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):

☒ No fee required.

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(1) Title of each class of securities to which transaction applies:

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(3) Per unit price or other underlying value of transaction computed pursuant to Exchange Act Rule 0-11 (set forth the amount on which the filing fee is calculated and state how it was determined):

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☐ Fee paid previously with preliminary materials.

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(1) Amount Previously Paid:

(2) Form, Schedule or Registration Statement No.:

(3) Filing Party:

(4) Date Filed:

60 Leveroni Court
Novato, California 94949

NOTICE OF ANNUAL MEETING OF STOCKHOLDERS

**To Be Held on June 26, 2020
at 8:00 a.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 26, 2020, at 8:00 a.m. Pacific Time virtually via the Internet at *www.virtualshareholdermeeting.com/RARE20* (the " ***Annual Meeting*** "). Instructions on how to participate in the Annual Meeting and demonstrate proof of stock ownership are posted at *www.virtualshareholdermeeting.com/RARE20* . The webcast of the Annual Meeting will be archived for one year after the date of the Annual Meeting at *www.virtualshareholdermeeting.com/RARE20* . Only stockholders who held stock at the close of business on the record date, April 27, 2020, 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 I 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, 2020; (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/RARE20* 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 29, 2020

WHETHER OR NOT YOU EXPECT TO ATTEND THE ANNUAL MEETING, PLEASE VOTE VIA THE INTERNET AS INSTRUCTED IN THE NOTICE OF INTERNET AVAILABILITY 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 26, 2020:**

**The Proxy Statement and Annual Report on Form 10-K for the year ended December 31, 2019 are
available at www.proxyvote.com.**

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Novato, California 94949

PROXY STATEMENT FOR
2020 ANNUAL MEETING OF STOCKHOLDERS

TO BE HELD ON JUNE 26, 2020
at 8:00 a.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 2020 Annual Meeting of Stockholders, to be held virtually via the Internet at *www.virtualshareholdermeeting.com/RARE20* on June 26, 2020, at 8:00 a.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, 2019 (the " ***Annual Report*** ") is first being mailed on or about April 29, 2020 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* 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 27, 2020 (the " ***Record Date*** "), are entitled to notice of and to vote at the Annual Meeting. On the Record Date, 59,652,532 shares of 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 cumulate votes 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 through the Internet (by going to *www.proxyvote.com* until 11:59 p.m. Eastern Time the day before the Annual Meeting) or, if you have received a paper copy of the proxy materials, over the telephone (by calling 1-800-690-6903 until 11:59 p.m. Eastern Time the day before the Annual Meeting) or by returning 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/RARE20* 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 vote before the Annual Meeting (i) in accordance with the voting instructions provided by the institution that holds their shares, which may provide for voting over the telephone or through the Internet, or (ii) by returning 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 who attend the Annual Meeting may also vote during the Annual Meeting by going to *www.virtualshareholdermeeting.com/RARE20* and following the instructions regarding voting. Brokerage firms, banks or other financial institutions that do not receive voting instructions from beneficial holders may vote these shares on behalf of the beneficial holders only with respect to "routine matters," or else return a proxy leaving these shares un-voted (a " ***broker non-vote*** "). The ratification of the selection of the independent registered public accounting firm (Proposal No. 2) is the only item on the agenda for the Annual Meeting that is considered routine. Abstentions and broker non-votes will be counted for the purpose of determining the presence or absence of a quorum. The required vote for each of the proposals expected to be acted upon at the Annual Meeting and the treatment of abstentions and broker non-votes under each proposal are described below:

Proposal No. 1 — Election of directors. Directors are elected by a plurality of the votes cast, with the three nominees obtaining the greatest number of affirmative votes being elected as directors. Broker non-votes and shares as to which a stockholder withholds voting authority are not considered votes cast and therefore will have no effect on the vote outcome. 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 must 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, in advance of the meeting, as discussed above, in order to reduce the likelihood that we will be forced to incur additional expenses soliciting proxies for the Annual Meeting.

Any registered holder of our common stock 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 the Annual Meeting virtually via the Internet to facilitate stockholder attendance and participation and have done so every year since our initial public offering. 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 for the Annual Meeting enhances stockholder access by allowing our stockholders to participate fully, and equally, from any location around the world at no cost. We believe the virtual format is the right choice for us, not only because it brings cost savings to us and our stockholders, but because it increases our ability to engage with all stockholders, regardless of size, resources, or physical location. We are aware of concerns that virtual meetings may diminish stockholder voice or reduce accountability and are taking steps to address these concerns. For example, our virtual meeting format enhances, rather than constrains, stockholder access, participation, and communication because the online format allows stockholders to communicate with us during the Annual Meeting so they can ask questions of our Board, management, and a representative from our independent registered public accounting firm. During the live Q&A session, we will answer questions as they come in, as time permits. We are committed to publishing and answering each question received following the Annual Meeting. Although the live webcast is available only to stockholders of the Record Date at the time of the Annual Meeting, the webcast of the Annual Meeting will be archived for the public for one year after the date of the Annual Meeting at www.virtualshareholdermeeting.com/RARE20.

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. Our stockholders can raise questions or concerns regarding the Company at any time by calling our Investor Relations department at (844) 280-7681 or contacting our Board by following the process described under “Corporate Governance—Stockholder Communications.”

PROPOSAL NO. 1 – ELECTION OF CLASS I 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 nine directors. The Class I directors (whose terms expire at the Annual Meeting) are Emil D. Kakkis, M.D., Ph.D., Shehnaaz Suliman, M.D., and Daniel G. Welch. The Class II directors (whose terms expire at the 2021 annual meeting of stockholders) are Deborah Dunsire, M.D., Michael Narachi, and Clay B. Siegall, Ph.D. The Class III directors (whose terms expire at the 2022 annual meeting of stockholders) are William Aliski, Lars Ekman, M.D., Ph.D., and Matthew K. Fust. The Class I directors elected at the Annual Meeting will hold office until the 2023 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, upon the recommendation of the Nominating and Corporate Governance Committee, has nominated Dr. Kakkis, Dr. Suliman and Mr. Welch for election as Class I directors at the Annual Meeting. Dr. Kakkis, Dr. Suliman and Mr. Welch have indicated their willingness to serve if elected. Should Dr. Kakkis, Dr. Suliman and Mr. Welch become unable or, for good cause, unwilling to serve, 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. As described more fully below under " – Board Diversity, Skills and Expertise", the Nominating and Corporate Governance Committee also considers each potential nominee's contribution to the Board's diversity of talents, skills, 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* 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. 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 54 under the caption "Stockholder Proposals."

Board Diversity, Skills and Expertise

The Nominating and Corporate Governance Committee assesses the effectiveness of its Board membership criteria on an ongoing basis and as part of the director selection and nomination process. Although we do not have a formal policy regarding board diversity, the Nominating and Corporate Governance Committee places significant emphasis on diversity and actively considers whether nominees assist in achieving a mix of Board members that represents a diversity of talents, skills, backgrounds, and expertise. The current composition of our Board reflects the importance of diversity to the Board.

Of our nine current directors,

- two directors are ***female*** ;
- one director is ***Asian American*** ;
- one director is ***LGBT*** ; and
- seven directors have ***significant professional experience outside of the United States*** .

Our Board also possesses broad expertise, qualifications, skills and experience that particularly align with our business and long-term strategy. For instance,

- All of our directors have ***executive level leadership experience in the biopharmaceutical industry*** ;
- All of our directors have ***public company governance experience*** ;
- Seven of our directors have experience with ***global business operations*** ;
- Seven of our directors have ***financial or capital markets experience*** ;
- Six of our directors have leadership experience in ***business development or corporate strategy*** ;
- Five of our directors have ***clinical development leadership experience*** ;
- Five of our directors have ***commercial leadership experience and with rare disease products in particular*** ;
- Four of our directors have ***scientific and research leadership experience*** ;
- Four of our directors have particular leadership experience related to ***global pricing, reimbursement and pricing approvals*** ;
- Three of our directors have ***regulatory leadership experience*** ; and
- Two of our directors have leadership experience ***building a rare disease product franchise*** .

Nominees and Incumbent Directors

The following table sets forth the following information for our director nominees 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	Director Since	Year Current Term Expires	Current Director Class
Director Nominees					
Emil D. Kakkis, M.D., Ph.D.	59	President and Chief Executive Officer	2010	2020	I
Shehnaaz Suliman, M.D.	48	Director	2019	2020	I
Daniel G. Welch	62	Chairman of the Board	2015	2020	I
Continuing Directors					
Deborah Dunsire, M.D.	57	Director	2017	2021	II
Michael Narachi	60	Director	2015	2021	II
Clay B. Siegall, Ph.D.	59	Director	2014	2021	II
William Aliski	72	Director	2011	2022	III
Lars Ekman, M.D., Ph.D.	70	Director	2016	2022	III
Matthew K. Fust	55	Director	2014	2022	III

Class I Directors Nominated for Election

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 its 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 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 Medical Genetics and was board certified in Pediatrics. He holds a B.A. in Biology from Pomona College and received combined M.D. and Ph.D. degrees from the UCLA School of Medicine's Medical Scientist Training Program where he received the Bogen prize for his research. We believe that Dr. Kakkis possesses specific expert knowledge of genetics and rare diseases and operational experience in the life sciences sector that qualify him to serve on our Board.

Shehnaaz Suliman, M.D., M.Phil., M.B.A. has served as a member of our Board since January 2019. Dr. Suliman has served as President and Chief Operating Officer of Alector, Inc., a publicly traded clinical stage biotechnology company, since December 2019 and previously served as Senior Vice President, Corporate Development and Strategy of Theravance Biopharma, Inc., a biopharmaceutical company, from July 2017 until March 2019. Prior to her position at Theravance, Dr. Suliman worked for Genentech, Inc., a biopharmaceutical company, as Group Leader and Project Team Leader in the R&D Portfolio Management and Operations Group from September 2010 to May 2015 and then as Vice President and Global Therapeutic Head, Roche Partnering from June 2015 to July 2017. Prior to Genentech, Dr. Suliman held various management roles of increasing responsibility at Gilead Sciences, Inc., a public biopharmaceutical company, between January 2005 and September 2010. Prior to Gilead, Dr. Suliman was an investment banker with Lehman Brothers and Petkevich & Partners, advising public and private companies on buy- and sell-side transactions. She is a member of the board of directors of 10X Genomics, Inc., a publicly traded life science technology company, and a member of the board of directors of Parvus Therapeutics, Inc., a private biopharmaceutical company. Dr. Suliman received her M.D. at the University of Cape Town Medical School, South Africa, and holds an M.B.A, with distinction, and M.Phil. in Development Studies degrees from Oxford University, where she was a Rhodes Scholar. We believe that Dr. Suliman is qualified to serve on our Board due to her extensive operational experience with global biopharmaceutical companies, and particularly her expertise in business development, corporate strategy and clinical drug development. Dr. Suliman was recommended to the Nominating and Corporate Governance Committee as a director candidate by another director and by a third-party search firm.

