>Nominees for Class II Director</i></b>					
Deborah Dunsire, M.D.	55	Director	2017	2018	II
Michael Narachi	58	Director	2015	2018	II
Clay B. Siegall, Ph.D.	57	Director	2014	2018	II
<b><i>Continuing Directors</i></b>					
Emil D. Kakkis, M.D., Ph.D.	57	President and Chief Executive Officer	2010	2020	I
Daniel G. Welch	60	Chairman of the Board	2015	2020	I
William Aliski	70	Director	2011	2019	III
Lars Ekman, M.D., Ph.D.	68	Director	2016	2019	III
Matthew K. Fust	53	Director	2014	2019	III

**Class II Directors Nominated for Election**

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

**Deborah Dunsire, M.D.** has served as a member of our Board since April 2017. Dr. Dunsire previously served as President and Chief Executive Officer and as a director of Xtuit Pharmaceuticals, Inc., a private biopharmaceutical company, from January 2017 to March 2018. Prior to her position at Xtuit, she served as President and Chief Executive Officer and a director of FORUM Pharmaceuticals Inc., a private pharmaceutical company, a position she held from July 2013 to May 2016. Prior to FORUM, Dr. Dunsire worked for Takeda Pharmaceutical Company Limited, a publicly traded pharmaceutical company, as a corporate officer from June 2010 to June 2011 and a director from June 2011 to June 2013. She served as President and Chief Executive Officer and as a director of Millennium Pharmaceuticals, Inc., a publicly traded biopharmaceutical company, between 2005 and 2008, when it was acquired by Takeda, and then as President and Chief Executive Officer of Millenium: The Takeda Oncology Company after the acquisition between 2008 and 2013. Prior to Millenium, Dr. Dunsire held various roles of increasing responsibility at Novartis Pharma AG between 1988 and 2005. Dr. Dunsire previously served as a director of Allergan, Inc., a publicly traded pharmaceutical company, between December 2006 and April 2015. She currently serves as a board member of Alexion Pharmaceuticals Inc. She obtained an MBBCh from the University of the Witwatersrand. We believe that Dr. Dunsire is qualified to serve on our Board due to her extensive experience in the biotechnology and pharmaceutical sectors, including service as the chief executive officer of pharmaceutical companies, which gives her the skills to provide us with operational and strategic insights.

**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. Orexigen filed for reorganization under Chapter 11 of the U.S. Bankruptcy Code in March 2018. Previously, Mr. Narachi served as Chairman, Chief Executive Officer and President of Ren



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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. Mr. Narachi currently serves as chair of the Emerging Companies Section Governing Board of BIO, the Biotechnology Innovation Organization, and previously served as a member of the Board of Directors of PhRMA, the Pharmaceutical Research and Manufacturers of America. He previously served as the chairman of the board of directors of Celladon Corporation, a publicly traded biotechnology company, from October 2013 to March 2016, and as a director of AMAG Pharmaceuticals, Inc., a publicly traded specialty pharmaceutical company, from November 2006 to April 2014. 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, all of which give him the skills to provide us with operational and strategic insights.

**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 1997. 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. Dr. Siegall also served as a director of Mirna Therapeutics, Inc., a publicly traded biotechnology company, from January 2013 to December 2016. 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.

**Additional Statement In Support of Dr. Siegall's Nomination.**

In considering Dr. Siegall's nomination, the Nominating and Corporate Governance Committee and the Board acknowledged his service on two public company boards of directors in addition to his service on the board of directors of Seattle Genetics, Inc., and considered whether such service, while serving as the President and Chief Executive Officer of Seattle Genetics, Inc., would allow him to dedicate sufficient time to, and focus on, his duties as a director of the Company. In determining to nominate Dr. Siegall, the Nominating and Corporate Governance Committee and the Board recognized the extensive value, skills, and experience that Dr. Siegall adds to our Board and the superior level of engagement he has demonstrated during his tenure as a member of our Board. For example, during 2017, he attended 100% of the aggregate of the meetings of the Board and the committees on which he is a member—a total of 15 meetings—and was an important contributor during each meeting. Accordingly, the Nominating and Corporate Governance Committee and the Board concluded that Dr. Siegall should continue to serve as a director based on his business experience, demonstrated reliability, and commitment to service on our Board.

**Class III Directors Continuing in Office Until 2019**

**William Aliski** has served as a member of our Board since January 2011. Mr. Aliski has served as a commercial consultant for early-stage orphan disease companies, including Ra Pharmaceuticals, Inc., from October 2016 to March 2017, Clementia Pharmaceuticals, Inc., from December 2015 to January 2017, 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

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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, a venture capital firm, where Dr. Ekman 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 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 Inc. 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 Atara Biotherapeutics, Inc., Dermira, Inc., and MacroGenics, Inc., which are publicly traded biopharmaceutical companies. He also served on the board of directors of Sunesis Pharmaceuticals, Inc. from May 2005 to May 2017. 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 2020**

**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 our 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 is also Founder, and serves as President, 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

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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 self-employed as a biotechnology advisor. Mr. Welch was an Executive Partner at Sofinnova Ventures, a biopharmaceutical venture capital firm, from January 2015 through January 2018. 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 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.

## **Vote Required**

The three nominees who receive the greatest number of affirmative votes will be elected as Class II 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.

## **Director Resignation Policy**

We have a director resignation policy, which is 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. The policy establishes that any director nominee who receives more withhold votes than for votes in an uncontested election of directors is required to tender his or her resignation promptly following the certification of the election results. Abstentions and broker non-votes are not counted as either a withhold or for vote. The Nominating and Corporate Governance Committee of the Board will promptly consider the tendered resignation and make a recommendation to the Board. The Board will act on the recommendation of the Nominating and Corporate Governance Committee no later than 90 days following the certification of the election results. The Board will promptly publicly disclose its decision and, if applicable, the reasons for rejecting the tendered resignation.

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

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**PROPOSAL NO. 2 RATIFICATION OF THE 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, 2018, and has further directed that we submit the selection of Ernst & Young LLP for ratification by our stockholders at the Annual Meeting.

We are not required to submit the selection of our independent registered public accounting firm for stockholder approval, but are submitting our 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 our best interests.

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 our 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 2017 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, 2017 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 or expected to be billed to us for the audit and other services rendered by Ernst & Young LLP, our independent registered public accounting firm, for the fiscal years ended December 31, 2017 and 2016.

	<b>2017</b>	<b>2016</b>
Audit fees <sup>(1)</sup>	\$ 1,314,000	\$ 1,195,000
Audit-related fees		
Tax fees <sup>(2)</sup>	156,000	108,000
All other fees <sup>(3)</sup>	2,000	2,000
<b>Total</b>	<b>\$ 1,472,000</b>	<b>\$ 1,305,000</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 reports on Form 10-K; the review of our interim financial statements included in our quarterly reports on Form 10-Q; consultation on technical accounting matters; 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.

(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 cast 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.**



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**REPORT OF THE AUDIT COMMITTEE**

The Audit Committee evaluates auditor performance, manages relations with our 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 our 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 our financial reporting process on behalf of the Board. Our management has the primary responsibility for the financial statements and reporting process, including our 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, 2017. This review included a discussion of the quality and the acceptability of our financial reporting, including the nature and extent of disclosures in the financial statements and the accompanying notes.

The Audit Committee also reviewed with our 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 our financial reporting and such other matters as are required to be discussed with the Committee pursuant to Auditing Standard No. 1301, *Communication with Audit Committees* issued by the PCAOB. The Audit Committee has received the written disclosures and the letter from the independent registered public accounting firm required by the PCAOB 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 us and management, including the matters required by the applicable rules of the PCAOB.

In addition to the matters specified above, the Audit Committee discussed with our 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 our 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 our audited financial statements should be included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017.

Submitted by the Audit Committee of the Board of Directors

Matthew Fust, Chairperson

William Aliski

Michael Narachi

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**PROPOSAL NO. 3 ADVISORY (NON-BINDING) VOTE TO APPROVE THE COMPENSATION OF OUR NAMED EXECUTIVE OFFICERS**

**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 the compensation of our named executive officers (a *say-on-pay vote*).

The say-on-pay vote is a non-binding vote on the compensation of our 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 say-on-pay vote is not a vote on our general compensation policies, compensation of our Board of Directors, or our compensation policies as they relate to risk management. The Dodd-Frank Act requires us to hold the say-on-pay vote at least once every three years, and we have determined to hold a say-on-pay vote every year.

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 24 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 us, 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 named 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 Company's definitive proxy statement for the 2018 Annual Meeting of Stockholders (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 Proxy Statement.

**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.**



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**CORPORATE GOVERNANCE**

**Director Independence**

Our Board currently consists of eight members. Our Board has determined that Mr. Aliski, Dr. Dunsire, Dr. Ekman, Mr. Fust, Mr. Narachi, Dr. Siegall and Mr. Welch qualify as independent directors in accordance with the NASDAQ listing requirements and rules. Dr. Kakkis is not considered independent because he is an employee of the Company. 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 or her family members has engaged in certain types of business dealings with us. 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. 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 that we are required to disclose, and any waivers of our Code of Business Conduct and Ethics that apply 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 our business or affairs 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 2017, our Board met five 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 the director was a member in our last fiscal year held during the period when the director served on the Board or the committees, as applicable.

The Board has a standing Audit Committee, Compensation Committee, Nominating and Corporate Governance Committee and Research and Development 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 (including the heightened independence standards that apply to Audit Committee and Compensation Committee members).

Four of the seven directors serving at the time of the 2017 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 encouraged to attend the annual meeting of stockholders.

