

## SCHEDULE 14A

Proxy Statement Pursuant to Section 14(a) of the Securities  
Exchange Act of 1934 (Amendment No.)



Filed by the Registrant



Filed by a Party other than the Registrant

**Check the appropriate box:**

<input type="checkbox"/>	Preliminary Proxy Statement
<input type="checkbox"/>	Confidential, for use of the Commission only (as permitted by Rule 14a-6(e)(2))
<input checked="" type="checkbox"/>	Definitive Proxy Statement
<input type="checkbox"/>	Definitive Additional Materials
<input type="checkbox"/>	Soliciting Material Pursuant to §240.14a-12

## ULTRAGENYX PHARMACEUTICAL INC.



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

*(Name of Person(s) Filing Proxy Statement, if other than the Registrant)*

**Payment of Filing Fee (Check all boxes that apply):**

<input checked="" type="checkbox"/>	No fee required
<input type="checkbox"/>	Fee paid previously with preliminary materials
<input type="checkbox"/>	Fee computed on table in exhibit required by Item 25(b) per Exchange Act Rules 14a-6(i)(1) and 0-11

A large, stylized green 'X' graphic that spans across the page, starting from the top right and extending towards the bottom left. It is composed of two thick, curved lines that meet at a central point.

# 2024 PROXY STATEMENT

June 18, 2024

9:30 a.m. Pacific Time

[www.virtualshareholdermeeting.com/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024)

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60 Leveroni Court  
Novato, California 94949

# Notice of Annual Meeting of Stockholders

To Be Held on  
June 18, 2024 at  
9:30 a.m. Pacific Time

Dear Stockholder:

You are cordially invited to attend the Annual Meeting of Stockholders of Ultragenyx Pharmaceutical Inc., a Delaware corporation (we, us, Ultragenyx or the Company), which will be held on June 18, 2024, at 9:30 a.m. Pacific Time virtually via the Internet at [www.virtualshareholdermeeting.com/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024) (Annual Meeting). Instructions on how to participate in the Annual Meeting and demonstrate proof of stock ownership are included in this proxy statement (Proxy Statement). The webcast of the Annual Meeting will be archived for one year after the date of the Annual Meeting at [www.virtualshareholdermeeting.com/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024). Only stockholders who held stock at the close of business on the record date, April 23, 2024, may vote at the Annual Meeting or any adjournment or postponement thereof.

In the event of a technical malfunction or other situation that the meeting chair determines may affect the ability of the Annual Meeting to satisfy the requirements for a meeting of stockholders to be held by means of remote communication under the Delaware General Corporation Law, or that otherwise makes it advisable to adjourn the Annual Meeting, the chair or secretary of the Annual Meeting will convene the meeting at 12:00 p.m. Pacific Time on the date specified above and at the Company's address specified above solely for the purpose of adjourning the meeting to reconvene at a date, time and physical or virtual location announced by the meeting chair. Under either of the foregoing circumstances, we will post information regarding the announcement on the Investors page of the Company's website at <https://ir.ultragenyx.com>.

At the Annual Meeting, you will be asked to consider and vote upon: (1) the election of the three Class II director nominees named in the Proxy Statement; (2) approval of our amended and restated 2023 Incentive Plan (A&R 2023 Plan), (3) the ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2024; (4) an advisory (non-binding) resolution to approve the compensation of our named executive officers; and (5) any other business that may properly come before the Annual Meeting or any adjournment or postponement thereof. No other items of business are expected to be considered, and no other director nominees will be entertained, at the Annual Meeting.

The accompanying Proxy Statement more fully describes the details of the business to be conducted at the Annual Meeting. Proposal 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 of the other proposals described in the Proxy Statement.

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We are pleased to make use of the U.S. Securities and Exchange Commission (SEC) 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 26, 2024

**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 MATERIALS, COMPLETE, DATE, SIGN, AND RETURN THE ENCLOSED PROXY CARD USING THE ENCLOSED RETURN ENVELOPE OR VOTING INSTRUCTION FORM PROVIDED WITH THE PRINTED PROXY MATERIALS, AS PROMPTLY AS POSSIBLE SO THAT YOUR SHARES MAY BE REPRESENTED AT THE ANNUAL MEETING. YOU MAY ALSO VOTE AT THE VIRTUAL ANNUAL MEETING**

**IMPORTANT NOTICE REGARDING THE AVAILABILITY OF PROXY MATERIALS FOR THE ANNUAL MEETING OF STOCKHOLDERS TO BE HELD ON JUNE 18, 2024:**

The Proxy Statement and Annual Report on Form 10-K for the year ended December 31, 2023 are available at [www.proxyvote.com](http://www.proxyvote.com).

**VOTING METHODS**



If you are an owner of record as of the record date, you may vote via any of the following methods:

**INTERNET**

Visit the website at:  
[www.proxyvote.com](http://www.proxyvote.com)



**TELEPHONE**

If you requested and received a proxy card, call toll-free at 1-800-690-6903



**MAIL**

If you requested and received a proxy card, sign, date and mail the proxy card in the enclosed envelope



**AT THE MEETING:**

Vote at the meeting by going to:  
[www.virtualshareholdermeeting.com/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024)

If you are a beneficial owner of shares held through a broker, bank or other owner of record, you must follow the voting instructions you receive from the owner of record to vote your shares.

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# Proxy Statement Overview

This overview highlights certain information contained elsewhere in this Proxy Statement and does not contain all of the information that you should consider. You should read the entire Proxy Statement carefully before voting. For more complete information regarding our business and 2023 performance, please review our 2023 Annual Report on Form 10-K as filed with the U.S. Securities and Exchange Commission (SEC) in February 2024.

## Meeting and Voting Information

		
<b>Meeting Date and Meeting Time</b>	<b>Record Date</b>	<b>Location</b>
June 18, 2024 9:30 a.m. Pacific Time	April 23, 2024	Live via audio webcast at <a href="http://www.virtualshareholdermeeting.com/RARE2024">www.virtualshareholdermeeting.com/RARE2024</a>

We intend to mail the Notice Regarding the Availability of Proxy Materials containing instructions on how to access this Proxy Statement and our 2023 Annual Report on Form 10-K beginning on or about April 26, 2024 to all stockholders entitled to vote at the Annual Meeting.

Proposals	Board Vote Recommendation	For More Information See Page
<b>Proposal 1</b> Election of Class II Directors	<b>FOR</b> each nominee	9
<b>Proposal 2</b> Approval of the A&R 2023 Plan	<b>FOR</b>	14
<b>Proposal 3</b> Ratification of the selection of Ernst & Young LLP as our independent auditor	<b>FOR</b>	22
<b>Proposal 4</b> Say on Pay	<b>FOR</b>	25

## Business Highlights

In 2023, we generated significant momentum across our clinical and commercial programs to position our company for what we expect to be a catalyst-rich 2024.

Our key financial and operational accomplishments in 2023 included:

**27%**  
increase in  
Dojolvi sales

**77%**  
increase in  
Crysvita sales in  
Latin America  
and Turkey

### Revenue and Commercial Highlights

We achieved \$434 million in total revenue in 2023 compared to total revenue of \$363 million in 2022, which represented 20% growth. We achieved continued strong growth in product sales in 2023 compared to the prior year. Revenue from product sales of Crysvita<sup>®</sup> in countries where we lead commercialization activities increased from \$43 million to \$76 million, or by 77%, compared to 2022, largely due to sales in Latin America. Total revenue from Crysvita<sup>®</sup> sales, including royalty revenue we received from our partner, Kyowa Kirin, from sales of the product in North America (following the transition of our commercialization responsibilities in North America to Kyowa Kirin in 2023) and in the European territory increased by 17% from \$279 million in 2022 to \$328 million in 2023. Global revenue from Dojolvi<sup>®</sup> increased by 27% from \$56 million in the prior year to \$71 million in 2023 and global revenue from Mepsevii<sup>®</sup> increased by 48% from \$21 million in 2022 to \$30 million in 2023.

In 2023, we continued to make progress on international regulatory and reimbursement negotiations across our marketed products and continued to add new approvals and positive reimbursement decisions, specifically in certain launch markets for Evkeeza.

## Program Highlights and Other Achievements

We hosted a Company Analyst Day in October 2023 where we announced meaningful updates and data from three of our key clinical programs: UX143 (setrusumab) monoclonal antibody for the treatment of Osteogenesis Imperfecta, GTX-102 antisense oligonucleotide (ASO) for the treatment of Angelman syndrome and UX701 for the treatment of Wilson Disease. We also continued to make important progress on our other clinical pipeline programs.

See page 40 for more information about our achievements in 2023.









## Corporate Responsibility Highlights

At Ultragenyx, we are committed to bringing novel products to patients for the treatment of rare and ultrarare diseases, with a focus on serious, debilitating genetic diseases. Our purpose is to lead the future of rare disease medicine as we seek to treat individuals afflicted by diseases with limited or no treatment options. We recognize that their lives and well-being are dependent upon our efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care. For more information about our Corporate Responsibility efforts and initiatives, please see our Corporate Responsibility Report for fiscal year 2023 (2023 Corporate Responsibility Report), which will be available on our website at [www.ultragenyx.com](http://www.ultragenyx.com) under "Ultra-Committed – Corporate Responsibility". Website references throughout this document are provided for convenience only, and the content on the referenced websites is not incorporated by reference into this document.

See page 31 for more information about our Corporate Responsibility highlights in 2023.

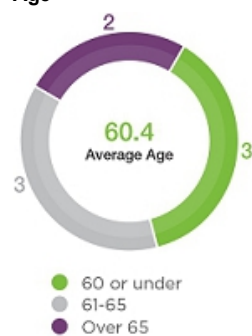
## Board of Directors

The following table provides summary information about each nominee for director at the Annual Meeting and our continuing directors, as of the date of this Proxy Statement.

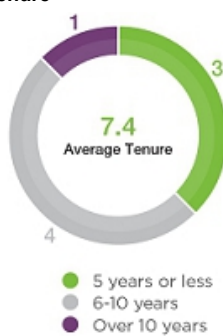
Nominee/Director Name	Age	Principal Occupation	Director Since	Year Current Term Expires	Current Director Class	Independent
<b>Director Nominees</b>						
<b>Deborah Dunsire, M.D.</b>	61	Board member and advisor to various life science companies	2017	2024	II	
<b>Michael Narachi</b>	64	Board member and advisor to various life science companies	2015	2024	II	
<b>Corsee D. Sanders, Ph.D.</b>	67	Board member and advisor to various life science companies	2021	2024	II	
<b>Continuing Directors</b>						
<b>Matthew K. Fust</b>	59	Board member and advisor to various life science companies	2014	2025	III	
<b>Amrit Ray, M.D.</b>	51	Board member and advisor to various life science companies	2022	2025	III	
<b>Emil D. Kakkis, M.D., Ph.D.</b>	63	President and Chief Executive Officer, Ultragenyx	2010	2026	I	
<b>Shehnaaz Suliman, M.D.</b>	52	Chief Executive Officer of ReCode Therapeutics	2019	2026	I	
<b>Daniel G. Welch</b>	66	Board member and advisor to various life science companies	2015	2026	I	

Board Composition Highlights

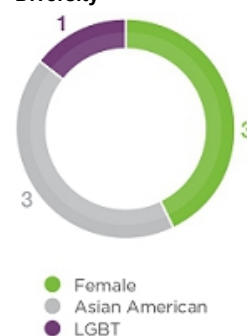
Age



Tenure



Diversity



Board Diversity

Our Nominating and Corporate Governance Committee places significant emphasis on diversity and actively considers whether potential candidates would 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. Currently, three of our directors (38%) self-identify as Asian, one (13%) self-identifies as LGBTQ+ and three (38%) are women. We also have a female chairperson of our Nominating and Corporate Governance Committee. In accordance with Nasdaq's board diversity listing standards, below is the aggregate statistical information about our Board's self-identified gender and racial characteristics and LGBTQ+ status as voluntarily confirmed to us by each of our directors.

Board Diversity Matrix (as of the date of this Proxy Statement)

Total Number of Directors:	8			
	Female	Male	Non-Binary	Did not Disclose Gender
<b>Gender</b>	3	5	—	—
<b>Number of Directors who identify in any of the categories below:</b>				
African American or Black	—	—	—	—
Alaskan Native or Native American	—	—	—	—
Asian	2	1	—	—
Hispanic or Latinx	—	—	—	—
Native Hawaiian or Pacific Islander	—	—	—	—
White	1	4	—	—
Two or More Races or Ethnicities	—	—	—	—
LGBTQ+	—	1	—	—
Did not disclose demographic background	—	—	—	—



## Board Diversity, Skills and Experience

Our Nominating and Corporate Governance Committee also considers numerous other skills, qualities and experiences when evaluating director nominees, including whether the nominee has specific strengths that would augment existing skills and experiences of the Board and in a manner that is aligned with the company's strategic direction. The following matrix highlights each director's primary skills or knowledge in these areas as identified by the Nominating and Corporate Governance Committee. The matrix does not encompass all of the knowledge, skills, experiences or attributes of our directors, and the fact that a particular knowledge, skill, experience or attribute is not listed does not mean that a director does not possess it. In addition, the absence of a particular knowledge, skill, experience or attribute with respect to any of our directors does not mean the director in question is unable to contribute to the decision-making process in that area. The type and degree of knowledge, skill and experience listed below may vary among the members of the Board. In response to stockholder feedback, we also provide below enhanced disclosure regarding the diversity of our Board.

	Welch	Kakkis	Dunsire	Fust	Narachi	Ray	Sanders	Suliman
<b>Skills and Experience</b>								
<i>Core Board Capabilities</i>								
Biopharma C-level leadership	✓	✓	✓	✓	✓	✓	✓	✓
Scientific and research leadership		✓				✓	✓	✓
Clinical development leadership		✓	✓		✓	✓	✓	✓
Regulatory leadership		✓			✓	✓	✓	
Rare disease commercial experience	✓	✓	✓					
Biopharma commercial leadership	✓		✓		✓	✓		
Global access, pricing and reimbursement	✓		✓		✓	✓		
Global business operations	✓	✓	✓	✓	✓	✓	✓	✓
Finance and capital markets	✓		✓	✓	✓			✓
Corporate strategy and/or business development leadership	✓	✓	✓		✓	✓		✓
Corporate governance and board experience	✓	✓	✓	✓	✓	✓	✓	✓
<i>Relevant / Advisory Capabilities</i>								
Compliance (GXP, commercial)	✓	✓	✓	✓	✓	✓	✓	
Corporate legal and IP	✓		✓		✓			✓
Government affairs and policy	✓	✓	✓		✓	✓		
Human resources and organizational development	✓	✓	✓	✓	✓	✓	✓	✓
Manufacturing/supply chain	✓	✓	✓		✓			
<b>Diversity</b>								
<b>Gender</b>								
Male	✓	✓		✓	✓	✓		
Female			✓				✓	✓
<b>Race/Ethnicity</b>								
Asian						✓	✓	✓
White	✓	✓	✓	✓	✓			
LGBTQ+				✓				

## Corporate Governance Overview

We are committed to maintaining good corporate governance practices and we periodically review our practices. We believe that good corporate governance, including the practices listed below, promotes the long term interests of our stockholders.

- All of our directors are independent, other than our President and Chief Executive Officer
- Director Resignation Policy that applies when a director fails to receive majority support in an uncontested election
- Completely independent Audit Committee, Nominating and Corporate Governance Committee and Compensation Committee
- Effective and active independent Chairman
- Annual Board and committee self-evaluations
- Active stockholder engagement program
- All our current directors attended at least 90% of board and committee meetings of which the director was a member in 2023
- Minimum stock ownership requirements for named executive officers and directors
- Director Overboarding Policy limiting the total number of public company boards that a director may serve to five total public company boards and public company CEO directors to three total public company boards
- Board and committees are authorized to engage outside advisors independently of management
- Clawback Policy that complies with Rule 10D-1 under the Exchange Act and also includes a discretionary recoupment provision that permits recovery of all incentive compensation (including time-based and performance-based equity awards) in the event of fraud or intentional misconduct (see section entitled "Clawback Policy" of this Proxy Statement for additional details)
- Prohibition against hedging and pledging transactions by our directors and employees, including our executive officers
- Corporate Governance Guidelines and robust Global Code of Conduct

## PROPOSAL 1

# Election of Class II Directors

Our Amended and Restated Certificate of Incorporation provides that the Board is to be divided into three classes as nearly equal in number as reasonably possible, with directors in each class generally serving three-year terms. The total Board size is currently fixed at eight directors. The Class II directors (whose terms expire at the Annual Meeting) are Deborah Dunsire M.D., Michael Narachi and Corsee D. Sanders, Ph.D. Dr. Dunsire and Mr. Narachi were most recently elected by stockholders at the 2021 Annual Meeting; Dr. Sanders was recommended by a third party search firm and appointed to the Board after the 2021 Annual Meeting. The Class III directors (whose terms expire at the 2025 Annual Meeting) are Matthew K. Fust and Amrit Ray, M.D. The Class I directors (whose terms expire at the 2026 Annual Meeting) are Emil D. Kakkis, M.D., Ph.D., Shehnaaz Suliman, M.D., and Daniel G. Welch. The Class II directors elected at the Annual Meeting will hold office until the 2027 annual meeting of stockholders or 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 (bylaws).

As described below, the Board, upon the recommendation of the Nominating and Corporate Governance Committee, has nominated the director nominees listed above for election as directors at the Annual Meeting. They have indicated their willingness and ability to serve if elected. Should any of them become unable or, for good cause, unwilling to serve, the persons named on the enclosed proxy card 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 above under "Proxy Statement Overview – Board Diversity, Skills and Experience", 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. In 2023, the Board adopted a "Rooney Rule" policy in the Company's Corporate Governance Guidelines, which provides that as part of the search process for each new director, the Nominating and Corporate Governance Committee includes women and minorities in the pool of candidates (and instructs any search firm the Nominating and Corporate Governance Committee engages to do so) and interviews at least one woman or one minority candidate.

Additionally, the Nominating and Corporate Governance Committee considers whether a nominee will be able to dedicate sufficient time to, and focus on, his or her duties as a member of the Board. The Board membership criteria are set forth in our Corporate Governance Guidelines, a copy of which is available on our website at [www.ultragenyx.com](http://www.ultragenyx.com) in the "Corporate Governance" subsection of the "Investors" tab. The Nominating and Corporate Governance Committee assesses its effectiveness in balancing these considerations in connection with its annual evaluation of the composition of the Board.

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 and in the same manner as director candidates recommended by other sources. 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 submit such recommendation to the Nominating and Corporate Governance Committee as described in the section titled "Stockholder Communications" and 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 deadline for nominating director candidates set forth below under the question "When are other proposals and director nominations for next year's annual meeting due?" in the section entitled "Additional Information".

## Nominees and Incumbent Directors

Information regarding our director nominees and our current directors, including their respective age as of the date of this Proxy Statement and their principal occupation, is set forth below.

### Class II Directors Nominated for Election

#### Deborah Dunsire, M.D.

##### Board member and advisor to various life science companies

**Age:** 61  
**Director since:** 2017  
**INDEPENDENT**  
**Committees:** 2  
**Other Public Directorships:** 1

Dr. Dunsire served as President and Chief Executive Officer of H. Lundbeck A/S, a pharmaceutical company from September 2018 until August 2023. 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 currently serves on the board of Syros Pharmaceuticals, a publicly traded biotechnology company, and previously served as a director of Allergan, Inc., a publicly traded pharmaceutical company, from December 2006 until April 2015 and a director of Alexion Pharmaceuticals Inc., a publicly traded biopharmaceutical company, from January 2018 until July 2021 when it was acquired by AstraZeneca. She obtained an MBCh from the University of the Witwatersrand.

##### Skills and Qualifications specific to Ultragenyx:

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

##### Board member and advisor to various life science companies

**Age:** 64  
**Director since:** 2015  
**INDEPENDENT**  
**Committees:** 2  
**Other Public Directorships:** 0

Mr. Narachi previously served as President and Chief Executive Officer of CODA Biotherapeutics, Inc., a private biotherapeutics company, from August 2018 through October 2022 and served as a director of CODA Biotherapeutics until the company's sale in March 2023. Between March 2009 and July 2018, Mr. Narachi served as President, Chief Executive Officer and 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, until his retirement in 2004. Mr. Narachi joined Amgen in 1984 and held various senior positions throughout the organization over a 20-year career including global development leader for Neupogen/Neulasta, Vice President of development and representative director for Amgen Japan; head of corporate strategic planning; Chief Operations Officer of Amgen Colorado; and vice president, licensing and business development. Mr. Narachi previously served on the board of directors of BIO, the Biotechnology Innovation Organization, as a member of the board of directors of PhRMA, the Pharmaceutical Research and Manufacturers of America and 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 holds a B.S. in Biology and an M.A. in Biology and Genetics from the University of California at Davis. He also holds an M.B.A. from the Anderson Graduate School of Management at University of California, Los Angeles.

##### Skills and Qualifications specific to Ultragenyx:

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.

**Corsee D. Sanders, Ph.D.****Board member and advisor to various life science companies**

**Age: 67**  
**Director since: 2021**  
**INDEPENDENT**  
**Committees: 2**  
**Other Public Directorships: 3**

Dr. Sanders currently serves as a board member and advisor to life science companies. Dr. Sanders currently serves as a member of the board of directors of Beigene Ltd., Molecular Templates Inc. and Legend Biotech Corporation, each a publicly traded biotechnology company, and AltruBio Inc., a private biopharmaceutical company. She currently serves as a co-chair of the Board of Advisors, and Chair of the Science and Technology Advisory Committee for the Fred Hutchinson Cancer Center. She most recently served as Executive Vice President at Juno Therapeutics, Inc., a biopharmaceutical company, from 2017 to 2018 and strategic advisor to the Chief Medical Officer of Celgene Corporation, a biopharmaceutical company, from 2018 to 2019 following Juno's acquisition by Celgene. Following the acquisition of Celgene by Bristol Myers Squibb, Dr. Sanders served as Transition Advisor to the Clinical Development team at BMS from 2019 to 2020. Prior to her role at BMS, she held positions of increasing responsibility at Genentech/Roche, a biotechnology company, from 1994 to 2017, including serving as Senior Vice President, Global Head of Clinical Operations and Industry Collaboration, from 2012 to 2017. Dr. Sanders holds a B.S. and M.S. in Statistics, *magna cum laude*, from the University of the Philippines. She also holds an M.A. and Ph.D. in Statistics from the Wharton Doctoral Program at the University of Pennsylvania.

**Skills and Qualifications specific to Ultragenyx:**

We believe that Dr. Sanders is qualified to serve on our Board due to her established and extensive experience in global clinical development and her role as an advisor and director to various companies in the biotechnology and pharmaceutical sectors, all of which give her the skills to provide us with operational and strategic insights.

**Class III Directors Continuing in Office Until 2025****Matthew Fust****Board member and advisor to various life science companies**

**Age: 59**  
**Director since: 2014**  
**INDEPENDENT**  
**Committees: 2**  
**Other Public Directorships: 3**

Mr. Fust is a board member and advisor to life science companies. Mr. Fust currently serves on the board of directors of Atara Biotherapeutics, Inc., Crinetics Pharmaceuticals, Inc. and Neumora Therapeutics Inc., all of which are publicly traded biopharmaceutical companies. Mr. Fust also previously served on the board of directors of Sunesis Pharmaceuticals, Inc., a biotechnology company, from May 2005 to May 2017, the board of Dermira, Inc., a biopharmaceutical company, from August 2014 to February 2020 and the board of MacroGenics, Inc., a biotechnology company, from March 2014 to May 2020. He retired as Executive Vice President of Onyx Pharmaceuticals, Inc., a biopharmaceutical company, where he served from January 2009 to 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 to 2002. From 1991 to 1996, Mr. Fust was a manager in the healthcare strategy practice at Andersen Consulting, a consulting company. Mr. Fust holds a B.A. in Accounting from the University of Minnesota and an M.B.A. from the Stanford Graduate School of Business.

**Skills and Qualifications specific to Ultragenyx:**

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.