Daniel G. Welch has served as a member of our Board since April 2015 and as Chairman of the Board since June 2015. Mr. Welch is currently Executive Chairman at Levo Therapeutics, Inc., a private biotechnology company, and Executive Chairman of Incarda Therapeutics, Inc., a private biotechnology firm, a position he has held since March 2019. Between January 2015 and January 2018, he was an Executive Partner at Sofinnova Ventures, a venture capital firm. Prior to Sofinnova, Mr. Welch served as Chairman, Chief Executive Officer, and President of InterMune, Inc., 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. In addition to serving as Executive Chairman at Levo Therapeutics and as Executive Chairman of Incarda Therapeutics, both private biotechnology firms, Mr. Welch currently 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 as chairman of the board of AveXis, Inc., a publicly traded biotechnology company, from January 2016 through its acquisition in May 2018, and 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.

Class II Directors Continuing in Office Until 2021

Deborah Dunsire, M.D. has served as a member of our Board since April 2017. Dr. Dunsire has served as President and Chief Executive Officer of H. Lundbeck A/S, a pharmaceutical company, since September 2018. She 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, 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 member of the board of directors of Alexion Pharmaceuticals Inc., a public biopharmaceutical company. 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 various 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 CODA Biotherapeutics, Inc., a private biotherapeutics company. Between March 2009 and July 2018, Mr. Narachi served as President and Chief Executive Officer and a director of Orexigen Therapeutics, Inc., a biotechnology company. 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 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 on the board of directors 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 gene therapy 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 service as the chief executive officer of various biotechnology companies, 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. Dr. Siegall previously served as a director of Alder BioPharmaceuticals, Inc., a publicly traded biotechnology company, from 2006 until October 2019 and 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.

Class III Directors Continuing in Office Until 2022

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 Audentes Therapeutics, Inc. from September 2018 to the present, Ra Pharmaceuticals, Inc. from October 2016 to March 2017, Clementia Pharmaceuticals, Inc. from December 2015 to January 2017 and May 2018 to the present, OxThera from January 2015 through April 2015, Prosensa during 2014, Adimab LLC from November 2013 until December 2013, NPS Pharmaceuticals from April 2013 through December 2014, Fidelity Biosciences from August 2012 until December 2012, and Enobia Pharma from September 2011 until March 2012. Before that, Mr. Aliski served as Senior Vice President and Chief Commercial Officer of FoldRx Pharmaceuticals, a rare disease company that is now a wholly-owned subsidiary of Pfizer Inc., from June 2009 until March 2011, as Director of Simon Kucher Partners, a global consulting firm, from January 2008 until June 2009, and as General Manager of BioMarin Europe at BioMarin Pharmaceuticals Inc. from December 2005 until January 2008. Mr. Aliski currently serves on the board of directors of Applied Genetic Technologies Corporation, a public biotechnology company, and on the board of directors of X4 Pharmaceuticals, Inc., a public biopharmaceutical company. 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, a public biopharmaceutical company, Chairman of the Board of Sophiris Bio, Inc., a public biopharmaceutical company, and Chairman of the Board of Prothena Corporation plc, a public neuroscience company. Dr. Ekman previously served on the boards of Spark Therapeutics, Inc. from 2015 until December 2019, Elan Corporation plc from 2007 through 2013, InterMune Inc. from 2006 through 2013, and Ocera Therapeutics, Inc. from 2009 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, Inc., 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 currently serves on the board of directors of Atara Biotherapeutics, Inc., Crinetics Pharmaceuticals, Inc. and MacroGenics, Inc. (“MacroGenics”), which are publicly traded biopharmaceutical companies. Mr. Fust has notified MacroGenics that he will not stand for re-election as a director of the company upon the expiration of his term at the MacroGenics 2020 annual meeting of stockholders. Mr. Fust also previously served on the board of directors of Sunesis Pharmaceuticals, Inc. from May 2005 to May 2017 and on the board of Dermira, Inc. from August 2014 to February 2020. 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.

Vote Required

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

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, 2020, 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 2019 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, 2019 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 of Directors and Committees—Board Committees" and "Report of the Audit Committee."

Ernst & Young LLP has audited our financial statements since our 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.

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, 2019 and 2018.

	2019	2018
Audit fees (1)	$ 1,490,000	$ 1,419,000
Audit-related fees	—	—
Tax fees (2)	120,000	198,000
All other fees (3)	—	2,000
Total	$ 1,610,000	$ 1,619,000

(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 in 2018 consisted 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.

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* . 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, 2019. 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 applicable rules of 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, 2019.

Submitted by the Audit Committee of the Board of Directors
Matthew Fust, Chairperson
William Aliski
Michael Narachi

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, our compensation policies as they relate to risk management, or our pay ratio.

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 performance, including continuous improvements in corporate performance and accomplishments that are expected to increase stockholder value. The Compensation Discussion and Analysis section starting on page 25 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 this proposal, we will consider our stockholders' concerns and evaluate what actions may be appropriate to address those concerns. 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. Unless the Board modifies its policy on the frequency of holding say-on-pay advisory votes, the next say-on-pay vote will occur in 2021.

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 2020 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.

CORPORATE GOVERNANCE

Director Independence

Our Board currently consists of nine members. Our Board has determined that Mr. Aliski, Dr. Dunsire, Dr. Ekman, Mr. Fust, Mr. Narachi, Dr. Siegall, Dr. Suliman, and Mr. Welch qualify as “independent” directors in accordance with 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.

Global Code of Conduct

We have adopted a Global Code of Conduct that applies to all of our employees, officers, and directors, including those officers responsible for financial reporting. Our Global Code of Conduct is available on our website, *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 Global Code of Conduct that we are required to disclose, and any waivers of our Global Code of Conduct that apply to our Chairman of the Board, any of our executive officers, or any member of our Board.

Anti-Hedging Policy

Our insider trading policy prohibits our directors and employees, including our executive officers, from engaging in hedging transactions that are designed to hedge, offset or transfer with respect to equity compensation received by a director or employee, all or a portion of the risk of a decline in the market price of shares of our stock.

Stockholder Communications

Generally, stockholders who have questions or concerns regarding the Company should contact our Investor Relations department at (844) 280-7681. 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. At the request of the Chairman of the Board, the Secretary to the Board reviews all correspondence addressed to the Chairman, organizes the correspondence, and provides it to the Chairman or to individual directors, as appropriate. Our independent directors have requested that certain items that are unrelated to the Board’s duties, such as spam, junk mail, mass mailings, solicitations, resumes, and job inquiries not be provided to directors.

BOARD OF DIRECTORS AND COMMITTEES

During fiscal 2019, our Board met five times and also acted by written consent two times. Each director, other than Deborah Dunsire, 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. During fiscal 2019, Dr. Dunsire attended 71% of the aggregate of the meetings of the Board and committees of which she was a member due to unavoidable scheduling conflicts. Dr. Dunsire participated in a debriefing after each missed meeting with at least one director who attended that meeting.

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 applicable independence standards (including the heightened independence standards that apply to Audit Committee and Compensation Committee members).

Six of the directors serving at the time of the 2019 Annual Meeting of Stockholders (the " ***2019 Annual Meeting*** ") 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.

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 eight of our nine 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 four times in 2019, 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 Global Code of Conduct; 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* under the “Corporate Governance” subsection of the “Investors” tab. The Audit Committee has been delegated the task of overseeing significant financial risks facing us and reporting back to the Board regarding the same. The Audit Committee met six times in fiscal 2019. All members of the Audit Committee satisfy the current independence and financial literacy standards promulgated by Nasdaq and the SEC, and the Board has determined that Mr. Fust qualifies as an “audit committee financial expert,” as the SEC has defined that term in Item 407 of Regulation S-K.

Compensation Committee. The members of the Compensation Committee are Mr. Aliski (Chairperson), Dr. Siegall, Mr. Narachi and Mr. Welch. The Compensation Committee annually reviews and approves corporate goals and objectives relevant to the compensation of our executive officers; evaluates the performance of our executive officers in light of such 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* under the “Corporate Governance” subsection of the “Investors” tab. The Compensation Committee has been delegated the task of overseeing risks related to our compensation policies and programs. 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 four times in fiscal 2019 and also acted by written consent four 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), Dr. Suliman, 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* under the “Corporate Governance” subsection of the

"Investors" tab. The Nominating and Corporate Governance Committee has been delegated the task of overseeing all risks facing us, other than those overseen by the Audit Committee and by the Compensation Committee, and reporting back to the Board regarding the same. The Nominating and Corporate Governance Committee met four times in fiscal 2019 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. Suliman. The Research and Development Committee assists the Board in its oversight of the 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 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* under the "Corporate Governance" subsection of the "Investors" tab. The Research and Development Committee met two times in fiscal 2019.

Compensation Committee Interlocks and Insider Participation

During fiscal 2019, the Compensation Committee consisted of Mr. Aliski, Dr. Siegall, Mr. Welch and Mr. Narachi, who joined the Compensation Committee in June 2019. 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.

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 I Directors).

Name	Age	Position
Emil D. Kakkis, M.D., Ph.D.	59	President and Chief Executive Officer, Director
Camille L. Bedrosian, M.D.	67	Chief Medical Officer and Executive Vice President
Erik Harris	50	Chief Commercial Officer and Executive Vice President
Dennis Huang	55	Chief Technical Operations Officer and Executive Vice President
Thomas Kassberg	59	Chief Business Officer and Executive Vice President
Karah Parschauer	42	General Counsel and Executive Vice President
John R. Pinion II	54	Chief Quality Officer and Executive Vice President of Translational Sciences
Shalini Sharp	45	Chief Financial Officer and Executive Vice President

Camille L. Bedrosian, M.D. has served as our Chief Medical Officer and Executive Vice President 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 A.B. 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.

Erik Harris has served as our Chief Commercial Officer and Executive Vice President since June 2019. Prior to his appointment as our Chief Commercial Officer, Erik served as our Senior Vice President and Head of North American Commercial Operations from July 2017 to June 2019. Prior to Ultragenyx, Mr. Harris spent six years at Crescendo Bioscience, a subsidiary of Myriad Genetics, Inc. At Crescendo he held various leadership positions, most recently as Vice President of Commercial from June 2014 to June 2017, leading all aspects of marketing, sales, managed care and customer service. Mr. Harris previously served as Vice President of Pulmonology Marketing at Intermune, Inc. Earlier in his career he held positions in the commercial organizations at Elan Pharmaceuticals, Inc., Genentech, Inc., and Bristol-Myers Squibb Company. To begin his professional career, Mr. Harris served as a Lieutenant Commander in Naval Aviation and Congressional Fellow for the United States Navy. Mr. Harris received his Masters of Business Administration at the Wharton School of Business, and Bachelor of Science at the United States Naval Academy.