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

We currently separate 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 seven of our eight 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 five times in 2017, 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 our 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 us. 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 us. 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 reports 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 financial risk assessment and management; establishes, maintains and oversees our Code of Business Conduct and Ethics; assists the Nominating and Corporate Governance Committee by overseeing our compliance program with respect to legal and regulatory requirements impacting areas of financial risk; annually reviews and reassesses the adequacy of the Audit Committee charter; and performs other duties, as specified in the Audit Committee Charter, a copy of which is available on our 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 us and reporting back to the Board regarding the same. The Audit Committee met six times in fiscal 2017. All members of the Audit Committee satisfy

the current independence and financial literacy standards promulgated by NASDAQ and the SEC, and the



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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.

*Compensation Committee.* The members of the Compensation Committee are Mr. Aliski (Chairperson), Dr. Siegall and Mr. Welch. The Compensation Committee annually reviews and approves corporate goals and objectives relevant to the compensation of our executive officers and individual corporate goals and objectives relevant to compensation of our Chief Executive Officer; 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 benefit 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; oversees the maintenance and presentation to the Board of management's plans for succession to senior management positions; and performs other duties, as specified in the Compensation Committee Charter, a copy of which is available on our 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 eight times in fiscal 2017 and also acted by written consent eight 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; oversees the maintenance and presentation to the Board of plans for succession to the position of Chief Executive Officer; assists the Compensation Committee in its oversight of succession planning for other senior management positions; oversees our compliance program; annually reviews and reassesses the adequacy of the Nominating and Corporate Governance Committee charter; and performs other duties, as specified in the Nominating and Corporate Governance Committee Charter, a copy of which is available on our 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 us and reporting back to the Board regarding the same. The Nominating and Corporate Governance Committee met six times in fiscal 2017 and also acted by written consent one time. All members of the Nominating and Corporate Governance Committee satisfy the current NASDAQ independence standards.

*Research and Development Committee.* The members of the Research and Development Committee are Dr. Ekman (Chairperson), Dr. Dunsire, Dr. Kakkis and Dr. Siegall. The Research and Development Committee assists the Board in its oversight of our strategic direction for our pipeline and investment in research and development; evaluates and advises on our key R&D activities and early pipeline development goals and strategy; assesses the resources and budget allocated to R&D spend and provides guidance regarding the investment of resources in pipeline growth;

evaluates and provides input with respect to the quality of the science being conducted and overall program execution; assesses the overall quality of the R&D programs and prospects for progression to monitor our pipeline to maintain product flow; evaluates our clinical-stage pipeline and its

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progress, as well as those operational execution initiatives that are important for filing and approval of products in our pipeline; and performs other duties, as specified in the Research and Development Committee Charter, a copy of which is available on our website at [www.ultragenyx.com](http://www.ultragenyx.com) under the Corporate Governance subsection of the Investors tab. The Research and Development Committee met two times in fiscal 2017.

**Compensation Committee Interlocks and Insider Participation**

During fiscal 2017, the Compensation Committee consisted of Mr. Aliski, Dr. Siegall and Mr. Welch. 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, their respective ages as of the date of this Proxy Statement 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 Class II Directors).

<b>Name</b>	<b>Age</b>	<b>Position</b>
Emil D. Kakkis, M.D., Ph.D.	57	President and Chief Executive Officer, Director
Camille L. Bedrosian, M.D.	65	Chief Medical Officer and Executive Vice President
Jayson Dallas, M.D.	50	Chief Commercial Officer and Executive Vice President
Dennis Huang	53	Chief Technical Operations Officer and Executive Vice President
Thomas Kassberg	57	Chief Business Officer and Executive Vice President
Karah Parschauer	40	General Counsel and Executive Vice President
John R. Pinion II	52	Chief Quality Officer and Executive Vice President of Translational Sciences
Shalini Sharp	43	Chief Financial Officer and Executive Vice President

**Camille L. Bedrosian, M.D.** has served as our Executive Vice President and Chief Medical Officer since January 2018. Prior to Ultragenyx, she served as Senior Vice President and Chief Medical Officer at Alexion Pharmaceuticals, Inc., a biopharmaceutical company, from May 2008 to January 2018. Between September 2002 and April 2008, she served as Vice President and Chief Medical Officer at Ariad Pharmaceuticals, Inc., an oncology company. From 1997 to 2002, Dr. Bedrosian served in the Clinical Research and Development Department of Wyeth/Genetics Institute, Inc., most recently as Senior Director, Oncology/Hematology. From 1986 to 1997, she was a Fellow, an Associate, and then Assistant Professor of Medicine in the Division of Hematology and Oncology at Duke University Medical Center and the Duke Comprehensive Cancer Center. Dr. Bedrosian received her B.A. degree from Harvard University/Radcliffe College in Chemistry, her M.S. in Biophysics from M.I.T., and her M.D. from Harvard Medical School.

**Jayson Dallas, M.D.** has served as our Executive Vice President since January 2016 and our Chief Commercial Officer since August 2015. Between August 2015 and January 2016, he served as our Senior Vice President. 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 has also served as a board member of Arena Pharmaceuticals Inc. since February 2017. 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 Executive Vice President since January 2016 and our Chief Technical Operations Officer since May 2015. Between May 2015 and January 2016, he served as our Senior Vice President. 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 Executive Vice President since January 2016 and our Chief Business Officer since November 2011. Between November 2011 and January 2016, he served as our Senior Vice President. 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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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.

**Karah Parschauer** has served as our General Counsel and Executive Vice President since June 2016. Prior to Ultragenyx, Ms. Parschauer served in various executive capacities, and most recently as Vice President, Associate General Counsel, at Allergan plc, a pharmaceutical company, from June 2005 until June 2016. Prior to Allergan, Ms. Parschauer was an attorney at Latham & Watkins LLP, where she practiced in the areas of mergers and acquisitions, securities offerings and corporate governance. Ms. Parschauer holds a B.A. in Biology from Miami University and a J.D. from Harvard Law School.

**John R. Pinion II** has served as our Chief Quality Officer and Executive Vice President of Translational Sciences since September 2017. Between January 2016 and September 2017, he served as our Executive Vice President of Analytical Sciences and Research, and between July 2015 and September 2017, as our Chief Quality Operations Officer. Between July 2015 and January 2016, he served as our Senior Vice President of Analytical Sciences and Research. Prior to Ultragenyx, Mr. Pinion served in various roles with increasing responsibilities at Genentech, a pharmaceutical company, between 2005 and June 2015, 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 to July 2015. Mr. Pinion holds a Bachelor of Science in Mechanical Engineering from the University of West Virginia.

**Shalini Sharp** has served as our Executive Vice President since January 2016 and our Chief Financial Officer since May 2012. Between May 2012 and January 2016, she served as our Senior Vice President. 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 and of Array BioPharma Inc. since April 2017. 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, 2017, we have not become, and are not currently proposed to be, a participant in 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 transactions 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, a related person is defined as any person described in Item 404(a) of Regulation S-K and includes any director, nominee for director or executive officer of the Company; a beneficial owner of more than five percent of any class of our voting securities; and a

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person who is an immediate family member of any such director, nominee for director, executive officer or more-than-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, nominee for director, executive officer or more-than-five percent beneficial owner).

Pursuant to this policy, if we want to enter into a transaction with a related person, our Chief Financial Officer (or General Counsel, in the case where the Chief Financial Officer has a direct or indirect interest in the transaction) will review the proposed transaction to determine if such transaction qualifies as a related-person transaction. If the Chief Financial Officer (or General Counsel, if applicable) determines that the proposed transaction is a related-person transaction, then the proposed transaction will be submitted to the Audit Committee for consideration at the next Audit Committee meeting; provided, however, that if the Chief Financial Officer (or General Counsel, if applicable), in consultation with the Chief Executive Officer, determines that it is not practicable or desirable to wait until the next meeting of the Audit Committee, then the Chief Financial Officer (or General Counsel, if applicable) shall submit the proposed transaction to the chairperson of the Audit Committee (who possesses delegated authority to act between meetings of the Audit Committee to pre-approve or ratify, as applicable, any related-person transaction in which the aggregate amount involved is expected to be less than \$1 million).

In the event that our Chief Executive Officer or Chief Financial Officer (or General Counsel, if applicable) becomes aware of a related-person transaction that has not been previously approved or previously ratified under our related-person transaction 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 or the chairperson of the Audit Committee, as applicable, will consider all of the relevant facts and circumstances available, including (if applicable) but not limited to: the benefits to us; 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. The Audit Committee or the chairperson of the Audit Committee, as applicable, will only approve related-person transactions that are in, or are not inconsistent with, the best interests of the Company and its stockholders, as the Audit Committee or the chairperson of the Audit Committee determines in good faith.

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 2017.





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**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 13, 2018 (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 options, 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 49,674,179 shares of our common stock outstanding as of April 13, 2018. 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 of any other person, except with respect to the percentage ownership of all directors and executive officers as a

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group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o Ultragenyx Pharmaceutical Inc., 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>		
Wellington Management Group LLP and affiliates <sup>(1)</sup>	5,859,120	11.8%
Capital Research Global Investors <sup>(2)</sup>	4,985,005	10.0%
Capital International Investors <sup>(3)</sup>	4,411,165	8.9%
BlackRock, Inc. <sup>(4)</sup>	3,731,887	7.5%
FMR LLC <sup>(5)</sup>	3,648,275	7.3%
The Vanguard Group <sup>(6)</sup>	3,166,465	6.4%
New Perspective Fund <sup>(7)</sup>	2,768,288	5.6%
<b>Directors and Named Executive Officers:</b>		
William Aliski <sup>(8)</sup>	90,235	*
Deborah Dunsire, M.D. <sup>(9)</sup>	5,833	*
Lars Ekman, M.D., Ph.D. <sup>(10)</sup>	16,458	*
Matthew K. Fust <sup>(11)</sup>	43,125	*
Michael Narachi <sup>(12)</sup>	30,625	*
Clay B. Siegall, Ph.D. <sup>(13)</sup>	38,125	*
Daniel G. Welch <sup>(14)</sup>	30,625	*
Emil D. Kakkis, M.D., Ph.D. <sup>(15)</sup>	3,276,249	6.6%
Jayson Dallas, M.D. <sup>(16)</sup>	94,323	*
Thomas Kassberg <sup>(17)</sup>	259,898	*
John R. Pinion II <sup>(18)</sup>	88,145	*
Shalini Sharp <sup>(19)</sup>	161,529	*
All executive officers and directors as a group <sup>(20)</sup> (15 persons)	4,245,642	8.4%

\* 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 8, 2018 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 December 29, 2017, Wellington Management Group LLP, Wellington Group Holdings LLP, and Wellington Investment Advisors Holdings LLP have shared voting power with respect to 4,505,983 of these shares and shared dispositive power with respect to 5,859,120 of these shares; and Wellington Management Company LLP has shared voting power with respect to 4,344,935 of these shares and shared dispositive power with respect to 5,334,743 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.