**Amrit Ray, M.D.****Board member and advisor to various life science companies**

**Age: 51**  
**Director since: 2022**  
**INDEPENDENT**  
**Committees: 1**  
**Other Public Directorships: 1**

Dr. Ray currently serves as a board member and advisor to life science companies. Previously, Dr. Ray served as Chief Patient Officer at Biohaven Pharmaceuticals, a public biopharmaceutical company, from March 2022 to December 2022 when acquired by Pfizer. Prior to his role at Biohaven, he served as Senior Adviser to Bain Capital Life Sciences, an investment company, from February 2021 to March 2022. Prior to Bain Capital, Dr. Ray served as Global President, Head of R&D and Medical, for Pfizer Essential Health and subsequently the Pfizer Upjohn division at Pfizer, Inc., a public pharmaceutical company, from 2017 to January 2021. Prior to Pfizer, he held positions of increasing responsibility at Johnson & Johnson, a public pharmaceutical company, including serving as Senior Vice President, External Affairs (Science and Medicine) in 2017, Senior Vice President, Chief Medical Officer of Janssen from 2012 to 2017 and Senior Vice President, Chief Safety Officer from 2009 to 2012. Dr. Ray currently serves on the board of directors of Fortrea, a publicly traded, global clinical research organization, and on the board of directors of several privately owned life science companies. Dr. Ray currently serves as a Trustee at the Board of the Hastings Center for Bioethics, and as a Visiting Professor of Practice, Faculty of Medical Sciences at Newcastle University in the United Kingdom. Dr. Ray holds a B.S., with Honours, in Immunology and a M.D. (M.B., Ch.B.) from the University of Edinburgh. He also holds an M.B.A. from the Tuck School of Business at Dartmouth College.

**Skills and Qualifications specific to Ultragenyx:**

We believe that Dr. Ray is qualified to serve on our Board due to his extensive experience in the life sciences industry, and particularly his research and development expertise.

## Class I Directors Continuing in Office until 2026

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

Founder, President and CEO

**Age: 63**  
**Director since: 2010**  
**NOT INDEPENDENT**  
**Committees: 1**  
**Other Public Directorships: 0**

Dr. Kakkis is our founder and has served as our President and Chief Executive Officer and as a member of our Board since our inception in April 2010. Dr. Kakkis served as our interim principal financial officer following the departure of the Company's Chief Financial Officer in November 2022 until the appointment of Mr. Horn as the Company's Chief Financial Officer in October 2023. Prior to Ultragenyx, from September 1998 to February 2009, Dr. Kakkis served 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 to 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 received the Termeer Visionary Leadership Award in 2019 and the Leadership Award from the California Life Sciences Association in 2021. 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 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.

#### Skills and Qualifications specific to Ultragenyx:

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

Chief Executive Officer of ReCode Therapeutics

**Age: 52**  
**Director since: 2019**  
**INDEPENDENT**  
**Committees: 2**  
**Other Public Directorships: 1**

Dr. Suliman has served as Chief Executive Officer of ReCode Therapeutics, a privately-held, integrated genetic medicines company, since January 2022. Prior to joining ReCode Therapeutics, Dr. Suliman served as President and Chief Operating Officer of Alector, Inc., a clinical stage biotechnology company, from December 2019 to December 2021 and previously served as Senior Vice President, Corporate Development and Strategy of Theravance Biopharma, Inc., a biopharmaceutical company, from July 2017 to 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 biopharmaceutical company, from 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. 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 from Oxford University, where she was a Rhodes Scholar.

#### Skills and Qualifications specific to Ultragenyx:

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.

### Daniel G. Welch

Board member and advisor to various life science companies

**Age: 66**  
**Director since: 2015**  
**INDEPENDENT CHAIRMAN**  
**Committees: 2**  
**Other Public Directorships: 3**

Mr. Welch is a board member and advisor to life science companies. Mr. Welch currently serves on the boards of Structure Therapeutics, Nuvation Bio Inc. and Prothena Corporation, each of which are publicly traded biotechnology companies. 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. 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 Intercept Pharmaceuticals, Inc from November 2015 to June 2021 and Seagen from July 2007 to December 2023. Mr. Welch holds a B.S. from the University of Miami and an M.B.A. from the University of North Carolina.

#### Skills and Qualifications specific to Ultragenyx:

We believe that Mr. Welch is a strong operating executive with operational and strategic expertise in the global pharmaceutical market, whose experience contributes valuable insight to the Board.

## Vote Required

The three nominees who receive the greatest number of affirmative votes will be elected as Class II directors. Shares as to which a stockholder withholds voting authority and broker non-votes, if any, 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 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. Broker non-votes, if any, 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 DIRECTOR NOMINEES IDENTIFIED ABOVE.**

## PROPOSAL 2

# Approval of the Ultragenyx Pharmaceutical Inc. Amended and Restated 2023 Incentive Plan

On April 20, 2024, the Board approved an amendment and restatement of the Ultragenyx Pharmaceutical Inc. 2023 Incentive Plan (the 2023 Plan and as amended and restated, the A&R 2023 Plan), subject to stockholder approval at the Annual Meeting. The A&R 2023 Plan provides for an additional 4.0 million shares of our common stock to be available for issuance thereunder. We believe that our equity compensation policies enable us to attract and retain a highly-skilled team of executives and aligns our executives' interests with those of our stockholders by rewarding short-term and long-term performance and tying compensation to increases in stockholder value. If the A&R 2023 Plan is approved by our stockholders, we intend to file a Form S-8 with the SEC following the Annual Meeting during the second or third quarter that covers the additional shares reserved for issuance under the A&R 2023 Plan.

## Share Reserve Under the A&R 2023 Plan

The maximum number of shares of our common stock authorized for issuance under the A&R 2023 Plan is (i) 8.5 million shares plus (ii) the number of shares subject to any award outstanding under the 2014 Incentive Plan (2014 Plan) or the 2011 Incentive Plan (2011 Plan and together with the 2014 Plan, the Prior Plans) as of June 7, 2023 that after June 7, 2023 are not issued because such award is forfeited, canceled, terminates, expires or otherwise lapses without being exercised, or is settled in cash. Based on the recent range of our stock price, our current compensation practices, our anticipated future awards, as well as our three-year burn rate, we believe the A&R 2023 Plan share reserve will be sufficient for us to grant equity awards for approximately one year based on our stock price and compensation philosophy and policies.

As part of the Compensation Committee's recommendation to the Board to approve the A&R 2023 Plan and the additional shares available for issuance under the A&R 2023 Plan, the Compensation Committee considered advice from Aon, its independent compensation consultant. The Compensation Committee also carefully analyzed our historical burn rate, anticipated future equity award needs to help drive our long-term strategic plan, and the dilutive impact of the A&R 2023 Plan's share reserve.

## Reasons for Seeking Stockholder Approval

We believe the following are important considerations for stockholders in determining whether to approve the A&R 2023 Plan:

- **Equity Awards are Essential to Talent Acquisition and Retention:** Equity awards, similar to those typically offered by our competitors are, and we believe will continue to be, an integral component of our overall compensation program, enabling us to attract qualified and skilled employees and directors, retain our existing employees, including our experienced management team, and provide incentives for our employees to exert maximum efforts for our success, ultimately contributing to the creation of shareholder value. If the A&R 2023 Plan is not approved by our stockholders at the Annual Meeting, we will not have adequate shares to continue to grant equity awards to all our employees and directors in 2025.
- **We are Committed to Executing on our Strategic Priorities:** The use of equity awards assists us and will continue to assist us in ensuring that our executives and employees are focused on long term value creation for our stockholders and in enabling us to attract and retain the talent needed to execute on our strategic priorities while managing our cash flow. We believe that the approval of the A&R 2023 Plan as described in this proposal is instrumental to our ongoing success and our ability to provide increased value to our stockholders.
- **We are Managing our Annual Burn Rate:** We carefully and thoughtfully manage our equity award use, balancing attraction, retention and incentivization of our employees against dilution and burn rate considerations. We have relied more heavily on the grant of restricted stock units (RSUs) and for our executives, Performance Stock Units (PSUs) as opposed to options in an effort to manage our burn rate.
- **Our Equity Program is Performance-Based:** The Compensation Committee believes that performance stock units (PSUs) aligns the objectives of management with those of our stockholders with respect to long-term performance and success of the Company. For 2023 and 2024, the equity grants to our executive officers, other than to our Chief Executive Officer, reflected an equal value split among option, RSUs and PSUs, and to further align our Chief Executive Officer's compensation with stockholder interests and to increase the portion of compensation tied to Company performance measures, the Compensation Committee changed the equity award value split for our Chief Executive Officer from an equal value split among options, RSUs and PSUs to 20% options, 20% RSUs and 60% PSUs.



## Key Features and Governance Best Practices

The A&R 2023 Plan reflects a number of provisions that protect stockholders and reflect corporate governance best practices, including the following:

- **No Repricing and No Reload Options:** Without stockholder approval, the A&R 2023 Plan prohibits the repricing of stock options and SARs (defined below), the exchange or substitution of one award for another award that has the effect of reducing the exercise or purchase price, and the cancellation or exchange of underwater awards for cash, another award or other property, except in the event of a change in our capitalization or a covered transaction (described below). Reload options are not permitted under the A&R 2023 Plan.
- **No Dividends on Unvested Awards:** The A&R 2023 Plan provides that dividends and dividend equivalent rights may never be paid on any unvested award.
- **No Liberal Share Recycling:** The A&R 2023 Plan prohibits liberal share recycling.
- **Limit on Non-Employee Director Compensation:** The A&R 2023 Plan contains an annual limit on cash and equity-based compensation that may be paid or granted, whether under the A&R 2023 Plan or otherwise, to our non-employee directors of \$900,000 (or \$1,500,000 in the calendar year that the non-employee director first joins the Board or if the non-employee director is serving as chairman or lead director of the Board).
- **Clawback Provision:** Awards under the A&R 2023 Plan are subject to our clawback policy. In addition, the Plan Administrator (as defined below) may cancel or limit awards under the A&R 2023 Plan if a participant is not in compliance with the applicable award agreement or if a participant breaches any confidentiality agreement with the Company.
- **No Automatic Single Trigger Acceleration:** In the event of a covered transaction, the A&R 2023 Plan does not provide for automatic single trigger acceleration.
- **Term and Exercise Price Limits on Options and SARs:** Options and SARs granted under the A&R 2023 Plan are subject to a maximum term of 10 years and may not be granted at a discount to the fair market value of our common stock on the grant date.
- **No Evergreen:** There is no automatic share reload or “evergreen” provision in the A&R 2023 Plan.

## Shares Remaining Under the Prior Plans

As of March 28, 2024, a total of 83,094,037 shares of our common stock were outstanding and the fair market value of our common stock was \$46.69 based on the closing sale price of our common stock on the Nasdaq Global Select Market as of March 28, 2024. The following table sets forth information regarding outstanding equity awards and shares available for future equity awards under the 2023 Plan (without giving effect to approval of the A&R 2023 Plan), the Prior Plans, our Employment Inducement Plan (the “Inducement Plan”), the Dimension Therapeutics, Inc. 2015 Stock Option and Incentive Plan (the “DT 2015 Plan”), the Dimension Therapeutics, Inc. 2013 Stock Plan (the “DT 2013 Plan”), and the shares available for future equity awards under our Amended and Restated 2014 Employee Stock Purchase Plan (ESPP) as of March 28, 2024.

	2023 Plan	2014 Plan	2011 Plan	DT 2015 Plan	DT 2013 Plan	Inducement Plan	Aggregate Under All Plans
Total shares underlying outstanding stock options	1,372,104	9,558,796	—	22,458	1,692	300,448	11,255,498
Weighted average exercise price of outstanding stock options	53.69	68.21	—	47.77	22.41	47.57	65.84
Weighted average remaining life of outstanding stock options	9.8	5.6	—	2.3	1.0	8.9	6.2
Total shares underlying outstanding RSUs	2,772,203	2,217,867	—	—	—	414,741	5,404,811
Total shares underlying outstanding PSUs (assuming target performance)	263,884	379,608	—	—	—	—	643,492
Total shares available for issuance (assuming outstanding PSUs vest at maximum performance)	837,971	—	—	—	—	114,829	952,800

## Dilution

Dilution is commonly measured by “overhang,” which generally refers to the total number of equity awards outstanding plus the total number of shares available for grant under our equity plans, divided by the sum of the total common stock outstanding, the number of equity awards outstanding and the total number of shares available for grant under our equity plans. If the A&R 2023 Plan is approved, our overhang will be approximately 22% as of March 28, 2024; however, many of the stock options attributing to our overhang are underwater. As shown above, we had approximately 11,255,498 stock options outstanding, with a weighted average exercise price of \$65.84 as of March 28, 2024. That included 6,322,256 options currently exercisable, with a weighted average exercise price of \$71.44, and 4,933,242 options not yet vested, with a weighted average exercise price of \$58.66. That compares to a closing stock price on March 28, 2024, of \$46.69. The 9,155,384 stock options currently underwater account for 9% of our overhang if the A&R 2023 Plan is approved.

## Historical Burn Rate

Our equity plan share usage over 2021, 2022 and 2023 represented a three-year average burn rate of 4.95%, as described in the table below.

Year	Weighted Average Common Stock Outstanding	Time-based Stock Options Granted	Performance-based Stock Options Granted	Performance-based Stock Options Earned	RSUs Granted	PSUs Granted	PSUs Earned	Annualized Burn Rate <sup>(1)</sup>
2021	67,795,540	1,217,820	0	0	738,905	62,000	168,274	3.13%
2022	69,914,225	2,293,950	1,827,449	0	1,347,125	166,730	28,990	5.25%
2023	73,543,862	2,123,256	0	152,593	2,447,170	362,470	27,581	6.46%
<b>Three-Year Average</b>								<b>4.95%</b>

(1) Annualized burn rate defined as: time-based stock options granted, performance-based stock options earned, RSUs granted and PSUs earned as a percentage of weighted average common shares outstanding.

## Summary of the A&R 2023 Plan

The following summary describes the material terms of the A&R 2023 Plan. This summary of the A&R 2023 Plan is not a complete description of all provisions of the A&R 2023 Plan and is qualified in its entirety by reference to the A&R 2023 Plan, which is attached hereto as Appendix A. Stockholders are encouraged to read the A&R 2023 Plan in its entirety.

## Purpose

The purpose of the A&R 2023 Plan is to advance the Company’s interests by providing for the grant to participants of equity and other incentive awards.

## Plan Administration

The Compensation Committee serves as the primary administrator of the A&R 2023 Plan, except that the Compensation Committee may, subject to applicable law, delegate authority to one or more members of the Board or one or more of our officers or employees (the Compensation Committee or such delegate, the Plan Administrator).

The Plan Administrator has the authority to, among other things, (i) interpret and construe the A&R 2023 Plan, any rules and regulations under the A&R 2023 Plan and the terms and conditions of any award granted under the A&R 2023 Plan, (ii) determine eligibility for and grant awards under the A&R 2023 Plan, (iii) determine, modify or waive the terms and conditions of awards under the A&R 2023 Plan, (iv) prescribe forms, rules and procedures relating to the A&R 2023 Plan, (v) establish and verify the extent of satisfaction of any performance goals or other conditions applicable to the grant, issuance, retention, vesting, exercisability or settlement of any award under the A&R 2023 Plan, and (vi) otherwise do all things necessary or appropriate to carry out the purposes of the A&R 2023 Plan. The Plan Administrator’s determinations under the A&R 2023 Plan are conclusive and binding.

## Authorized Shares

Subject to adjustment as described below, the maximum number of shares of our common stock that may be delivered in satisfaction of awards under the A&R 2023 Plan is (i) 8.5 million, plus (ii) the number of shares of common stock subject to any award under a Prior Plan as of June 7, 2023 that become available as a result of the termination, cancellation or forfeiture of such awards under such Prior Plan after June 7, 2023.

Shares of our common stock to be issued under the A&R 2023 Plan may be authorized but unissued shares of our common stock or previously issued shares acquired by us. If any shares of our common stock underlying awards that are settled in cash, canceled, forfeited or otherwise expire or lapse without being exercised (to the extent applicable), the shares of common stock allocable to the terminated portion of such award will again be available for issuance under the A&R 2023 Plan.

However, notwithstanding the foregoing, the following shares of common stock will not be available for issuance under the Plan: (a) shares withheld from an award under the A&R 2023 Plan or a Prior Plan to satisfy the tax withholding obligations with respect to such award, (b) shares withheld from an award under the A&R 2023 Plan or a Prior Plan in payment of the exercise price of an award requiring exercise, (c) shares repurchased on the open market by us using proceeds from the exercise price paid with respect to awards under the A&R 2023 Plan or a Prior Plan, or (d) gross shares subject to an SAR granted under the A&R 2023 Plan or a Prior Plan that are not issued in connection with the stock-settlement of such SAR.

## Limits on Non-Employee Director Compensation

The aggregate dollar value of equity-based (based on the grant date fair value of equity-based awards determined for financial reporting purposes) and cash compensation granted under the A&R 2023 Plan or otherwise to any one non-employee director during any fiscal year will not exceed \$900,000, with up to \$1,500,000 to be permitted for a non-employee director in the fiscal year he or she first joins our Board or is first designated as Chairman of our Board or Lead Director.

## Eligibility

The Plan Administrator will select participants from among our, and our affiliates', executives, employees in good performance standing, directors, consultants and advisors who are in a position to contribute significantly to our success and the success of our affiliates. Eligibility for options intended to be incentive stock options, or ISOs, is limited to our employees or the employees of certain of our affiliates. As of March 28, 2024, there were approximately 1,269 employees (including eight executive officers) and seven non-employee directors who would be eligible to participate in the A&R 2023 Plan. As of March 28, 2024, no consultants or advisors would be eligible to participate in the A&R 2023 Plan.

## Awards Under the A&R 2023 Plan

The A&R 2023 Plan provides for grants of stock options, SARs, restricted and unrestricted stock, stock units (including RSUs), performance awards, cash awards and other awards convertible into or otherwise based on shares of our common stock. Dividend equivalents may also be provided in connection with an award under the A&R 2023 Plan (other than stock options and SARs), provided that such dividend equivalents will be subject to the same limits or restrictions as the awards to which they relate, and will not be payable until such awards vest.

## Stock Options

A stock option is an award that entitles the participant to receive, upon exercise, shares of our common stock upon payment of the exercise price. The exercise price of an option may not be less than the fair market value (or, in the case of an ISO granted to a ten percent shareholder, 110% of the fair market value) of a share of our common stock on the date of grant. The Plan Administrator will determine the time or times at which stock options become exercisable and the terms on which they remain exercisable. The maximum term of a stock option is 10 years (or, in the case of an ISO granted to a ten percent shareholder, five years). Reload stock options are prohibited under the A&R 2023 Plan.

## SARs

SARs entitle the participant to receive, upon exercise, shares of our common stock or cash equal to the excess of the value of the shares subject to the SAR over the exercise price. The exercise price of an SAR may not be less than the fair market value of a share of our common stock on the date of grant. The Plan Administrator will determine the time or times at which SARs become exercisable and the terms on which they remain exercisable. The maximum term of an SAR is 10 years.

## Restricted and Unrestricted Stock

A restricted stock award is an award of our common stock subject to restrictions requiring that it be redelivered or offered for sale to the Company if specified conditions are not satisfied, while an unrestricted stock award is an award of our common stock that is not subject to any restrictions. The Plan Administrator will determine the terms of awards of restricted and unrestricted stock.

## Stock Units

A stock unit award is denominated in shares of our common stock and entitles the participant to receive stock or cash measured by the value of the shares in the future. An RSU is a stock unit award that is subject to the satisfaction of performance conditions or other vesting conditions. The Plan Administrator will determine the terms of awards of stock units, including RSUs.

## Performance Awards

A performance award is an award under the A&R 2023 Plan where the vesting, settlement or exercisability is subject to specified performance criteria.

## Cash Awards

Cash awards are awards under the A&R 2023 Plan that are denominated in cash.

## Termination of Employment

The Plan Administrator will determine the effect of termination of employment or service on an award under the A&R 2023 Plan. Unless otherwise expressly provided by the Plan Administrator, upon a termination of a participant's employment all unvested options then held by the participant and other awards requiring exercise will terminate and all other unvested awards will be forfeited and all vested stock options and SARs then held by the participant will remain outstanding for three months, or one year in the case of death, or, in each case, until the applicable expiration date, if earlier. All stock options and SARs held by a participant immediately prior to the participant's termination of employment will immediately terminate upon termination of employment if the termination is for cause or occurs in circumstances that, in the determination of the Plan Administrator, would have constituted grounds for the participant's employment to be terminated for cause.

## Transferability

Awards under the A&R 2023 Plan may not be transferred except by will or by the laws of descent and distribution. For awards other than ISOs, the Plan Administrator may permit the transfer not for value to any transferee eligible to be covered by the provisions of Form S-8.

## Clawback; Recovery of Compensation

Awards granted under the A&R 2023 Plan are subject to forfeiture, termination and rescission, and a participant will be obligated to return to us the value received with respect to awards, to the extent provided by the Plan Administrator in an award agreement, pursuant to Company policy relating to the recovery of erroneously-paid incentive compensation (including our Clawback Policy), or as otherwise required by law or applicable stock exchange listing standards.

## Covered Transaction

In the event of a covered transaction (as defined in the A&R 2023 Plan), the Plan Administrator may, among other things, provide for (i) continuation or assumption of outstanding awards, (ii) new grants in substitution of outstanding awards, (iii) the accelerated vesting or delivery of shares under awards or for a cash-out of outstanding awards, in each case on such terms and with such restrictions as it deems appropriate. However, if the award is not continued or assumed or a new grant is not substituted for the award, then stock options and SARs will become fully exercisable and all other awards shall become vested (with any performance based on target or actual performance as determined by the Plan Administrator).

## Prohibition Against Repricing

We will not, without stockholder approval (except in the case of a change in our capitalization, as described below), (i) reduce the exercise price of a stock option or SAR, (ii) other than in the case of covered transaction, at any time when the exercise price of a stock option or SAR is above the fair market value of a share of stock, cancel and re-grant or exchange such stock option or SAR for cash or a new award having a lower (or no) exercise price, or (iii) take any other action with respect to an award that would be treated as a repricing under generally accepted accounting principles.

## Adjustment

In the event of a stock dividend, stock split or combination of shares including a reverse stock split, recapitalization or other change in our capital structure that constitutes an equity restructuring within the meaning of the Financial Accounting Standards Board, Accounting Standards Codification Topic 718, *Compensation — Stock Compensation*, the Plan Administrator will make appropriate adjustments to the maximum number of shares that may be delivered under the A&R 2023 Plan, and will also make appropriate adjustments to the number and kind of shares of stock or securities subject to awards, the exercise prices of such awards or any other terms of awards affected by such change. The Plan Administrator will also make the types of adjustments described above to take into account distributions and other events other than those listed above if it determines that such adjustments are appropriate to avoid distortion and preserve the value of awards.

## Amendment and Termination

The Plan Administrator will be able to amend the A&R 2023 Plan or outstanding awards for any purpose which may at the time be permitted by law, or terminate the A&R 2023 Plan as to future grants of awards, except that the Plan Administrator will not be able to alter the terms of an award if it would affect materially and adversely a participant's rights under the award without the participant's consent (unless expressly provided in the A&R 2023 Plan or the right to alter the terms of an award was expressly reserved by the Plan Administrator at the time the award was granted). Stockholder approval will be required for any amendment to the A&R 2023 Plan to the extent such approval is required by law, including the Internal Revenue Code of 1986, as amended (the "Code"), or applicable stock exchange requirements. No awards may be made under the A&R 2023 Plan after 10 years from June 7, 2023.

## New Plan Benefits

As described above, the selection of participants who will receive awards under the A&R 2023 Plan and the size and types of awards will be determined by the Plan Administrator in its discretion. Therefore, the amount of any future awards under the A&R 2023 Plan is not yet determinable and it is not possible to predict the benefits or amounts that will be received by, or allocated to, particular individuals or groups of employees.

## Awards Granted Under the 2023 Plan

No awards made under the 2023 Plan prior to the date of the Annual Meeting were granted subject to stockholder approval of the A&R 2023 Plan. Pursuant to SEC rules, the following table sets forth information with respect to awards that have been granted under the 2023 Plan to the groups named below as of March 28, 2024, with PSUs based on achievement of target performance. No associate of any director, executive officer or director nominee has received awards under the 2023 Plan, and no person has received more than 5% of all awards under the 2023 Plan.