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 currently serves on the board of directors of CytoDel, Inc., a private biopharmaceutical company. 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 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., a biopharmaceutical company, 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 has served on the board of directors of Arcturus Therapeutics, Ltd., a publicly traded RNA medicines company, since June 2019 and on the board of directors of Evolus, Inc., a publicly traded performance beauty company, since July 2019. 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 B.S. of Science in Mechanical Engineering from the University of West Virginia.

Shalini Sharp has served as our Executive Vice President since January 2016 and as 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 Sutro Biopharma, Inc., a public biotechnology company, since November 2018, a board member of Precision Biosciences Inc., a public biotechnology company, since December 2018 and a board member of Neurocrine Biosciences, Inc., a public biopharmaceutical company, since February 2020. She previously served as a board member of Array BioPharma Inc. from April 2017 until June 2019 and as a board member of Agenus from May 2012 until June 2018. 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, 2019, 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 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" included 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.

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DELINQUENT SECTION 16(A) BENEFICIAL OWNERSHIP REPORTS

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 a 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 2019, except that Ms. Sharp filed one late Form 4, which reported shares sold (rather than withheld as erroneously reported originally) to pay the required tax withholdings due to the vesting of restricted stock units.

SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The following table sets forth information relating to the beneficial ownership of our common stock as of April 20, 2020 (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 59,581,451 shares of our common stock outstanding as of April 20, 2020. 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 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
Stockholders Owning Greater than 5%:		
Capital International Investors (1)	6,594,662	11.1
Capital Research Global (2)	6,362,695	10.7
The Vanguard Group (3)	4,853,159	8.1
BlackRock, Inc. (4)	4,733,084	7.9
T. Rowe Price Associates, Inc. (5)	4,491,769	7.5
Capital World Investors (6)	3,237,295	5.4
Federated Hermes, Inc. (7)	2,972,008	5.0
Directors and Named Executive Officers:		
William Aliski (8)	198,460	*
Deborah Dunsire, M.D. (9)	36,625	*
Lars Ekman, M.D., Ph.D. (10)	42,250	*
Matthew K. Fust (11)	58,431	*
Michael Narachi (12)	52,250	*
Clay B. Siegall, Ph.D. (13)	59,750	*
Shehnaaz Suliman, M.D. (14)	16,889	*
Daniel G. Welch (15)	52,250	*
Emil D. Kakkis, M.D., Ph.D. (16)	3,467,662	5.8
Camille Bedrosian, M.D. (17)	66,870	*
Thomas Kassberg (18)	324,016	*
John R. Pinion II (19)	173,139	*
Shalini Sharp (20)	190,082	*
All executive officers and directors as a group (21) (16 persons)	5,010,537	8.2%

* 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 14, 2020 by Capital International Investors, a division of Capital Research and Management Company (“CRMC”). The Schedule 13G/A reported that, as of December 31, 2019, Capital International Investors has sole voting power with respect to 6,313,443 of these shares and sole dispositive power with respect to 6,594,662 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 CRMC and Capital Bank and Trust Company, as well as the following CRMC subsidiaries: 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, 16th Floor, Los Angeles, CA 90025.

(2) Based on information set forth in a Schedule 13G/A filed with the SEC on February 14, 2020 by Capital Research Global Investors, a division of CRMC. The Schedule 13G/A reported that, as of December 31, 2019, 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. Capital Research Global Investors disclaims beneficial ownership of these shares pursuant to Rule 13d-4 of the Exchange Act. The principal business address for Capital Research Global Investors is listed in such filing as 333 South Hope Street, Los Angeles, CA 90071.

(3) Based on information set forth in a Schedule 13G/A filed with the SEC on February 12, 2020 by The Vanguard Group. The Schedule 13G/A reported that, as of December 31, 2019, The Vanguard Group has

sole voting power with respect to 115,218 shares, shared voting power with respect to 8,812 shares, sole dispositive power with respect to 4,736,484 shares, and shared dispositive power with respect to 116,675 shares. The principal business address for The Vanguard Group is listed in such filing as 100 Vanguard Blvd., Malvern, PA 19355.

(4) Based on information set forth in a Schedule 13G/A filed with the SEC on February 6, 2020 by BlackRock, Inc. The Schedule 13G/A reported that, as of December 31, 2019, BlackRock, Inc. has sole voting power with respect to 4,568,813 shares and sole dispositive power with respect to 4,733,084 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 14, 2020 by T. Rowe Price Associates, Inc. The Schedule 13G/A reported that, as of December 31, 2019, T. Rowe Price Associates, Inc. has sole voting power with respect to 926,616 of these shares and sole dispositive power with respect to 4,491,769 of these shares. The principal business address for T. Rowe Price Associates, Inc. is listed in such filing as 100 E. Pratt Street, Baltimore, MD 21202.

(6) Based on information set forth in a Schedule 13G filed with the SEC on February 14, 2020 by Capital World Investors, a division of CRMC. The Schedule 13G reported that, as of December 31, 2019, Capital World 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. Capital World Investors disclaims beneficial ownership of these shares pursuant to Rule 13d-4 of the Exchange Act. Capital World Investors divisions of CRMC and Capital International Limited collectively provide investment management services under the name “Capital World Investors”. The principal business address for Capital World Investors is listed in such filing as 333 South Hope Street, Los Angeles, CA 90071.

(7) Based on information set forth in a Schedule 13G filed with the SEC on February 13, 2020 by Federated Hermes, Inc. (“Federated”) and the Trust and Trustees, as defined below. The Schedule 13G reported that, as of December 31, 2019, Federated has sole voting and sole dispositive power with respect to all shares beneficially owned as a result of acting as the parent holding company to companies that act as investment advisor to various investment companies. Federated is the parent holding company of Federated Equity Management Company of Pennsylvania and Federated Global Investment Management Corp. (the “Investment Advisers”), which act as investment advisers to registered investment companies. The Investment Advisers are wholly owned subsidiaries of FII Holdings, Inc., which is wholly owned subsidiary of Federated. All of Federated’s outstanding voting stock is held in the Voting Shares Irrevocable Trust (the “Trust”) for which Thomas R. Donahue, Rhodora J. Donahue and J. Christopher Donahue act as trustees (collectively, the “Trustees”). The Trustees have collective voting control that they exercise over Federated. Federated, the Trust, and each of the Trustees expressly disclaim beneficial ownership of these shares pursuant to Rule 13d-4 of the Exchange Act. The principal business address for Federated is listed in such filing as 1001 Liberty Avenue, PA 15222-3779.

(8) Consists of (a) 90,350 shares of common stock held by the William and Carolyn Aliski Trust, (b) 9,686 shares of common stock held in a grantor annuity trust, (c) 18,000 shares of common stock held in a grantor annuity trust, (d) 44,924 shares of common stock held by Mr. Aliski, (e) 32,500 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (f) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020. Mr. Aliski shares voting and dispositive power over the 90,350 shares of common stock held by the William and Carolyn Aliski Trust; each of Mr. Aliski’s spouse and nephew is a trustee of such trust. Mr. Aliski has sole voting and dispositive power over the 27,686 shares of common stock held by him in each of the grantor annuity trusts; Mr. Aliski is the trustee of the trusts. Mr. Aliski also has sole voting and dispositive power over the 32,500 shares of common stock issuable pursuant to stock options held by Mr. Aliski and the 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units.

(9) Consists of (a) 4,875 shares of common stock, (b) 28,750 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(10) Consists of (a) 6,750 shares of common stock, (b) 32,500 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(11) Consists of (a) 11,750 shares of common stock, (b) 43,681 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(12) Consists of (a) 6,750 shares of common stock, (b) 42,500 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(13) Consists of (a) 6,750 shares of common stock, (b) 50,000 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(14) Consists of (a) 13,889 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (b) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(15) Consists of (a) 6,750 shares of common stock, (b) 42,500 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 3,000 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(16) 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) 586,520 shares of common stock held by Dr. Kakkis, (c) 317,009 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (d) 4,392 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020. 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 586,520 shares of common stock held by him, the 317,009 shares of common stock issuable pursuant to stock options held by Dr. Kakkis and the 4,392 shares of common stock issuable pursuant to the vesting of restricted stock units.

(17) Consists of (a) 8,100 shares of common stock, (b) 58,052 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 718 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(18) Consists of (a) 78,480 shares of common stock, (b) 243,819 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 1,717 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(19) Consists of (a) 15,871 shares of common stock, (b) 155,801 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 1,467 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(20) Consists of (a) 85,441 shares of common stock, (b) 102,924 shares of common stock issuable pursuant to stock options exercisable within 60 days of April 20, 2020 and (c) 1,717 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

(21) Consists of (a) 3,561,475 shares of common stock held by our directors and executive officers, (b) 1,411,390 shares of common stock issuable pursuant to stock options held by our directors and executive officers that are exercisable within 60 days of April 20, 2020 and (c) 37,672 shares of common stock issuable pursuant to the vesting of restricted stock units within 60 days of April 20, 2020.

EXECUTIVE COMPENSATION

Compensation Discussion and Analysis

The following compensation discussion and analysis describes the material elements of compensation earned in fiscal 2019 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, 2019 are:

- Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer;
- Shalini Sharp, Chief Financial Officer and Executive Vice President (1) ;
- John R. Pinion II, Chief Quality Officer and Executive Vice President of Translational Sciences;
- Camille L. Bedrosian, M.D., Chief Medical Officer and Executive Vice President; and
- Thomas Kassberg, Chief Business Officer and Executive Vice President.

These persons constitute our principal executive officer, our principal financial officer and our three other most highly paid executive officers serving during fiscal 2019.

(1) In March 2020, we announced that Shalini Sharp had informed us of her decision to voluntarily resign from her position as our Executive Vice President and Chief Financial Officer, effective as of the earlier of (i) the date her successor Chief Financial Officer commences employment with us and (ii) September 2, 2020 (the “ ***Officer Resignation Date*** ”). Ms. Sharp’s employment with us will terminate on the date that is six months from the Officer Resignation Date, or such earlier date following the Officer Resignation Date as mutually agreed between Ms. Sharp and the Company (the “ ***Separation Date*** ”). During the period between the Officer Resignation Date and the Separation Date (the “ ***Transition Period*** ”), Ms. Sharp will continue to be an employee of the Company but will no longer have the powers, duties and responsibilities commensurate with the position of Executive Vice President and Chief Financial Officer. During the Transition Period, Ms. Sharp’s primary responsibility will be to transition her duties and institutional knowledge to the new Chief Financial Officer, and to provide assistance on or lead projects as requested by our Chief Executive Officer and/or the new Chief Financial Officer.