- (2) Based on information set forth in a Schedule 13G/A filed with the SEC on February 14, 2018 by Capital Research Global Investors. The Schedule 13G/A reported that, as of December 29, 2017, 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 filed with the SEC on January 10, 2018 by Capital International Investors. The Schedule 13G reported that, as of December 29, 2017, Capital International

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Investors has sole voting power with respect to 4,139,700 of these shares and sole dispositive power with respect to 4,411,165 of these shares. Capital International Investors disclaims beneficial ownership of these shares pursuant to Rule 13d-4 of the Exchange Act. Capital International Investors divisions of Capital Research and Management Company ( CRMC ) and Capital Bank and Trust, as well as the following CRMC subsidiaries: Capital Guardian Trust Company, Capital International Limited, Capital International Sarl, Capital International K.K. and Capital International, Inc., collectively provide investment management services under the name Capital International Investors. The principal business address for Capital International Investors is listed in such filing as 11100 Santa Monica Boulevard, 16<sup>th</sup> Floor, Los Angeles, CA 90025.

- (4) Based on information set forth in a Schedule 13G/A filed with the SEC on January 23, 2018 by BlackRock, Inc. The Schedule 13G/A reported that, as of December 31, 2017, BlackRock, Inc. has sole voting power with respect to 3,650,795 shares and sole dispositive power with respect to 3,731,887 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/A filed with the SEC on February 13, 2018 by FMR LLC and Abigail P. Johnson. The Schedule 13G/A reported that, as of December 31, 2017, FMR LLC has sole voting power with respect to 461,534 of these shares and sole dispositive power with respect to 3,648,275 of these shares. FMR LLC has no shared voting or shared dispositive power with respect to any of the shares. The principal business address for FMR LLC and Abigail P. Johnson is listed in such filing as 245 Summer Street, Boston, MA 02210.
- (6) Based on information set forth in a Schedule 13G/A filed with the SEC on February 9, 2018 by The Vanguard Group. The Schedule 13G/A reported that, as of December 31, 2017, The Vanguard Group has sole voting power with respect to 77,102 shares, shared voting power with respect to 3,850 shares, sole dispositive power with respect to 3,088,533 shares, and shared dispositive power with respect to 77,932 shares. The principal business address for The Vanguard Group is listed in such filing as 100 Vanguard Blvd., Malvern, PA 19355.
- (7) Based on information set forth in a Schedule 13G filed with the SEC on February 14, 2018 by New Perspective Fund, an investment company registered under the Investment Company Act of 1940, which is advised by Capital Research and Management Company ( CRMC ). CRMC manages equity assets for various investment companies through three divisions, Capital Research Global Investors, Capital World Investors, and Capital International Investors. These divisions generally function separately from each other with respect to investment research activities and they make investment decisions and proxy voting decisions for the investment companies on a separate basis. Under certain circumstances, New Perspective Fund may vote the shares of the fund. These shares may also be reflected in a filing made by Capital Research Global Investors, Capital International Investors, and/or Capital World Investors. The principal business address for New Perspective Fund is listed in such filing as 333 South Hope Street, Los Angeles, CA 90071.
- (8) Consists of (a) 71,485 shares of common stock and (b) 18,750 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.

- (9) Consists of shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.
  
- (10) Consists of (a) 1,875 shares of common stock and (b) 14,583 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.
  
- (11) Consists of (a) 13,194 shares of common stock and (b) 29,931 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.
  
- (12) Consists of (a) 1,875 shares of common stock and (b) 28,750 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.

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- (13) Consists of (a) 1,875 shares of common stock and (b) 36,250 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.
- (14) Consists of (a) 1,875 shares of common stock and (b) 28,750 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018.
- (15) Consists of (a) 2,559,741 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009, (b) 405,980 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) 155,303 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018 and (e) 5,525 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018. Dr. Kakkis shares voting and dispositive power over the 2,559,741 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 405,980 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 155,303 shares of common stock issuable pursuant to stock options held by Dr. Kakkis and the 5,525 shares of common stock issuable pursuant to the vesting of restricted stock units.
- (16) Consists of (a) 4,748 shares of common stock, (b) 88,725 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018 and (c) 850 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018.
- (17) Consists of (a) 68,648 shares of common stock, (b) 189,150 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018 and (c) 2,100 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018.
- (18) Consists of (a) 3,620 shares of common stock, (b) 83,775 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018 and (c) 750 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018.
- (19) Consists of (a) 46,913 shares of common stock, (b) 112,516 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 13, 2018 and (c) 2,100 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018.
- (20) Consists of (a) 3,186,713 shares of common stock 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) 895,279 shares of common stock issuable pursuant to stock options held by our directors and executive officers that are exercisable within 60 days of April 13, 2018 and (d) 13,950 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 13, 2018.





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**EXECUTIVE COMPENSATION**

**Compensation Discussion and Analysis**

The following compensation discussion and analysis describes the material elements of compensation earned in fiscal 2017 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, 2017 are:

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

Shalini Sharp, Chief Financial Officer and Executive Vice President;

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

Thomas Kassberg, Chief Business Officer and Executive Vice President; and

John R. Pinion II, Chief Quality Officer and Executive Vice President of Translational Sciences.

These persons constitute our principal executive officer, our principal financial officer and our three other most highly paid executive officers serving during fiscal 2017.

***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 to both 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 us to attract and retain highly qualified executives with the skills and experience required for the achievement of business goals;

align compensation elements with our 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: a base salary, an annual bonus opportunity and equity-based compensation. The Compensation Committee believes that cash compensation in the form of base salary and an annual bonus opportunity provides our executive officers with short-term rewards for success in achieving annual goals and objectives, and that long-term compensation through the award of stock options, restricted stock units or other equity awards aligns the objectives of management with those of our stockholders with respect to long-term performance and success.

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In setting compensation levels for our executive officers, the Compensation Committee does not formulaically benchmark against any one specific reference point. Instead, it considers a variety of factors, including peer group survey data for the 25<sup>th</sup>, 50<sup>th</sup>, and 75<sup>th</sup> percentiles, tenure, role, responsibilities, performance, and local competitive market practices. Compensation paid to our named executive officers is delivered primarily through at-risk pay, based on both short-term and long-term incentives, including the achievement of corporate and individual goals and objectives.

In addition to our compensation elements, the following compensation program features are designed to align our executive team's interests 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;

We prohibit our employees, including our executive team, from pledging our securities;

We do not offer our executive team any substantially enhanced benefits or perquisites when compared with our overall employee population;

We have a clawback policy, which permits us to recover performance-based cash and equity compensation paid to our current or former executive officers in certain cases; see [Clawback Policy](#) ;

We require our executive officers and directors to hold shares of our common stock in order to align their long-term interests with those of our stockholders; see [Minimum Stock Ownership Requirements](#) ;

We have double-trigger vesting of outstanding equity awards following covered transactions under our employment arrangements with our executive officers. See [Summary Compensation Table Narrative Disclosure to Summary Compensation Table Covered Transaction](#) ; and

We have established a long-term incentive program applicable to all current employees, including our executive officers, to further tie compensation to performance and focus employee efforts on corporate goals and objectives; see [Equity Compensation Equity Grants in Fiscal 2017 Long-Term Incentive Program](#)

***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 our 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 our named executive officers. While the Compensation Committee draws on a number of resources, including input from the Board, the Chief Executive Officer and its independent compensation consultants, to make decisions regarding our executive compensation program, ultimate decision-making authority rests with the Compensation Committee. The Compensation Committee retains discretion over base salaries, annual bonuses, and equity compensation for executive officers. The Compensation Committee relies upon the judgment of its members in making compensation decisions, after

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reviewing our performance 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 independent, external compensation consultant, Radford, an Aon Hewitt Company ( ***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 our peers in the industry and other technical considerations, including tax- and accounting-related matters. The Compensation Committee regularly evaluates Radford's performance 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 competitive market considerations 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 other non-employee directors of the Board to evaluate the performance of the Chief Executive Officer and determine compensation actions for the Chief Executive Officer.

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

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. In order to evaluate the level of compensation for our named executive officers for 2017, our Compensation Committee, using information provided by Radford, established a peer group of publicly traded, national and regional companies in the biopharmaceutical and biotechnology industries based on a balance of the following criteria:

companies with drugs in late-stage development, as well as recently commercial companies;

companies with comparable market capitalizations (i.e., in the range of \$1.0 billion to \$7.5 billion);

companies with headcounts between 100 and 500; and

companies located in biotech hubs, such as the San Francisco bay area, San Diego, Massachusetts and the New York/New Jersey tristate area.