Name and Position	Stock Options Granted	RSUs and PSUs Granted
<b>Emil D. Kakkis, M.D., Ph.D.</b> , President and Chief Executive Officer	70,094	153,712
<b>Howard Horn</b> , Chief Financial Officer and Executive Vice President, Corporate Strategy	34,200	38,800
<b>Erik Harris</b> , Chief Commercial Officer and Executive Vice President	34,200	38,800
<b>John Pinion</b> , Chief Quality Officer and EVP, Translational Sciences	31,400	35,600
<b>Karah Parschauer</b> , Chief Legal Officer & Corporate Affairs, Executive Vice President	31,400	35,600
All current executive officers as a group (8)	298,294	412,512
All current directors who are not executive officers as a group (7)	0	0
Deborah Dunsire, M.D.	0	0
Michael Narachi	0	0
Corsee D. Sanders, Ph.D.	0	0
All current employees, including all current officers who are not executive officers, as a group (1,208)	1,371,414	3,036,457

## U.S. Federal Income Tax Consequences

The following is a summary of the U.S. federal income tax treatment applicable to us and the participants who receive awards under the A&R 2023 Plan based on the federal income tax laws in effect on the date of this Proxy Statement. This summary is not intended to be exhaustive and does not address all matters relevant to a particular participant based on their specific circumstances. The summary expressly does not discuss the income tax laws of any state, municipality, or non-U.S. taxing jurisdiction, or the gift, estate, excise (including the rules applicable to deferred compensation under Section 409A of the Code), or other tax laws other than U.S. federal income tax law. Because individual circumstances may vary, we recommend that all participants consult their own tax advisor concerning the tax implications of awards granted under the A&R 2023 Plan.

## Stock Option Grants

Stock options granted under the A&R 2023 Plan may be either ISOs, which satisfy the requirements of Section 422 of the Code, or non-statutory stock options (NSOs), which are not intended to meet such requirements. The U.S. federal income tax treatment for the two types of options differs as follows:

## Incentive Stock Options

No taxable income is recognized by the participant at the time of the grant of an ISO, and no taxable income is recognized for ordinary income tax purposes at the time the ISO is exercised, although taxable income may arise at that time for alternative minimum tax purposes. Unless there is a disqualifying disposition, as described below, the participant will recognize long-term capital gain in an amount equal to the excess of (i) the amount realized upon the sale or other disposition of the purchased shares over (ii) the exercise price paid for the shares.

A disqualifying disposition occurs if the disposition is less than two years after the date of grant or less than one year after the exercise date. If there is a disqualifying disposition of the shares, then the excess of (i) the fair market value of those shares on the exercise date or (if less) the amount realized upon such sale or disposition over (ii) the exercise price paid for the shares will be taxable as ordinary income to the participant. Any additional gain or loss recognized upon the disposition will be a capital gain or loss.

If the participant makes a disqualifying disposition of the purchased shares, then we will be entitled to an income tax deduction, for the taxable year in which such disposition occurs, equal to the amount of ordinary income recognized by the participant as a result of the disposition. We will not be entitled to any income tax deduction if the participant makes a qualifying disposition of the shares.

## Non-Statutory Stock Options

No taxable income is recognized by a participant upon the grant of an NSO. The participant in general will recognize ordinary income, in the year in which the NSO is exercised, equal to the excess of the fair market value of the purchased shares on the exercise date over the exercise price paid for the shares, and the participant will be required to satisfy the tax withholding requirements applicable to such income. We will be entitled to an income tax deduction equal to the amount of ordinary income recognized by the participant with respect to the exercised NSO.

## SARs

No taxable income is recognized upon receipt of an SAR. The holder will recognize ordinary income in the year in which the SAR is exercised, in an amount equal to the excess of (i) the fair market value of the underlying shares of common stock on the exercise date over (ii) the base price in effect for the exercised right, and the holder will be required to satisfy the tax withholding requirements applicable to such income. We will be entitled to an income tax deduction equal to the amount of ordinary income recognized by the holder in connection with the exercise of the SAR.

## Stock Awards

Participants will recognize ordinary income at the time unrestricted stock awards are granted in an amount equal to the excess of (i) the fair market value of the shares on the grant date over (ii) the cash consideration (if any) paid for the shares. The holder will be required to satisfy the tax withholding requirements applicable to the income.

No taxable income is recognized at the time restricted stock awards are issued, but the participant will have to report as ordinary income, as and when those shares subsequently vest, an amount equal to the excess of (i) the fair market value of the shares on the vesting date over (ii) the cash consideration (if any) paid for the shares. The participant may, however, elect under Section 83(b) of the Code to include as ordinary income in the year the unvested shares are issued an amount equal to the excess of (a) the fair market value of those shares on the issue date over (b) the cash consideration (if any) paid for such shares. If the Section 83(b) election is made, the participant will not recognize any additional income as and when the shares subsequently vest.

We will be entitled to an income tax deduction equal to the amount of ordinary income recognized by the participant at the time such ordinary income is recognized by the participant.

## Other Awards

Generally, no taxable income is recognized upon receipt of stock units (including RSUs), performance awards or cash awards. The holder will recognize ordinary income in the year in which the shares subject to the award are actually issued or in the year in which the award is settled in cash. The amount of that income will be equal to the fair market value of the shares on the date of issuance or the amount of the cash paid in settlement of the award, and the holder will be required to satisfy the tax withholding requirements applicable to the income.

We will be entitled to an income tax deduction equal to the amount of ordinary income recognized by the holder at the time the shares are issued or the cash amount is paid.

## Deductibility of Executive Compensation

Section 162(m) of the Code limits the deductibility for federal income tax purposes of certain compensation paid to any “covered employee” in excess of \$1 million. For purposes of Section 162(m), the term “covered employee” includes any individual who serves as chief executive officer, chief financial officer or one of the other three most highly compensated executive officers for 2017 or any subsequent calendar year. It is expected that compensation deductions for any covered employee with respect to awards under the A&R 2023 Plan will be subject to the \$1 million annual deduction limitation.

## Vote Required

Approval of the A&R 2023 Plan requires the affirmative vote of a majority of the votes cast on this proposal. Because abstentions and broker non-votes, if any, 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. 2.**

## PROPOSAL 3

# 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, 2024, 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 our 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 Audit Committee; any pre-approval decisions made by the Chairman are reported 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 2023 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, 2023 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 are expected to be available to respond to appropriate stockholder questions.

## Fees for Independent Registered Public Accounting Firm

The following is a summary of the aggregate audit fees billed or expected to be billed by Ernst & Young LLP for the indicated fiscal years and the fees billed by Ernst & Young LLP for all other services rendered during the indicated fiscal years.

	2023	2022
Audit fees <sup>(1)</sup>	\$ 2,240,000	\$ 2,009,000
Audit-related fees	—	—
Tax fees <sup>(2)</sup>	77,000	97,000
All other fees	—	—
<b>TOTAL</b>	<b>\$ 2,317,000</b>	<b>\$ 2,106,000</b>

(1) Audit fees consist of the aggregate fees billed for professional services rendered for the audit of our annual financial statements included in our annual reports on Form 10-K; the review of our interim financial statements included in our quarterly reports on Form 10-Q; consultation on technical accounting matters; assistance with registration statements filed with the SEC; and the issuance of comfort letters and consents.

(2) Tax fees principally include fees for tax compliance and tax advice.



## 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, if any, 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. 3.

## Report of the Audit Committee

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

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

The Audit Committee oversees our financial reporting process on behalf of the Board. Our management has the primary responsibility for the financial statements and reporting process, including our system of internal controls over financial reporting. In fulfilling its oversight responsibilities, the Audit Committee reviewed and discussed with management the audited financial statements included in the Annual Report on Form 10-K for the fiscal year ended December 31, 2023. 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 discussed with our independent registered public accounting firm, which is responsible for expressing an opinion on the conformity of the audited financial statements with generally accepted accounting principles 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 and the SEC. The Audit Committee has received the written disclosures and the letter from the independent registered public accounting firm required by the applicable rules of 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.

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.

Based 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, 2023.

Submitted by the Audit Committee of the Board of Directors

Matthew Fust, Chairperson  
Corsee D. Sanders, Ph.D.  
Michael Narachi

## PROPOSAL 4

# Advisory (Non-Binding) Vote to Approve the Compensation of our Named Executive Officers

## Background

Section 14A of the Securities Exchange Act of 1934, as amended (Exchange Act), as adopted pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act (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 (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 talented 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 of our strategic plan that are expected to increase stockholder value. The Compensation Discussion and Analysis section starting on page 39 provides a more detailed discussion of the executive compensation program and compensation philosophy.

The vote under this Proposal No. 4 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 2025.

Stockholders will be asked at the Annual Meeting to approve the following resolution pursuant to this Proposal No. 4:

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 2024 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, if any, 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. 4.**

# Corporate Governance

## Director Independence

Our Board currently consists of eight members. Our Board has determined that Dr. Dunsire, Mr. Fust, Mr. Narachi, Dr. Ray, Dr. Sanders, 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. Former director Lars Ekman, M.D., Ph.D., whose term expired at the 2023 Annual Meeting of Stockholders (2023 Annual Meeting), was independent during the period he served on the Board. 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.

## Director Overboarding Policy

Our Board believes that all members of the Board must have sufficient time to focus on his or her Board duties. Our Corporate Governance Guidelines limit the total number of public company boards that a director may serve as follows:

- *Director who is not a public company Chief Executive Officer: five total public company boards*
- *Director who serves as a Chief Executive Officer of a public company: three total public company boards*

All of our directors are currently in compliance with our policy.

## Global Code of Conduct

We have adopted a Global Code of Conduct that applies to all of our employees, officers, and directors, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Our Global Code of Conduct is available on our website, [www.ultragenyx.com](http://www.ultragenyx.com), under the “Corporate Governance” subsection of the “Investors” tab. We intend to promptly disclose on our website any future changes or amendments to, or waivers of, to the Global Code of Conduct that we are required to disclose under applicable rules. Our Board is responsible for applying and interpreting the Global Code of Conduct in situations where questions are presented to it.

## Anti-Hedging and Anti-Pledging 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 or other equity securities of the Company held (directly or indirectly) by a director or employee, all or a portion of the risk of a decline in the market price of shares of our stock. Our insider trading policy also prohibits our director and employees, including our executive officers, from pledging Company securities as collateral for a loan.

## 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 Corporate Secretary 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 inappropriate or 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 2023, our Board met five times. Each of our current directors attended at least 90% of the aggregate meetings of the Board and meetings of the committees of which the director was a member in fiscal 2023 held during the period when the director served on the Board or the committees, as applicable.

The Board has a standing Audit Committee, Compensation Committee, Nominating and Corporate Governance Committee, and Research and Development Committee. All members of the Audit, Compensation, and Nominating and Corporate Governance Committees are non-employee directors whom the Board has determined are independent under applicable independence standards (including the heightened independence standards that apply to Audit Committee and Compensation Committee members).

Eight of the directors serving at the time of the 2023 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

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, and met four times in 2023, and the Chairman presides at these sessions. We believe this structure provides effective oversight of our management and the Company.

The Board believes that its programs for risk oversight, as described under “Role of the Board in Risk Oversight” below, would be effective under a variety of leadership frameworks. Accordingly, the Board’s risk oversight function did not significantly impact its selection of the current leadership structure.

### Role of the Board in Risk Oversight

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 assessing what level of risk is acceptable and appropriate for us, taking into account the immediacy of any such risks, and evaluating risks and circumstances across various timeframes, including the short, medium and long term. 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 periodically evaluates and discusses potential emerging risks with members of senior management as well as third-party advisors and experts. In 2023, the Board and the committees, as appropriate, reviewed with management the various risks and mitigation strategies related to the macroeconomic environment, cost containment strategies, prioritization of the Company’s pipeline programs, cybersecurity risks and the Company’s initiatives related to Corporate Responsibility and sustainability matters. The Board also delegates to Board committees oversight of selected elements of risk as set forth below.

### Board Committees

Our Board currently has a standing Audit Committee, Compensation Committee, Nominating and Governance Committee and Research and Development Committee. Each of these committees operates under a written charter which sets forth the functions and responsibilities of the committee, a copy of which is available on our website at [www.ultragenyx.com](http://www.ultragenyx.com) under the “Corporate Governance” subsection of the “Investors” tab.

**Audit Committee****Members:**

Matthew K. Fust (Chairperson)

Michael Narachi

Corsee D. Sanders, Ph.D.

*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*

**Meetings held during 2023: Six****Key Responsibilities**

The Audit Committee has been delegated the task of overseeing significant financial risks facing us and steps management has undertaken to mitigate these risks. The Audit Committee reports back to the Board regarding these risks. In 2023, the Audit Committee reviewed with management, in particular, the risks and mitigation strategies related to cybersecurity and the security programs related to our information technology systems. The Audit Committee is also responsible for the following:

- 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 charter

Compensation Committee	Key Responsibilities
<p><b>Members:</b> Michael Narachi (Chairperson) Deborah Dunsire, M.D. Daniel G. Welch</p> <p><i>All members of the Compensation Committee satisfy the current Nasdaq and SEC independence standards</i></p> <p><b>Meetings held during 2023: Four</b></p>	<p>The Compensation Committee has been delegated the task of overseeing risks related to our compensation policies and programs. In 2023, the Compensation Committee reviewed with management in particular the risks and mitigation strategies related to human capital management, succession planning for senior management positions, and strategies related to the Company's management of outstanding equity awards. The Compensation Committee is also responsible for the following:</p> <ul style="list-style-type: none"><li>• Annually reviews and approves corporate goals and objectives relevant to the compensation of our executive officers</li><li>• Evaluates the performance of our executive officers in light of such goals and objectives, and determines the compensation of our executive officers</li><li>• Appoints, compensates, and oversees the work of any compensation consultant, legal counsel, or other advisor retained by the Compensation Committee</li><li>• Conducts the independence assessment outlined in Nasdaq rules with respect to any compensation consultant, legal counsel, or other advisor retained by the Compensation Committee</li><li>• Oversees, and has the authority to administer, our compensation and benefit plans</li><li>• Reviews and approves our policies and procedures for the grant of equity-based awards</li><li>• Reviews and makes recommendations to the Board with respect to director compensation</li><li>• 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</li><li>• Oversees the maintenance and presentation to the Board of management's plans for succession to senior management positions</li><li>• Annually reviews and reassesses the adequacy of the Compensation Committee charter and performs other duties, as specified in the charter</li></ul>

Nominating and Corporate Governance Committee	Key Responsibilities
<p><b>Members:</b> Shehnaaz Suliman, M.D. (Chairperson) Matthew K. Fust Daniel G. Welch</p> <p><i>All members of the Nominating and Corporate Governance Committee satisfy the current Nasdaq and SEC independence standards</i></p> <p><b>Meetings held during 2023: Four</b></p>	<p>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. In 2023, the Nominating and Corporate Governance Committee reviewed with management, in particular, the risks and mitigation strategies related to Corporate Responsibility matters, our director search process and the Company's governance structure to assess the continued appropriateness of the classified board and other structural elements for the Company. The Nominating and Corporate Governance Committee is also responsible for the following:</p> <ul style="list-style-type: none"><li>• Develops and recommends to the Board criteria for Board and committee membership</li><li>• Establishes procedures for identifying and evaluating Board candidates, including nominees recommended by stockholders; identifies individuals qualified to become members of the Board</li><li>• Recommends to the Board the persons to be nominated for election as directors and to each of the Board's committees</li><li>• Develops and recommends to the Board a set of corporate governance guidelines</li><li>• Oversees the maintenance and presentation to the Board of plans for succession to the position of Chief Executive Officer</li><li>• Assists the Compensation Committee in its oversight of succession planning for other senior management positions</li><li>• Oversees our compliance program</li><li>• Annually reviews and reassesses the adequacy of the Nominating and Corporate Governance Committee charter and performs other duties, as specified in the charter</li></ul>

## Research and Development Committee

### Key Responsibilities

**Members:**

Amrit Ray, M.D. (Chairperson)  
Emil Kakkis, M.D., Ph.D.  
Deborah Dunsire, M.D.  
Corsee D. Sanders, Ph.D.  
Shehnaaz Suliman, M.D.

**Meetings held during 2023:** Three

The Research and Development Committee assists the Board in its oversight of the strategic direction for our pipeline and investment in research and development. In 2023, the Research and Development Committee reviewed with management in particular the risks and mitigation strategies related to the Company's prioritization of its clinical and pre-clinical programs. The Research and Development Committee is also responsible for the following:

- 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
- Performs other duties as specified in the Research and Development Committee Charter

## Compensation Committee Interlocks and Insider Participation

During fiscal 2023, the Compensation Committee consisted of Dr. Dunsire and Messrs. Narachi and Welch. None of the members of our Compensation Committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our Board or Compensation Committee.



## Corporate Responsibility Matters\*

At Ultragenyx, our purpose is to lead the future of rare disease medicine as we seek to treat individuals afflicted by diseases with limited or no treatment options, and we recognize that their lives and well-being are dependent upon our collective efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care. Our strong commitment to ethics, integrity and corporate responsibility is centered around partnering with the rare disease community, improving access to treatments, maintaining a people-first culture, and investing in innovation.

Our 2023 Corporate Responsibility report will be available in digital format on our website at [www.ultragenyx.com](http://www.ultragenyx.com) under “Ultra-Committed – Corporate Responsibility”. Website references throughout this document are provided for convenience only, and the content on the referenced websites is not incorporated by reference into this document. Our 2023 Corporate Responsibility Report is based on our Corporate Responsibility framework, which centers on six pillars: Innovation, Patients, People, Communities, Planet and Governance. The contents of our Corporate Responsibility Reports are not deemed to be part of this Proxy Statement or incorporated by reference herein.

### Innovation

We are committed to delivering novel, disease modifying treatments with speed and urgency to rare disease communities with limited or no treatment options. In the coming years, we anticipate developing six to seven clinical therapies with the potential to reach up to 150,000 patients.

In 2023, we advanced key clinical programs including our programs for UX143 in osteogenesis imperfecta (OI), GTX-102 for in Angelman syndrome and UX701 for Wilson disease. See “Executive Compensation – Compensation and Discussion Analysis - Business Highlights” for a description of the advances we made in 2023 in these programs.

We are committed to helping the rare disease community by sharing our science and expertise to advance future drug development, whether by us or others. We host a recurring Rare Bootcamp, where we share our knowledge, expertise, insights and connections to help patient families, foundations and other organizations seeking to develop novel treatments for rare diseases. Our bootcamp is designed for incredibly determined patient families and advocates who have started funding their own rare disease research and are looking to better coordinate and build structure around their efforts. Since 2017, we have held eight bootcamps with over 135 individuals from more than 100 organizations attending.

### Patients

We are committed to supporting the broader rare disease community through our efforts to develop novel therapies, share our science and expertise, achieve broad access to screening and treatment, and partner with policy makers for meaningful change. We believe that the greatest impact we can have on the lives of individuals with rare disease is to make our treatments accessible and affordable, and to engage and support rare disease families along their journey.

We believe that pricing should not be a barrier to accessing treatment and we are committed to pricing our medicines responsibly. We set our drug prices with global pricing in mind and in the U.S., we use our best efforts to help make it possible that no patient foregoes treatment for financial reasons. In 2023, we continued to align U.S. price increases consistent with the Consumer Price Index for the commercial products where we lead promotion activities.

We believe policy and regulation are critical to the cost-effective and timely development and commercialization of treatments for individuals living with rare diseases. Our Global Policy Committee identifies priority areas for our engagement and advocacy, aiming to educate, inspire and influence key decision-makers in the rare disease field. The committee’s objective is to enable all individuals with a rare disease to receive an accurate diagnosis, quality care and access to available therapy as quickly as possible.

We are a founding member of the Rare Disease Company Coalition (RDCC), a coalition of 23 companies that educates policymakers on the distinct considerations of life science companies operating in the rare disease space and focuses on three priorities: supporting robust development and innovation, promoting accessibility, and enabling earlier diagnosis.

## People

We are committed to working to maintain a diverse, inclusive, safe and healthy environment. We are also committed to fair and equitable compensation practices that are transparent and free from bias. We strive to provide employees with a workplace and work environment where they can do their best work and where they want to stay long term. Our voluntary employee turnover rate in 2023 was 10.5%, which is below the U.S. and global averages for our industry according to benchmark reports from Aon/Radford.

### Employee Diversity Data\*

<b>Number of Total Employees</b>	<b>1,276</b>
% of employees who are women	56%
% of employees with a disability	4%
<b>Age Breakdown (Total Employees)</b>	
<30 years of age	6%
30-50 years of age	63%
>50 years of age	31%
<b>Number of Employees on the Executive Leadership Team</b>	<b>9</b>
Number of XLT members who are women	1
Number of XLT members who self-reported and identified as racially or ethnically diverse	2
<b>Number of Women in Management Positions</b>	<b>220</b>
% of women in all management positions (as % of total management positions**)	50%
% of women VP and above (as % of total management positions**)	44%
% of women in STEM-related positions (as % of total STEM positions)	56%
<b>Number of U.S. Employees</b>	<b>1,089</b>
% of U.S. employees who are women	57%
% of U.S. employees who self-reported as veterans	1%
% of U.S. employees who self-reported and identified as racially or ethnically diverse***	46%*
Asian	28%
Black/African American	6%
Hispanic or Latino	9%
White	54%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	4%
Not Specified	0.4%
Number of U.S. employees in management positions who self-reported and identified as racially or ethnically diverse***	390
Asian	26%
Black/African American	3%
Hispanic or Latino	6%
White	63%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	1%
Not specified	2%

\* As of December 31, 2023. U.S. employee data consistent with company's submission on the U.S. Federal Employer Information Report Equal Opportunity (EEO-1) Form. Due to rounding, the percent breakdowns for racial/ethnic minorities add up to >100%.

\*\* Total management positions include all management positions at Ultragenyx, including employees in manager roles.

\*\*\* Based on employees' self-reporting race and ethnicity.

We are committed to fair and equitable compensation practices within a pay-for-performance framework and believe these principles are critical in supporting our culture and achieving our mission. As part of this commitment, we regularly conduct thorough pay analyses designed to assess and uphold equitable compensation practices. These assessments take into account various factors such as performance, experience, level, tenure and location to identify any potential pay disparities among employees in similar roles. To strengthen our endeavors, we leverage software that enhances our capacity to effectively monitor and manage pay equity practices. This includes utilizing data analytics, which enable a more comprehensive examination of compensation across roles, departments and demographic groups. With real-time monitoring capabilities, we track adherence to our pay equity principles throughout the hiring process and employment lifecycle, facilitating prompt identification of any emerging issues.

## Communities

We are committed to supporting initiatives that provide (1) impactful resources for the rare disease community, (2) public health and access to care, (3) Science, Technology, Engineering, the Arts and Mathematics (STEAM) education, and (4) local, at-risk communities. In 2023, we approved over \$2.5 million in charitable donations, independent medical education and health-related grants across 21 countries. We offer employees the opportunity to take two paid volunteer days each year (16 hours), so that they can spend time giving back to our communities and contribute to local initiatives. In 2023, we introduced our Ultragenyx Inaugural Days of Service where more than 50% of our employees worldwide registered to be a part of numerous impactful volunteer activities.

In addition to our philanthropic efforts, we support the rare disease community through educational initiatives, patient advocacy, research and access to information. Our specific focus is on patient and professional organizations that provide vital support to the rare disease community.

## Planet

We are committed to developing an environmental strategy that minimizes our environmental footprint across our business. In 2023, we continued to implement improvements to reduce our environmental footprint and to develop our environmental strategy.

A few of our initiatives are described below:

- We purchased 100% renewable electricity through Marin Clean Energy's Deep Green program for our corporate headquarters campus in Novato, California. Through the program, we are purchasing Green-e® certified renewable electricity from solar and wind sources and avoiding greenhouse gas (GHG) emissions associated with our electricity use. Purchasing renewable electricity supports the City of Novato's Climate Change Action Plan, which outlines strategies for the city to achieve a GHG reduction target of 40% below 2005 levels by 2035. In 2023, Ultragenyx purchased 1,955+ MWh of renewable electricity through this initiative, which avoided an estimated 473 MT CO<sub>2</sub>e.
- Electric vehicle (EV) charging stations are offered at our California and Massachusetts office complexes to support employees who drive electric vehicles. As of spring 2024, there were 38 EV charging connections across those facilities.
- In 2022, our laboratory operations team implemented a new management system designed to enhance the tracking and review of frozen sample inventory, which improved cross-functional collaboration with scientists and study leads and increased space efficiency, reclaiming nearly an entire freezer's worth of additional storage. Concurrently, the team embarked on replacing outdated sample freezers with newer, more energy-efficient models, a move expected to reduce energy consumption over time. Building on these sustainable practices, in 2023, we launched a My Green Lab certification pilot at our Novato, California, lab, with significant support from lab users and leadership. We currently plan to implement eco-friendly changes in 2024 with the goal to eventually extend the certification to more labs.

## Governance

We are committed to maintaining responsible corporate governance practices to promote the long term interest of our stockholders and other stakeholders. A summary of our corporate governance practices is described in this Proxy Statement under "– Corporate Governance."