Executive Summary

During 2019, we made significant progress in executing our strategic priorities in building and growing our commercial business and advancing our product candidates. We had a number of major financial and operational accomplishments during the year, including the following:

- *Significant Revenue Growth* . We achieved $103.7 million in total revenue during 2019, a 101% increase compared to total revenue in 2018, largely driven by continued strong growth in Crysvita ® (burosumab) sales during the year. In 2019, we recognized $87.3 million in total Crysvita revenue compared to $18.9 million in Crysvita revenue in the prior year after our product launch in April 2018.
- *Regulatory Approvals and Filings Related to Crysvita* . We made advances with obtaining regulatory approval for Crysvita in Latin America and expanding the product label and indication for the product in the U.S. In March 2019, Brazil’s National Health Surveillance Agency approved Crysvita for the treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients one year of age and older. In September 2019, the U.S. Food and Drug

Administration (the " ***FDA*** ") approved a label expansion for Crysvita to include new clinical data demonstrating superiority of treatment with Crysvita versus oral phosphate and active vitamin D (conventional therapy) in pediatric patients with XLH, and improvement in stiffness, and maintenance of efficacy of Crysvita in adult patients with longer-term treatment. The indication has also been expanded to include infants as young as six months of age. Finally, in December 2019, we submitted a supplemental Biologics License Application (" ***sBLA*** ") to the FDA for Crysvita for the treatment of FGF23-related hypophosphatemia associated with phosphaturic mesenchymal tumors, or tumor-induced osteomalacia or TIO, which cannot be curatively resected or localized. The FDA has accepted for review the sBLA and has assigned a Prescription Drug User Fee Act (" ***PDUFA*** ") target date of June 18, 2020.

- *UX007 New Drug Application (NDA) accepted for filing by the FDA.* In October 2019, we announced that the FDA has accepted for review our NDA for UX007 (triheptanoin) for the treatment of long-chain fatty acid oxidation disorders (LC-FAOD), a group of genetic disorders in which the body is unable to convert long-chain fatty acids into energy. The FDA has assigned a PDUFA target date of July 31, 2020.

- *Positive Data Results from Phase 1/2 Studies of Gene Therapy Product Candidates.* We continued to make significant progress with the clinical development of our product candidates, including in particular our gene therapy product candidates. We announced in September 2019 positive data from the second dose cohort of our ongoing Phase 1/2 study of DTX401, an adeno-associated virus (" ***AAV*** ") based gene therapy for the treatment of glycogen storage disease type Ia (GSDIa). All three patients in Cohort 2 showed a clinical response with improvements in glucose control and other metabolic parameters compared to baseline, and all three patients in Cohort 1 continued to demonstrate long-term, durable responses. At the end of 2019, we received topline positive safety and efficacy data from Cohort 3 and longer-term data from Cohort 2 of our ongoing Phase 1/2 study of DTX301, an investigational AAV gene therapy for the treatment of ornithine transcarbamylase (OTC) deficiency. The data demonstrated that up to six of the nine patients demonstrated a response as of the data cutoff date of December 9, 2019.

- *Business Development Achievements.* We entered into a partnership with GeneTx Biotherapeutics LLC (" ***GeneTx*** ") in August 2019 to develop GeneTx's GTX-102, an antisense oligonucleotide (" ***ASO*** ") for the treatment of Angelman syndrome, a serious, debilitating, rare neurogenetic disorder that affects approximately 1 in 15,000 people worldwide. GeneTx filed an Investigational New Drug (" ***IND*** ") application for GTX-102 with the FDA in 2019. The IND is now active and the Phase 1/2 study is expected to begin in the first half of 2020. The FDA granted Orphan Drug Designation and Rare Pediatric Disease Designation to GTX-102 for the treatment of Angelman syndrome. We also expanded our existing collaboration with Arcturus Therapeutics Holdings, Inc. (" ***Arcturus*** "), a leading messenger RNA medicines company, in June 2019 to discover and develop mRNA, DNA and siRNA therapeutics for up to 12 rare disease targets. Our most advanced mRNA program is UX053 for the treatment of Glycogen Storage Disease Type III, a disease caused by a glycogen debranching enzyme (AGL) deficiency that results in glycogen accumulation in the liver and muscle.

- *Successful Financings* . We achieved a successful follow-on public offering in February 2019 and received net proceeds of approximately $330 million. In December 2019, we sold our royalty interest in Crysvita in the European territory to an affiliate of Royalty Pharma for $320 million.

Stockholder Outreach

Overview

Our stockholder outreach program is a year-long effort by our management to engage with our stockholders in a continuous and meaningful way and is consistent with our commitment to engagement, communication, and transparency. The Compensation Committee views stockholder feedback as an important factor in developing and improving our executive compensation program.

Stockholder Advisory Vote on Executive Compensation

Each year, our stockholders are provided the opportunity to cast an advisory vote on the compensation of our named executive officers, or the "say-on-pay" vote, and the Compensation Committee considers the outcome of the prior year's say-on-pay vote when making decisions relating to the compensation of our named executive officers and our executive compensation programs. Following our 2019 Annual Meeting, our Board and the Compensation Committee determined that management should conduct enhanced outreach efforts in response to the results of our say-on-pay vote at the 2019 Annual Meeting, which received 68.1% support. While this result reflects broad support of our compensation philosophy and our pay practices, it represents a decline from 2018 and 2017 results (74.6% and 83.9% support, respectively). We gave thoughtful consideration to this development, and to better understand our stockholders' perspective concerning our executive compensation, we expanded our stockholder engagement activities. During 2019 and in the beginning of 2020, we reached out to stockholders representing over 75% of our outstanding shares as of the record date for the 2019 Annual Meeting, including each of our largest stockholders. In response to our outreach efforts, we met with stockholders who responded to our engagement request, which holders represented a majority of our outstanding shares. Participants at these meetings included our Chief Financial Officer and General Counsel; our Board Chairman also participated in some of the discussions.

During the course of our engagement efforts, we learned that the driver for nearly all the votes cast "AGAINST" our 2019 say-on-pay proposal was disapproval by one institutional investor and several of its affiliates of the evergreen feature in our 2014 Incentive Plan and 2014 Employee Stock Purchase Plan. Our 2014 Incentive Plan and 2014 Employee Stock Purchase Plan, including the evergreen provisions within the plans, were approved by our stockholders in connection with our initial public offering in 2014. In response to prior feedback, we previously publicly disclosed that we will not seek to extend the evergreen provisions, nor adopt new ones, upon expiration of the current evergreen provisions after January 1, 2024.

The Compensation Committee carefully reviewed feedback from our recent engagement efforts and did not make any material changes to its compensation practices as a direct result of the vote. The Compensation Committee reexamined in particular the evergreen provisions in our 2014 Incentive Plan and 2014 Employee Stock Purchase Plan. Following this review, the Compensation Committee determined that the evergreen provision has been crucial in supporting our growth in the company's development stage. Our employees have increased by almost 40% in the past two years, which increase has enabled us to successfully execute on various goals in our strategic business plan and enhance stockholder value, including our key achievements in 2019 that we described above. The evergreen provision supported our success and achievement of these business goals in enabling us to recruit and retain talented employees in our competitive biotech industry by ensuring we have enough shares to grant equity awards to new hires and continuing employees. In addition, the Compensation Committee noted that several of our peers in the biotech industry that have been public for approximately the same period as us and for whom we are competing for this critical talent have similar evergreen provisions in their equity plans. Without the benefit of our sunsetting evergreen provisions, we would be constrained in our ability to attract and retain a skilled and motivated workforce in our competitive industry.

That said, both the Compensation Committee and our Board understand the concern of potential dilution as a result of the evergreen provisions and are committed to acting as careful stewards of stockholder value. As such, the Compensation Committee designed our compensation program to incorporate various best practices, including, among others, a fulsome clawback provision, double trigger change in control provisions, robust stock ownership guidelines for our named executive officers and directors, anti-hedging and anti-pledging policies, prohibitions on excise tax gross-ups, prohibitions on awards of discretionary short-term incentive bonuses and performance-based long-term incentive awards. Our compensation program is thoughtfully designed to promote achievement of our business strategy while minimizing stockholder dilution. Our engagement efforts in 2019 and early 2020 confirmed that a large majority of our stockholders support our compensation program and pay levels. Indeed, other than the institutional investor (and its affiliates) described above that objected to our sunsetting evergreen provisions, none of the other stockholders we met with expressed disapproval of our executive compensation program or practices.

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 (" ***RSUs*** "), or other equity awards aligns the objectives of management with those of our stockholders with respect to long-term performance and success of the Company.

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, 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.

What We Don't Do

- We do not offer any tax gross-up payments to our executive team for any change-of-control payments.
- As discussed above under " – Corporate Governance", we prohibit our executive officers from hedging our securities.
- 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.

What We Do

- 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".
- 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—Annual Equity Grants in Fiscal 2019."

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 reviewing our corporate performance and carefully evaluating an executive's performance during the year against established goals, operational performance, and business responsibilities. In addition, the Compensation Committee utilizes discretion in the assessment process to respond to and adjust to a dynamic business environment.

Compensation Consultant

The Compensation Committee retains the services of an independent, external compensation consultant, Radford, an Aon 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 annually evaluates Radford's performance and determines whether to engage Radford or another compensation consultant 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, 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 2019, 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 recently filed NDAs or with early commercial organizations;
- companies with comparable market capitalizations (i.e., in the range of $750 million to $7.5 billion);
- companies with annual revenues of less than $150 million;
- companies with employee headcounts between 300 and 1,200 employees; and
- companies located in biotech hubs, such as the San Francisco bay area, San Diego, Massachusetts and the New York/New Jersey tristate area.

Our peer group for 2019, referred to as our 2019 peer group, which is used to evaluate compensation actions for the 2019 fiscal year was selected by the Compensation Committee based on Radford's recommendation in June 2018 and is comprised of the following 20 publicly-traded companies in the pharmaceutical and biotechnology industries:

ACADIA Pharmaceuticals Inc.	FibroGen, Inc.	Radius Health
Agios Pharmaceuticals, Inc.	Halozyme Therapeutics, Inc.	Sage Therapeutics
Amicus Therapeutics, Inc.	Intercept Pharmaceuticals, Inc.	Sarepta Therapeutics, Inc.
Array BioPharma Inc.	Ionis Pharmaceuticals, Inc.	Spark Therapeutics, Inc.
bluebird bio, Inc.	Neurocrine Biosciences, Inc.	TESARO, Inc.
Clovis Oncology, Inc.	Portola Pharmaceuticals, Inc.	Theravance Biopharma, Inc.
Exelixis Inc.	Puma Biotechnology, Inc.	

We believe that the compensation practices of our 2019 peer group provided us with appropriate benchmarks for evaluating the compensation of our named executive officers for 2019 because of the developmental, market and organizational characteristics we shared with our peer group. At the time that we selected our 2019 peer group we were at approximately the 41 st percentile in terms of market capitalization, the 97 th percentile in terms of employees relative to the peer group and the 47 th percentile in terms of revenue.