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Our peer group for 2017, referred to as our 2017 peer group, used to evaluate compensation actions for the 2017 fiscal year was selected by the Compensation Committee with Radford's support in August 2016 and is comprised of the following 20 publicly-traded companies in the pharmaceutical and biotechnology industries:

ACADIA Pharmaceuticals Inc.	Intercept Pharmaceuticals, Inc.	Prothena Corporation Plc
Agios Pharmaceuticals, Inc.	Ionis Pharmaceuticals, Inc.	Puma Biotechnology, Inc.
Alnylam Pharmaceuticals, Inc.	Juno Therapeutics, Inc.	Sarepta Therapeutics, Inc.
ARIAD Pharmaceuticals, Inc.	Kite Pharma, Inc.	Spark Therapeutics, Inc.
bluebird bio, Inc.	Neurocrine Biosciences, Inc.	TESARO, Inc.
Exelixis Inc.	Novavax, Inc.	Theravance Biopharma, Inc.
Halozyme Therapeutics, Inc.	Portola Pharmaceuticals, Inc.	

We believe that the compensation practices of our 2017 peer group provided us with appropriate compensation benchmarks for evaluating the compensation of our named executive officers for 2017 because of the developmental, market and organizational characteristics we shared with our peer group. At the time that we selected our 2017 peer group we were at approximately the 60<sup>th</sup> percentile in terms of market capitalization and the 52<sup>nd</sup> percentile in terms of the number of employees relative to the peer group.

***Annual Performance Reviews***

Our Compensation Committee conducts an annual performance review of our named executive officers and approves their compensation. By the first quarter of each year, base salaries and equity awards for the fiscal year are approved and, for purposes of determining potential payments under our corporate bonus plan (the ***bonus plan***), annual corporate goals and individual performance objectives are established and set forth in writing. After the end of each year, our Compensation Committee determines the amounts that will be paid to our executive officers under our bonus plan after carefully (i) reviewing overall corporate performance; (ii) performing an evaluation of each named executive officer's annual performance against established corporate goals; (iii) reviewing each individual executive officer's contributions to achievement of the corporate goals; and (iv) in the case of executive officers other than our Chief Executive Officer, reviewing the achievement of individual performance objectives.

Our Compensation Committee, with input from the Board, evaluates our Chief Executive Officer's individual performance, determines whether to adjust his base salary and determines the amount of his bonus, if any, under our bonus plan. Our Compensation Committee typically determines adjustments in base salary and the amount of any bonus 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.

***Base Salary******Overview***

The Compensation Committee believes it is important to provide adequate fixed compensation to our executive officers working in a highly volatile and competitive industry. The Compensation Committee's choice of actual pay

levels versus our competitive market 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. In



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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 corporate 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;

executive officer compensation levels at other similar companies to ensure competitiveness; and

recruiting and retention market dynamics.

*2017 Base Salaries*

The Compensation Committee engaged Radford to conduct a comprehensive benchmarking study reporting on compensation levels and practices, including equity, relative to our 2017 peer group. Radford prepared an Executive Compensation Assessment report in October 2016 that provided a competitive assessment of our executive compensation program as compared to the 2017 peer group data for base salaries, target total cash compensation and equity compensation. The report demonstrated that the base salary level for our CEO was below the 50<sup>th</sup> percentile as compared to the 2017 peer group.

The Compensation Committee approved an increase to the Chief Executive Officer's base salary effective March 1, 2017, to position his base salary more competitively with our 2017 peer group in recognition of the Chief Executive Officer's outstanding performance, his contribution to the increase in our market value and the need to retain his services to lead us in achieving our corporate goals and business objectives.

For 2017, increases in base salaries for our named executive officers, other than our CEO, were approximately 3.4-3.6% annualized. Beginning in 2017, the effective date for annual salary increases was moved from January 1 to March 1, so that all elements of direct compensation can be reviewed and assessed at the same point in the year. For 2017 only, annual salary increases were positively prorated by 16.6% to account for this two-month delay, resulting in effective increases in our named executive officer's base salaries ranging from 3.9-4.2%, other than our Chief Executive Officer. Increases in base salaries for 2017, other than with respect to our Chief Executive Officer, were a result of standard annual merit increases that were based on the achievement of corporate and individual performance goals and objectives during 2016 (and were similar to merit-based increases for our employees generally), as well as Radford peer group data regarding 2017 salary increases.



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The following table shows the increases in base salaries for our named executive officers between fiscal 2016 and fiscal 2017:

<b>Name</b>	<b>Title</b>	<b>Fiscal 2016 Base Salary</b>	<b>Fiscal 2017 Base Salary</b>	<b>Percentage (%) Increase<sup>(1)</sup></b>
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	\$ 567,200	\$ 655,000	15.5%
Shalini Sharp	Chief Financial Officer and Executive Vice President	\$ 441,000	\$ 459,305	4.2%
Jayson Dallas, M.D.	Chief Commercial Officer and Executive Vice President	\$ 433,000	\$ 450,973	4.2%
Thomas Kassberg	Chief Business Officer and Executive Vice President	\$ 405,600	\$ 421,500	3.9%
John R. Pinion II	Chief Quality Officer and Executive Vice President of Translational Sciences	\$ 399,185	\$ 415,754	4.2%

(1) Reflects extra two months of positive pro-ration for change in increase date from January to March.

**Annual Bonus***Overview*

Our bonus plan provides an opportunity for cash bonus payments based upon the attainment of annual performance goals. For all executive officers, except the Chief Executive Officer, the goals relate to both corporate and individual performance. Corporate performance goals include corporate, financial, and operational measures or objectives. Individual performance goals focus on individual contributions intended to drive achievement of the corporate goals and build and manage the executive officers' functions. The Chief Executive Officer's bonus is based solely on corporate performance.

Generally, by the end of each fiscal year, the Compensation Committee assesses corporate performance, and determines an overall percentage of goal achievement, for such year. This corporate performance score then determines the size of the bonus pool applicable to corporate performance from which annual bonuses are to be paid to executive officers. A corporate performance score at or below 50% results in the corporate component of the bonus pool not being funded. If an executive officer receives a performance rating that he or she only partially meets expectations, then the individual component of the bonus is paid out at 50%; if an executive officer receives an unsatisfactory performance rating, then the individual component of the bonus is not paid out to that officer. Subject to the rights contained in any agreement between the Company and the executive officer, an executive officer must be employed by the Company on the bonus payment date to be eligible to receive a bonus payment.

The individual goals for each executive officer, other than the Chief Executive Officer, are adopted at the beginning of each performance year by the Chief Executive Officer and communicated to each executive officer. The Compensation Committee considers the individual performance of each executive officer (other than our Chief Executive Officer) and our overall corporate 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. All executive officers are assigned annual bonus targets,

expressed as a percent of base salary, based on each executive officer's accountability, scope of responsibilities and potential impact on performance, as well as peer group competitive data for similarly situated positions. The annual bonus is weighted 75% for corporate performance and 25% for individual performance. The annual bonus for the Chief Executive Officer is weighted 100% for corporate performance. With respect to the corporate component of the bonus, the maximum payout is 150% of target. With respect to the individual component of the bonus, the maximum payout is 120% of target. Actual payouts are based upon achievement with respect to established goals (for corporate performance) and individual performance rating (for individual performance).

**Table of Contents***Fiscal 2017 Bonuses*

Annual corporate goals for fiscal 2017 were proposed by our executive officers and approved by our Compensation Committee in early 2017. Individual objectives for our executive officers for 2017 were proposed by each executive officer, with review, input and confirmation from our Chief Executive Officer.

For 2017, our Compensation Committee set the annual targets for our executive officers' bonuses as a percent of base salary generally targeting the 50th percentile of our 2017 peer group, which resulted in no change to the targets from 2016. The target bonuses, as a percentage of base salary, for the named executive officers for fiscal 2017 are set forth in the following table.

<b>Name</b>	<b>Title</b>	<b>Target Bonus for Fiscal 2017 (% of Base Salary)</b>
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	60%
Shalini Sharp	Chief Financial Officer and Executive Vice President	40%
Jayson Dallas, M.D.	Chief Commercial Officer and Executive Vice President	40%
Thomas Kassberg	Chief Business Officer and Executive Vice President	40%
John R. Pinion II	Chief Quality Officer and Executive Vice President of Translational Sciences	40%

In December 2017, the Compensation Committee assessed our overall 2017 performance against the achievement of the corporate goals to determine a total percentage of achievement between 0% and 150%. The Compensation Committee considered the following general performance goals in assessing overall performance for the 2017 fiscal year:

**Preclinical and Clinical Development Goals (90% weighting):** These goals established target performance in our preclinical pipeline, as well as in each of our clinical programs, with a focus on moving product candidates through the regulatory review and approval process and preparing for commercialization.

**Corporate Development Goals (10% weighting):** These goals set targets for enhancing corporate infrastructure to support global commercialization launch preparation efforts and talent, increasing our preclinical or clinical pipeline, and demonstrating fiscal responsibility.

In establishing these goals, the Compensation Committee selected performance goals that were considered aggressive, meaning that they are goals that were considered achievable, but only with a high degree of diligence and success in execution.

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. The Compensation Committee then

evaluated the relative importance of the weightings of each goal. After such review and evaluation was complete, the Compensation Committee determined that some goals were not fully realized, and other goals were achieved at levels greater than 100%. The Compensation Committee then determined an overall percentage of achievement for all goals combined, which for fiscal year 2017 was 100%.

In December 2017, in addition to assessing the foregoing corporate goals, the Compensation Committee assessed our Chief Executive Officer's individual accomplishments, as well as the individual accomplishments of

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other executive officers. The individual achievements for Ms. Sharp, Mr. Kassberg, Dr. Dallas, and Mr. Pinion are summarized below:

The individual performance achievements for Shalini Sharp, our Chief Financial Officer and Executive Vice President, for fiscal 2017 included implementing an at-the-market registered offering; managing our tax structure; and assisting with the acquisition of gene therapy clinical and preclinical programs.

The individual performance achievements for Jayson Dallas, our Chief Commercial Officer and Executive Vice President, for fiscal 2017 included continuing to build the global commercial function with the recruitment of senior commercial leadership; and developing a patient services hub.