\* Our Corporate Responsibility commitments, goals and other forward-looking statements about our corporate responsibility activities are aspirational and may change, and there is no guarantee or promise that they will be met.

## Executive Officers

Our current executive officers, their respective ages as of the date of this Proxy Statement, and positions are set forth in the following table. Biographical information regarding each executive officer (other than Dr. Kakkis) is set forth following the table. Biographical information for Dr. Kakkis is set forth above under Proposal No. 1 (Election of Class II Directors). Mr. Crombez was appointed as our Chief Medical Officer and Executive Vice President effective March 2023 and Mr. Horn joined the Company as our Chief Financial Officer, Corporate Strategy and Executive Vice President effective October 2023.

Name	Age	Position
Emil D. Kakkis, M.D., Ph.D.	63	President and Chief Executive Officer, Director
Eric Crombez, M.D.	51	Chief Medical Officer and Executive Vice President
Erik Harris	54	Chief Commercial Officer and Executive Vice President
Howard Horn	46	Chief Financial Officer, Corporate Strategy and Executive Vice President
Dennis Huang	59	Chief Technical Operations Officer and Gene Therapy Operations and Executive Vice President
Thomas Kassberg	63	Chief Business Officer and Executive Vice President
Karah Parschauer	46	Chief Legal Officer & Corporate Affairs and Executive Vice President
John R. Pinion II	57	Chief Quality Officer and Executive Vice President of Translational Sciences

**Dr. Eric Crombez** has served as our Chief Medical Officer, Executive Vice President since March 2023. Dr. Crombez previously served as our Chief Medical Officer for gene therapy and inborn errors of metabolism, overseeing global clinical development and execution for the Company's gene therapy programs, after he joined the Company following the acquisition of Dimension Therapeutics, a biotechnology company, in November 2017. At Dimension Therapeutics, Dr. Crombez served as Chief Medical Officer from December 2014 to November 2017 where he led the clinical development efforts for clinical gene therapy programs in hemophilia B, hemophilia A, ornithine transcarbamylase (OTC) deficiency and glycogen storage disease type Ia (GSDIa). Previously, he worked at Shire plc, a biopharmaceutical company, in its Human Genetics Therapy business unit. Dr. Crombez is an appointed industry representative on the FDA Cellular, Tissue and Gene Therapies Advisory Committee. He previously served as assistant professor, Department of Pediatrics, Division of Medical Genetics at the David Geffen School of Medicine at the University of California, Los Angeles (UCLA). Dr. Crombez is a board-certified clinical geneticist and completed residencies in pediatrics and medical genetics and a fellowship in clinical biochemical genetics at the UCLA School of Medicine. Dr. Crombez obtained his B.S. in biology from the University of Michigan, Ann Arbor, and his M.D. from Wayne State University School of Medicine, Detroit.

**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, Inc., a molecular diagnostic company, most recently as Vice President of Commercial. Earlier in his career, Mr. Harris served as Vice President of Marketing at InterMune, Inc., a biotechnology company, and also held positions in the commercial organizations at Elan Pharmaceuticals, Inc., Genentech, Inc., and Bristol-Myers Squibb Company. At the start of his professional career, Mr. Harris served as a Lieutenant Commander in Naval Aviation and Congressional Fellow for the United States Navy. Mr. Harris currently serves on the board of directors of Denali Therapeutics Inc., a public biopharmaceutical company. Mr. Harris holds a B.S. from the United States Naval Academy and an M.B.A. from the Wharton School of Business.

**Howard Horn** has served as our Chief Financial Officer, Corporate Strategy and Executive Vice President since October 2023. He previously served as Chief Financial Officer and as a member of the founding management team at Vir Biotechnology, a public biotech company from March 2017 to April 2023, where he led the accounting, finance, investor relations and facilities functions. Prior to Vir Biotechnology, Mr. Horn was at Biogen, a biotechnology company, where he served first as Vice President, Strategic Corporate Finance and led Biogen's corporate capital allocation processes, and then as Vice President, Business Planning and led Biogen's resource allocation processes across all functions and regions. Previously, he held positions of increasing responsibility as a consultant in the Pharmaceutical and Medical Products Practice at McKinsey & Company, and as an equity research analyst in the Life Sciences group at UBS Group AG. Mr. Horn received his B.A. in Economics from Princeton University and his M.B.A. from the Wharton School of the University of Pennsylvania.

**Dennis Huang** has served as our Executive Vice President since January 2016, our Chief Technical Operations Officer since May 2015 and as our Chief Technical Operations Officer and Gene Therapy Operations since December 2021. From May 2015 to 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. From November 2011 to 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 to 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 to 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 Chief Legal Officer & Corporate Affairs, Executive Vice President since February 2023, as our Chief Legal Officer and Executive Vice President since December 2021 and 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 to 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 currently serves on the board of directors of Evolus, Inc., a publicly traded performance beauty company, and the board of directors of Tenaya Therapeutics, a publicly traded biotechnology company. 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. From January 2016 to September 2017, he served as our Executive Vice President of Analytical Sciences and Research, and from July 2015 to September 2017, as our Chief Quality Operations Officer. From July 2015 to 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, from 2005 to 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 currently serves on the board of directors of Aroa Biosurgery, a soft tissue regeneration company listed on the Australian Securities Exchange. Mr. Pinion holds a B.S. of Science in Mechanical Engineering from the University of West Virginia.

## Certain Relationships and Related-Person Transactions

### Related-Person Transactions

Since January 1, 2023, we have not become, and are not currently proposed to be, a participant in any transactions required to be disclosed in the Proxy Statement 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, prior to entering into a transaction with a related person, our Chief Financial Officer (or Chief Legal Officer, 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 Chief Legal Officer, 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 Chief Legal Officer, 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 Chief Legal Officer, 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 Chief Legal Officer, 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.

## Delinquent Section 16(a) 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 written representations from our directors and executive officers that no Form 5 was required, other than one Form 4 to report one transaction with respect to Theodore Huizenga, which were not timely filed due to administrative error, 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 2023.

# 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 12, 2024 (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 and nominees;
- 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 person has sole or shared voting power or investment power as well as any shares that the person 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 below 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 83,103,882 shares of our common stock outstanding as of April 12, 2024. 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
<b>Stockholders Owning Greater than 5%:</b>		
The Vanguard Group <sup>(1)</sup>	8,673,432	10.4%
RTW Investments, LP <sup>(2)</sup>	6,887,086	8.3%
BlackRock, Inc. <sup>(3)</sup>	5,238,400	6.3%
The Wellington Management Group LLP <sup>(4)</sup>	4,415,029	5.3%
Sands Capital Management, LLC <sup>(5)</sup>	4,118,008	5.0%
<b>Directors and Named Executive Officers:</b>		
Deborah Dunsire, M.D. <sup>(6)</sup>	70,210	*
Matthew K. Fust <sup>(7)</sup>	61,590	*
Michael Narachi <sup>(8)</sup>	85,835	*
Amrit Ray, M.D. <sup>(9)</sup>	30,543	*
Corsee D. Sanders, Ph.D. <sup>(10)</sup>	28,095	*
Shehnaaz Suliman, M.D. <sup>(11)</sup>	61,585	*
Daniel G. Welch <sup>(12)</sup>	85,835	*
Emil D. Kakkis, M.D., Ph.D. <sup>(13)</sup>	3,258,197	3.9%
Erik Harris <sup>(14)</sup>	139,045	*
Howard Horn	0	0
Karah Parschauer <sup>(15)</sup>	150,086	*
John R. Pinion II <sup>(16)</sup>	290,650	*
All current executive officers and directors as a group <sup>(17)</sup> (15 persons)	4,966,353	5.8%

\* 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 13, 2024 by The Vanguard Group. The Schedule 13G/A reported that, as of December 29, 2023, The Vanguard Group has shared voting power with respect to 33,125 shares, sole dispositive power with respect to 8,556,929 shares, and shared dispositive power with respect to 116,503 shares. The principal business address for The Vanguard Group is listed in such filing as 100 Vanguard Blvd., Malvern, PA 19355.

(2) Based on information set forth in a Schedule 13G/A jointly filed with the SEC on February 14, 2024 by RTW Investments, LP (RTW Investments) and Roderick Wong, M.D.. The Schedule 13G/A reported that, as of December 31, 2023, RTW Investments and Dr. Wong have shared voting power and shared dispositive power with respect to 6,887,086 shares. The filing indicates that Dr. Wong is the managing partner and chief investment officer of RTW Investments. The principal business address for each of RTW Investments and Dr. Wong is listed in such filing as 40 10th Avenue, Floor 7, New York, NY 10014.

- (3) Based on information set forth in a Schedule 13G/A filed with the SEC on January 29, 2024 by BlackRock, Inc. The Schedule 13G/A reported that, as of December 31, 2023, BlackRock, Inc. has sole voting power with respect to 4,967,673 shares and sole dispositive power with respect to 5,238,400 shares. The principal business address for BlackRock Inc. is listed in such filing as 50 Hudson Yards, New York, NY 10001.
- (4) Based on information set forth in a Schedule 13G/A filed with the SEC on February 8, 2024 by Wellington Management Group LP and certain of its affiliates. The Schedule 13G/A reported that, as of December 29, 2023, Wellington Management Group LP, Wellington Group Holdings LLP and Wellington Investment Advisors Holdings LLP have shared voting power with respect to 4,100,801 shares and shared dispositive power with respect to 4,415,029 shares, and Wellington Management Company LLP has shared voting power with respect to 4,084,307 shares and shared dispositive power with respect to 4,331,419 shares. The principal business address for the above entities is listed in such filing as c/o Wellington Management Company LLP, 280 Congress Street, Boston, MA 02210. The filing indicates that Wellington Management Group LLP is the parent holding company of certain holding companies and the Wellington Investment Advisers, are owned of record by clients of the Wellington Investment Advisers. Wellington Investment Advisors Holdings LLP controls directly, or indirectly through Wellington Management Global Holdings, Ltd., the Wellington Investment Advisers. Wellington Investment Advisors Holdings LLP is owned by Wellington Group Holdings LLP. Wellington Group Holdings LLP is owned by Wellington Management Group LLP.
- (5) Based on information set forth in a Schedule 13G jointly filed with the SEC on February 13, 2024 by Sands Capital Management, LLC (SCM) and Frank M. Sands ("Sands"). The Schedule 13G reported that, as of December 31, 2023, SCM and Sands have shared voting power with respect to 2,955,657 shares and shared dispositive power with respect to 4,118,008 shares. The filing states that Sands holds ultimate voting and investment power over securities held by SCM. The principal business address for each of SCM and Sands is listed in such filing as 1000 Wilson Blvd., Suite 3000, Arlington, VA 22209.
- (6) Consists of (a) 15,870 shares of common stock, (b) 50,480 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (7) Consists of (a) 11,000 shares of common stock and (b) 46,730 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (8) Consists of (a) 17,745 shares of common stock and (b) 64,230 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (9) Consists of (a) 5,007 shares of common stock and (b) 19,970 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 5,566 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (10) Consists of (a) 3,827 shares of common stock and (b) 20,408 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (11) Consists of (a) 10,995 shares of common stock and (b) 46,730 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (12) Consists of (a) 17,745 shares of common stock and (b) 64,230 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table, and (c) 3,860 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.
- (13) Consists of (a) 2,263,985 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009, (b) 460,315 shares of common stock held by Dr. Kakkis and (c) 533,897 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table. Dr. Kakkis shares voting and dispositive power over the 2,263,985 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 460,315 shares of common stock held by him and the 533,897 shares of common stock issuable pursuant to stock options held by Dr. Kakkis.
- (14) Consists of (a) 22,888 shares of common stock and (b) 116,157 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table.
- (15) Consists of (a) 24,665 shares of common stock and (b) 125,421 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table.
- (16) Consists of (a) 46,593 shares of common stock and (b) 244,057 shares of common stock issuable pursuant to stock options exercisable within 60 days of the date of this table.
- (17) Consists of (a) 3,167,494 shares of common stock, (b) 1,768,468 shares of common stock issuable pursuant to stock options that are exercisable within 60 days of the date of this table, and (c) 30,391 shares of common stock issuable pursuant to the vesting of RSUs within 60 days of the date of this table.



# Executive Compensation

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## Compensation Discussion and Analysis

The following compensation discussion and analysis describes the material elements of compensation earned in fiscal 2023 by each of the executive officers identified below, 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, 2023 are:



These persons constitute our principal executive officer, our principal financial officer and our three other most highly paid executive officers serving during fiscal 2023. Mr. Horn joined the Company as Chief Financial Officer and principal financial officer effective October 16, 2023. Dr. Kakkis served as interim principal financial officer in 2023 prior to Mr. Horn’s appointment.

## Business Highlights

In 2023, we generated significant momentum across our clinical and commercial programs to position our company for what we expect to be a catalyst-rich 2024.

## Revenue and Commercial Highlights

We achieved \$434 million in total revenue in 2023 compared to total revenue of \$363 million in 2022, which represented 20% growth. We achieved continued strong growth in product sales in 2023 compared to the prior year. Revenue from product sales of Crysvida<sup>®</sup> in countries where we lead commercialization activities increased from \$43 million to \$76 million, or by 77%, compared to 2022, largely due to sales in Latin America. Total revenue from Crysvida<sup>®</sup> sales, including royalty revenue we received from our partner, Kyowa Kirin, from sales of the product in North America (following the transition of our commercialization responsibilities in North America to Kyowa Kirin in 2023) and in the European territory increased by 17% from \$279 million in 2022 to \$328 million in 2023. Global revenue from Dojolvi<sup>®</sup> increased by 27% from \$56 million in the prior year to \$71 million in 2023 and global revenue from Mepsevii<sup>®</sup> increased by 48% from \$21 million in 2022 to \$30 million in 2023.

In 2023, we continued to progress international regulatory and reimbursement negotiations across our marketed products and continued to add new approvals and positive reimbursement decisions, specifically in certain launch markets for Evkeeza. For instance, in December 2023, we received approval from the European Commission for an expanded indication for Evkeeza in children aged five years and older with homozygous familial hypercholesterolemia (HoFH) and in January 2024, we announced that we had achieved marketing authorization for Evkeeza in Japan for HoFH.

## Program Highlights

**Meaningful Data Announcements.** We hosted a company Analyst Day in October 2023 where we announced meaningful updates and data from three of our key clinical programs:

- **UX143 (setrusumab) monoclonal antibody for the treatment of Osteogenesis Imperfecta (OI):** We presented additional interim data from the Phase 2 portion of our Phase 2/3 *Orbit* study in UX143, a fully human monoclonal antibody administered by IV that inhibits sclerostin and is being developed for the treatment of OI, or brittle bone disease. The data we presented demonstrated that UX143 significantly reduced incidence of fractures in patients with OI with at least six months of follow-up and continued to demonstrate ongoing and meaningful improvements in lumbar spine bone mineral density. As of the data cut-off for our October 2023 announcement, there were no treatment-related serious adverse events observed in the study.
- **GTX-102 antisense oligonucleotide (ASO) for the treatment of Angelman syndrome.** We presented data from the extension cohorts in our ongoing Phase 1/2 study for GTX-102, an ASO administered by intrathecal injection that inhibits expression of the paternal UBE3A antisense and is being developed for the treatment of Angelman syndrome, a debilitating and rare neurogenetic disorder caused by loss-of-function of the maternally inherited allele of the UBE3A gene. The data we presented demonstrated clinically meaningful improvements across multiple domains. We presented quantitative data that showed improvements across multiple clinical domains compared to natural history data, where available, and clinical changes were associated with quantitative changes in EEG. We also presented long-term data that demonstrated that patients who stopped and restarted the treatment reacquired previously gained developmental skills when they were re-dosed with the current regimen. As of the data cut-off date for our October 2023 announcement, there had been no additional treatment-related serious adverse events (SAEs) since the previously disclosed event in November 2022. In January 2024, GTX-102 was subsequently accepted into the Priority Medicines (PRIME) program by the European Medicines Agency (EMA). PRIME is granted by the EMA to medicines that show the potential to benefit patients with unmet needs based on early clinical data. We provided a safety and efficacy update from some of the expansion cohort patients with a minimum of Day 170 data in April 2024.
- **UX701 for the treatment of Wilson Disease:** UX701 is an adeno-associated virus 9 (AAV 9) type gene therapy, administered by a one-time IV infusion that is being developed for the treatment of patients with Wilson disease. We presented interim data that demonstrated that four out of five patients in the first cohort (5.0 x 10<sup>12</sup> GC/kg) of our pivotal seamless Phase 1/2/3 *Cyprus2+* study for UX701 had reductions in urinary copper and were tapering off of chelators and/or zinc therapy, including two of three earlier treated patients in the cohort that were then completely off standard of care therapy. As of the data cut-off date for our October 2023 announcement, UX701 had been generally well tolerated with no treatment-related SAEs. In February 2024, we announced that we have completed enrollment and dosing of 15 patients in the three dose escalating cohorts of the first stage of the pivotal *Cyprus2+*. During this stage, the safety and efficacy of UX701 is being evaluated that will enable a dose to be selected for further evaluation in the second, randomized, placebo-controlled stage of this seamless pivotal study.

**Continued Progress on Other Clinical Programs.** In addition to our meaningful progress on UX143, GTX-102 and UX701 as described above, we continued to make important progress on our other clinical programs:

- **DTX401 for the treatment of glycogen storage disease type Ia (GSDIa):** In May 2023, we announced that the last patient had been dosed in our Phase 3 study for DTX401, an adeno-associated virus 8 (AAV8), gene therapy clinical candidate, administered by a one-time IV infusion that is being developed for the treatment of patients with glycogen storage disease type Ia (GSDIa). The 48-week study has fully enrolled patients eight years of age and older, randomized 1:1 to DTX401 or placebo.
- **DTX301 for the treatment of ornithine transcarbamylase (OTC) deficiency:** We continued randomizing and dosing patients in our ongoing Phase 3 study for DTX301, an AAV8 gene therapy product candidate, administered by a one-time IV infusion that is being developed for the treatment of patients with OTC deficiency.
- **UX111 for the treatment of Sanfilippo syndrome type A or MPS IIIA.** We continued discussions with the FDA seeking an accelerated review path for UX111, an AAV 9 gene therapy product candidate administered by a one-time IV infusion that provides the cross-correcting enzyme that enables the break down of Heparan sulfate (HS) that is being developed for the treatment of patients with Sanfilippo syndrome type A, or MPS IIIA. In February 2024, we presented new data from the ongoing pivotal *Transpher A* study evaluating the efficacy and safety of UX111 in children with MPS IIIA at the 20<sup>th</sup> Annual *WORLDSymposium*<sup>TM</sup> that demonstrated that the observed reductions of HS exposure in cerebrospinal fluid can predict improved long-term cognitive function in patients with MPS IIIA following treatment with UX111.

### Other Corporate Achievements

- **Opening of Gene Therapy Manufacturing Facility.** In June 2023, we announced the official opening of our gene therapy manufacturing facility in Bedford, Massachusetts. The new, state-of-the-art facility is expected to enable us to produce gene therapy treatments reliably and at scale, leveraging our proprietary Pinnacle PCLTM (producer cell line) manufacturing platform. Our Pinnacle PCL platform is designed to enable greater efficiency in producing adeno-associated virus (AAV) gene therapies with improved product quality and yield, lower cost and increased speed of production. We subsequently successfully manufactured our first batch of cGMP material at the facility.

- **Successful Financing.** In October 2023, we completed an underwritten public offering in which we received total proceeds of \$326 million, net of underwriting discounts and commissions.

## Stockholder Outreach

### 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. We received 95% support for our say-on-pay proposal at our 2023 Annual Meeting, representing strong support of our executive compensation programs and an increase over the 88% support we received at our 2022 Annual Meeting. We did not make any changes to our executive compensation programs as a result of the say-on-pay vote.

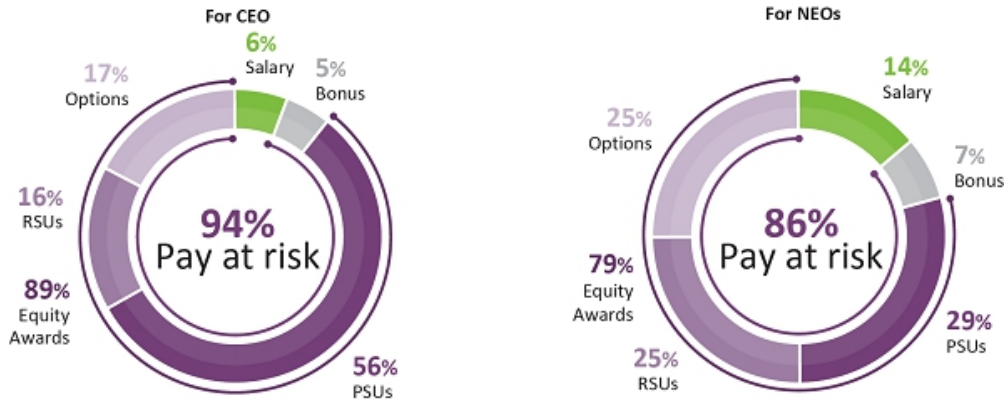
### Stockholder Engagement

We regularly engage with our stockholders to solicit their feedback on a variety of topics, including executive compensation, corporate governance, corporate responsibility and other topics in order to gain a better understanding of their perspective on these issues. Stockholder feedback is important, and the information we glean from these engagements is highly valued. Our stockholder engagement team has consisted of certain independent directors and members of our legal, investor relations and human resources expertise areas. Stockholders also regularly meet with members of our senior management team to discuss our strategy and review our business, goals and performance. In 2023, we engaged with several shareholders who owned, in aggregate, a meaningful percentage of outstanding shares. The feedback we received from those conversations were conveyed to the rest of our senior management team and the board and relevant board committees, as appropriate.

## Compensation Highlights

As described below under “Compensation Philosophy and Objectives”, our Compensation Committee believes that executive compensation should be directly linked to short-term and long-term performance. A few of the key decisions made by the Compensation Committee aligned with such philosophy are as follows:

- **Modest base salary adjustments:** Base salary increases for our current executives (excluding Mr. Horn who joined the Company in October 2023) were 3.5%. Salary increases were generally based on competitive market positioning and individual performance and were aligned to increases provided to all employees in good performance standing.
- **Annual bonuses linked to pre-determined milestone performance goals and individual accomplishments.** Our Chief Executive Officer received a below target bonus of 75% of his base salary based solely on corporate performance. Annual bonuses for our other NEOs ranged from 47% to 55% of their base salary when the impact of individual performance is factored in.
- **Our equity mix included performance-based equity:** We maintained our equity mix at one-third of each PSUs, RSUs and stock options for NEOs other than our Chief Executive Officer and for our Chief Executive Officer, we increased the percentage of PSUs to 60% for 2023 annual grants.
- **PSU payouts linked to performance:** Our relative TSR-based 2021 PSUs paid out below target, at 87%. We did not achieve the threshold level of performance under our revenue-based 2022 PSUs and as such, such PSUs were forfeited with no payout.
- **Pay mix is highly “at risk”:** The percentage of pay that is “at risk” for our CEO and NEOs (excluding Mr. Horn who joined us in October 2023) is 94% and 86%, respectively, helping us align pay with performance.



## 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 primary 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, RSUs and PSUs 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 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 Do	
	Our Compensation Committee is comprised solely of independent directors.
	Our Compensation Committee engages its own independent, external compensation consultant to assist the committee in its review of executive and director compensation practices.
	We proactively engage with our stockholders throughout the year.
	We have a clawback policy that permits recovery of all incentive compensation (including time-based and performance-based equity awards) in the event of fraud or intentional misconduct by our current or former executive officers; 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 2023” and “—Fiscal 2024 Compensation – Equity Grants.”
	We hold an annual say-on-pay vote for stockholders.

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 employees, including 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.
	We prohibit the repricing of outstanding stock options without stockholder approval.
	The A&R 2023 Plan does not include an “evergreen” feature.

## 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. The Compensation Committee may delegate its authority to one or more subcommittees. The Compensation Committee may also delegate authority to review and approve the compensation of our employees to certain of our executive officers.

### Compensation Consultant

The Compensation Committee retains the services of an independent, external compensation consultant, Aon's Human Capital Solutions Practice, a division of Aon plc (Aon). 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 Aon's performance and determines whether to engage Aon or another compensation consultant and has the final authority to engage and terminate Aon's services. In 2023, the cost of Aon's consulting services related to executive compensation, director compensation and equity plan design and considerations provided to the Compensation Committee was approximately \$237,000. In addition, in 2023, management also engaged Aon to provide survey data relating to broad-based compensation matters. The aggregate cost of such other consulting services provided in 2023 by Aon and other affiliates of Aon (not related to Aon's executive compensation, director compensation and equity consulting services provided to the Compensation Committee) was approximately \$27,000.