Annual Performance Reviews

Our Compensation Committee conducts an annual performance review of our named executive officers and approves their compensation. By the end of 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 (1) reviewing overall corporate performance; (2) evaluating each named executive officer's annual performance against established corporate goals; and (3) in the case of executive officers other than our Chief Executive Officer, reviewing the achievement of individual performance objectives.

At its first regularly scheduled meeting each year, 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 equity awards and his bonus, if any, under our bonus plan.

Our Compensation Committee may also review and adjust 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 determining appropriate base salary levels for a given executive officer, the Compensation Committee considers the following factors:

- individual performance of, and overall management of the function by, 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.

The effective date of annual merit increases to base salary is March 1.

2019 Base Salaries

The Compensation Committee engaged Radford to conduct a competitive review and analysis of our current executive compensation program relative to our 2019 peer group. Radford prepared an Executive Compensation Assessment report in September 2018 that provided a competitive assessment of our executive compensation program as compared to the 2019 peer group data for base salaries, target total cash compensation, and equity compensation.

For 2019, each of Dr. Bedrosian and Ms. Sharp received increases in base salaries of approximately 3%, annualized. These increases were based on their individual performance against goals and objectives during 2018 (and were similar to merit-based increases for our employees generally), as well as Radford's trends report of standard annual merit salary increases for 2019; Dr. Bedrosian's increase was prorated as she had commenced employment with us on January 29, 2018. In September 2018, Dr. Kakkis and Messrs. Pinion and Kassberg each received increases of approximately 6%, 4.5% and 9%, respectively, to their base salaries in order to more closely align their salaries at the market 50th percentile of our 2019 peer group and as a result did not receive further increases in their base salaries in 2019.

The following table shows the increases in base salaries for our named executive officers between fiscal 2018 and fiscal 2019:

Name	Title	Fiscal 2018 Base Salary	Fiscal 2019 Base Salary as of March 1, 2019	Percentage Increase (%)
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	$ 714,000	$ 714,000	NA
Shalini Sharp	Chief Financial Officer and Executive Vice President	$ 473,084	487,277	3.0
John R. Pinion II	Chief Quality Officer and Executive Vice President, Translational Sciences	$ 447,690	$ 447,690	NA
Camille L. Bedrosian, M.D.	Chief Medical Officer and Executive Vice President	$ 500,000	$ 513,849	2.8
Thomas Kassberg	Chief Business Officer and Executive Vice President	$ 474,000	$ 474,000	NA

Annual Bonus

Overview

Our bonus plan provides an opportunity for cash bonus awards 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 business, financial, and operational measures or objectives. Individual performance goals focus on individual contributions that drive achievement of the corporate goals and provide leadership for the executive officers' respective functions. The Chief Executive Officer's bonus is based solely on corporate performance.

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, except that 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).

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 determines the size of the bonus pool applicable to the corporate component of the bonus plan. A 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.

Fiscal 2019 Bonuses

Annual corporate goals for fiscal 2019 were proposed by our executive officers and approved by our Board in early 2019 upon the recommendation of our Compensation Committee. Individual objectives for our executive officers for 2019 were proposed by each executive officer, with review, input and confirmation from our Chief Executive Officer.

For 2019, our Compensation Committee set the annual targets for our executive officers' bonuses as a percent of base salary generally targeting the market 50th percentile of our 2019 peer group, which resulted in increasing the bonus targets from 60% to 70% for our Chief Executive Officer and from 40% to 45% for all of our other named executive officers. The target bonuses, as a percentage of base salary, for the named executive officers for fiscal 2019 are set forth in the following table.

Name	Title	Target Bonus for Fiscal 2019 (% of Base Salary)
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	70%
Shalini Sharp	Chief Financial Officer and Executive Vice President	45%
John R. Pinion II	Chief Quality Officer and Executive Vice President, Translational Sciences	45%
Camille L. Bedrosian, M.D.	Chief Medical Officer and Executive Vice President	45%
Thomas Kassberg	Chief Business Officer and Executive Vice President	45%

In December 2019, the Compensation Committee assessed our overall 2019 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 performance goals, as well as the relative weighting of these goals, in assessing overall performance for the 2019 fiscal year:

Goal	Weighting	Achievement against Goal
1. Product Portfolio and Performance • Achieving targets for global revenue in our territories and number of patients on products in target territories • Negotiating, preparing, and making regulatory submissions in target territories and obtaining market authorizations for two products in key territories outside of the United States • Improving supply reliability and inventory management for key products • Defining clinical studies and obtaining FDA alignment and acceptance of study designs • Completing studies and treatment of trial cohorts and releasing topline results	***75%***	Partially Achieved
2. Pipeline • Completing Phase 0 activities to enable pre-clinical program to be ready for IND filing • Advancing translational research programs to stage where decision can be made whether to advance program • Completing development and nonclinical testing related to manufacturing process for a specified product candidate	***15%***	Achieved
3. General Corporate • Maintaining operating cash usage within 5% of budget • Executing on all commercial and development goals while maintaining a culture of high ethics and compliance, with no adverse regulatory actions • Retaining key contributors • Completing planned phases for all major capital projects subject to program timelines and budgetary considerations	***5%***	Achieved
4. Business Development • Completing significant collaboration with strategic partner	***5%***	Achieved

In establishing these goals, the Compensation Committee selected performance goals that it 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 performance against these goals, the Compensation Committee reviewed each goal and determined whether or not it was achieved. The Compensation Committee then referred to the relative importance of the goals, based on the previously established weightings of each goal. After completing this assessment, the Compensation Committee determined that some goals were not completely achieved, and other goals were exceeded. The Compensation Committee also considered additional key corporate achievements that were not represented in the 2019 corporate goals, including a successful financing and the achievement of an expanded collaboration with a third party partner. The Compensation Committee then determined an overall percentage of achievement for all goals combined, which resulted in 96% achievement for fiscal year 2019.

In December 2019, in addition to assessing the foregoing corporate goals, the Compensation Committee assessed the individual accomplishments of our named executive officers, other than our Chief Executive

Officer, for purposes of determining the individual component of their annual bonus. Key individual achievements for these named executive officers are summarized below:

Named Executive Officer	Key 2019 Achievements
Shalini Sharp *Chief Financial Officer and EVP*	• Raised $350 million in gross proceeds from equity offering • Managed budget to within 5% while achieving significant procurement savings • Increased scope of internal audit, optimized tax strategies and supported international entities and collaborations with similar sized finance and accounting team • Implemented IT projects supporting commercialization efforts and growth
John R. Pinion II *Chief Quality Officer and EVP, Translational Sciences*	• Transition of certain translational research projects to IND enabling studies • Successful health authority inspections • Start up of new analytical lab, translational science lab and gene therapy lab projects • Partnered with third party partner to ensure reliable quality supply of Crysvita and improvements in partner's quality system
Camille L. Bedrosian, M.D. *Chief Medical Officer and EVP*	• Onboarding of key leadership talent • Led and supported teams in submission and FDA acceptance of NDA for UX007 based on Phase 2 data • Led and supported teams to prepare for sBLA submissions for Crysvita resulting in FDA approved label expansion and potential indication expansion • Successful pre-IND meeting with FDA for pipeline candidate and led and supported development of other pre-IND product programs • Supported business development efforts to assess GeneTx program for Angelman Syndrome and other business development partnership opportunities • Developed strategic Medical Affairs plans
Thomas Kassberg *Chief Business Officer and EVP*	• Completed transactions with GeneTx and expanded partnership with Arcturus • Advanced discussions with potential third party partners and collaborators • Continued to develop strong alliance management capabilities • Completed planned construction phases for capital products on schedule and within budget variances

Dr. Kakkis evaluated the performance of Ms. Sharp, Mr. Pinion, Dr. Bedrosian and Mr. Kassberg 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 2019.

Achievement of Goals and Relationship to Compensation Awarded

The overall 96% achievement score for the 2019 corporate goals, combined with Dr. Kakkis' assessment of the individual performance and achievement of Ms. Sharp, Mr. Pinion, Dr. Bedrosian and Mr. Kassberg during fiscal 2019, resulted in the Compensation Committee approving bonus awards for performance in 2019 as set forth in the following table:

Name	Title	Total Fiscal 2019 Bonus	Corporate Component Score (Weighting 75%; 100% for CEO)	Individual Component Score (Weighting 25%)
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	$ 479,808	96%	N/A
Shalini Sharp	Chief Financial Officer and Executive Vice President	$ 218,178	96%	110%
John R. Pinion II	Chief Quality Officer and Executive Vice President of Translational Sciences	$ 205,490	96%	120%
Camille L. Bedrosian, M.D.	Chief Medical Officer and Executive Vice President	$ 230,076	96%	110%
Thomas Kassberg	Chief Business Officer and Executive Vice President	$ 206,901	96%	100%

The bonuses awarded under our 2019 annual incentive program were paid in March 2020.

Equity Compensation

Overview

Stock Options, Restricted Stock Units and Performance Stock Units. Executive officers are eligible to receive equity compensation in the form of stock options, RSUs and/or performance stock units (" ***PSUs*** "). The Compensation Committee grants stock options, RSUs and/or PSUs annually 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 an annual 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. The Compensation Committee also grants stock options and RSUs to new executive officer hires. In determining the form and value of a new hire grant to executive officers, the Compensation Committee considers the expected 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 new hire executives at peer companies holding comparable positions, and the competitive market dynamics at the time of hire.

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 we make 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, encouraging the retention of executive officers by maintaining their focus on our long-term performance, as well as on the achievement of specific performance goals. 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 RSU 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.

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.

Annual Equity Grants in Fiscal 2019

Effective March 1, 2019, the Compensation Committee approved annual equity awards for our named executive officers. These awards were granted as a 50%/25%/25% value split among stock options, RSUs and PSUs, respectively, which approximated the market 50th percentile of our 2019 peer group and allowed us to align with the market direction of including RSUs in annual grants to remain competitive while maintaining an emphasis on pay and performance that naturally results from stock options and PSUs.

In an effort to increase retentive value and align executive compensation with the Company's performance related to product commercialization, the Compensation Committee added PSUs as part of the annual equity grant in March 2019 described above (the " ***2019 PSUs*** ") with a performance condition based on fiscal 2019 revenue, calculated in accordance with generally accepted accounting principles (" ***GAAP*** "). The revenue target for the 2019 PSUs was $107 million, with a threshold revenue target of $80 million for any PSUs to be earned and a maximum revenue target of $140 million. 50% of the 2019 PSUs subject to the award would be earned upon achievement of the threshold level, 100% of the 2019 PSUs subject to the award would be earned upon achievement of the target revenue and 150% of 2019 PSUs subject to the award would be earned upon achievement of the maximum target. The actual percentage of 2019 PSUs earned for performance between the threshold and maximum was determined on a straight-line interpolated basis. Once the achievement of the performance metric is certified by the Compensation Committee, 33% of the earned PSUs subject to the award would vest on the later of the certification date and March 1, 2020 and 67% of the earned PSUs subject to the award would vest thereafter on the first anniversary of such date.