The individual performance achievements for Thomas Kassberg, our Chief Business Officer and Executive Vice President, for fiscal 2017 included developing and implementing an alliance management function and a facilities organizational plan; and acquiring gene therapy clinical and preclinical programs.

The individual performance achievements for John Pinion, our Chief Quality Officer and Executive Vice President of Translational Sciences, for fiscal 2017 included accepting the added responsibilities for translational sciences, including pharmacology, toxicology and research; advancing implementation of a scalable, flexible, and right-sized quality system and IT infrastructure; successfully attracting and retaining key contributors; and successfully managing multiple regulatory inspections.

Dr. Kakkis evaluated the performance of Ms. Sharp, Dr. Dallas, Mr. Kassberg, and Mr. Pinion after considering the above achievements and provided a proposed bonus amount for each such officer to the Compensation Committee in light of such officer's achievements during 2017.

*Achievement of Goals and Relationship to Compensation Awarded*

The overall 100% achievement score for the 2017 corporate goals, combined with Dr. Kakkis' assessment of the individual performance and achievement of Ms. Sharp, Dr. Dallas, Mr. Kassberg, and Mr. Pinion during fiscal 2017, resulted in the Compensation Committee approving bonus awards for performance in 2017 as set forth in the following table:

<b>Name</b>	<b>Title</b>	<b>Total Fiscal 2017 Bonus</b>	<b>Corporate Component Score (Weighting 75%; 100% for CEO)</b>	<b>Individual Component Score (Weighting 25%)</b>
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Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	\$ 393,000	100%	N/A
Shalini Sharp	Chief Financial Officer and Executive Vice President	\$ 188,315	100%	110%
Jayson Dallas, M.D.	Chief Commercial Officer and Executive Vice President	\$ 180,389	100%	100%
Thomas Kassberg	Chief Business Officer and Executive Vice President	\$ 168,600	100%	100%
John R. Pinion II	Chief Quality Officer and Executive Vice President of Translational Sciences	\$ 170,459	100%	110%

The bonuses awarded under our 2017 annual incentive program were paid in March 2018.



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### ***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 units. The Compensation Committee grants stock options and restricted stock units to executive officers to recognize their contributions to the achievement of corporate objectives, to align their interests with those of our stockholders by creating value tied to the performance of our stock price and for retention purposes. In determining the form 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 our overall performance relative to corporate objectives.

Under the terms of our 2014 Incentive Plan, pursuant to which all equity grants are currently made, the exercise price of any stock options awarded 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 before 2017 we have made annual grants to existing officers and employees in or around May of each year and to new hires on a fixed schedule within one month of the commencement of their employment. In 2017, we began making annual equity grants in or around March of each year to allow management and the Compensation Committee to review all elements of compensation at the same point in the year. We also do not have any program, plan or practice to time award dates of 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 special retention or performance objectives.

Authority to make equity grants to executive officers rests with the Compensation Committee. Recommendations for equity grant guidelines are made by Radford based on grant values for similarly situated executive positions in our peer group companies and accounting for dilution constraints. Our CEO recommends grants for individual executives within those guidelines. The Compensation Committee then reviews and considers our CEO's recommendation and approves the final grant amounts.

We believe that annual equity awards serve as a useful performance recognition mechanism. Our typical option awards to executive officers (including our 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 our 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 annual equity awards encourage the retention of executive officers and maintain their focus on our long-term performance, as well as on the achievement of specific performance goals.

In addition to the new hire and annual equity awards described above, the Compensation Committee considers grants of other equity awards, as needed, to align business strategy with our compensation practices.

#### *Equity Grants in Fiscal 2017*

In March 2017, the Compensation Committee approved annual equity awards for our executive officers and employees. These annual equity awards are awarded as a mix of stock options and restricted stock units at a 75%/25%

ratio. For the 2017 fiscal year, the Compensation Committee reviewed proposed annual equity awards for our named executive officers at both the 50<sup>th</sup> percentile and the 75<sup>th</sup> percentile of our 2017 peer group, based on grant value. In recognition of corporate and individual performance, our Chief Executive Officer recommended equity grants at the high end of the range based on recommendations from Radford. Radford s

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recommendations, on which our equity guidelines are based, utilize market data regarding long-term incentives and equity compensation from our 2017 peer group.

The table below sets forth all annual equity awards granted in fiscal 2017 to our named executive officers. No new hire equity awards were granted to our executive officers in fiscal 2017.

<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.	March 1, 2017	78,000	13,000
Shalini Sharp	March 1, 2017	28,000	4,000
Jayson Dallas, M.D.	March 1, 2017	30,000	4,500
Thomas Kassberg	March 1, 2017	25,000	3,000
John R. Pinion II	March 1, 2017	18,000	2,700

**Long-Term Performance Award**

In December 2017, the Compensation Committee approved performance stock unit awards for all employees that were eligible to receive equity awards, including our named executive officers. The Compensation Committee approved these performance stock unit awards, after consulting with Radford, in order to directly link the growth expectations for the Company to rewards with a focus on sustainable operating performance.

The performance metrics applicable to the performance stock unit awards are:

Approval and launch in the US of a broad label (for 1 year old and up and no disease restrictions) for Crysvida in 2018;

New drug application (NDA) or marketing authorization application (MAA) submission or a new commercial product in a major market, in either case, by the end of 2019; and

First 12 months of US commercial net sales of Crysvida following approval and launch exceeding forecast by 35% (to be achieved by no later than the end of 2019).

Once the achievement of a performance metric is certified by the Compensation Committee, 33.3% of the performance stock unit awards will convert into time-based restricted stock units, with half of these awards vesting on the first anniversary of the certification date and the other half vesting on the second anniversary of the certification.

The table below sets forth the performance stock units granted in fiscal 2017 to our named executive officers.

<b>Name</b>	<b>Date of Grant</b>	<b>Number of Performance Stock Units</b>
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Emil D. Kakkis, M.D., Ph.D.	December 22, 2017	10,300
Shalini Sharp	December 22, 2017	4,300
Jayson Dallas, M.D.	December 22, 2017	4,300
Thomas Kassberg	December 22, 2017	4,300
John R. Pinion II	December 22, 2017	4,300

The values of the equity grants awarded to executive officers for the 2017 fiscal year, as well as all compensation actions taken with respect to the named executive officers in fiscal 2017, are reflected in the Summary Compensation Table.

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***Changes for Fiscal 2018***

*Say-on-Pay*

At our 2017 Annual Meeting, approximately 84% of the votes cast by our stockholders approved the compensation paid to our named executive officers as described in our proxy statement filed with the SEC in connection with that meeting. Our Board and the Compensation Committee reviewed the results of this vote and the evaluation of our compensation practices by third parties in connection with assessing the compensation of our executive officers for 2018. Based on this review, the Compensation Committee implemented changes to its compensation practices, adopted a clawback policy (as described below), and made a supplemental grant of a long-term performance award (as described above). In accordance with the preference expressed by our stockholders to conduct an advisory vote on executive compensation every year, the next advisory vote after the Annual Meeting will occur as part of our 2019 Annual Meeting.

*Peer Group*

The Compensation Committee reviews our list of peer companies annually to determine if revisions are needed to reflect changes in our development status and in market capitalization, developments relative to our peer companies and other factors. The Compensation Committee engaged Radford to assist in reviewing our 2017 peer group and in suggesting revisions, as appropriate.

Based on Radford's assessment and recommendations, the Compensation Committee selected 22 publicly traded companies in the pharmaceutical and biotechnology industries to serve as our new list of peer companies for 2018, referred to as our 2018 peer group, by balancing the following criteria:

companies with drugs in late-stage development, as well as recently commercial companies;

companies with comparable market capitalizations (i.e., in the range of \$750 million to \$7.5 billion); and

companies with headcounts between 150 and 800.

Our 2018 peer group is comprised of the following 22 companies in the pharmaceutical and biotechnology industries:

ACADIA Pharmaceuticals Inc.	Intercept Pharmaceuticals, Inc.	Radius Health
Agios Pharmaceuticals, Inc.	Ionis Pharmaceuticals, Inc.	Sage Therapeutics
Alnylam Pharmaceuticals, Inc.	Juno Therapeutics, Inc.	Sarepta Therapeutics, Inc.
bluebird bio, Inc.	Kite Pharma, Inc.	Spark Therapeutics, Inc.
Clovis Oncology, Inc.	Neurocrine Biosciences, Inc.	TESARO, Inc.
Exelixis Inc.	Portola Pharmaceuticals, Inc.	Theravance Biopharma, Inc.

FibroGen, Inc.

Prothena Corporation Plc

Halozyme Therapeutics, Inc.

Puma Biotechnology, Inc.

We believe that the compensation practices of our 2018 peer group provided us with appropriate compensation benchmarks for evaluating the compensation of our named executive officers for 2018.

*Base Salaries*

For 2018, increases in base salaries for our named executive officers were approximately 3% annualized. Increases in base salaries for 2018 were a result of standard annual merit increases based on the

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achievement of corporate and individual performance goals and objectives during 2017, as well as Radford peer group data regarding 2018 salary increases.

### *Annual Bonuses*

For 2018, the Compensation Committee determined to maintain the targets for 2018 bonuses at the 2017 levels for all executive officers.

### *Equity Compensation*

We expect to make our annual equity grants in or around March of each year to allow management and the Compensation Committee to review all elements of compensation at one point in the year. After reviewing equity compensation for 2018, the Compensation Committee determined that target equity compensation for our named executive officers should be positioned between the 50<sup>th</sup> and 75<sup>th</sup> percentile of our peer group. In keeping with this strategy, 2018 equity grants to our executive officers were generally at the 50<sup>th</sup> percentile of our 2018 peer group.

### *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. Reliable and competitive health, welfare and vacation benefits ensure that we have a productive and focused workforce.