Our Compensation Committee has assessed the independence of Aon consistent with Nasdaq listing standards and has concluded that the engagement of Aon does not raise any conflict of interest.

### Chief Executive Officer

The Chief Executive Officer and other executive officers, attends Compensation Committee meetings and works with the Compensation Committee Chairman and Aon 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 Aon 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 and the Chief Executive Officer does not participate in the portions of meetings in which his compensation is discussed and determined.

## Defining and Comparing Compensation to Peer Group 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. Market data is one element considered by the Compensation Committee when making executive compensation decisions, but the Compensation Committee does not set compensation levels based solely on market data. Rather, the Compensation Committee reviews the 25<sup>th</sup>, 50<sup>th</sup> and 75<sup>th</sup> percentiles of relevant market data as one frame of reference in making its executive compensation decisions. Final executive compensation decisions reflect a variety of factors, including each executive's experience, performance rating, the relative importance of the executive's role within the organization, as well as where each executive's pay level falls relative to the market data.

In order to evaluate the level of compensation for our named executive officers for 2023, our Compensation Committee, using information provided by Aon, 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 emphasis on orphan pharmaceutical products;
- companies with comparable market capitalizations (i.e., in the range of \$2 billion to \$16 billion);
- companies with revenue of between \$200 million and \$1 billion; and
- companies with headcounts between 300 to 3,000 employees.

Our 2023 peer group is comprised of the following 21 companies in the pharmaceutical and biotechnology industries, reflecting the addition of Alkermes plc, Apellis Pharmaceuticals, Inc., BioCryst Pharmaceuticals, Inc., Corcept Therapeutics Inc. and the removal of Acceleron Pharma, Inc., Agios Pharmaceuticals and Nektar Therapeutics from our 2022 peer group.

ACADIA Pharmaceuticals Inc.	Biomarin Pharmaceutical Inc.	Insmed Incorporated
Alkermes plc	Blueprint Medicines Corporation	Ionis Pharmaceuticals, Inc.
Alnylam Pharmaceuticals Inc.	Corcept Therapeutics Inc.	Jazz Pharmaceuticals plc
Amicus Therapeutics	Exelixis Inc.	Neurocrine Biosciences, Inc.
Apellis Pharmaceuticals, Inc.	FibroGen, Inc.	PTC Therapeutics, Inc.
BioCryst Pharmaceuticals, Inc.	Global Blood Therapeutics, Inc.	Sarepta Therapeutics, Inc.
Biohaven Pharmaceutical Holding Company Ltd.	Halozyme Therapeutics	United Therapeutics Corporation

We believe that the compensation practices of our 2023 peer group provided us with appropriate benchmarks for evaluating the compensation of our named executive officers for 2023 because of the developmental, market and organizational characteristics we shared with our peer group. At the time that we selected our 2023 peer group we were at approximately the 40<sup>th</sup> percentile of the peer group in terms of market capitalization, the 79<sup>th</sup> percentile in terms of employees headcount and the 31<sup>st</sup> 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), target bonuses, 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 setting competitive levels which are essential to retain these key executives. 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.

### 2023 Base Salaries

The Compensation Committee engaged Aon to conduct a competitive review and analysis of our current executive compensation program relative to our 2023 peer group. Aon prepared an Executive Compensation Assessment report in December 2022 that provided a competitive assessment of our executive compensation program as compared to the 2023 peer group data for base salaries, target total cash compensation, and equity compensation.

For 2023, increases in base salaries for our named executive officers, were 3.5% annualized, excluding Mr. Horn who joined the Company in October 2023. Mr. Horn's base salary was established in connection with his appointment. The overall 2023 merit budget was based on an Aon trend report regarding projected market merit spends for 2023. Individual increases in base salaries were also based on achievement of 2022 individual goals.

The following table shows the increases in base salaries for our named executive officers between fiscal 2022 and fiscal 2023. Mr. Horn joined the Company in October 2023 and as such, did not have a fiscal 2022 base salary.

Name	Title	Fiscal 2022 Base Salary (as of December 31, 2022)	Fiscal 2023 Base Salary (as of December 31, 2023)	Percentage Increase (%)
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	\$ 800,000	\$ 828,000	3.5
Howard Horn	Chief Financial Officer, Corporate Strategy and Executive Vice President	—	590,000	—
Erik Harris	Chief Commercial Officer and Executive Vice President	569,300	589,000	3.5
John R. Pinion II	Chief Quality Officer and Executive Vice President, Translational Sciences	536,100	555,000	3.5
Karah Parschauer	Chief Legal Officer & Corporate Affairs and Executive Vice President	519,600	538,000	3.5

## Annual Bonus

### Overview

Our annual incentive program 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 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. In 2023, the Company modified its bonus structure for all employees, including executive officers other than the Chief Executive Officer, such that the bonus payout for employees (excluding the Chief Executive Officer) is based on the employee's annual target bonus percentage multiplied by the corporate performance achievement multiplied by the individual performance achievement. Prior to the change, the annual bonus was weighted 75% for corporate performance and 25% for individual performance for executives other than the Chief Executive Officer. The annual bonus for the Chief Executive Officer continues to be 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 minimum payout is 0% and the maximum payout is 133% of the Company achievement. Actual payouts are based upon achievement with respect to established goals (for corporate performance) and for performance in three categories (for individual performance) – achievement of individual goals, key deliverables of the role and demonstration of the Company's values and behaviors.

Generally, after 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.

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 2023 Bonuses

Annual corporate goals for fiscal 2023 were proposed by our executive officers and approved by our Compensation Committee in early 2022. Individual objectives for our executive officers for 2023 were proposed by each executive officer, with review, input and confirmation from our Chief Executive Officer.

The target bonuses, as a percentage of base salary, for the named executive officers for fiscal 2023 are set forth in the following table. The target bonus for Dr. Kakkis increased from 75% of base salary in 2022 to 80% of base salary in 2023 to align with market practices.

Name	Target Bonus for Fiscal 2023 (% of Base Salary)
Emil D. Kakkis, M.D., Ph.D.	80
Howard Horn*	50
Erik Harris	50
John R. Pinion II	50
Karah Parschauer	50

\* As Mr. Horn commenced employment with the Company after September 30, 2023, he was not eligible to receive an annual bonus for fiscal year 2023.

In February 2024, the Compensation Committee assessed our overall 2023 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 2023 fiscal year:

Goal	Weighting	Achievement Against Goal	Weighted Achievement
<b>1. Commercial Products</b>			
<ul style="list-style-type: none"> <li>Achieve targets for global revenue in our territories and number of patients on products in target territories</li> </ul>	35%	91%	32%
<b>2. Research &amp; Development</b>			
<ul style="list-style-type: none"> <li>Achieve enrollment objectives for pivotal trials for specified product candidates</li> </ul>	52%	89%	46%
<b>3. Gene Therapy Manufacturing</b>			
<ul style="list-style-type: none"> <li>Complete engineering runs and GMP runs for drug product and drug supply</li> </ul>	5%	150%	8%
<b>4. General Corporate</b>			
<ul style="list-style-type: none"> <li>Maintaining operating cash usage within 5% of budget</li> <li>Retaining over 80% of employees rated as "meets expectations" or above</li> <li>Achieve threshold scores in annual employee engagement survey: overall engagement score of 80%+, inclusion and diversity score of 75%+ and employee well-being score of 75%+</li> <li>Execute all goals while maintaining ethical and compliant culture with no adverse regulatory actions</li> </ul>	8.5%	50%	4%

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. The Compensation Committee also considered additional key corporate achievements that were not represented in the 2023 corporate goals, including the successful analyst day hosted by the Company in October 2023, completion of a financing transaction and successful execution of a complex clinical pipeline which involved foregoing achievement on some corporate goals to prioritize the Company's key clinical programs. For all goals combined, including additional achievements by the Company not previously defined, the Compensation Committee determined an overall 94% achievement for fiscal year 2023.

In February 2024, in addition to assessing the foregoing corporate goals, the Compensation Committee (after consultation with our Chief Executive Officer) assessed the individual accomplishments of our named executive officers (other than Mr. Horn) for purposes of determining the individual component of their annual bonus, other than our Chief Executive Officer whose annual bonus is based solely on the corporate goals described above. Key individual achievements for these named executive officers are summarized below.



Named Executive Officer	Key 2023 Achievements
<b>Erik Harris</b> Chief Commercial Officer and EVP	<ul style="list-style-type: none"> <li>Achieved continued growth in product sales to support achievement of global revenue targets</li> <li>Achieved operating margin and operating expenses for global commercial team within specified variance</li> <li>Executed commercial planning for launch readiness for pipeline programs</li> <li>Submission of initial country dossiers for Evkeeza in specified countries</li> </ul>
<b>John R. Pinion II</b> Chief Quality Officer and EVP, Translational Sciences	<ul style="list-style-type: none"> <li>Led and sponsored non-clinical activities to enable development and advancement of translational science portfolio</li> <li>Successfully executed IND enabling studies for specified pre-clinical programs</li> <li>Sponsored and hosted successful R&amp;D day</li> <li>Leadership support for Company's planned spinout of the Alzheimer's program</li> <li>Executive sponsor for Company's DEIB action committee</li> </ul>
<b>Karah Parschauer</b> Chief Legal Officer & Corporate Affairs and EVP	<ul style="list-style-type: none"> <li>Successfully managed and/or resolved litigation and investigations</li> <li>Supported successful analyst day and subsequent financing</li> <li>Assumed additional responsibilities with patient advocacy team</li> <li>Successful execution of government affairs matters related to newborn screening, state biomarker testing and other rare disease legislative initiatives</li> </ul>

Dr. Kakkis evaluated the performance of Mr. Harris, Mr. Pinion and Ms. Parschauer 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 2023.

### Achievement of Goals and Relationship to Compensation Awarded

The overall 94% achievement score for the 2023 corporate goals, combined with Dr. Kakkis' assessment of the individual performance and achievement of Mr. Harris, Mr. Pinion and Ms. Parschauer during fiscal 2023 resulted in the Compensation Committee approving bonus awards for performance in 2023 as set forth in the following table.

Name	Title	Corporate Component Score (100% for CEO)	Individual Component Score	Total Fiscal 2023 Bonus	Bonus Achieved (as % of Base Salary)
Emil D. Kakkis, M.D., Ph.D.	President and Chief Executive Officer	94	—	\$622,700	75%
Erik Harris	Chief Commercial Officer and Executive Vice President	94	101	\$279,600	47%
John R. Pinion II	Chief Quality Officer and Executive Vice President of Translational Sciences	94	113	\$294,800	53%
Karah Parschauer	Chief Legal Officer & Corporate Affairs and Executive Vice President	94	118	\$298,400	55%

The bonuses awarded under our 2023 annual incentive program were paid in March 2024.

## 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 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.

Under the terms of our 2023 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 and typically our annual grants occur during an open trading window. 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 grants are made by Aon based on grant values for similarly situated executive positions in our peer group companies and accounting for dilution constraints and management of our burn rate. 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. The Compensation Committee also determines and approves the final grant amounts to our CEO.

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 2023

In March 2023, the Compensation Committee approved equity grants that reflected an equal value split among options, RSUs and PSUs for our named executive officers other than our Chief Executive Officer, who instead received 60% of his grant in the form of PSUs, with the remaining portion split equally among options and RSUs, and Mr. Horn, who received new hire grants of options and RSUs upon the commencement of his employment. The increased portion of our Chief Executive Officer's annual equity grant that is performance-based is intended to enhance the alignment with stockholder interests.



The PSU awards granted to our executive officers in March 2023 (2023 PSUs) consist of a revenue portion (50% of the PSUs) with a two-year performance period, a relative total stockholder return (TSR) portion (25% of the PSUs) with a three-year performance period and a strategic performance portion (25% of the PSUs) with a two-year performance period. The revenue-based 2023 PSUs will vest based upon achievement of revenue-based targets during the period beginning January 1, 2023 and ending December 31, 2024, with all of the earned revenue-based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2025. The revenue-based PSUs will be earned at 50% of target for threshold performance and 200% of target for maximum performance, with no PSUs being earned for below threshold performance.

The relative TSR-based 2023 PSUs will vest based upon our TSR performance relative to the TSR of the companies in the Nasdaq Biotechnology Index during the period beginning January 1, 2023 and ending December 31, 2025, with all of the earned relative TSR-based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2026. The relative TSR-based PSUs will be earned as follows based on our percentile ranking among the Nasdaq Biotechnology Index during the performance period:

Level of Performance	TSR Percentile	Earned TSR PSUs
Threshold	25 <sup>th</sup>	25%
Target	50 <sup>th</sup>	100%
Stretch	75 <sup>th</sup>	150%
Maximum	90 <sup>th</sup>	200%

The strategic performance based 2023 PSUs will vest upon achievement of specified strategic performance objectives during the period between January 1, 2023 and ending December 31, 2024, with all of the earned strategic performance based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2025. The strategic performance based PSUs will be earned as follows based on the number of strategic goals achieved during the performance period. No PSUs are earned under the strategic performance based 2023 PSUs for below threshold performance.

Level of Performance	Number of Strategic Goals Achieved	Earned Strategic PSUs
Threshold	2 out of 5	50%
Target	3 out of 5	100%
Stretch	4 out of 5	150%
Maximum	5 out of 5	200%

The table below sets forth all equity awards granted in fiscal 2023 to our named executive officers.

Name	Date of Grant	Number of Options	Number of RSUs	Target Number of PSUs
Emil D. Kakkis, M.D., Ph.D.	3/1/2023	83,500	46,200	138,700
Howard Horn <sup>(1)</sup>	10/9/2023	140,469	80,336	—
Erik Harris	3/1/2023	39,400	22,000	22,000
John R. Pinion II	3/1/2023	39,400	22,000	22,000
Karah Parschauer	3/1/2023	39,400	22,000	22,000

(1) Mr. Horn received a new hire sign-on equity award consisting of options and RSUs upon commencement of his employment with the Company.

## Performance of 2022 PSUs

The PSU awards granted to our executive officers in March 2022 (2022 PSUs) consisted of a revenue portion with a two-year performance period (80% of the PSUs) and a relative TSR portion with a three-year performance period (20% of the PSUs). The revenue-based 2022 PSUs vested based upon achievement of the following revenue-based targets (and application of linear interpolation) during the period beginning January 1, 2022 and ending December 31, 2023.

Level of Performance	Aggregate Revenue	Earned Revenue-Based PSU Percentage
<b>Actual</b>	<b>\$ 798M</b>	<b>0%</b>
Threshold	\$ 815M	50%
Target	\$ 907M	100%
Maximum	\$ 1,075M	200%

In February 2024, based on the Company's 2022 and 2023 revenue performance measured in accordance with U.S. GAAP, the Compensation Committee certified that the threshold level of revenue-based 2022 PSUs had not been achieved and as a result, the revenue-based portion of the 2022 PSUs were forfeited. The performance period for the relative TSR-based 2022 PSUs extends through December 31, 2024, and if earned, such portion will vest in early 2025.

## Performance of 2021 PSUs

The PSU awards granted to our executive officers in March 2021 (2021 PSUs) consisted of a revenue portion with a two-year performance period (80% of the PSUs) and a relative TSR portion with a three-year performance period (20% of the PSUs).

In February 2023, based on the Company's 2021 and 2022 revenue performance measured in accordance with U.S. GAAP, the Compensation Committee certified that the revenue-based 2021 PSUs vested at 60.5% of target, and such earned 2021 PSUs vested on March 1, 2023, as described in our 2023 proxy statement. The relative TSR-based 2021 PSUs vested based upon our TSR performance relative to the TSR of the companies in the Nasdaq Biotechnology Index during the period beginning January 1, 2021 and ending December 31, 2023, with all of the earned relative TSR-based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2024. The relative TSR-based PSUs was earned as follows based on our percentile ranking among the Nasdaq Biotechnology Index during the performance period:

Level of Performance	TSR Percentile	Earned TSR PSUs
Threshold	25 <sup>th</sup>	25%
<b>Actual</b>	<b>46<sup>th</sup></b>	<b>87%</b>
Target	50 <sup>th</sup>	100%
Stretch	75 <sup>th</sup>	150%
Maximum	90 <sup>th</sup>	200%

Based on the Company's ranking relative to the TSR of the companies in the Nasdaq Biotechnology Index of the 46<sup>th</sup> percentile during the performance period, the Compensation Committee certified on March 1, 2024 that the relative TSR-based 2021 PSUs were earned at 87% of target, and such earned 2021 PSUs vested on such certification date.

## Fiscal 2024 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 Aon to assist in reviewing our peer group and in suggesting revisions, as appropriate.

Based on Aon'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 2024, referred to as our 2024 peer group, by balancing the following criteria:

- companies with emphasis on orphan pharmaceutical products;
- companies with comparable market capitalizations (i.e., in the range of \$2 billion to \$16 billion);
- companies with revenue of between \$200 million and \$1.4 billion; and
- companies with headcounts between 300 to 4,000 employees.

Our 2024 peer group is comprised of the following 20 companies in the pharmaceutical and biotechnology industries, reflecting the addition of BridgeBio Pharma, Inc. Intra-Cellular Therapies Inc. and Sage Therapeutics, Inc. and the removal of Alnylam Pharmaceuticals Inc., Biohaven Pharmaceutical Holding Company, Ltd., Global Blood Therapeutics, Inc. and United Therapeutics Corporation from our 2023 peer group.

ACADIA Pharmaceuticals Inc.	BridgeBio Pharma, Inc.	Ionis Pharmaceuticals, Inc.
Alkermes plc	Corcept Therapeutics Inc.	Jazz Pharmaceuticals plc
Amicus Therapeutics	Exelixis Inc.	Neurocrine Biosciences, Inc.
Apellis Pharmaceuticals, Inc.	FibroGen, Inc.	PTC Therapeutics, Inc.
BioCryst Pharmaceuticals, Inc.	Halozyme Therapeutics	Sage Therapeutics, Inc.
BioMarin Pharmaceutical Inc.	Insmed Incorporated	Sarepta Therapeutics, Inc.
Blueprint Medicines Corporation	Intra-Cellular Therapies Inc.	

We believe that the compensation practices of our 2024 peer group provided us with appropriate compensation benchmarks for evaluating the compensation of our named executive officers for 2024.

### Base Salaries

For 2024, increases in base salaries for our named executive officers, were 4% annualized for Dr. Kakkis to align to the external market peer group, 3% annualized for Mr. Harris and Mr. Pinion to align to the market and 6% annualized for Ms. Parschauer for assuming additional responsibilities and to align to the market. Mr. Horn did not receive an increase in base salary for 2024 as he had joined the Company in October 2023. The overall 2024 merit budget was based on an Aon trend report regarding projected market merit budgets and spend for 2024.

### Annual Bonuses

For 2024, the Compensation Committee maintained the target annual bonus of 80% for our Chief Executive Officer and maintained the target bonus amount of 50% for all other named executive officers. No other significant changes were made to the bonus plan for 2024.

### Equity Compensation

For 2024, the equity grants to our executive officers, other than our Chief Executive Officer, continued to reflect an equal value split among options, RSUs and PSUs. For our Chief Executive Officer, the Compensation Committee maintained the equity split of 60% PSUs, 20% options and 20% RSUs.

The PSU awards granted to our executive officers in 2024 (2024 PSUs) consist of a revenue portion with a two-year performance period (1/3 of the PSUs), a relative TSR portion with a three-year performance period (1/3 of PSUs) and a strategic performance portion (1/3 of the PSUs) with a three-year performance period. The revenue-based 2024 PSUs will vest based upon achievement of revenue-based targets during the period beginning January 1, 2024 and ending December 31, 2025, with all of the earned revenue-based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2026. The relative TSR-based 2024 PSU awards will vest based upon our TSR performance relative to the TSR of the companies in the Nasdaq Biotechnology Index during the period beginning January 1, 2024 and ending December 31, 2026, with all of the earned relative TSR-based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2027. The strategic-based 2024 PSU awards will vest based on achievement of specified strategic goals during the period beginning January 1, 2024 and ending December 31, 2026, with all

of the earned strategic based PSUs vesting on the later of (i) the date in which the Compensation Committee certifies such achievement and (ii) March 1, 2027. The PSUs will be earned at 50% of target for threshold performance (or 25% of target for the relative TSR-based PSUs) and 200% of target for maximum performance, with no PSUs becoming earning for below threshold performance.

## Employee Benefits 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.

In addition, our named executive officers are eligible to participate in a retirement savings plan (401(k) Plan), which is a tax-qualified defined contribution plan pursuant to the Internal Revenue Code that allows participants to contribute certain amounts of their annual compensation, subject to limits prescribed by the Internal Revenue Service. All of our employees are eligible to participate in the 401(k) Plan on the same terms as the named executive officers.

Our named executive officers, along with our other highly-compensated U.S. based employees and non-employee directors of our Board, are also eligible to participate in our nonqualified deferred compensation plan. Please see "Nonqualified Deferred Compensation Plan" below for a summary of the plan.

## Clawback Policy

Our Board has adopted a Clawback Policy intended to comply with the listing standards from Nasdaq implementing Exchange Act Rule 10D-1. In the event the Company is required to prepare an accounting restatement of the Company's financial statements due to material non-compliance with any financial reporting requirement under the federal securities laws, the Company will recover, on a reasonably prompt basis, the excess incentive-based compensation received by any covered executive officer, including the named executive officers, during the prior three fiscal years that exceeds the amount that the named executive officer otherwise would have received had the incentive-based compensation been determined based on the restated financial statements. The Clawback Policy also permits recoupment of all incentive compensation, including time-based and performance-based equity awards and bonuses, in the event of fraud or intentional misconduct by a current or former executive officer, including the named executive officers.

## Minimum Stock Ownership Requirements

We maintain 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 shares of our common stock with value equivalent to 3x the annual retainer for Board members, 3x base salary for our Chief Executive Officer and 1x base salary for the other named executive officers. Shares that count toward satisfaction of these guidelines include shares owned outright by the individual (including RSUs that have vested), shares retained after an option exercise or issuance under another type of equity award granted under the company's equity incentive plans, shares retained after purchase under the ESPP and shares held in trust for the benefit of the individual. Unexercised options and unvested and unearned RSUs and PSUs do not count towards satisfaction of these guidelines. These guidelines were required to be achieved by the end of 2022 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. As of December 31, 2023, each of our Board members and named executive officers who were required to be in compliance with the guidelines by the end of 2023 had met the ownership guidelines.

## Risk Management and Mitigation

In reviewing the compensation structure in fiscal 2023, 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 2023, our corporate goals were reviewed and approved by the Board in early 2023, 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 benefit 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 incorporated by reference in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023.

Submitted by the Compensation Committee of the Board of Directors

Michael Narachi, Chairman  
Deborah Dunsire, M.D.  
Daniel G. Welch

## Summary Compensation Table

The following table sets forth the compensation earned during the years ended December 31, 2023, 2022, and 2021 by our Chief Executive Officer, our Chief Financial Officer and our next three highest-paid executives. We refer to these officers as our named executive officers.

Name and Principal Position	Year	Salary	Bonus <sup>(1)</sup>	Stock Awards <sup>(2)</sup>	Option Awards <sup>(3)</sup>	Non-Equity Incentive Plan Compensation <sup>(4)</sup>	All Other Compensation <sup>(5)</sup>	Total
<b>Emil D. Kakkis, M.D., Ph.D.</b> President and Chief Executive Officer	2023	\$ 823,692	\$ —	\$ 9,456,663	\$ 2,161,782	\$ 622,656	\$ 13,200	\$ 13,077,993
	2022	796,154	—	7,463,295	3,310,681	558,000	12,200	12,140,330
	2021	768,846	—	5,410,512	2,835,580	499,100	8,700	9,522,738
<b>Howard Horn<sup>(6)</sup></b> Chief Financial Officer, Corporate Strategy and Executive Vice President	2023	124,808	60,000	2,867,459	2,888,773	—	—	5,941,040
<b>Erik Harris</b> Chief Commercial Officer and Executive Vice President	2023	585,969	—	2,169,750	1,020,050	279,598	13,200	4,068,567
	2022	566,331	—	2,007,768	892,550	269,706	12,200	3,748,555
	2021	543,846	—	1,653,212	866,427	245,025	8,700	3,317,210
<b>John R. Pinion II,</b> Chief Quality Officer and Executive Vice President, Translational Sciences	2023	552,092	—	2,169,750	1,020,050	294,761	13,200	4,049,853
	2022	533,315	—	2,007,768	892,550	260,678	12,200	3,706,511
	2021	509,939	—	1,653,212	866,427	224,942	8,700	3,263,220
<b>Karah Parschauer</b> Chief Legal Officer & Corporate Affairs and Executive Vice President	2023	535,169	—	2,169,750	1,020,050	298,375	13,200	4,036,544

(1) The amount reported in this column represents a sign-on bonus under Mr. Horn's offer letter.