Based on the Company's fiscal 2019 revenues calculated in accordance with GAAP of $103.7 million, the Compensation Committee certified in February 2020 that 93.9% of the target revenue for the 2019 PSU Awards had been achieved and as such 33% of the earned 2019 PSUs vested on March 1, 2020 and the remaining earned PSUs subject to the award will vest on March 1, 2021, subject to the named executive officer's continued service.

The table below sets forth all annual equity awards granted in fiscal 2019 to our named executive officers.

Name	Date of Grant	Number of Options	Number of Restricted Stock Units	Target Number of Performance Stock Units
Emil D. Kakkis, M.D., Ph.D.	March 1, 2019	66,000	20,000	20,000
Shalini Sharp	March 1, 2019	23,000	8,000	6,000
John R. Pinion II	March 1, 2019	23,000	20,000	6,000
Camille L. Bedrosian, M.D.	March 1, 2019	21,500	7,000	6,000
Thomas Kassberg	March 1, 2019	21,500	7,000	6,000

The values of the equity grants awarded to executive officers for the 2019 fiscal year, as well as all compensation actions taken with respect to the named executive officers in fiscal 2019, are reflected in the Summary Compensation Table.

Certification of PSU Vesting for 2018 Awards

In December 2017, the Compensation Committee approved PSUs for all employees who were eligible to receive equity awards, including new employees to be hired before June 30, 2018 (the " ***2018 PSUs*** ").

The metrics applicable to the 2018 PSUs were:

- Approval and launch in the U.S. of a broad label (for 1 year old and up and no disease restrictions) for Crysvita in 2018 (" ***Metric 1*** ");
- 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 (" ***Metric 2*** "); and
- First 12 months of US commercial net sales of Crysvita following approval and launch exceeding forecast by 35% (to be achieved by no later than the end of 2019) (" ***Metric 3*** "). The 12-month measurement period ended on April 30, 2019 based on timing of the approval and launch of Crysvita.

For awards granted to new employees hired between January 1, 2018 and June 30, 2018, the metrics applicable to the PSUs were only Metric 2 and Metric 3.

Once the achievement of a performance metric is certified by the Compensation Committee, 33.3% (or 50% if only two metrics apply) of the 2018 PSUs converted into time-based RSUs, 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 date. During fiscal 2019, the Compensation Committee certified achievement of Metric 3 of the 2018 PSUs in May 2019 and achievement of Metric 2 in October 2019. Metric 1 of the 2018 PSUs had previously been achieved in April 2018.

Fiscal 2020 Compensation

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, market capitalization, changes in individual peer companies, and other factors. The Compensation Committee engaged Radford to assist in reviewing our peer group and in suggesting revisions, as appropriate.

Based on Radford's assessment and recommendations, the Compensation Committee selected 20 publicly traded companies in the pharmaceutical and biotechnology industries to serve as our new list of peer companies for 2020, referred to as our 2020 peer group, by balancing the following criteria:

- companies with recently filed NDAs or early commercial organizations;
- companies with comparable market capitalizations (i.e., in the range of $1.5 billion to $12 billion);
- companies with revenue less than $500 million; and
- companies with headcounts between 300 and 1,200.

Our 2020 peer group is comprised of the following 20 companies in the pharmaceutical and biotechnology industries:

ACADIA Pharmaceuticals Inc.	Exelixis Inc.	Portola Pharmaceuticals, Inc.
Agios Pharmaceuticals, Inc.	FibroGen, Inc.	PTC Therapeutics, Inc.
Alnylam Pharmaceuticals Inc.	Halozyme Therapeutics, Inc.	Puma Biotechnology, Inc.
Amicus Therapeutics	Intercept Pharmaceuticals, Inc.	Sage Therapeutics
Array BioPharma	Ionis Pharmaceuticals, Inc.	Sarepta Therapeutics, Inc.
bluebird bio, Inc.	Nektar Therapeutics	Theravance Biopharma, Inc.
Clovis Oncology, Inc.	Neurocrine Biosciences, Inc.	

We believe that the compensation practices of our 2020 peer group provided us with appropriate compensation benchmarks for evaluating the compensation of our named executive officers for 2020.

Base Salaries

For 2020, increases in base salaries for our named executive officers, other than Mr. Pinion, were approximately 3% annualized and were 4% annualized for Mr. Pinion. The overall 2020 merit budget was based on a Radford trend report regarding projected market merit spends for 2020. Individual increases in base salaries were based on achievement of 2019 individual goals.

Annual Bonuses

For 2020, the Compensation Committee determined to retain the target amounts used for 2019 bonuses of 70% for our Chief Executive Officer and of 45% for all other named 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. For 2020, equity grants to our executive officers reflected a 50/25/25 value split among options, RSUs, and PSUs, respectively

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, participation is 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 are eligible to participate in the 401(k) Plan on the same terms as the named executive officers.

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.

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 2019, 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. The effective date of annual merit increases to salary is March 1st. 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

Our Board has 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 our named executive officers and Board members who have been named executive officers and Board members as of the date the policy was implemented and within five years of appointment for newly appointed named executive officers and Board members.

Risk Management and Mitigation

In reviewing the compensation structure in fiscal 2019, 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 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 2019, our corporate goals were reviewed and approved by the Board in early 2019, upon the recommendation of the Compensation Committee, 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, 2019.

Submitted by the Compensation Committee of the Board of Directors
William Aliski, Chairman
Michael Narachi
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, 2019, 2018, and 2017 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 (1)	Stock Awards (2)	Option Awards (3)	Non-Equity Incentive Plan Compensation (4)	All Other Compensation (5)	Total
Emil D. Kakkis, M.D., Ph.D.	2019	$714,000	—	$2,702,000	$2,650,784	$ 479,808	$ 39,887	$6,586,479
President and Chief	2018	$679,952	$ —	$ 944,385	$2,753,513	$ 428,400	$ 39,377	$4,845,627
Executive Officer	2017	$638,115	$ —	$1,319,303	$4,295,234	$ 393,000	$ 39,247	$6,684,899
Shalini Sharp,	2019	$484,548	$ —	$ 945,700	$ 923,758	$ 218,178	$ 33,337	$2,605,521
Chief Financial Officer	2018	$470,435	$ —	$ 339,010	$1,019,820	$ 198,695	$ 32,827	$2,060,787
and Executive Vice President	2017	$455,785	$ —	$ 424,043	$1,541,879	$ 188,315	$ 32,697	$2,642,719
John R. Pinion II,	2019	$447,690	$ —	$1,756,300	$ 923,578	$ 205,490	$ 37,472	$3,370,530
Chief Quality Officer	2018	$430,320	$ —	$ 217,935	$ 728,443	$ 188,030	$ 36,346	$1,601,074
and Executive Vice	2017	$412,568	$ —	$ 308,603	$ 991,208	$ 170,459	$ 36,491	$1,919,329
President, Translational Sciences								
Camille L. Bedrosian, M.D.	2019	$511,186	$ —	$ 878,150	$ 863,513	$ 230,076	$ 13,891	$2,496,816
Chief Medical Officer	2018	$442,308	$210,000	$ 990,000	$2,906,983	$ 189,274	$ 107,727	$4,846,292
and Executive Vice President								
Thomas Kassberg	2019	$474,000	$ —	$ 878,150	$ 863,513	$ 206,901	$ 33,937	$2,456,501
Chief Business Officer	2018	$441,340	$ —	$ 242,150	$ 786,718	$ 189,600	$ 32,852	$1,692,660
and Executive Vice President	2017	$418,443	$ —	$ 335,243	$1,376,678	$ 168,600	$ 32,697	$2,331,661

(1) The amounts reported in this column represent sign-on bonuses.

(2) The amounts reported in this column for a fiscal year represent the grant date fair value of the RSUs and PSUs granted to our named executive officers during the fiscal year, as computed in accordance with ASC Topic 718, not including any estimates of forfeitures, and, with respect to the PSUs, assuming the most probable outcome of the performance conditions as of the grant date. The assumptions used in calculating the grant date fair value of the RSUs and PSUs reported in this column are set forth in the notes to our financial statements included in our Annual Report. The amounts reported in this column reflect the accounting cost for these RSUs and PSUs and do not correspond to the actual economic value that may be received by the named executive officers from the RSUs and the PSUs. The value of the PSUs reported in this column for 2019, assuming achievement of the maximum performance level, is as follows: $2,206,500 for Dr. Kakkis and $607,950 for Ms. Sharp, Mr. Pinion, Dr. Bedrosian and Mr. Kassberg.

(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. 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 2019 fiscal year reflect the following payments to our named executive officers: Dr. Kakkis—$8,400 for 401(k) matching contributions, $3,375 for health savings account contributions, $21,562 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us, and $6,550 for key person life insurance premiums paid by us; Ms. Sharp—$8,400 for 401(k) matching contributions, $3,375 for health savings account contributions and $21,562 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us; Mr. Pinion—$8,400 for 401(k) matching contributions, $29,072 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us; Dr. Bedrosian—$11,162 for medical, dental, vision and life/accidental death & dismemberment premiums paid by us, and $2,729 in housing grossup payments; and Mr. Kassberg—$8,400 for 401(k) matching contributions, $3,375 for health savings account contributions and $21,562 for medical, dental, vision and life/accidental death & dismemberment premiums and $600 for fitness expenses paid or reimbursed by us.

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.

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, 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.

- if Dr. Bedrosian is terminated by us without cause or resigns employment with us due to a constructive termination, she will be entitled to: (i) accelerated vesting of any equity-based compensation awards granted to her in connection with her employment as if she remained employed by us for an additional twelve (12) months following the date of termination; (ii) 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 (iii) 12 months of base salary continuation and a pro-rated annual bonus at the target in effect as of the date of termination.

Covered Transaction

Pursuant to the employment agreement with Dr. Kakkis, as amended and the offer letters with Ms. Sharp, Mr. Pinion, Dr. Bedrosian and Mr. Kassberg (including any amendments) 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 (or, 24 months in the case of Dr. Bedrosian) 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;

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

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

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

For purposes of each of the offer letters with Ms. Sharp, Mr. Pinion and Mr. Kassberg, “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;
- 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 the offer letter with Dr. Bedrosian, “cause” means her:

- commission of a felony or any crime involving dishonesty, breach of trust, or physical harm to any person;
- willful engagement in conduct that is in bad faith and materially injurious to us, including but not limited to misappropriation of trade secrets, fraud, or embezzlement;
- material breach of any agreement with us that is not cured within 10 days of written notice by us to her; 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 by us to her.

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, Mr. Pinion and Mr. Kassberg, “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 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.