The retirement savings plan for which our named executive officers are eligible (401(k) Plan) is a tax-qualified retirement savings plan pursuant to which the named executive officers can 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 eligible compensation 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. All of our employees, except for those employees that participated in the 401(k) plan of Dimension Therapeutics, Inc. prior to our acquisition of Dimension, are eligible to participate in the 401(k) Plan on the same terms as the named executive officers. Employees that participated in the Dimension 401(k) plan prior to the acquisition can continue to participate in that plan, which we assumed.

### *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. For tax years beginning prior to December 31, 2017, there was an exception to this deduction limitation for qualified performance-based compensation as defined under the Section 162(m) rules then in effect. Beginning with 2018, this exception was repealed and the list of executive officers covered by Section 162(m) has been expanded. 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.



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### ***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 2017, the largest portion of compensation to each of our named executive officers was in the form of equity compensation.

### ***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 March (provided that the named executive officer must have been employed by September 30 to receive an annual equity grant).

### ***Clawback Policy***

In December 2017, the Board adopted a Clawback Policy, which permits us to recover up to 100% of any performance-based cash and equity compensation that we pay to our current or former executive officers during the one-year period preceding the date on which we are required to prepare an accounting restatement if the Board determines that an act or omission of such executive officers contributed to the circumstances requiring the restatement and such act or omission involved fraud or intentional misconduct by the officer.

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

The Compensation Committee adopted stock ownership guidelines in order to align the long-term interests of our executive officers and directors with those of our stockholders. The guidelines require holding our common stock with value equivalent to 3x the annual retainer for Board members, 3x the base salary for our Chief Executive Officer and 1x the base salary for the other named executive officers. These guidelines are to be achieved by the end of 2021 for current named executive officers and Board members and within five years of appointment for newly appointed named executive officers and Board members.

There are no minimum stock ownership guidelines for our non-named executive officers or employees, although non-named executive officers 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, reviewed, and updated 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 2017, the Compensation Committee also considered how our compensation policies may affect our risk profile and how compensation policies may be used to mitigate risks facing us. More specifically, the Compensation Committee considered the general design philosophy of our 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 us 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 our overall performance, which reduces the ability and incentive for

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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 2017, our corporate goals were reviewed and approved by the Compensation Committee in early 2017 and are considered to be generally of the nature that would not encourage or reward excessive risk taking. Additionally, the Compensation Committee monitors our performance throughout the year and has the ability to intervene in instances where our actions vis-à-vis our performance goal attainment would be considered unduly risky, so that the Compensation Committee may act 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 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 and in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017.

Submitted by the Compensation Committee of the Board of Directors

William Aliski, Chairman

Clay B. Siegall, Ph.D.

Daniel G. Welch

**Summary Compensation Table**

The following table sets forth the compensation earned during the years ended December 31, 2017, 2016, and 2015 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 <sup>(1)</sup>	Stock Awards <sup>(2)</sup>	Option Awards <sup>(3)</sup>	Non-Equity	All	Total
						Incentive Plan Compensation <sup>(4)</sup>	Other Compensation <sup>(5)</sup>	
Emil D. Kakkis, M.D., Ph.D. <i>President and Chief Executive Officer</i>	2017	\$ 638,115	\$	\$ 1,319,303	\$ 4,295,234	\$ 393,000	\$ 39,247	\$ 6,684,899
	2016	\$ 567,200	\$	\$ 755,099	\$ 2,722,360	\$ 340,320	\$ 38,383	\$ 4,423,362
	2015	\$ 535,000	\$	\$ 967,746	\$ 3,578,620	\$ 280,875	\$ 38,419	\$ 5,400,660
Shalini Sharp, <i>Chief Financial Officer and Executive Vice President</i>	2017	\$ 455,785	\$	\$ 424,043	\$ 1,541,879	\$ 188,315	\$ 32,697	\$ 2,642,719
	2016	\$ 441,000	\$	\$ 282,280	\$ 1,025,693	\$ 185,220	\$ 32,218	\$ 1,966,411
	2015	\$ 420,000	\$	\$ 373,516	\$ 1,378,004	\$ 182,700	\$ 28,082	\$ 2,382,302
Jayson Dallas, M.D. <sup>(6)</sup> <i>Chief Commercial</i>	2017	\$ 447,517	\$ 50,000	\$ 468,443	\$ 1,652,013	\$ 180,389	\$ 36,345	\$ 2,834,707
	2016	\$ 433,000	\$ 50,000	\$ 239,938	\$ 1,324,864	\$ 181,860	\$ 32,403	\$ 2,262,065

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<i>Officer and Executive Vice President</i>	2015	\$ 164,279	\$ 100,000	\$ 1,164,500	\$ 6,266,173	\$ 74,724	\$ 40,603	\$ 7,810,279
Thomas Kassberg	2017	\$ 418,443	\$	\$ 335,243	\$ 1,376,678	\$ 168,600	\$ 32,697	\$ 2,331,661
<i>Chief Business Officers and Executive Vice President</i>	2016	\$ 405,600	\$	\$ 282,280	\$ 1,025,693	\$ 166,296	\$ 35,561	\$ 1,915,430
	2015	\$ 390,000	\$	\$ 373,516	\$ 1,378,004	\$ 165,750	\$ 30,996	\$ 2,338,266
John R. Pinion II <sup>(6)</sup>	2017	\$ 412,568	\$	\$ 308,603	\$ 991,208	\$ 170,459	\$ 36,491	\$ 1,919,329
<i>Chief Quality Officer and Executive Vice President of Translational Sciences</i>	2016	\$ 399,185	\$	\$ 211,710	\$ 1,222,295	\$ 167,658	\$ 34,614	\$ 2,035,462
	2015	\$ 171,432	\$	\$ 936,525	\$ 6,731,608	\$ 79,945	\$ 10,681	\$ 7,930,191

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- (1) The amounts reported in this column represent retention or sign-on bonuses. For fiscal 2017, the reported amount was a retention bonus for Dr. Dallas. Please see Narrative Disclosure to Summary Compensation Table Employment Arrangements with Our Named Executive Officers.
- (2) The amounts reported in this column for a fiscal year represent the grant date fair value of the restricted stock units and performance stock units granted to our named executive officers during the fiscal year, 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 restricted stock units and performance stock units reported in this column are set forth in the notes to our financial statements included in our Annual Report. Note that the amounts reported in this column reflect the accounting cost for these restricted stock units and performance 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 and the performance stock units. The value of the performance stock units reported in this column, assuming that the highest level of performance conditions will be achieved, is as follows: Dr. Kakkis \$494,709; Ms. Sharp \$206,529; Dr. Dallas \$206,529; Mr. Kassberg \$206,529; and Mr. Pinion \$206,529.
- (3) The amounts reported in this column for a fiscal year represent the grant date fair value of the stock options granted to our named executive officers during the fiscal year, 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. 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.
- (4) Amounts for a fiscal year represent cash bonuses earned in that fiscal year and paid in the subsequent fiscal year based on achievement of performance goals and other factors deemed relevant by our Compensation Committee.
- (5) Amounts reported in this column for the 2017 fiscal year reflect the following payments to our named executive officers: Dr. Kakkis \$8,100 for 401(k) matching contributions, \$3,375 for health savings account contributions, \$21,222 for medical, dental, vision and life/accidental death & dismemberment premiums and \$6,550 for key person life insurance premiums paid by us; Ms. Sharp \$8,100 for 401(k) matching contributions, \$3,375 for health savings account contributions and \$21,222 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us; Dr. Dallas \$7,954 for 401(k) matching contributions and \$28,391 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us; Mr. Kassberg \$8,100 for 401(k) matching contributions, \$3,375 for health savings account contributions and \$21,222 for life/accidental death & dismemberment premiums paid by us; and Mr. Pinion \$8,100 for 401(k) matching contributions and \$28,391 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us.
- (6) Dr. Dallas commenced employment with us in August 2015, and Mr. Pinion commenced employment with us in July 2015.

***Narrative Disclosure to Summary Compensation Table***

*Employment Arrangements with Our Named Executive Officers*

Dr. Kakkis, our Chief Executive Officer, is party to an employment agreement with us that provides for base salary. Dr. Kakkis is also eligible for an annual bonus opportunity and to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the 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.

Each of our other named executive officers is party to an offer letter with us that provides for base salary, an annual bonus opportunity and an initial grant of equity. They are eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letters, their employment is at will and may be terminated either by us or by them, with or without advance notice, for any reason or for no reason at all. The offer letter for Dr. Dallas provided for

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reimbursement of relocation expenses, a sign-on bonus and a retention bonus of \$100,000, payable in two installments, subject to continued employment. The first installment of the retention bonus was paid in 2016, and the second installment of \$50,000 was paid in August 2017.

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;

if Dr. Kakkis resigns his employment with us for good reason following a change in control (as defined below), then he shall be entitled to receive 24 months of base salary continuation; provided, however, that Dr. Kakkis must first provide us written notice of the good reason within six months of the event constituting good reason and a period of 20 days to cure the good reason and we must fail to cure the good reason during such 20-day period; and

if Ms. Sharp, Dr. Dallas, Mr. Kassberg or Mr. Pinion is terminated by us without cause or resigns employment with us due to a constructive termination, each executive will be entitled to: (i) an extended 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 letters with Ms. Sharp and Mr. Kassberg, as amended, and the offer letters with Dr. Dallas and Mr. Pinion, 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 awards shall accelerate with respect to 100% of the then-unvested awards.

*Definitions*

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

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



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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, Dr. Dallas, Mr. Kassberg and Mr. Pinion, **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;

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' 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' 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, Dr. Dallas, Mr. Kassberg, and Mr. Pinion, "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 or

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condition within 30 days of receiving such written objection, and (iii) the executive resigns his or her 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.