(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 realized by the named executive officers from the RSUs and the PSUs. The value of the PSUs reported in this column for 2023, assuming achievement of the maximum performance level, was as follows: \$14,695,265 for Dr. Kakkis and \$2,330,900 for each Mr. Harris, Mr. Pinion and Ms. Parschauer. Mr. Horn was not granted any PSUs in 2023.

(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 annual 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 under our annual incentive program.

(5) Amounts reported in this column for the 2023 fiscal year reflects 401(k) matching contributions.

(6) Mr. Horn joined the Company as Chief Financial Officer, Corporate Strategy and Executive Vice President in October 2023.

## 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 and participation 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. Pursuant to Mr. Horn's offer letter, he received a \$60,000 sign-on bonus in 2023, which is subject to repayment in the event his employment is terminated for "cause" (as defined below) or as a result of his resignation that is not a "constructive termination" (as defined below) within 12 months of his start date.

Each of these employment arrangements and offer letters, as amended, 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 as reflected in the amended arrangements.

## Involuntary Termination of Employment

Pursuant to their employment arrangements or offer letters, 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). Certain benefits are enhanced in the event the qualifying termination occurs within a specified period of time (12 months for Dr. Kakkis, and 18 months for Mr. Horn, Mr. Harris, Mr. Pinion and Ms. Parschauer) after the consummation of a “covered transaction” (as defined below), which we refer to as the covered transaction protection period. 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 (and not as a result of death or disability) or he resigns for good reason, he will be entitled to receive (i) the sum of 24 months of his base salary and his target bonus for the year in which the termination occurs (or, if such termination occurs within the covered transaction protection period, the sum of 24 months of his base salary and 2x his target bonus), (ii) reimbursement for monthly COBRA premiums for the 24-month period following his termination, (iii) if such termination occurs within the covered transaction protection period, accelerated vesting of any unvested equity-based compensation; and
- if Mr. Horn, Mr. Harris, Mr. Pinion or Ms. Parschauer is terminated by us without cause (and not as a result of death or disability) or upon a resignation due to a constructive termination, the executive will be entitled to the following severance benefits: (i) extension of the exercise period applicable to any options to purchase our stock held by the executive at the time of termination until 12 months from the date of such termination (or, if earlier, until the expiration of the term of the option set forth in the applicable option award agreement), (ii) the sum of 12 months of the executive’s base salary and the executive’s target bonus for the year in which the termination occurs (or, if such termination occurs within the covered transaction period, the sum of 18 months of the executive’s base salary and 1.5x the executive’s target bonus), (iii) reimbursement for monthly COBRA premiums for the 12-month period following the executive’s termination (or, if such termination occurs within the covered transaction protection period, for the 18-month period following the executive’s termination), (iv) if such termination occurs within the covered transaction period, accelerated vesting of any unvested equity-based compensation, and (v) if such termination occurs within the covered transaction period, extension of the exercise period applicable to any outstanding options held by the executive until 12 months from the date of such termination (or, if earlier, until the expiration of the term of the option set forth in the applicable option award agreement).



## 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 Mr. Horn, Mr. Harris, Mr. Pinion and Ms. Parschauer "cause" means the named executive officer's:

- willful engagement in conduct that is materially injurious to the Company (for Mr. Harris) or 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;
- willful refusal to implement or follow a material policy or directive (for Mr. Horn and Mr. Harris) or a material violation of any of our policies (for Mr. Pinion and Ms. Parschauer), in each case other than for Ms. Parschauer and Mr. Pinion, after notice from the Board and a reasonable opportunity to cure (if deemed curable);
- material breach of any agreement entered into with us (subject to notice from the Board and a reasonable opportunity to cure (if deemed curable) for Mr. Horn); or
- unauthorized use or disclosure of confidential information or trade secrets of ours or of our affiliates (subject to notice from the Board and a reasonable opportunity to cure (if deemed curable) for Mr. Horn).

For purposes of Dr. Kakkis' employment agreement, "good reason" means any of the following events without his consent, subject to standard notice and cure requirements:

- 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 each of the offer letters with Mr. Horn, Mr. Harris, Mr. Pinion and Ms. Parschauer, "constructive termination" means the occurrence of any of the following events without the named executive officer's consent, subject to standard notice and cure requirements:

- a material reduction or change in the executive's job duties, responsibilities and requirements from the executive's job duties, responsibilities and requirements immediately prior to such reduction or change, taking into account the differences in job title and duties that are normally occasioned by reason of an acquisition of one company by another;
- a material reduction of the executive's base salary (other than an equal, across-the-board reduction in the compensation of all similarly-situated employees of ours or the surviving entity that is approved by the Board); or
- a requirement that the executive relocate to a principal office that increases his or her one-way commute by more than 50 miles relative to the executive's immediately preceding principal office.

## Grants of Plan-Based Awards

The following table sets forth certain information regarding grants of plan-based awards to the named executive officers during fiscal 2023.

Name	Grant Date	Estimated Future Payouts Under Non-Equity Incentive Plan Awards <sup>(1)</sup>			Estimated Future Payouts Under Equity Incentive Plan Awards <sup>(2)</sup>			Stock Awards: Number of Shares of Stock or Units Granted <sup>(3)</sup>	Option Awards: Number of Securities Underlying Options Granted <sup>(#)</sup>	Exercise Price of Option Awards (\$/Share)	Grant Date Fair Value of Stock and Option Awards <sup>(4)</sup>
		Threshold (\$)	Target (\$)	Maximum (\$)	Threshold (#)	Target (#)	Maximum (#)				
Emil D. Kakkis, M.D., Ph.D.	3/1/2023	331,200	662,400	993,600	—	—	—	—	—	—	—
	3/1/2023	—	—	—	—	—	—	46,200	83,500	45.65	2,161,782
	3/1/2023	—	—	—	60,681	138,700	277,400	—	—	—	7,347,633
Howard Horn <sup>(5)</sup>	10/9/2023	—	—	—	—	—	—	—	140,469	35.68	2,888,773
	10/9/2023	—	—	—	—	—	—	80,366	—	—	2,867,459
Erik Harris		—	294,600	587,727	—	—	—	—	—	—	—
	3/1/2023	—	—	—	—	—	—	—	39,400	45.65	1,020,050
	3/1/2023	—	—	—	—	—	—	22,000	—	—	1,004,300
John R. Pinion II	3/1/2023	—	—	—	9,625	22,000	44,000	—	—	—	1,165,450
	3/1/2023	—	277,500	553,613	—	—	—	—	—	—	—
	3/1/2023	—	—	—	—	—	—	—	39,400	45.65	1,020,050
Karah Parschauer	3/1/2023	—	—	—	—	—	—	—	—	—	—
	3/1/2023	—	269,000	536,655	—	—	—	—	—	—	—
	3/1/2023	—	—	—	—	—	—	—	39,400	45.65	1,020,050
	3/1/2023	—	—	—	—	—	—	22,000	—	—	1,004,300
	3/1/2023	—	—	—	9,625	22,000	44,000	—	—	—	1,165,450

(1) The amounts in these columns represent the threshold (for Dr. Kakkis only), target and maximum amount of each named executive officer's cash payments under our 2023 annual incentive program as established by the Board and described in "Compensation Discussion and Analysis" above. Actual payments made for fiscal 2023 are provided in the Summary Compensation Table.

(2) The amounts in these columns represent the threshold, target and maximum level of achievement for the 2023 PSUs granted under our 2014 Incentive Plan. The PSU awards consist of a revenue portion (50% of the PSUs) with a two-year performance period, a relative total stockholder return (TSR) portion (25% of the PSUs) with a three-year performance period and a strategic performance portion (25% of the PSUs) with a two-year performance period. See "— 2023 Annual Equity Grants in Fiscal 2023" above for a description of the 2023 PSUs.

(3) The amounts in this column represents the RSUs granted under our 2014 Incentive Plan and our 2023 Incentive Plan during 2023.

(4) This column reflects the aggregate grant date fair value of equity awards granted in 2023 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 Note 12 to our financial statements included in our Annual Report for fiscal 2023.

(5) Represents the new hire option and RSU awards made to Mr. Horn upon his commencement of employment with the Company. As Mr. Horn commenced employment with the Company after September 30, 2023, he was not eligible to receive a cash payment under the 2023 annual non-equity incentive program.

## Outstanding Equity Awards at December 31, 2023

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2023.

Name	Grant Date	Option Awards <sup>(1)</sup>				Stock Awards				
		Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Option Exercise Price (\$)	Option Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#) <sup>(2)</sup>	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 (#) <sup>(3)</sup>	Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, Units or Other Rights That Have Not Vested (\$)	
<b>Emil D. Kakkis, M.D., Ph.D.</b>	3/1/2023	—	83,500	45.65	3/1/2033	46,200	2,209,284	138,700	6,632,634	
	3/1/2022	40,408	51,952	67.37	3/1/2032	39,030	1,866,415	10,408	497,711	
	3/1/2021	24,750	11,250	142.47	3/1/2031	12,132	580,152	—	—	
	3/1/2020	52,594	3,506	56.08	3/1/2030	4,250	203,235	—	—	
	3/1/2019	66,000	—	67.55	3/1/2029	—	—	—	—	
	3/1/2018	94,500	—	48.43	3/1/2028	—	—	—	—	
	3/1/2017	78,000	—	88.80	3/1/2027	—	—	—	—	
	6/1/2016	63,700	—	70.57	6/1/2026	—	—	—	—	
	5/21/2015	68,300	—	84.89	5/21/2025	—	—	—	—	
<b>Howard Horn</b>	10/9/2023	—	140,469	35.68	10/9/2033	80,366	3,843,102	—	—	
<b>Erik Harris</b>	3/1/2023	—	39,400	45.65	3/1/2033	22,000	1,052,040	22,000	1,052,040	
	3/1/2022	10,894	14,006	67.37	3/1/2032	10,500	502,110	2,800	133,896	
	3/1/2021	7,563	3,437	142.47	3/1/2031	3,707	177,269	—	—	
	3/1/2020	20,625	1,375	56.08	3/1/2030	1,950	93,249	—	—	
	6/19/2019	12,000	—	63.27	6/19/2029	—	—	—	—	
	3/1/2019	13,000	—	67.55	3/1/2029	—	—	—	—	
	3/1/2018	3,900	—	48.43	3/1/2028	—	—	—	—	
	7/6/2017	30,000	—	63.28	7/6/2027	—	—	—	—	
<b>John R. Pinion II</b>	3/1/2023	—	39,400	45.65	3/1/2033	22,000	1,052,040	22,000	1,052,040	
	3/1/2022	10,894	14,006	67.37	3/1/2032	10,500	502,110	2,800	133,896	
	3/1/2021	7,563	3,437	142.47	3/1/2031	3,707	177,269	—	—	
	3/1/2020	20,625	1,375	56.08	3/1/2030	1,950	93,249	—	—	
	3/1/2019	23,000	—	67.55	3/1/2029	—	—	—	—	
	3/1/2018	27,000	—	48.43	3/1/2028	—	—	—	—	
	3/1/2017	18,000	—	88.80	3/1/2027	—	—	—	—	
	6/3/2016	11,000	—	69.53	6/3/2026	—	—	—	—	
	6/1/2016	17,800	—	70.57	6/1/2026	—	—	—	—	
7/16/2015	90,000	—	124.87	7/16/2025	—	—	—	—		
<b>Karah Parschauer</b>	3/1/2023	—	39,400	45.65	3/1/2033	22,000	1,052,040	22,000	1,052,040	
	3/1/2022	10,894	14,006	67.37	3/1/2032	10,500	502,110	2,800	133,896	
	3/1/2021	7,563	3,437	142.47	3/1/2031	3,707	177,269	—	—	
	3/1/2020	20,625	1,375	56.08	3/1/2030	1,950	93,249	—	—	
	3/1/2019	21,500	—	67.55	3/1/2029	—	—	—	—	
	3/1/2018	17,000	—	48.43	3/1/2028	—	—	—	—	
	3/1/2017	12,000	—	88.80	3/1/2027	—	—	—	—	
	6/20/2016	17,664	—	54.50	6/20/2026	—	—	—	—	

(1) The options vest with respect to 1/4th of the shares underlying the option on the one-year anniversary of the applicable grant date, and with respect to 1/48th 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) Amounts in this column reflects unvested RSUs as well as earned but unvested PSUs. The RSUs vest with respect to 1/4th of the underlying shares on each anniversary of the grant date over a four-year period. For (i) Dr. Kakkis, 3,132 of the total earned PSUs listed in the March 1, 2021 row vested on March 1, 2024 and for (ii) each of Mr. Harris, Mr. Pinion and Ms. Parschauer, 957 of the total earned PSUs listed in the March 1, 2021 row vested on March 1, 2024. 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 set forth in the table are reported at target achievement. 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 2023.

Name	Option Awards		Stock Awards	
	Number of Shares Acquired on Exercise (#)	Value Realized on Exercise (\$) <sup>(1)</sup>	Number of Shares Acquired on Vesting (#)	Value Realized on Vesting (\$) <sup>(2)</sup>
Emil D. Kakkis, M.D., Ph.D.	47,853	1,272,193	35,477	1,619,525
Howard Horn	—	—	—	—
Erik Harris	—	—	11,363	521,022
John R. Pinion II	—	—	14,488	661,377
Karah Parschauer	—	—	11,238	513,015

(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 2023.

## Nonqualified Deferred Compensation

In June 2021, we adopted a non-qualified deferred compensation plan (Deferred Compensation Plan). Our Deferred Compensation Plan permits highly-compensated U.S. based employees, including our named executive officers, as well as non-employee members of the Board to defer up to 75% of their base salary and up to 100% of director compensation and other types of compensation, including annual cash bonuses, RSUs and PSUs awarded.

Generally, a deferral election must be made no later than December 31 of the previous year, and is irrevocable. Deferrals with respect to salary are deducted from the participant's salary in equal installments for the period of January 1 to December 31 of each year. These deferral elections are for the salary earned by the participant for the particular salary pay period during that year, which would otherwise be payable to the participant in such pay period. The election to defer salary under the Deferred Compensation Plan is in addition to any deferral election made by the participant under our 401(k) Plan. Deferrals for performance-based annual bonuses are for those bonuses earned during the year for which the election applies, which are payable the following year. The Deferred Compensation Plan is intended to provide participants with a tax deferral opportunity for compensation paid by us. The deferred amounts are not subject to income tax or income tax withholding when earned and deferred, but are fully taxable (and withheld appropriately) when distributed.

The Deferred Compensation Plan authorizes the Company to make matching contributions at our sole discretion. The participant is 100% vested at all times in his or her deferred cash account (including any company matching contributions), and deferrals of any compensation subject to vesting (such as RSUs and PSUs) shall vest in accordance with the provisions of the underlying award. No matching contributions were made by the Company in 2023.

The Deferred Compensation Plan provides for distribution of deferred compensation and earnings thereon upon a participant's separation from service with us, his or her retirement, a date specified by the participant in his or her compensation deferral agreement, the death of a participant (in such a case, to the designated beneficiary) or a "change in control." Payment distributions can be made in a lump sum, annual installments of up to five years at the participant's election or for "specified date accounts only", installments of up to 15 years, at the participant's election.

The Deferred Compensation Plan credits gains and losses to deferred amounts based upon “deemed” investments in mutual funds investing in equity instruments or debt securities chosen by each participant (which the participant may change at any time) from a “menu” of fund options provided by us. The investment returns credited to participants’ accounts in the Deferred Compensation Plan correspond to actual returns of the chosen funds. The performance of the mutual funds fluctuates with the conditions of the capital markets and the economy generally, and is affected by prevailing interest rates and credit risks. The investment options under the Deferred Compensation Plan include:

Fund	2023 Rate of Return (%)
PIMCO VIT Short-Term Admin	5.9
Vanguard VIF Total Bond Market Index	5.6
Western Asset Core Plus Vit I	6.8
DFA VIT Inflation-Protected Secs Instl.	4.0
Empower Multi-Sector Bond Investor (MXLMX)	7.9
American Funds IS <sup>®</sup> Capital World Bond 2	6.1
Vanguard VIF Equity Income	8.1
Fidelity <sup>®</sup> VIP Index 500 Initial	26.1
Invesco VI Equally Wtd S&P 500 I	13.7
T. Rowe Price Blue Chip Growth Port	49.3
American Century Vp Mid Cap Value I (AVIPX)	6.1
Empower S&P Mid Cap 400 <sup>®</sup> Index Inv (MXMDX)	15.8
MFS <sup>®</sup> VIT Mid Cap Growth Init	21.3
DFA VA US Targeted Value	20.0
Empower S&P Smallcap 600 <sup>®</sup> Index Inv (MXISX)	15.5
Clearbridge Variable Small Cap Growth I (QLMSIX)	8.4
Vanguard VIF Total Intl Stock Market Index	15.5
Vanguard VIF International	14.7
American Funds IS <sup>®</sup> New World 2	16.0
MFS <sup>®</sup> VIT III Global Real Estate Initial	11.5

For fiscal year 2023, other than Mr. Pinion, none of our named executive officers participated in the Deferred Compensation Plan.

Name	Executive Contributions in Last FY (\$)	Company Contributions in Last FY (\$)	Aggregate Earnings in Last FY (\$)	Aggregate Withdrawals/ Distributions (\$)	Aggregate Balance at Last FYE (\$)
Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer	—	—	—	—	—
Howard Horn Chief Financial Officer, Corporate Strategy and Executive Vice President	—	—	—	—	—
Erik Harris, Chief Commercial Officer and Executive Vice President	—	—	—	—	—
John R. Pinion II, Chief Quality Officer and Executive Vice President, Translational Sciences	183,239 <sup>(1)</sup>	—	34,125	—	217,365
Karah Parschauer, Chief Legal Officer & Corporate Affairs and Executive Vice President	—	—	—	—	—

(1) Represents the value of 4,014 RSUs as of the applicable vesting date that were deferred into the Deferred Compensation Plan upon the partial vesting of such RSUs.

## 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, as described above under “—Narrative Disclosure to Summary Compensation Table—Involuntary Termination of Employment” and “—Narrative Disclosure to Summary Compensation Table—Covered Transaction, has been estimated in the tables below. The tables below do not include the values of any amounts that a named executive officer may receive under the Deferred Plan as a result of a termination of employment or a change in control, as all amounts under the Deferred Plan are fully vested benefits.

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, 2023. The closing price of our common stock on The Nasdaq Global Select Market as of December 29, 2023, the last trading day of 2023, was \$47.82, which was used as the value of our common stock for purposes of the following tables. 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, 2023 by the difference between the closing price of our common stock as of December 31, 2023 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, 2023. The value of the RSU and PSU vesting acceleration was calculated by multiplying the number of unvested RSUs, earned but unvested PSUs and unearned PSUs (based on an assumed target level of performance) subject to vesting acceleration as of December 31, 2023 by the closing price of our common stock as of the last trading day of 2023. The value of COBRA reimbursements was calculated for each of the tables below based on the elections for each named executive officer in effect in December 2023 and the applicable COBRA premiums for December 2023.

### 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, 2023, the last business day of the fiscal year. Qualifying termination includes a termination by the Company without cause or a resignation by Dr. Kakkis for good reason.

Potential Payments Upon Termination or Change of Control	Qualifying Termination	Qualifying Termination following a Covered Transaction
Base Salary	\$1,656,000	\$ 1,656,000
Bonus	662,400	1,324,800
Acceleration of equity awards	—	12,170,625
COBRA reimbursements	55,969	55,969
<b>TOTAL</b>	<b>\$2,374,369</b>	<b>\$15,207,394</b>

### Howard Horn

The following table describes the potential payments upon employment termination for Howard Horn, our Chief Financial Officer, Corporate Strategy and Executive Vice President, as if his employment terminated as of December 31, 2023, the last business day of the fiscal year. Qualifying termination includes a termination by the Company without cause or a resignation by Mr. Horn due to a constructive termination.

Potential Payments Upon Termination or Change of Control	Qualifying Termination	Qualifying Termination following a Covered Transaction
Base Salary	\$590,000	\$ 885,000
Bonus	295,000	442,500
Acceleration of equity awards	—	5,548,396
COBRA reimbursements	405	607
<b>TOTAL</b>	<b>\$885,405</b>	<b>\$6,876,503</b>

## Erik Harris

The following table describes the potential payments upon employment termination for Erik Harris, our Chief Commercial Officer and Executive Vice President as if his employment terminated as of December 31, 2023, the last business day of the fiscal year. Qualifying termination includes a termination by the Company without cause or a resignation by Mr. Harris due to a constructive termination.

Potential Payments Upon Termination or Change of Control	Qualifying Termination	Qualifying Termination following a Covered Transaction
Base Salary	\$589,000	\$ 883,500
Bonus	294,500	441,750
Acceleration of equity awards	—	3,096,102
COBRA reimbursements	8,621	12,931
<b>TOTAL</b>	<b>\$892,121</b>	<b>\$4,434,283</b>

## 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, 2023, the last business day of the fiscal year. Qualifying termination includes a termination by the Company without cause or a resignation by Mr. Pinion due to a constructive termination.

Potential Payments Upon Termination or Change of Control	Qualifying Termination	Qualifying Termination following a Covered Transaction
Base Salary	\$555,000	\$ 832,500
Bonus	277,500	416,250
Acceleration of equity awards	—	3,096,102
COBRA reimbursements	38,552	57,828
<b>TOTAL</b>	<b>\$871,052</b>	<b>\$4,402,680</b>

## Karah Parschauer

The following table describes the potential payments upon employment termination for Karah Parschauer, our Chief Legal Officer & Corporate Affairs and Executive Vice President as if her employment terminated as of December 31, 2023, the last business day of the fiscal year. Qualifying termination includes a termination by the Company without cause or a resignation by Ms. Parschauer due to a constructive termination.

Potential Payments Upon Termination or Change of Control	Qualifying Termination	Qualifying Termination following a Covered Transaction
Base Salary	\$538,000	\$ 807,000
Bonus	269,000	403,500
Acceleration of equity awards	—	3,096,102
COBRA reimbursements	27,985	41,977
<b>TOTAL</b>	<b>\$834,985</b>	<b>\$4,348,579</b>

## Equity Compensation Plan Information

The table below discloses information as of December 31, 2023 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)(1)	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	3,000	\$21.00	—
2014 Incentive Plan	13,374,469	\$68.12	—
2014 Employee Stock Purchase Plan	—	—	6,609,795
2023 Equity Incentive Plan	33,585	—	5,359,901
Equity compensation plans not approved by security holders			
Dimension Therapeutics, Inc. 2015 Stock Option and Incentive Plan(1)	23,680	\$47.88	—
Dimension Therapeutics, Inc. 2013 Stock Plan(2)	3,454	\$25.11	—
Employment Inducement Plan	681,040	\$47.57	130,996
<b>TOTAL</b>	<b>14,119,228</b>	<b>\$67.43</b>	<b>12,100,692</b>

(1) Amounts in this column include outstanding stock options, RSUs and PSUs (assuming target performance for any unearned PSUs).

(2) 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 compensation review prepared by Aon in February 2023 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 2023.

A summary of the non-employee director cash compensation arrangements for fiscal 2023 is set forth below:

	Annual Retainer
<b>Board of Directors:</b>	
Chairman	\$85,000
Non-Chairman members	\$50,000
<b>Audit Committee:</b>	
Chairman	\$25,000
Non-Chairman members	\$12,500
<b>Compensation Committee:</b>	
Chairman	\$20,000
Non-Chairman members	\$10,000
<b>Nominating and Corporate Governance Committee:</b>	
Chairman	\$10,000
Non-Chairman members	\$ 5,000
<b>Research and Development Committee:</b>	
Chairman	\$14,000
Non-Chairman members	\$ 7,000

In 2024, upon recommendation of the Compensation Committee, the Board increased the annual cash compensation for certain committee members as follows: from \$10,000 to \$12,000 for the Chairperson of the Nominating & Corporate Governance Committee, from \$5,000 to \$6,000 for the other members of the Nominating & Corporate Governance Committee, from \$14,000 to \$15,000 for the Chairman of the Research and Development Committee and from \$7,000 to \$7,500 for the other members of the Research and Development Committee.