For purposes of the offer letter with Dr. Bedrosian, "constructive termination" means the occurrence of any of the following events without her consent if (i) she provides us with written objection (or notice) to the event or condition within 90 days following the occurrence of the event or condition, (ii) we do not reverse or otherwise cure the event or condition within 30 days of receiving such written objection, and (iii) she resigns her employment with us within 30 days following the expiration of that cure period:

- a material reduction or change in her job duties, responsibilities, authority or requirements from her job duties, responsibilities, authority or requirements immediately prior to such reduction or change;
- a material reduction of her 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);
- a requirement that she relocate to a principal office that increases her one-way commute by more than 50 miles relative to her immediately preceding principal office; or
- a material breach of her offer letter agreement (including the failure to grant the new hire equity set forth in her offer letter within 30 days of her hire date) or any other agreement between her and us.

Shalini Sharp Transition Agreement

In March 2020, we announced that Shalini Sharp had informed us of her decision to voluntarily resign from her position as our Executive Vice President and Chief Financial Officer, effective as of the Officer Resignation Date. In connection with her resignation, Ms. Sharp and the Company entered into a transition letter agreement on March 5, 2020 (the " ***Transition Agreement*** "). Pursuant to the Transition Agreement, during the Transition Period, Ms. Sharp will continue to be an employee of the Company but will no longer have the powers, duties and responsibilities commensurate with the position of Executive Vice President and Chief Financial Officer and her primary responsibility will be to transition her duties and institutional knowledge to the new Chief Financial Officer, and to provide assistance on or lead projects as requested by our Chief Executive Officer and/or the new Chief Financial Officer. Prior to the Transition Period, Ms. Sharp will continue to receive her current annual base salary of $501,900, subject to standard withholdings and deductions. During the Transition Period, she will receive a reduced annual base salary of $380,000, subject to standard withholdings and deductions, and continue to remain eligible for all regular employee benefits. She will remain eligible to receive her annual cash incentive bonus for 2020, with the final amount to be determined based on the achievement of the Company's corporate performance goals, to be paid at the same time that bonuses for fiscal year 2020 performance are paid to the Company's other executives. Any options to purchase shares in the Company stock, shares of RSUs and PSUs previously granted to Ms. Sharp will continue to vest and become exercisable during the Transition Period in accordance with their terms and all vested equity awards will remain exercisable until 120 days after the Separation Date.

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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 2019.

Name	Grant Date	Estimated Future Payouts Under Non-Equity Incentive Plan Awards		Estimated Future Payouts Under Equity Incentive Plan Awards			Stock Awards: Number of Shares of Stock or Units Granted (#)(4)	Option Awards: Number of Securities Underlying Options Granted (#)	Exercise Price of Option Awards ($/ Share)	Grant Date Fair Value of Stock and Option Awards ($)(5)
		Target ($)(1)	Maximum ($)(2)	Threshold (#)(3)	Target (#)(3)	Maximum (#)(3)				
Emil D. Kakkis, M.D., Ph.D.		499,800	749,700	—	—	—	—	—	—	—
	3/1/2019	—	—	—	—	—	—	66,000	67.55	2,650,784
	3/1/2019	—	—	—	—	—	20,000	—	—	1,351,000
	3/1/2019	—	—	10,000	20,000	30,000	—	—	—	1,351,000
Shalini Sharp		219,275	312,466	—	—	—	—	—	—	—
	3/1/2019	—	—	—	—	—	—	23,000	67.55	923,758
	3/1/2019	—	—	—	—	—	8,000	—	—	540,400
	3/1/2019	—	—	3,000	6,000	9,000	—	—	—	405,300
John R. Pinion II		201,461	287,081	—	—	—	—	—	—	—
	3/1/2019	—	—	—	—	—	—	23,000	67.55	923,758
	3/1/2019	—	—	—	—	—	20,000	—	—	540,400
	3/1/2019	—	—	3,000	6,000	9,000	—	—	—	405,300
Camille L. Bedrosian, M.D.		231,232	329,506	—	—	—	—	—	—	—
	3/1/2019	—	—	—	—	—	—	21,500	67.55	863,513
	3/1/2019	—	—	—	—	—	7,000	—	—	472,850
	3/1/2019	—	—	3,000	6,000	9,000	—	—	—	405,300
Thomas Kassberg		213,300	303,953	—	—	—	—	—	—	—
	3/1/2019	—	—	—	—	—	—	21,500	67.55	863,513
	3/1/2019	—	—	—	—	—	7,000	—	—	472,850
	3/1/2019	—	—	3,000	6,000	9,000	—	—	—	405,300

(1) The amount represents the target amount of each named executive officer's cash payments under our 2019 annual incentive program as established by the Board and described in "Compensation Discussion and Analysis" above. Actual payments made for fiscal 2019 are provided in the Summary Compensation Table.

(2) The amount represents the maximum amount of each named executive officer's cash payments under our 2019 annual incentive program as established by the Board and described in "Compensation Discussion and Analysis" above. Actual payments made for fiscal 2019 are provided in the Summary Compensation Table.

(3) The amount represents the PSUs granted under our 2014 Incentive Plan. 50% of the PSUs would be earned upon achievement of the threshold level, 100% of the PSUs would be earned upon achievement of the target revenue and 150% of PSUs subject to the award would be earned upon achievement of the maximum target.

(4) The amounts represent the RSUs granted under our 2014 Incentive Plan.

(5) This column reflects the aggregate grant date fair value of equity awards granted in 2019 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.

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Outstanding Equity Awards at December 31, 2019

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2019.

		Option Awards				Stock Awards			
Name	Grant Date	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	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 Unearned Shares, Units or Other Rights That Have Not Vested (#)(3)	Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, Units or Other Rights That Have Not Vested ($)
Emil D. Kakkis, M.D., Ph.D.	3/1/2019	—	66,000	67.55	3/1/2029	20,000	854,200	20,000	854,200
	3/1/2018	41,344	53,156	48.43	3/1/2028	14,625	624,634	—	—
	12/22/2017	—	—	—	—	8,585	366,665	—	—
	3/1/2017	53,625	24,375	88.80	3/1/2027	6,500	277,615	—	—
	6/1/2016	55,757	7,963	70.57	6/1/2026	2,675	114,249	—	—
	5/21/2015	68,000	—	84.89	5/21/2025	—	—	—	—
	11/1/2013	47,853	—	6.86	11/1/2023	—	—	—	—
Shalini Sharp	3/1/2019	—	23,000	67.55	3/1/2029	8,000	341,680	6,000	256,260
	3/1/2018	15,313	19,687	48.43	3/1/2028	5,250	224,228	—	—
	12/22/2017	—	—	—	—	3,584	153,073	—	—
	3/1/2017	19,250	8,750	88.80	3/1/2027	2,000	85,420	—	—
	6/1/2016	21,000	3,000	70.57	6/1/2026	1,000	42,710	—	—
	5/21/2015	26,300	—	84.89	5/21/2025	—	—	—	—
	11/1/2013	2,998	—	6.86	11/1/2023	—	—	—	—
John R. Pinion II	3/1/2019	—	23,000	67.55	3/1/2029	20,000	854,200	6,000	256,260
	3/1/2018	11,813	15,187	48.43	3/1/2028	3,750	160,163	—	—
	12/22/2017	—	—	—	—	3,584	153,073	—	—
	3/1/2017	12,375	5,625	88.80	3/1/2027	1,350	57,659	—	—
	6/1/2016	15,575	2,225	70.57	6/1/2026	750	32,033	—	—
	6/3/2016	9,625	1,375	69.53	6/3/2026	—	—	—	—
	7/6/2015	90,000	—	124.87	7/6/2025	—	—	—	—
Camille L. Bedrosian, M.D.	3/1/2019	—	21,500	67.55	3/1/2029	7,000	298,897	6,000	256,260
	1/30/2018	42,167	45,833	55.00	1/30/2028	16,375	699,376	—	—
Thomas Kassberg	3/1/2019	—	21,500	67.55	3/1/2029	7,000	298,897	6,000	256,260
	3/1/2018	13,125	16,875	48.43	3/1/2028	4,500	192,195	—	—
	12/22/2017	—	—	—	—	3,584	153,073	—	—
	3/1/2017	17,188	7,812	88.80	3/1/2027	1,500	64,065	—	—
	6/1/2016	21,000	3,000	70.57	6/1/2026	1,000	42,710	—	—
	5/21/2015	26,300	—	84.89	5/21/2025	—	—	—	—
	11/1/2013	39,878	—	6.86	11/1/2023	—	—	—	—
	11/17/2011	109,734	—	0.31	11/17/2021	—	—	—	—

(1) The option vests with respect to 1/4 th of the shares underlying the option on the one-year anniversary of the grant date, and with respect to 1/48 th 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 RSUs vest with respect to 1/4 th of the underlying shares on each anniversary of the grant date over a four-year period; provided, however, that for (i) Dr. Kakkis, Ms. Sharp, Mr. Pinion and Mr. Kassberg, approximately 1/5 of the RSUs listed in the December 22, 2017 row will vest on each of April 19, 2020, May 6, 2020, October 14, 2020, May 6, 2021 and October 14, 2021 and for (ii) Dr. Bedrosian, with respect to 2,875 of the total RSUs listed in the January 30, 2018 row, approximately 1/4 of such 2,875 RSUs will vest on each on each of May 6, 2020, October 14, 2020, May 6, 2021 and October 14, 2021. 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 PSUs vest as described above under "Compensation Discussion and Analysis—Equity Compensation." Please see the section entitled "—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 2019.

	Option Awards		Stock Awards	
Name and Principal Position	**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	—	—	15,365	975,854
Shalini Sharp, Chief Financial Officer and Executive Vice President	43,843	1,986,773	5,566	352,274
John R. Pinion II, Chief Quality Officer and Executive Vice President, Translational Sciences	—	—	5,266	328,307
Camille L. Bedrosian, M.D., Chief Medical Officer and Executive Vice President	—	—	4,500	219,825
Thomas Kassberg, Chief Business Officer and Executive Vice President	—	—	5,066	318,499

(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 2019.

Nonqualified Deferred Compensation

During fiscal 2019, 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, RSU, and PSU vesting acceleration was calculated for each of the tables below based on the assumption that the change in control and executive's employment termination occurred on December 31, 2019. The closing price of our common stock on The Nasdaq Global Select Market as of December 31, 2019, the last trading day of 2019, was $42.71, 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 31, 2019 by the difference between the closing price of our common stock as of December 31, 2019 and the exercise price for such unvested option shares. No value is attributed to unvested options subject to acceleration which have exercise prices above the closing market price of our common stock as of December 31, 2019. The value of the RSU and PSU vesting acceleration was calculated by multiplying the number of unvested RSUs and PSUs subject to vesting acceleration as of December 31, 2019 by the closing price of our common stock as of December 31, 2019.

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 31, 2019, the last business day of the fiscal year.