**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 2017.

Name	Grant Date	Estimated Future Payouts		Equity Incentive Plan Awards Target (#)(3)	Stock Awards:		Exercise Price of Option Awards (\$/Share)	Grant Date Fair Value of Stock and Option Awards (\$)(5)
		Under Target (\$)(1)	Non-Equity Plan Awards Maximum (\$)(2)		Number of Shares of Stock or Units Granted (#)(4)	Option Awards: Number of Securities Underlying Options Granted (#)		
Emil D. Kakkis, M.D., Ph.D.	3/1/2017	393,000	589,500			78,000	88.80	4,295,234
	3/1/2017				13,000			1,154,400
	12/22/2017			10,300				164,903
Shalini Sharp	3/1/2017	183,722	261,804			28,000	88.80	1,541,879
	3/1/2017				4,000			355,200
	12/22/2017			4,300				68,843
		180,389	257,055					

Jayson Dallas, M.D.	3/1/2017				30,000	88.80	1,652,013
	3/1/2017			4,500			399,600
	12/22/2017			4,300			68,843
Thomas Kassberg		168,600	240,255				
	3/1/2017				25,000	88.80	1,376,678
	3/1/2017				3,000		266,400
	12/22/2017			4,300			68,843
John R. Pinion II		166,302	236,980				
	3/1/2017				18,000	88.80	991,208
	3/1/2017				2,700		239,760
	12/22/2017			4,300			68,843

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- (1) The amount represents the target amount of each named executive officer's cash payments under our 2017 annual incentive program as established by the Board and described in Compensation Discussion and Analysis above. Actual payments made for fiscal 2017 are provided in the Summary Compensation Table.
- (2) The amount represents the maximum amount of each named executive officer's cash payments under our 2017 annual incentive program as established by the Board and described in Compensation Discussion and Analysis above. Actual payments made for fiscal 2017 are provided in the Summary Compensation Table.
- (3) The amounts represent the performance stock units granted under our 2014 Incentive Plan.
- (4) The amounts represent the restricted stock units granted under our 2014 Incentive Plan.
- (5) This column reflects the aggregate grant date fair value of equity awards granted in 2017 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 and option awards reported in this column are set forth in the notes to our financial statements included in our Annual Report.

Table of Contents**Outstanding Equity Awards at December 31, 2017**

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2017.

Name	Grant Date	Option Awards				Stock Awards			Equity Incentive Plan Awards:
		Number of Securities Underlying Unexercised Options (#)	Number of Securities Underlying Unexercised Options (#)	Option Exercise Price (\$)	Option Expiration Date(1)	Number of Shares or Units of Stock That Have Not Vested (#)(2)	Market Value of Shares or Units of Stock That Have Not Vested (\$)	Equity Incentive Plan Awards: Number of Shares, Units or Other Rights That Have Not Vested (#)(3)	Market or Payout Value of Unearned Shares, Units or Other Rights That Have Not Vested (\$)
Emil D. Kakkis, M.D., Ph.D.	12/22/17							10,300	477,714
	3/1/17		78,000	88.80	3/1/27	13,000	602,940		
	6/1/16	23,887	39,813	70.57	6/1/26	8,025	372,200		
	5/21/15	44,110	24,190	84.89	5/21/25	5,700	264,366		
	11/1/13	47,853		6.86	11/1/23				
Shalini Sharp	12/22/17							4,300	199,434
	3/1/17		28,000	88.80	3/1/27	4,000	185,520		
	6/1/16	9,000	15,000	70.57	6/1/26	3,000	139,140		
	5/21/15	618	2,354	84.89	5/21/25	2,200	102,036		
	11/1/13	36,880		6.86	11/1/23				
	8/2/12	32,163		0.81	8/2/22				
Jayson Dallas, M.D.	12/22/17							4,300	199,434
	3/1/17		30,000	88.80	3/1/27	4,500	208,710		
	6/3/16	4,125	6,875	69.53	6/3/26				
	6/1/16	7,575	12,625	70.57	6/1/26	2,550	118,269		
	8/4/15	52,500	37,500	116.45	8/4/25	5,000	231,900		
Thomas Kassberg	12/22/17							4,300	199,434
	3/1/17		25,000	88.80	3/1/27	3,000	139,140		
	6/1/16	9,000	15,000	70.57	6/1/26	3,000	139,140		
	5/21/15	16,985	9,315	84.89	5/21/25	2,200	102,036		

	11/1/13	39,878		6.86	11/1/23		
	11/17/11	109,734		0.31	11/17/21		
John R. Pinion II	12/22/17					4,300	199,434
	3/1/17		18,000	88.80	3/1/27	2,700	125,226
	6/3/16	4,125	6,875	69.53	6/3/26		
	6/1/16	6,675	11,125	70.57	6/1/26	2,250	104,355
	7/16/15	54,375	35,625	124.87	8/4/25	3,750	173,925

- (1) The option vests with respect to 1/4<sup>th</sup> of the shares underlying the option on the one-year anniversary of the grant date, and with respect to 1/48<sup>th</sup> of the shares underlying the option, on each monthly anniversary thereafter, subject to the holder's continued service to us through each such vesting date. Please see the section entitled [Narrative Disclosure to Summary Compensation Table Covered Transaction](#) for accelerated vesting provisions that apply on certain terminations of employment.
- (2) The restricted stock units vest with respect to 1/4<sup>th</sup> of the underlying shares on each anniversary of the grant date over a four-year period. Please see the section entitled [Narrative Disclosure to Summary Compensation Table Covered Transaction](#) for accelerated vesting provisions that apply on certain terminations of employment.
- (3) The performance stock units vest as described above under [Compensation Discussion and Analysis Equity Compensation Equity Grants in Fiscal 2017 Long-Term Performance Award](#). Please see the section entitled

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Narrative Disclosure to Summary Compensation Table Covered Transaction for accelerated vesting provisions that apply on certain terminations of employment.

**Option Exercises and Stock Vested**

The following table sets forth certain information concerning the option awards exercised and stock awards vested for our named executive officers during fiscal 2017.

Name and Principal Position	Option Awards		Stock Awards	
	Number of Shares Acquired on Exercise (#)	Value Realized on Exercise (\$)(1)	Number of Shares Acquired on Vesting (#)	Value Realized on Vesting (\$)(2)
Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer			5,525	315,222
Shalini Sharp, Chief Financial Officer and Executive Vice President	24,000	2,027,881	2,100	119,827
Jayson Dallas, M.D., Chief Commercial Officer and Executive Vice President			3,350	204,594
Thomas Kassberg, Chief Business Officer and Executive Vice President			2,100	119,827
John R. Pinion II, Chief Quality Officer and Executive Vice President of Translational Sciences			2,625	163,133

(1) Value realized 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 on each exercise date.

(2) Value realized is equal to the closing price of our common stock on The NASDAQ Global Select Market on each vesting date multiplied by the number of stock awards that vested.

**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 2017.

**Nonqualified Deferred Compensation**

During fiscal 2017, 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.



**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, restricted stock unit, and performance 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 29, 2017. The closing price of our common stock on The NASDAQ Global Select Market as of

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December 29, 2017, the last trading day of 2017, was \$46.38, which was used as the value of our common 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 29, 2017 by the difference between the closing price of our common stock as of December 29, 2017 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 29, 2017. The value of the restricted stock unit and performance stock unit vesting acceleration was calculated by multiplying the number of unvested restricted stock units and performance stock units subject to vesting acceleration as of December 29, 2017 by the closing price of our common stock as of December 29, 2017.

**Dr. Emil Kakkis**

The following table describes the potential payments upon employment termination for Emil Kakkis, our President and Chief Executive Officer, as if his employment terminated as of December 29, 2017, 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>Resignation for Good Reason following a Change in Control</b>	<b>Termination by Company without Cause following a Covered Transaction</b>	<b>Resignation due to a Constructive Termination following a Covered Transaction</b>
Base Salary	\$ 1,310,000	\$ 1,310,000	\$ 1,310,000	\$ 1,310,000	
Acceleration of equity awards				1,717,220	1,717,220
<b>Total</b>	<b>\$ 1,310,000</b>	<b>\$ 1,310,000</b>	<b>\$ 1,310,000</b>	<b>\$ 3,027,220</b>	<b>\$ 1,717,220</b>

**Ms. Shalini Sharp**

The following table describes the potential payments upon employment termination for Shalini Sharp, our Chief Financial Officer and Executive Vice President, as if her employment terminated as of December 29, 2017, 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>Resignation due to a Constructive Termination</b>	<b>Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction</b>
Base Salary	\$ 459,305	\$ 459,305	\$ 459,305
Acceleration of equity awards			626,130

<b>Total</b>	\$ 459,305	\$ 459,305	\$ 1,085,435
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**Table of Contents*****Dr. Jayson Dallas***

The following table describes the potential payments upon employment termination for Jayson Dallas, our Chief Commercial Officer and Executive Vice President, as if his employment terminated as of December 29, 2017, the last business day of the fiscal year.

<b>Potential Payments Upon</b>			<b>Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction</b>
<b>Termination or Change of Control</b>	<b>Termination by Company without Cause</b>	<b>Resignation due to a Constructive Termination</b>	
Base Salary	\$ 450,973	\$ 450,973	\$ 450,973
Acceleration of equity awards			\$ 758,313
<b>Total</b>	<b>\$ 450,973</b>	<b>\$ 450,973</b>	<b>\$ 1,209,286</b>

***Mr. Thomas Kassberg***

The following table describes the potential payments upon employment termination for Thomas Kassberg, our Chief Business Officer and Executive Vice President, as if his employment terminated as of December 29, 2017, the last business day of the fiscal year.