Under the non-employee director compensation policy for fiscal 2023, each non-employee director who was initially appointed or elected to the Board received an equity award with a target value of \$800,000, comprised 50% of options to purchase shares of our common stock and 50% of RSUs on the date he or she first becomes a non-employee director. The options vest monthly over a three-year period and the RSUs vest annually in equal amounts over a three year period, in each case subject to the director's continued service to us through each such vesting date. In addition, for fiscal year 2023, on the date of the annual meeting of stockholders, each continuing non-employee director received an annual equity award at a target value of \$400,000, comprised of 50% options to purchase shares of our common stock and 50% RSUs, each of which would vest in full upon the earlier of (1) our subsequent annual meeting of stockholders and (2) the first anniversary of the date of grant, subject to the director's continued service to us through such vesting date. The exercise price of all of the foregoing options was equal to the fair market value of a share of our common stock on the date of grant.

In 2024, upon recommendation of the Compensation Committee, the Board maintained the target values of annual awards for non-employee directors but decreased the target values of awards for newly appointed directors from \$800,000 to \$600,000.

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 2023 by our non-employee directors.

Name	Fees Earned in Fiscal 2023	Stock Awards <sup>(1)</sup>	Option Awards <sup>(2)</sup>	Total
Daniel G. Welch	\$ 100,000	\$200,025	\$199,867	\$499,892
Deborah Dunsire, M.D.	60,000	200,025	199,867	459,892
Lars Ekman, M.D., Ph.D. <sup>(3)</sup>	32,000	—	—	32,000
Matthew K. Fust	80,000	200,025	199,867	479,892
Michael Narachi	82,500	200,025	199,867	482,392
Amrit Ray, M.D.	60,500	200,025	199,867	460,392
Corsee D. Sanders, Ph.D.	69,500	200,025	199,867	469,392
Shehnaaz Suliman, M.D.	67,000	200,025	199,867	466,892

- (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, 2023, our then non-employee directors had the following outstanding RSUs: Mr. Welch – 3,860; Dr. Dunsire – 3,860; Dr. Ekman - 0; Mr. Fust – 3,860; Mr. Narachi – 3,860; Dr. Ray – 7,273; Dr. Sanders – 5,158; and Dr. Suliman – 3,860.
- (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 2023, 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, 2023, our then non-employee directors had the following outstanding options: Mr. Welch – 64,230; Dr. Dunsire – 50,480; Dr. Ekman - 0; Mr. Fust – 46,730; Mr. Narachi – 64,230; Dr. Ray – 22,880; Dr. Sanders – 20,610; and Dr. Suliman – 46,730.
- (3) Dr. Ekman retired from the Board effective immediately after the 2023 Annual Meeting on June 7, 2023.

## 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 2023, the annual total compensation for Dr. Emil Kakkis, our Chief Executive Officer and President, was \$13,077,993 and for our median employee was \$353,878, resulting in a pay ratio of 37:1.

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 2023 on the calculation date, (B) the target bonus for 2023, and (C) the accounting value of any equity awards granted during 2023, 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, 2023, whether employed on a full-time, part-time, or seasonal basis. For any permanent employees who were only employed for part of the 2023 fiscal year, we annualized their compensation to present a more accurate representation of their comparative annual compensation. On October 1, 2023, we had 1,300 employees. The total compensation of our identified median employee using the same methodology we use for our named executive officers as set forth in the Summary Compensation Table.

The SEC's rules for identifying the median employee and calculating the pay ratio based on that employee's annual total compensation allow companies to adopt a variety of methodologies, to apply certain exclusions, and to make reasonable estimates and assumptions that reflect their employee populations and compensation practices. As a result, the pay ratio reported by other companies may not be comparable to the pay ratio reported above, as other companies have different employee populations and compensation practices and may utilize different methodologies, exclusions, estimates and assumptions in calculating their own pay ratios.

## Pay versus Performance

As required by Section 953(a) of the Dodd-Frank Wall Street Reform and Consumer Protection Act, and Item 402(v) of Regulation S-K, we are providing the following information about the relationship between Compensation Actually Paid (CAP) and certain financial performance of the Company. For further information concerning the Company's pay for performance philosophy and how the Company's aligns executive compensation with the Company's performance, refer to "Executive Compensation – Compensation Discussion and Analysis."

Year	Summary Compensation Table Total for PEO <sup>(1)</sup>	Compensation Actually Paid to PEO <sup>(2)</sup>	Average Summary Compensation Table Total for Non-PEO NEOs <sup>(3)</sup>	Average Compensation Actually Paid to Non-PEO NEOs <sup>(4)</sup>	Value of Initial Fixed \$100 Investment Based On:		Net Income (Loss) (in thousands) <sup>(7)</sup>	Revenue (in thousands) <sup>(8)</sup>
					Total Shareholder Return <sup>(5)</sup>	Peer Group Total Shareholder Return <sup>(6)</sup>		
2023	\$ 13,077,993	\$ 11,293,489	\$ 4,524,001	\$ 4,829,669	\$ 112	\$ 115	\$ (606,639)	\$ 434,249
2022	\$ 12,140,330	\$ 3,311,432	\$ 3,911,773	\$ (175,590)	\$ 108	\$ 111	\$ (707,421)	\$ 363,329
2021	\$ 9,522,738	\$ (2,152,804)	\$ 3,314,256	\$ (1,211,638)	\$ 197	\$ 125	\$ (454,025)	\$ 351,406
2020	\$ 5,144,220	\$ 22,726,743	\$ 2,706,102	\$ 8,015,000	\$ 324	\$ 126	\$ (186,566)	\$ 271,030

(1) The dollar amounts reported are the amounts in the "Total" column of the Summary Compensation Table in each applicable year for Dr. Kakkis, who served as our Chief Executive Officer during 2020, 2021, 2022 and 2023.

(2) The dollar amounts reported represent the amount of CAP, as computed in accordance with SEC rules. The dollar amounts do not reflect the actual amount of compensation earned by or paid during the applicable year. In accordance with SEC rules, the following adjustments were made to total compensation to determine the CAP:

Compensation Actually Paid to PEO	2023	2022	2021	2020
Summary Compensation Table Total	\$ 13,077,993	\$ 12,140,330	\$ 9,522,738	\$ 5,144,220
Less, value of "Stock Awards" and "Option Awards" reported in Summary Compensation Table	(11,618,444)	(10,773,976)	(8,246,092)	(3,709,589)
Plus, year end fair value of outstanding and unvested equity awards granted during the year		(6,264,762)	4,152,033	10,163,438
Plus (less), year over year change in fair value of outstanding and unvested equity awards granted in prior years	(1,167,400)	(2,934,552)	(5,753,632)	9,346,926
Plus (less), year over year change in fair value of equity awards granted in prior years that vested in the year	(286,732)	(1,385,132)	(1,827,851)	1,781,748
Less, prior year-end fair value of any equity awards that failed to meet vesting conditions in the year	—	—	—	—
<b>Compensation Actually Paid to Dr. Kakkis</b>	<b>\$ 11,293,489</b>	<b>\$ 3,311,432</b>	<b>\$ (2,152,804)</b>	<b>\$ 22,726,743</b>

(3) The dollar amounts reported represent the average of the amounts reported for the Company's named executive officers (NEOs) as a group (excluding our CEO) in the "Total" column of the Summary Compensation Table in each applicable year. The names of each of the NEOs (excluding our CEO) included for purposes of calculating the average amounts in each applicable year are as follows: (i) for 2023, Mr. Horn, Mr. Harris, Mr. Pinion and Ms. Parschauer, (ii) for 2022 and 2021, Dr. Camille Bedrosian, Mr. Harris, Mr. Pinion and Mardi Dier; and (iii) for 2020, Dr. Bedrosian, Ms. Dier, Mr. Harris, Mr. Pinion, and Shalini Sharp.

(4) The dollar amounts reported represent the average amount of CAP to the NEOs as a group (excluding our CEO), as computed in accordance with SEC rules. The dollar amounts do not reflect the actual average amount of compensation earned by or paid to the NEOs as a group (excluding our CEO) during the applicable year. In accordance with the SEC rules, the following adjustments were made to average total compensation for the NEOs as a group (excluding our CEO) for each year to determine the compensation actually paid, using the same methodology described above in Note 2:

<b>Compensation Actually Paid to Non-PEO NEOs</b>	<b>2023</b>	<b>2022</b>	<b>2021</b>	<b>2020</b>
Summary Compensation Table Total	\$ 4,524,001	\$ 3,911,773	\$ 3,314,256	\$ 2,706,102
Less, value of "Stock Awards" and "Option Awards" reported in Summary Compensation Table	(3,831,408)	(3,130,468)	(2,519,639)	(2,027,088)
Plus, year end fair value of outstanding and unvested equity awards granted during the year	4,476,201	1,264,910	1,268,695	4,150,071
Plus (less), year over year change in fair value of outstanding and unvested equity awards granted in prior years	(237,537)	(784,234)	(2,359,539)	2,748,610
Plus (less), year over year change in fair value of equity awards granted in prior years that vested in the year	(101,588)	(573,020)	(915,411)	437,305
Less, prior year-end fair value of any equity awards that failed to meet vesting conditions in the year	—	(864,551)	—	—
<b>Compensation Actually Paid to Non-PEO NEOs</b>	<b>\$ 4,829,669</b>	<b>\$ (175,590)</b>	<b>\$ (1,211,638)</b>	<b>\$ 8,015,000</b>

- (5) Cumulative TSR is calculated by dividing (a) the sum of (i) the cumulative amount of dividends for the measurement period, assuming dividend reinvestment, and (ii) the difference between the Company's share price at the end and the beginning of the measurement period by (b) the Company's share price at the beginning of the measurement period. The beginning of the measurement period for each year in the table is December 31, 2019.
- (6) Represents the weighted peer group TSR, weighted according to the respective companies' stock market capitalization at the beginning of each period for which a return is indicated. The peer group used for this purpose is the following published industry index: Nasdaq Biotechnology Index.
- (7) The dollar amounts reported represent the amount of net income calculated in accordance with GAAP reflected in the Company's audited financial statements for the applicable year.
- (8) The dollar amounts reported represent the amount of revenue calculated in accordance with GAAP reflected in the Company's audited financial statements for the applicable year.

## Performance Measures

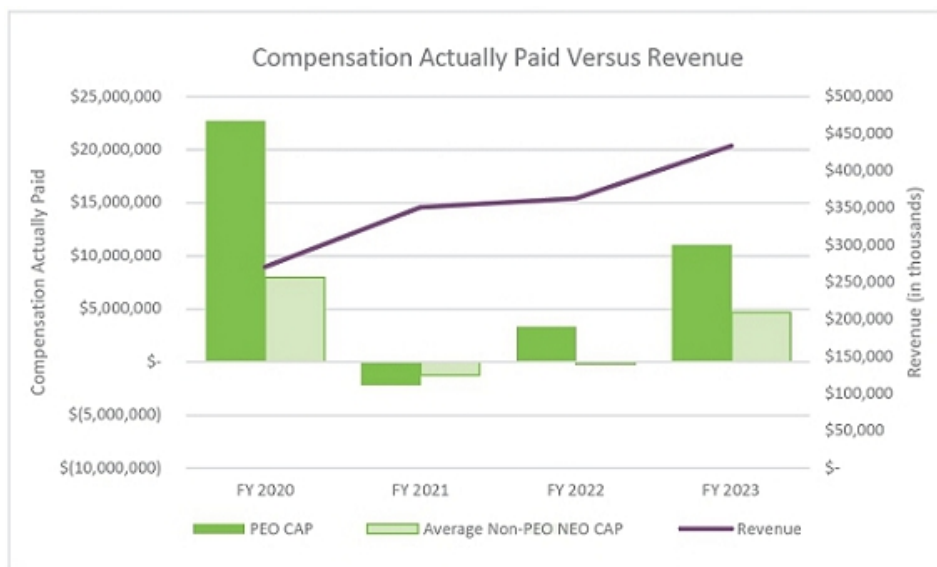
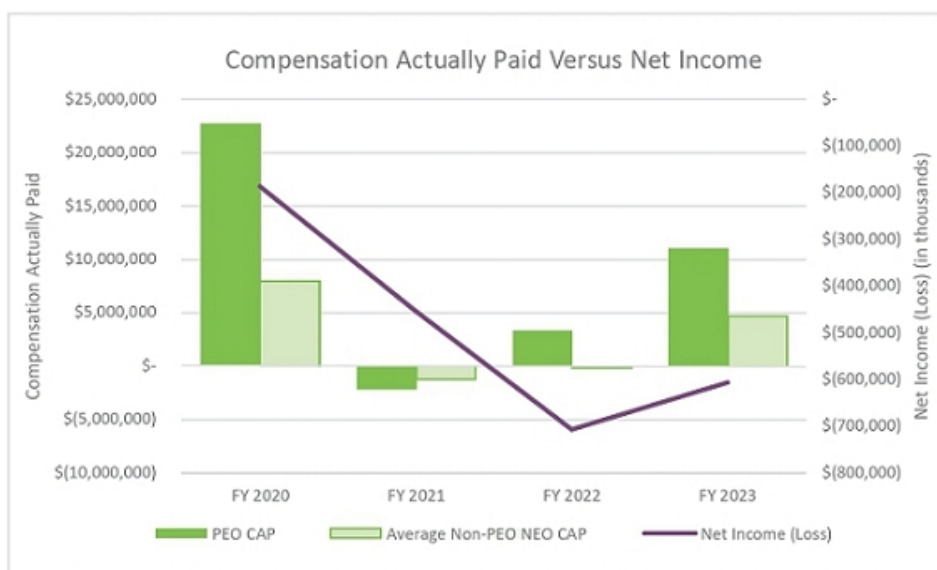
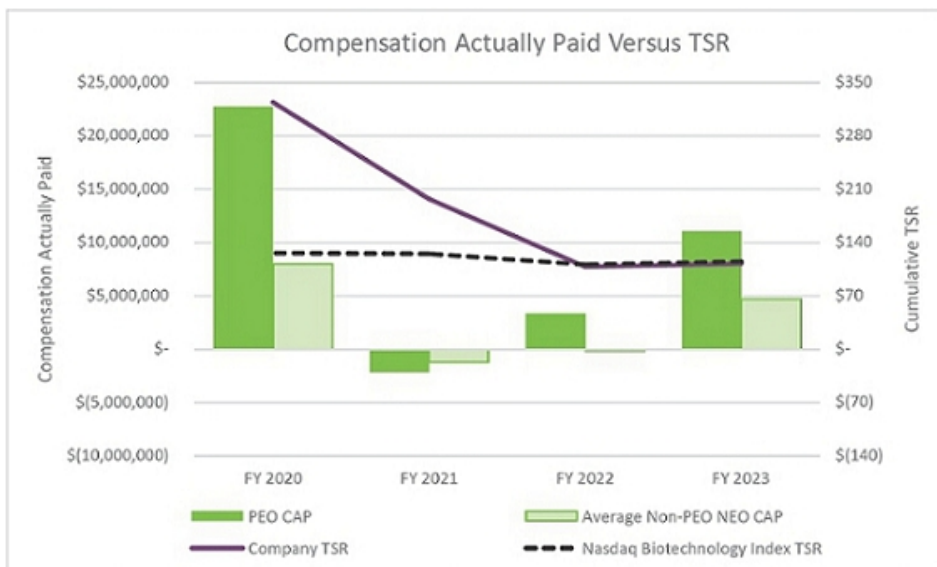
As described in greater detail in "Executive Compensation – Compensation Discussion and Analysis," the Company's executive compensation program reflects a variable pay-for-performance philosophy. The metrics that the Company uses for both our long-term and short-term incentive awards are selected based on an objective of incentivizing our NEOs to increase the value of our enterprise for our shareholders. The most important performance measures used by the Company to link executive compensation actually paid to the Company's NEOs, for the most recently completed fiscal year, to the Company's performance are as follows:

- Total Revenue
- Relative Total Stockholder Return
- Net Cash Used in Operations
- Clinical Development of Product Candidates

## Description of the Relationship between Pay and Performance

As described in more detail in the section "Executive Compensation – Compensation Discussion and Analysis," the Company's executive compensation program reflects a variable pay-for-performance philosophy. While the Company utilizes several performance measures to align executive compensation with Company performance, all of those Company measures are not presented in the Pay versus Performance table. Moreover, the Company generally seeks to incentivize long-term performance, and therefore does not specifically align the Company's performance measures with compensation that is actually paid (as computed in accordance with SEC rules) for a particular year. In accordance with SEC rules, the Company is providing the following descriptions of the relationships between information presented in the Pay versus Performance table.

The following graphs provide visual representations of the relationship between both the CAP of our PEO and the average CAP of our non-PEO NEOs and our (i) TSR, (ii) net income, and (iii) Revenue.



## Additional Information

### Questions and Answers About these Proxy Materials and Voting

#### Why did I receive a one-page notice in the mail regarding the Internet availability of proxy materials instead of a full set of proxy materials?

Pursuant to rules adopted by the SEC, we have elected to provide access to our proxy materials over the Internet. Accordingly, we are sending an Important Notice Regarding the Availability of Proxy Materials (the Notice of Internet Availability) to our stockholders of record. All stockholders will have the ability to access the proxy materials on the website referred to in the Notice of Internet Availability free of charge or request to receive a printed set of the proxy materials for the Annual Meeting. Instructions on how to access the proxy materials over the Internet or to request a printed copy may be found in the Notice of Internet Availability.

We intend to mail the Notice of Internet Availability beginning on or about April 26, 2024 to all stockholders of record entitled to vote at the Annual Meeting.

#### What if I received more than one Notice of Internet Availability?

If you receive more than one Notice of Internet Availability, your shares may be registered in more than one name or are registered in different accounts. Please follow the voting instructions on each Notice of Internet Availability and cast your vote with respect to each set of proxy materials to ensure that all of your shares are voted.

#### When and where will the Annual Meeting be held?

The Annual Meeting will be held on June 18, 2024, at 9:30 a.m. Pacific Time virtually via the Internet at [www.virtualshareholdermeeting.com/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024). 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. We believe 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. 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. 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 have taken steps to address these concerns. For example, we believe that 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. Stockholders can ask questions of our Board, management, and a representative from our independent registered public accounting firm during the meeting. During the live Q&A session, we will answer questions as they come in, as time permits, and in accordance with the meeting rules of conduct that will be available at the virtual meeting website. Following the Annual Meeting, we intend to publish and answer any questions received that comply with the meeting of conduct and that are not answered at the meeting. Although the live webcast is available only to stockholders as of the Record Date (defined below) 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/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024).

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”.

To participate in the Annual Meeting, you must access the meeting website above, enter your 16-digit control number found on your Notice of Internet Availability, proxy card or voting instruction form, and follow the instructions on the website. If your shares are held in street name and your Notice of Internet Availability or voting instruction form indicates that you may vote those shares through [www.proxyvote.com](http://www.proxyvote.com), then you may access, participate in and vote at the Annual Meeting with the 16-digit access code indicated on that Notice of Internet Availability or voting instruction form. Otherwise, stockholders who hold their shares in street name should contact their bank, broker or other nominee (preferably at least five days before the Annual Meeting) and obtain a “legal proxy” in order to be able to attend, participate in or vote at the Annual Meeting.

## What am I voting on?

At the Annual Meeting, you will be asked to consider and vote upon:

1. The election of the three directors named in the Proxy Statement as Class II directors;
2. The approval of the A&R 2023 Plan;
3. The ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2024; and
4. An advisory (non-binding) resolution to approve the compensation of our named executive officers.

## What if another matter is properly brought before the Annual Meeting?

The Board of Directors is not aware of any other matter that will be presented for consideration at the Annual Meeting. If any other matters are properly brought before the Annual Meeting, the persons named in the accompanying proxy will vote on those matters in accordance with their best judgment.

## What is the Board of Director's voting recommendation?

The Board of Directors recommends that you vote your shares:

- **"FOR"** the election of each of the Class II director nominees;
- **"FOR"** the approval of the A&R 2023 Plan;
- **"FOR"** the ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2024; and
- **"FOR"** the approval, on an advisory basis, of the compensation of our named executive officers

## How many votes do I have?

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.

## When is the record date for the Annual Meeting?

The Board of Directors has fixed the close of business on April 23, 2024 as the record date for the Annual Meeting (Record Date).

## How many shares must be represented in order to hold the Annual Meeting?

A quorum of stockholders is necessary to hold a valid stockholder meeting. A quorum will be present if stockholders holding at least a majority of the outstanding shares entitled to vote are present or represented by proxy at the Annual Meeting. On the Record Date, there were 83,133,341 shares outstanding and entitled to vote.

Your shares will be counted towards the quorum if you submit a valid proxy by mail, over the phone or via the Internet (or one is submitted on your behalf by your broker, bank or other nominee) or if you attend the Annual Meeting. In addition, abstentions and broker non-votes will be counted towards the quorum requirement. If there is no quorum, the chair of the Annual Meeting may adjourn the meeting to another date.

## How do I vote?

With regard to Proposal No. 1, the election of directors, you may vote "For" all the nominees to the Board or you may "Withhold" your vote for all the nominees or any individual nominee you specify. With regard to Proposals No. 2, 3 and 4 you may vote "For" or "Against" or abstain from voting.

The procedures for voting depend on whether your shares are registered in your name or are held by a bank, broker or other nominee:

## Registered Holders: Shares Registered in Your Name

If you are a stockholder of record, you may vote at the Annual Meeting, which will be held virtually via the Internet, vote by proxy over the telephone, vote by proxy via the Internet, or vote by proxy using a proxy card that you may request or that we may elect to deliver at a later time. Whether or not you plan to attend the Annual Meeting, we urge you to vote by proxy to ensure your vote is counted. You may still attend the Annual Meeting and vote online even if you have already voted by proxy.

- To vote via the Internet, vote at [www.proxyvote.com](http://www.proxyvote.com) prior to 11:59 p.m. Eastern Time the day before the Annual Meeting.
- If you have received a paper copy of the proxy materials, vote over the telephone by calling 1-800-690-6903 prior to 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/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024) and following the instructions regarding voting.

## Beneficial Holders: Shares Registered in Name of Broker, Bank or Other Nominee

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 as follows:

- In accordance with the voting instructions provided by the institution that holds their shares, which may provide for voting over the telephone or via the Internet, or
- 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/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024) and following the instructions regarding voting.

## What if I return a proxy card or otherwise vote but do not make specific choices?

If you are a stockholder of record and return a signed and dated proxy card or otherwise vote without marking voting selections, your shares will be voted in accordance with the Board's recommendations as describe under "*What is the Board of Directors' voting recommendations?*". If any other matter is properly presented at the Annual Meeting, your proxy holder (one of the individuals named on your proxy card) will vote your shares in his or her discretion.

## Are my shares voted if I do not provide a proxy or voting instructions?

If you are a stockholder of record and do not provide a proxy, you must attend the Annual Meeting in order to vote. If you hold shares through an account with a bank or broker and do not provide voting instructions, the bank or broker is permitted to vote your shares only on certain proposals considered "routine" matters. Whether a proposal is considered "routine" or "non-routine" is subject to stock exchange rules and final determination by the stock exchange. Even with respect to routine matters, some brokers are choosing not to exercise discretionary voting authority. Uninstructed shares that banks and brokers do not vote are counted as "broker non-votes." As a result, we urge you to direct your broker, fiduciary or custodian how to vote your shares to ensure that your interests are represented at the Annual Meeting.

## What vote is required to approve each proposal and how are votes counted?

### 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. Shares as to which a stockholder withholds voting authority and broker non-votes, if any, are not considered votes cast and therefore will have no effect on the vote outcome. As described above under "Proposal No. 1 – Election of Class II Directors," 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.

### Each of the Other Proposals

Each of the other proposals 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, if any, will have no effect on the vote outcome.



## Can I change my vote after submitting my proxy?

Yes. If you are the registered holder of your shares, you 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. Simply attending the Annual Meeting will not, by itself, revoke or change your proxy.

If you hold your shares beneficially through a brokerage firm, bank or other financial institution, you should contact your brokerage firm, bank or other financial institution for information on how to change or revoke your proxy.

## Who is paying for this proxy solicitation?

We are making these proxy materials available to you in connection with the solicitation of proxies by the Board of Directors of the Company. 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. We have retained Innisfree, a proxy solicitation firm, for assistance in connection with the Annual Meeting at an estimated cost of up to approximately \$25,000 plus reasonable out-of-pocket expense. 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.

## When are stockholder proposals for inclusion in our proxy statement for next year's annual meeting due?

Stockholders wishing to present proposals for inclusion in our proxy statement for the 2025 Annual Meeting of Stockholder (2025 Annual Meeting) pursuant to Rule 14a-8 of the Exchange Act must submit their proposals so that they are received by us at our principal executive offices no later than the close of business (5:00 p.m. Pacific Time) on December 27, 2024. However, if our 2025 Annual Meeting is advanced or delayed by more than 30 days before or after the anniversary date of the 2024 annual meeting, then the deadline will be a reasonable time prior to the time that we begin to print and mail our proxy materials.