Potential Payments Upon Termination or Change of Control	Termination by Company without Cause	Termination by Company Upon Death or Disability	Resignation for Good Reason following a Change in Control	Termination by Company without Cause following a Covered Transaction	Resignation for Good Reason following a Covered Transaction
Base Salary	$ 1,428,000	$ 1,428,000	$ 1,428,000	$ 1,428,000	—
Acceleration of equity awards	—	—	—	$ 3,091,563	$ 3,091,563
Total	$ 1,428,000	$ 1,428,000	$ 1,428,000	$ 4,519,563	$ 3,091,563

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 31, 2019, the last business day of the fiscal year.

Potential Payments Upon Termination or Change of Control	Termination by Company without Cause	Resignation due to a Constructive Termination	Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction
Base Salary	$ 487,277	$ 487,277	$ 487,277
Acceleration of equity awards	—	—	$ 1,103,370
Total	$ 487,277	$ 487,277	$ 1,590,647

Mr. John R. Pinion II

The following table describes the potential payments upon employment termination for John R. Pinion II, our Chief Quality Officer and Executive Vice President, Translational Sciences, as if his employment terminated as of December 31, 2019, the last business day of the fiscal year.

Potential Payments Upon Termination or Change of Control	Termination by Company without Cause	Resignation due to a Constructive Termination	Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction
Base Salary	$ 447,690	$ 447,690	$ 447,690
Acceleration of equity awards	—	—	$ 1,513,386
Total	$ 447,690	$ 447,690	$ 1,961,076

Dr. Camille L. Bedrosian

The following table describes the potential payments upon employment termination for Camille Bedrosian, our Chief Medical Officer and Executive Vice President, as if her employment terminated as of December 31, 2019, the last business day of the fiscal year.

Potential Payments Upon Termination or Change of Control	Termination by Company without Cause	Resignation due to a Constructive Termination	Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction
Base Salary	$ 513,849	$ 513,849	$ 513,849
Bonus	231,232	231,232	231,232
Acceleration of equity awards	412,835	412,835	1,254,606
Total	$ 1,157,916	$ 1,157,916	$ 1,999,687

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 31, 2019, the last business day of the fiscal year.

Potential Payments Upon Termination or Change of Control	Termination by Company without Cause	Resignation due to a Constructive Termination	Termination by Company without Cause or resignation due to a Constructive Termination following a Covered Transaction
Base Salary	$ 474,000	$ 474,000	$ 474,000
Acceleration of equity awards	—	—	$ 1,007,273
Total	$ 474,000	$ 474,000	$ 1,481,273

EQUITY COMPENSATION PLAN INFORMATION

The table below discloses information as of December 31, 2019 with respect to our equity compensation plans that have been approved by stockholders and equity compensation plans that have not been approved by stockholders.

Plan Category	Number of Securities to be Issued upon Exercise of Outstanding Options, Warrants and Rights(a)	Weighted-Average Exercise Price of Outstanding Options, Warrants and Rights	Number of Securities Remaining Available for Future Issuance under Equity Compensation Plans (Excluding Securities Reflected in Column (a))
Equity compensation plans approved by security holders:			
2011 Equity Incentive Plan, as amended	443,992	$ 5.26	—
2014 Incentive Plan	7,255,223	$ 67.67	2,710,617
2014 Employee Stock Purchase Plan			2,703,237
Equity compensation plans not approved by security holders (1)			
Dimension Therapeutics, Inc. 2015 Stock Option and Incentive Plan	71,651	$ 36.64	—
Dimension Therapeutics, Inc. 2013 Stock Plan	39,888	$ 26.61	—
Total	7,810,754	$ 62.51	

(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 December 2018 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 2019. The assessment found that the cash and equity compensation levels for non-employee directors was positioned generally at the 50 th percentile as compared to our 2019 peer group and as a result, the Compensation Committee made no changes at that time to the annual retainers and equity awards.

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A summary of the non-employee director compensation arrangements for fiscal 2019 is set forth below:

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

In 2020, to align the cash compensation levels for non-employee directors serving as chairman or members of certain committees to the 50th percentile as compared to our peer group in 2020, the Compensation Committee increased the annual retainer for the chairman of the Compensation Committee and the chairman of the Research and Development Committee to $20,000 and $14,000, respectively, and for the members of the Compensation Committee and Research and Development Committee to $10,000 and $7,000, respectively.

Under the non-employee director compensation policy for fiscal 2019, each non-employee director who is initially appointed or elected to the Board receives an option grant to purchase up to 20,000 shares of our common stock under our stock option plan on the date he or she first becomes a non-employee director, which will vest monthly over a three-year period, subject to the holder’s continued service to us through each such vesting date. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director is eligible to receive an annual option grant to purchase up to 5,000 shares of our common stock and an annual grant of 3,000 RSUs, 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 RSUs are 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 2019 by our non-employee directors.

Name	Fees Earned in Fiscal 2019	Stock Awards (1)	Option Awards (2)	Total
Daniel G. Welch	$ 92,500	$ 176,220	$ 161,670	$430,390
William Aliski	75,000	176,220	161,670	412,890
Deborah Dunsire, M.D.	56,000	176,220	161,670	393,890
Lars Ekman, M.D., Ph.D.	62,000	176,220	161,670	399,890
Matthew K. Fust	80,000	176,220	161,670	417,890
Michael Narachi	66,250	176,220	161,670	404,140
Clay B. Siegall, Ph.D.	60,500	176,220	161,670	398,390
Shehnaaz Suliman, M.D.	57,000	176,220	710,878	944,098

(1) The amounts reported in this column for a fiscal year represent the grant date fair value of the RSUs 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 RSUs reported in this column are set forth in the notes to our financial statements included in our Annual Report. The amounts reported in this column reflect the accounting cost for these RSUs, and do not correspond to the actual economic value that may be received by the non-employee directors from the RSUs. As of December 31, 2019, our non-employee directors had the following outstanding RSUs: Mr. Welch – 3,000; Mr. Aliski – 3,000; Dr. Dunsire – 3,000; Dr. Ekman—3,000; Mr. Fust—3,000; Mr. Narachi – 3,000; Dr. Siegall – 3,000; and Dr. Suliman – 3,000.

(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 2019, 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. 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, 2019, our non-employee directors had the following outstanding options: Mr. Welch – 42,500; Mr. Aliski – 32,500; Dr. Dunsire – 28,750; Dr. Ekman – 32,500; Mr. Fust – 43,681; Mr. Narachi – 42,500; Dr. Siegall – 50,000; and Dr. Suliman – 25,000.

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. This 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 2019, the annual total compensation for Dr. Emil Kakkis, our Chief Executive Officer and President, was $6,586,479 and for our median employee was $323,253, resulting in a pay ratio of 20:1.

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 2019 on the calculation date, (B) the target bonus for 2019, and (C) the accounting value of any equity awards granted during 2019, 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, 2019, whether employed on a full-time, part-time, or seasonal basis. For any permanent employees who were only employed for part of the 2019 fiscal year, we annualized their compensation to present a more accurate representation of their comparative annual compensation. On October 1, 2019, we had 736 employees.

OTHER BUSINESS

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

STOCKHOLDER PROPOSALS

Stockholder proposals intended to be included in the proxy statement for the 2021 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 30, 2020. 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 2021 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 2020 annual meeting (that is, between February 26, 2021 and March 28, 2021); *provided, however* , that if the 2021 annual meeting date is advanced by more than 30 days before or delayed by more than 60 days after the anniversary date of the 2020 annual meeting, then such notice must be received on or before 10 days after the day on which the date of the 2021 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, 2019, 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* . 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) 475-6876. Similarly, if you share an address with another stockholder and have received multiple copies of our proxy statement and annual report or Notice, you may write or call us at the address and phone number above to request delivery of a single copy of these materials in the future. If your stock is held through a brokerage firm, bank or other financial institution and you prefer to receive separate copies of our proxy statement and annual report or Notice, as applicable, either now or in the future, please contact your brokerage firm, bank or other financial institution.

EACH STOCKHOLDER IS URGED TO VOTE VIA THE INTERNET AS INSTRUCTED IN THE NOTICE OF INTERNET AVAILABILITY 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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ULTRAGENYX PHARMACEUTICAL INC.
60 LEVERONI COURT
NOVATO, CA 94949

VOTE BY INTERNET
Before The Meeting - Go to **www.proxyvote.com**

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

During The Meeting - Go to **www.virtualshareholdermeeting.com/RARE20**

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

VOTE BY PHONE - 1-800-690-6903
Use any touch-tone telephone to transmit your voting instructions up until 11:59 P.M. Eastern Time the day before the meeting date. Have your proxy card in hand when you call and then follow the instructions.

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

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

D11838-P35297 KEEP THIS PORTION FOR YOUR RECORDS

THIS PROXY CARD IS VALID ONLY WHEN SIGNED AND DATED. DETACH AND RETURN THIS PORTION ONLY

ULTRAGENYX PHARMACEUTICAL INC.

Company Proposals

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

Nominees:	For	Withhold
1a. Emil D. Kakkis, M.D., Ph.D.	☐	☐
1b. Shehnaaz Suliman, M.D.	☐	☐
1c. Daniel G. Welch	☐	☐

The Board of Directors recommends you vote FOR proposals 2 and 3:

	For	Against	Abstain
2. Ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2020.	☐	☐	☐
3. Advisory (non-binding) vote to approve the compensation of our named executive officers.	☐	☐	☐

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

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

Signature [PLEASE SIGN WITHIN BOX] Date

Signature (Joint Owners) Date

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Important Notice Regarding the Availability of Proxy Materials for the Annual Meeting:
The Notice and Proxy Statement and Annual Report on Form 10-K for the year ended December 31, 2019 are available at www.proxyvote.com.

D11839-P35297

ULTRAGENYX PHARMACEUTICAL INC.

Annual Meeting of Stockholders

June 26, 2020 8:00 a.m., Pacific Time

This proxy is solicited by the Board of Directors of Ultragenyx Pharmaceutical Inc.

The stockholder(s) hereby appoint(s) Emil D. Kakkis, M.D., Ph.D. and Karah Parschauer, or either of them, as proxies, each with the power to appoint his or her substitute, and hereby authorize(s) them to represent and to vote, as designated on the reverse side of this ballot, all of the shares of Common Stock of Ultragenyx Pharmaceutical Inc. that the stockholder(s) is/are entitled to vote at the 2020 Annual Meeting of Stockholders to be held at 8:00 a.m., Pacific Time, on June 26, 2020, and at any adjournment or postponement thereof.

This proxy, when properly executed, will be voted in the manner directed herein. If the proxy is properly executed and delivered, but no such direction is made, this proxy will be voted in accordance with the Board of Directors' recommendations and in the discretion of the proxy holders on all other matters that may come before the meeting or any adjournment or postponement thereof (including, if applicable, for the election of a person to the Board of Directors if any nominee named in Proposal No. 1 becomes unavailable for election at the Annual Meeting).

Address Changes/Comments: ______________________________

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

Continued and to be signed on reverse side