<b>Potential Payments Upon</b>			<b>Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction</b>
<b>Termination or Change of Control</b>	<b>Termination by Company without Cause</b>	<b>Resignation due to a Constructive Termination</b>	
Base Salary	\$ 421,500	\$ 421,500	\$ 421,500
Acceleration of equity awards			\$ 579,750
<b>Total</b>	<b>\$ 421,500</b>	<b>\$ 421,500</b>	<b>\$ 1,001,250</b>

***Mr. John Pinion***

The following table describes the potential payments upon employment termination for John Pinion, our Chief Quality Officer and Executive Vice President of Translational Sciences, as if his employment terminated as of December 29, 2017, the last business day of the fiscal year.

<b>Potential Payments Upon</b>			<b>Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction</b>
<b>Termination or Change of Control</b>	<b>Termination by Company without Cause</b>	<b>Resignation due to a Constructive Termination</b>	
Base Salary	\$ 415,754	\$ 415,754	\$ 415,754
Acceleration of equity awards			\$ 602,940
<b>Total</b>	\$ 415,754	\$ 415,754	\$ 1,018,694

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

The table below discloses information as of December 31, 2017 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(a)</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>
Equity compensation plans approved by security holders:			
2011 Equity Incentive Plan, as amended	711,577	\$ 6.29	
2014 Incentive Plan	5,586,810	\$ 75.28	1,070,108
2014 Employee Stock Purchase Plan			1,782,441
Equity compensation plans not approved by security holders <sup>(1)</sup>			
Dimension Therapeutics, Inc. 2015 Stock			
Option and Incentive Plan	269,641	\$ 37.09	
Dimension Therapeutics, Inc. 2013 Stock Plan	161,792	\$ 24.21	
<b>Total</b>	<b>6,729,820</b>	<b>\$ 62.75</b>	<b>2,852,549</b>

- (1) In connection with our acquisition of Dimension Therapeutics, Inc. on November 7, 2017, we assumed these plans and outstanding option awards thereunder (whether or not then vested or exercisable). The assumed awards continue to have, and are subject to, the same terms and conditions as were applicable prior to the acquisition as set forth in the applicable plan (including any applicable award agreement, other agreement or other document evidencing such awards), except that the awards are exercisable for shares of our common stock with exercise prices adjusted to reflect the terms of the acquisition, all as set forth in the merger agreement. No new awards can be made under these plans.

**DIRECTOR COMPENSATION**

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 Board of Director Compensation Review prepared by Radford in November 2016 provided a competitive assessment of our compensation practices for non-employee directors in connection with the Compensation Committee's evaluation of the level of compensation for our non-employee directors for 2017. The assessment found that the cash compensation levels for non-employee directors was below the 50<sup>th</sup> percentile as compared to our 2017 peer group and that our equity compensation levels for non-employee directors was positioned between the 50<sup>th</sup> and 75<sup>th</sup> percentiles as

compared to our 2017 peer group. In order to better align director compensation with that of our executive officers for the 2017 fiscal year, the Compensation Committee increased cash compensation to align with the 50<sup>th</sup> percentile of our 2017 peer group. For equity compensation, the Compensation Committee made no change to the mix of options and restricted stock units for annual grants and retained the use of options for appointment awards, which continues to keep the equity compensation between the 50<sup>th</sup> and 75<sup>th</sup> percentiles of our 2017 peer group.

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A summary of the non-employee director compensation arrangements for fiscal 2017 is set forth below:

	<b>Annual Retainer</b>
<b>Board of Directors:</b>	
Chairman	\$ 80,000
Non-Chairman members	\$ 50,000
<b>Audit Committee:</b>	
Chairman	\$ 20,000
Non-Chairman members	\$ 10,000
<b>Compensation Committee:</b>	
Chairman	\$ 15,000
Non-Chairman members	\$ 7,500
<b>Nominating and Corporate Governance Committee:</b>	
Chairman	\$ 10,000
Non-Chairman members	\$ 5,000
<b>Research and Development Committee:</b>	
Chairman	\$ 12,000
Non-Chairman members	\$ 6,000

Under the non-employee director compensation policy for fiscal 2017, each non-employee director who was initially appointed or elected to the Board received an option grant to purchase up to 15,000 shares of our common stock under our stock option plan on the date he or she first become 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 was eligible to receive an annual option grant to purchase up to 3,750 shares of our common stock and an annual grant of 1,875 restricted stock units, each of which will vest in full upon the earlier of (a) our 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 and restricted stock units were granted at fair market value as of the date of grant.

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

The following table shows the compensation earned in fiscal 2017 by our non-employee directors.

Name	Fees Earned in			Total
	Fiscal 2017	Stock Awards <sup>(1)</sup>	Option Awards <sup>(2)</sup>	
Daniel G. Welch	\$ 92,500	\$ 123,113	\$ 142,961	\$ 358,574
William Aliski	75,000	123,113	142,961	341,074
Deborah Dunsire, M.D. <sup>(3)</sup>	42,000	123,113	654,521	819,634
Lars Ekman, M.D., Ph.D.	62,000	123,113	142,961	328,074
Matthew K. Fust	80,000	123,113	142,961	346,074
Michael Narachi	65,000	123,113	142,961	331,074
Clay B. Siegall, Ph.D.	63,500	123,113	142,961	329,574



- (1) The amounts reported in this column for a fiscal year represent the grant date fair value of the restricted stock units granted to our non-employee directors during the fiscal year, 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 restricted stock units reported in this column are set forth in the notes to our financial statements included in our Annual Report. Note that the amounts reported in this column reflect the

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- 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. As of December 31, 2017, our non-employee directors had the following outstanding restricted stock units: Mr. Welch 1,875; Mr. Aliski 1,875; Dr. Dunsire 1,875; Dr. Ekman 1,875; Mr. Fust 1,875; Mr. Narachi 1,875; and Dr. Siegall 1,875.
- (2) The amounts reported in this column represent the grant date fair value of the stock options granted to our non-employee directors during fiscal 2017, 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. 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, 2017, our non-employee directors had the following outstanding options: Mr. Welch 32,500; Mr. Aliski 34,000; Dr. Dunsire 18,750; Dr. Ekman 22,500; Mr. Fust 33,681; Mr. Narachi 32,500; and Dr. Siegall 40,000.
- (3) Dr. Dunsire's appointment to the Board was effective April 6, 2017.

**CEO PAY RATIO**

We are required by SEC rules adopted under the Dodd-Frank Act to disclose the ratio of our median employee's annual total compensation to the annual total compensation of our principal executive officer. The new disclosure provides a measure of the equitability of pay within our company. We believe our compensation philosophy and process yield an equitable result for all of our employees. For 2017, the annual total compensation for Dr. Emil Kakkis, our Chief Executive Officer and President, was \$6,684,899, and for our median employee was \$297,348, resulting in a pay ratio of 22:1.

In accordance with Item 402(u) of Regulation S-K, we identified the median employee by (i) aggregating for each applicable employee (A) base salary for 2017 on the calculation date, (B) the target bonus for 2017, and (C) the accounting value of any equity awards granted during 2017, and (ii) ranking this annual compensation measure for our employees from lowest to highest. This calculation encompasses individuals, excluding our CEO, employed by us on October 1, 2017, whether employed on a full-time, part-time, or seasonal basis. For any permanent employees who were only employed for part of the 2017 fiscal year, we annualized their compensation to present a more accurate representation of their comparative annual compensation. Employees located in jurisdictions outside of the United States, or non-U.S. employees, were excluded from the calculation in accordance with the regulation's de minimis exemption for non-U.S. employees totaling up to five percent of our total employees. On October 1, 2017, we had 437 employees, 15 of which were non-U.S. employees that were excluded from the calculation and 422 of which were U.S. employees. The number of non-U.S. employees excluded from the calculation by jurisdiction is as follows: Switzerland 10; France 1; Germany 1; Italy 1; Spain 1; and Sweden 1.

The process described above led to the inclusion of an even number of employees in the calculations, which resulted in two median employees, rather than a single median employee. Because the SEC rules require that we identify a single median employee, we selected the median employee who did not take a leave of absence during the year in order to identify a more representative median employee whose compensation reflects a full year of employment.

We acquired Dimension Therapeutics, Inc. on November 7, 2017. No employees of Dimension are included in the above calculations because they were not employed by us on October 1, 2017, the date we used to identify the median employee.

**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

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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.

### **STOCKHOLDER PROPOSALS**

Stockholder proposals intended to be included in the proxy statement for the 2019 annual meeting of stockholders pursuant to SEC Rule 14a-8 must be received by our corporate Secretary no later than the close of business on December 28, 2018. In order to be considered for inclusion in our proxy statement, these proposals must satisfy the requirements of SEC Rule 14a-8.

Our 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 2019 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 our corporate Secretary between 90 and 120 days prior to the one-year anniversary date of the 2018 annual meeting (that is, between February 19, 2019 and March 21, 2019); *provided, however*, that if the 2019 annual meeting date is advanced by more than 30 days before or delayed by more than 60 days after the anniversary date of the 2018 annual meeting, then such notice must be received on or before 10 days after the day on which the date of the 2019 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 bylaws. Proposals not meeting the requirements set forth in our 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 our corporate Secretary at 60 Leveroni Court, Novato, California 94949.

### **DELIVERY OF PROXY MATERIALS**

**Our annual report to stockholders for the fiscal year ended December 31, 2017, including audited financial statements, accompanies this Proxy Statement. Copies of our Annual Report and the exhibits thereto are available from us 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](http://www.sec.gov). We 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 of our stockholders. This delivery method can result in meaningful cost savings for us. In order to take advantage of this opportunity, we 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 our 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.

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**EACH STOCKHOLDER IS URGED TO 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, BY COMPLETING, DATING, SIGNING AND RETURNING THE ENCLOSED PROXY CARD USING THE ENCLOSED RETURN ENVELOPE, AS PROMPTLY AS POSSIBLE SO THAT YOUR SHARES MAY BE REPRESENTED AT THE ANNUAL MEETING.**

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