Proposals for inclusion in our proxy statement for the 2025 Annual Meeting should be sent to the Company's Corporate Secretary at 60 Leveroni Court, Novato, California 94949 and must satisfy the requirements of Rule 14a-8 of the Exchange Act. We reserve the right to exclude from our proxy statement any proposals not meeting such requirement.

## When are other proposals and director nominations for next year's annual meeting due?

With respect to director nominations and proposals of other business (other than those to be included in our proxy statement pursuant to and in compliance with Rule 14a-8), our bylaws provide that stockholders who intend to present a stockholder proposal or director nomination at the 2025 Annual 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 Annual Meeting (that is, between February 18, 2025 and the close of business (5:00 p.m. Pacific Time) on March 20, 2025). If the 2025 Annual Meeting date is advanced by more than 30 days before or delayed by more than 60 days after the anniversary date of the Annual Meeting, then such notice must be received on or before the close of business on the later of 90th day before the date of the 2025 Annual Meeting or the 10th day after the day on which the date of the 2025 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 (which include timing and information required under Rule 14a-19 of the Exchange Act).

If a stockholder fails to meet these deadlines and fails to satisfy the requirements of Rule 14a-4 under the Exchange Act, we may exercise discretionary voting authority under proxies we solicit to vote on any such proposal as we determine appropriate. Proposals and nominations not meeting the requirements set forth in our bylaws will not be entertained 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. We reserve the right to reject, rule out of order, or take other appropriate action with respect to any nomination or proposal that does not comply with these and other applicable requirements.

## How can I find out the result of the voting at the Annual Meeting?

Preliminary voting results will be announced at the Annual Meeting. In addition, final voting results will be published in a current report on Form 8-K that we expect to file with the SEC within four business days after the Annual Meeting.

## Other Business

We know of no other matters to be submitted to a vote of stockholders at the Annual Meeting. 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 above.

## Forward-Looking Statements

Certain of the statements made in this Proxy Statement are “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended, Section 21E of the Exchange Act and the Private Securities Litigation Reform Act of 1995, including, among others, statements related to our expectations and projections regarding our future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for our clinical programs and clinical studies, future regulatory interactions, goals and other statements regarding our corporate responsibility activities, anticipated effects of our executive compensation structure and programs, and the components and timing of regulatory submissions. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, business and operating results, risks related to reliance on third party partners to conduct certain activities on the Company’s behalf, uncertainty and potential delays related to clinical drug development, smaller than anticipated market opportunities for the Company’s products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the Company’s future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx’s products and drug candidates. The Company expressly disclaims any obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see our Annual Report on Form 10-K for the fiscal year ended December 31, 2023 filed with the SEC in February 2024, and our subsequent annual and periodic reports filed with the SEC.

## Delivery Of Proxy Materials

**Our annual report to stockholders for the fiscal year ended December 31, 2023, including audited financial statements, accompanies this Proxy Statement. We will provide copies of our Annual Report without charge upon written request of a stockholder to our investor relations department at 60 Leveroni Court, Novato, California 94949.** Copies of these materials are also available online through the SEC at [www.sec.gov](http://www.sec.gov). We may satisfy SEC rules regarding delivery of proxy materials, including the Proxy Statement, Annual Report and Notice of Internet Availability by delivering a single copy of the proxy materials to stockholders who have the same address and do not participate in electronic delivery of proxy materials, a procedure adopted by the SEC called “householding.” This delivery method can result in meaningful cost savings for us and conservation of natural resources. Under this procedure, only one copy of the proxy materials will be delivered to multiple stockholders who share an address, unless contrary instructions are received from one or more stockholders at that address. We undertake to deliver promptly upon written or oral request a separate copy of the Proxy statement, Annual Report or Notice of Internet Availability to a stockholder at a shared address to which a single copy of these materials was delivered. If you hold stock as a registered holder and prefer to receive separate copies of these materials either now or in the future, please contact our investor relations department at 60 Leveroni Court, Novato, California 94949 or by telephone at (415) 483-8800. Similarly, if you share an address with another stockholder and have received multiple copies of the proxy materials, 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 received a single copy of the proxy materials and prefer to receive separate copies of the proxy materials, either now or in the future, or if you received multiple copies of the proxy materials and prefer to receive a single copy 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 MATERIALS, BY COMPLETING, DATING, SIGNING AND RETURNING THE ENCLOSED PROXY CARD USING THE ENCLOSED RETURN ENVELOPE OR VOTING INSTRUCTION FORM PROVIDED WITH THE PRINTED PROXY MATERIALS, AS PROMPTLY AS POSSIBLE SO THAT YOUR SHARES MAY BE REPRESENTED AT THE ANNUAL MEETING.**

## APPENDIX A

# Ultragenyx Pharmaceutical Inc. Amended and Restated 2023 Incentive Plan

## 1. Defined Terms

Exhibit A, which is incorporated by reference, defines the terms used in the Plan and sets forth certain operational rules related to those terms.

## 2. Purpose

The Plan has been established to advance the interests of the Company by providing for the grant to Participants of Stock-based and other incentive Awards. The Plan was originally approved by the Board on April 20, 2023 effective on the Original Effective Date and was amended and restated effective as June 18, 2024 (the “**Effective Date**”).

## 3. Administration

The Administrator has discretionary authority, subject only to the express provisions of the Plan, to (a) interpret and construe the Plan, any rules and regulations under the Plan and the terms and conditions of any Award; (b) determine eligibility for and grant Awards; (c) determine, modify or waive the terms and conditions of any Award; (d) prescribe forms, rules and procedures relating to the Plan; (e) establish and verify the extent of satisfaction of any Performance Criteria or other conditions applicable to the grant, issuance, retention, vesting, exercisability or settlement of any Award; (f) determine the extent to which adjustments are required pursuant to Section 7; (g) approve corrections in the documentation or administration of any Award; and otherwise do all things necessary or appropriate to carry out the purposes of the Plan. Determinations of the Administrator made under the Plan will be conclusive and will bind all parties.

## 4. Limits on Awards Under the Plan

### (a) Number of Shares.

(i) The maximum number of shares of Stock that may be delivered in satisfaction of Awards under the Plan is (A) 8,500,000, plus (B) the number of shares of Stock subject to any Award outstanding under a Prior Plan as of the Original Effective Date that after the Original Effective Date are not issued because such Award is forfeited, canceled, terminates, expires or otherwise lapses without being exercised (to the extent applicable), or is settled in cash. Up to 8,500,000 shares of Stock may be issued in satisfaction of ISOs, but nothing in this Section 4(a) will be construed as requiring that any, or any fixed number of, ISOs be awarded under the Plan. The limits set forth in this Section 4(a) shall be construed to comply with Section 422.

(ii) For purposes of this Section 4(a), if an outstanding Award is forfeited, canceled, terminates, expires or otherwise lapses without being exercised (to the extent applicable), or is settled in cash, the shares of Stock allocable to the terminated portion of such Award or such forfeited shares of Stock shall again be available for issuance under the Plan. Notwithstanding the foregoing, the following shares shall not be available for issuance under the Plan: (A) shares withheld from an Award under the Plan or a Prior Plan to satisfy the tax withholding obligations with respect to such Award, (B) shares withheld from an Award under the Plan or a Prior Plan in payment of the exercise price of an Award requiring exercise, (C) shares repurchased on the open market by the Company using proceeds from the exercise price paid with respect to Awards under the Plan or a Prior Plan, or (D) gross shares subject to an SAR granted under the Plan or a Prior Plan that are not issued in connection with the Stock-settlement of such SAR.

(b) Type of Shares. Stock delivered by the Company under the Plan may be authorized but unissued Stock or previously issued Stock acquired by the Company. No fractional shares of Stock will be delivered under the Plan.

(c) Limit on Non-Employee Director Compensation. The aggregate dollar value of equity-based (based on the grant date fair value of equity-based Awards determined for financial reporting purposes) and cash compensation granted under the Plan or otherwise to any non-employee director shall not exceed \$900,000 during any calendar year for services provided as a non-employee director; provided, however, that in the calendar year in which a non-employee director first joins the Board or during any calendar year in which a non-employee director is designated as Chairman of the Board or Lead Director, the maximum aggregate dollar value of equity-based and cash compensation granted to the non-employee director may be up to \$1,500,000.

## 5. Eligibility and Participation

The Administrator will select Participants from among key Employees and directors of, and consultants and advisors to, the Company and its Affiliates. Eligibility for ISOs is limited to individuals described in the first sentence of this Section 5 who are employees of the Company or of a “parent corporation” or “subsidiary corporation” of the Company as those terms are defined in Section 424 of the Code. Eligibility for Stock Options other than ISOs is limited to individuals described in the first sentence of this Section 5 who are providing direct services on the date of grant of the Stock Option to the Company or to a subsidiary of the Company that would be described in the first sentence of Section 1.409A-1(b)(5)(iii)(E) of the Treasury Regulations.

## 6. Rules Applicable to Awards

### (a) All Awards.

- (i) **Award Provisions.** The Administrator will determine the terms of all Awards, subject to the limitations provided herein. By accepting (or, under such rules as the Administrator may prescribe, being deemed to have accepted) an Award, the Participant will be deemed to have agreed to the terms of the Award and the Plan. Notwithstanding any provision of this Plan to the contrary, awards of an acquired company that are converted, replaced or adjusted in connection with the acquisition may contain terms and conditions that are inconsistent with the terms and conditions specified herein, as determined by the Administrator.
- (ii) **Term of Plan.** No Awards may be made after 10 years from the Effective Date, but previously granted Awards may continue beyond that date in accordance with their terms. Notwithstanding the foregoing, no ISOs may be granted after 10 years from Date of Adoption.
- (iii) **Transferability.** Neither ISOs nor, except as the Administrator otherwise expressly provides in accordance with the second sentence of this Section 6(a)(iii), other Awards may be transferred other than by will or by the laws of descent and distribution. During a Participant’s lifetime, ISOs (and, except as the Administrator otherwise expressly provides in accordance with the second sentence of this Section 6(a)(iii), SARs and NSOs) may be exercised only by the Participant. The Administrator may permit the gratuitous transfer (i.e., transfer not for value) of Awards other than ISOs to any transferee eligible to be covered by the provisions of Form S-8 (under the Securities Act), subject to such limitations as the Administrator may impose.
- (iv) **Vesting, etc.** The Administrator will determine the time or times at which an Award will vest or become exercisable and the terms on which a Stock Option or SAR will remain exercisable. Without limiting the foregoing, the Administrator may at any time accelerate the vesting or exercisability of an Award, regardless of any adverse or potentially adverse tax or other consequences resulting from such acceleration. Unless the Administrator expressly provides otherwise, however, the following rules will apply if a Participant’s Employment ceases:
  - (A) Immediately upon the cessation of the Participant’s Employment and except as provided in (B) and (C) below, each Stock Option and SAR that is then held by the Participant or by the Participant’s permitted transferees, if any, will cease to be exercisable and will terminate and all other Awards that are then held by the Participant or by the Participant’s permitted transferees, if any, to the extent not already vested will be forfeited.
  - (B) Subject to (C) and (D) below, all Stock Options and SARs held by the Participant or the Participant’s permitted transferees, if any, immediately prior to the cessation of the Participant’s Employment, to the extent then exercisable, will remain exercisable for the lesser of (i) a period of three months or (ii) the period ending on the latest date on which such Stock Option or SAR could have been exercised without regard to this Section 6(a)(iv), and will thereupon immediately terminate.
  - (C) All Stock Options and SARs held by a Participant or the Participant’s permitted transferees, if any, immediately prior to the Participant’s death, to the extent then exercisable, will remain exercisable for the lesser of (i) the one year period ending with the first anniversary of the Participant’s death or (ii) the period ending on the latest date on which such Stock Option or SAR could have been exercised without regard to this Section 6(a)(iv), and will thereupon immediately terminate.
  - (D) All Stock Options and SARs (whether or not exercisable) held by a Participant or the Participant’s permitted transferees, if any, immediately prior to the cessation of the Participant’s Employment will immediately terminate upon such notice of cessation of Employment if the termination is for Cause or occurs in circumstances that in the sole determination of the Administrator would have constituted grounds for the Participant’s Employment to be terminated for Cause.
- (v) **Additional Restrictions.** The Administrator may cancel, rescind, withhold or otherwise limit or restrict any Award at any time if the Participant is not in compliance with all applicable provisions of the Award agreement and the Plan, or if the Participant breaches any agreement with the Company or its Affiliates with respect to confidentiality. Without limiting the generality of the foregoing, the Administrator may recover Awards made under the Plan and payments under or gain in respect of any Award to the extent required to comply with Company policy, including the Company’s Clawback Policy.
- (vi) **Taxes.** The delivery, vesting and retention of Stock, cash or other property under an Award are conditioned upon

full satisfaction by the Participant of all tax withholding requirements with respect to the Award. The Administrator will prescribe such rules for the withholding of taxes as it deems necessary. The Administrator may, but need not, hold back shares of Stock from an Award or permit a Participant to tender previously owned shares of Stock in satisfaction of tax withholding requirements (but not in excess of the maximum statutory withholding rate).

- (vii) Dividend Equivalents, Etc. The Administrator may provide for the payment of amounts (on terms and subject to conditions established by the Administrator, except to the extent provided otherwise in this Section 6(a)(vii)) in lieu of cash dividends or other cash distributions with respect to Stock subject to an Award whether or not the holder of such Award is otherwise entitled to share in the actual dividend or distribution in respect of such Award. Any entitlement to dividend equivalents or similar entitlements will be established and administered either consistent with an exemption from, or in compliance with, the requirements of Section 409A. Dividends or dividend equivalent amounts payable in respect of Awards shall be subject to the same limits or restrictions as the Awards to which they relate and shall not be payable until such Awards vest. Notwithstanding the foregoing, no Stock Options or SARs shall provide for payment or accrual of dividends or dividend equivalents.
  - (viii) Rights Limited. Nothing in the Plan will be construed as giving any person the right to continued employment or service with the Company or its Affiliates, or any rights as a stockholder except as to shares of Stock actually issued under the Plan. The loss of existing or potential profit in Awards will not constitute an element of damages in the event of termination of Employment for any reason, even if the termination is in violation of an obligation of the Company or any Affiliate to the Participant.
  - (ix) Coordination with Other Plans. Awards under the Plan may be granted in tandem with, or in satisfaction of or substitution for, other Awards under the Plan or awards made under other compensatory plans or programs of the Company or its Affiliates. For example, but without limiting the generality of the foregoing, awards under other compensatory plans or programs of the Company or its Affiliates may be settled in Stock (including, without limitation, Unrestricted Stock) if the Administrator so determines, in which case the shares delivered will be treated as awarded under the Plan (and will reduce the number of shares thereafter available under the Plan in accordance with the rules set forth in Section 4).
  - (x) Section 409A. Each Award will contain such terms as the Administrator determines, and will be construed and administered, such that the Award either qualifies for an exemption from the requirements of Section 409A or satisfies such requirements.
- (b) Stock Options and SARs.
- (i) Time and Manner of Exercise. Unless the Administrator expressly provides otherwise, no Stock Option or SAR will be deemed to have been exercised until the Administrator receives a notice of exercise (in form acceptable to the Administrator), which may be an electronic notice, signed (including electronic signature in form acceptable to the Administrator) by the appropriate person and accompanied by any payment required under the Award. A Stock Option or SAR exercised by any person other than the Participant will not be deemed to have been exercised until the Administrator has received such evidence as it may require that the person exercising the Award has the right to do so.
  - (ii) Exercise Price. The exercise price (or the base value from which appreciation is to be measured) of each Award requiring exercise will be no less than 100% (or in the case of an ISO granted to a ten-percent shareholder within the meaning of Section 422, 110%) of the Fair Market Value of the Stock subject to the Award, determined as of the date of grant, or such higher amount as the Administrator may determine in connection with the grant.
  - (iii) Payment of Exercise Price. Where the exercise of an Award is to be accompanied by payment, payment of the exercise price will be by cash or check acceptable to the Administrator or by such other legally permissible means, if any, as may be acceptable to the Administrator.
  - (iv) Maximum Term. Stock Options and SARs will have a maximum term not to exceed 10 years from the date of grant (or five years from the date of grant in the case of an ISO granted to a ten-percent shareholder described in Section 6(b)(ii) above); provided, however, that, if a Participant still holding an outstanding but unexercised NSO or SAR 10 years from the date of grant (or, in the case of an NSO or SAR with a maximum term of less than 10 years, such maximum term) is prohibited by applicable law from exercising such Stock Options or SARs, and if at such time the Stock is publicly traded (as determined by the Administrator), the maximum term of such Award will instead be deemed to expire on the 30<sup>th</sup> day following the date the Participant is no longer prohibited from engaging in such open market sales.
  - (v) No Repricing without Stockholder Approval. The Company shall not, without stockholder approval (except in the case of a change in the Company's capitalization (as described in Section 7(b)), (A) reduce the exercise price of a Stock Option or SAR, (B) other than in the case of Covered Transaction, at any time when the exercise price of a Stock Option or SAR is above the Fair Market Value of a share of Stock, cancel and re-grant or exchange such Stock Option or SAR for cash or a new Award having a lower (or no) exercise price, or (c) take any other action with respect to an Award that would be treated as a repricing under generally accepted accounting principles.
  - (vi) No Reload Grants. Stock Options shall not be granted under the Plan in consideration for, and shall not be conditioned upon the delivery of, shares of Stock to the Company in payment of the exercise price and/or tax withholding obligation under any other employee stock option.

## 7. Effect of Certain Transactions

- (a) Mergers, etc. Except as otherwise provided in an Award agreement, the following provisions will apply in the event of a Covered Transaction:
- (i) Assumption or Substitution. If the Covered Transaction is one in which there is an acquiring or surviving entity, the Administrator may (but, for the avoidance of doubt, need not) provide (i) for the assumption or continuation of some or all outstanding Awards or any portion thereof or (ii) for the grant of new awards in substitution thereof by the acquiror or survivor or an affiliate of the acquiror or survivor. Notwithstanding any provision of this Section 7, in the event of a Covered Transaction in which the acquiring or surviving entity does not assume or continue outstanding Awards or provide for the grant of new awards in substitution thereof, all Awards that are not assumed, continued or substituted for shall be treated as follows effective immediately prior to the Covered Transaction: (A) in the case of a Stock Option or SAR, the Participant shall have the ability to exercise such Stock Option or SAR, including any portion of the Stock Option or SAR not previously exercisable, (B) in the case of any Award the vesting of which is in whole or in part subject to Performance Criteria, all conditions to the grant, issuance, retention, vesting or transferability of, or any other restrictions applicable to, such Award shall immediately lapse and the Participant shall have the right to receive a payment based on target level achievement or actual performance through a date determined by the Committee, as determined by the Committee, and (C) in the case of any other Award all conditions to the grant, issuance, retention, vesting or transferability of, or any other restrictions applicable to, such Award shall immediately lapse. In no event shall any action be taken pursuant to this Section 7(a)(i) that would change the payment or settlement date of an Award in a manner that would result in the imposition of any additional taxes or penalties pursuant to Section 409A of the Code.
  - (ii) Cash-Out of Awards. Subject to Section 7(a)(v) below the Administrator may (but, for the avoidance of doubt, need not) provide for payment (a "cash-out"), with respect to some or all Awards or any portion thereof, equal in the case of each affected Award or portion thereof to the excess, if any, of (A) the fair market value of one share of Stock (as determined by the Administrator in its reasonable discretion) times the number of shares of Stock subject to the Award or such portion, over (B) the aggregate exercise or purchase price, if any, under the Award or such portion (in the case of an SAR, the aggregate base value above which appreciation is measured), in each case on such payment terms (which need not be the same as the terms of payment to holders of Stock) and other terms, and subject to such conditions, as the Administrator determines; provided, however, that if the fair market value of one share of Stock (as determined by the Administrator in its reasonable discretion) does not exceed the exercise price of such Award, then the Award shall be cancelled without any payment of consideration therefor.
  - (iii) Acceleration of Certain Awards. Subject to Section 7(a)(v) below, the Administrator may (but, for the avoidance of doubt, need not) provide that any Award requiring exercise will become exercisable, in full or in part and/or that the delivery of any shares of Stock remaining deliverable under any outstanding Award of Stock Units (including Restricted Stock Units and Performance Awards to the extent consisting of Stock Units) will be accelerated in full or in part, in each case on a basis that gives the holder of the Award a reasonable opportunity, as determined by the Administrator, following exercise of the Award or the delivery of the shares, as the case may be, to participate as a stockholder in the Covered Transaction.
  - (iv) Termination of Awards Upon Consummation of Covered Transaction. Except as the Administrator may otherwise determine in any case, each Award will automatically terminate (and in the case of outstanding shares of Restricted Stock, will automatically be forfeited) upon consummation of the Covered Transaction, other than Awards assumed or continued pursuant to Section 7(a)(i) above.
  - (v) Additional Limitations. Any share of Stock and any cash or other property delivered pursuant to Section 7(a)(ii) or Section 7(a)(iii) above with respect to an Award may, in the discretion of the Administrator, contain such restrictions, if any, as the Administrator deems appropriate to reflect any Performance Criteria or other vesting conditions to which the Award was subject and that did not lapse (and were not satisfied) in connection with the Covered Transaction. For purposes of the immediately preceding sentence, a cash-out under Section 7(a)(ii) above or acceleration under Section 7(a)(iii) above will not, in and of itself, be treated as the lapsing (or satisfaction) of a Performance Criteria or other vesting condition. In the case of Restricted Stock that does not vest and is not forfeited in connection with the Covered Transaction, the Administrator may require that any amounts delivered, exchanged or otherwise paid in respect of such Stock in connection with the Covered Transaction be placed in escrow or otherwise made subject to such restrictions as the Administrator deems appropriate to carry out the intent of the Plan.
- (b) Changes in and Distributions With Respect to Stock.
- (i) Basic Adjustment Provisions. In the event of a stock dividend, stock split or combination of shares (including a reverse stock split), recapitalization or other change in the Company's capital structure that constitutes an equity restructuring within the meaning of FASB ASC 718, the Administrator will make appropriate adjustments to the maximum number of shares specified in Section 4(a) that may be delivered under the Plan and will also make appropriate adjustments to the number and kind of shares of stock or securities subject to Awards then outstanding or subsequently granted, any exercise prices relating to Awards and any other provision of Awards affected by such change.
  - (ii) Certain Other Adjustments. The Administrator may also make adjustments of the type described in Section 7(b)(i) above to take into account distributions to stockholders

other than those provided for in Section 7(a) and 7(b)(i), or any other event, if the Administrator determines that adjustments are appropriate to avoid distortion in the operation of the Plan, having due regard for the qualification of ISOs under Section 422 and the requirements of Section 409A, where applicable.

- (iii) Continuing Application of Plan Terms. References in the Plan to shares of Stock will be construed to include any stock or securities resulting from an adjustment pursuant to this Section 7.

## 8. Legal Conditions on Delivery of Stock

The Company will not be obligated to deliver any shares of Stock pursuant to the Plan or to remove any restriction from shares of Stock previously delivered under the Plan until: (a) the Company is satisfied that all legal matters in connection with the issuance and delivery of such shares have been addressed and resolved; (b) if the outstanding Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (c) all conditions of the Award have been satisfied or waived. The Company may require, as a condition to exercise of the Award, such representations or agreements as counsel for the Company may consider appropriate to avoid violation of the Securities Act or any applicable state or non-U.S. securities law. Any Stock required to be issued to Participants under the Plan will be evidenced in such manner as the Administrator may deem appropriate, including book-entry registration or delivery of stock certificates. In the event that the Administrator determines that Stock certificates will be issued to Participants under the Plan, the Administrator may require that certificates evidencing Stock issued under the Plan bear an appropriate legend reflecting any restriction on transfer applicable to such Stock, and the Company may hold the certificates pending lapse of the applicable restrictions.

## 9. Amendment and Termination

The Administrator may at any time or times amend the Plan or any outstanding Award for any purpose which may at the time be permitted by law, and may at any time terminate the Plan as to any future grants of Awards; provided, that except as otherwise expressly provided in the Plan the Administrator may not, without the Participant's consent, alter the terms of an Award so as to affect materially and adversely the Participant's rights under the Award, unless the Administrator expressly reserved the right to do so at the time the Award was granted. Any amendments to the Plan will be conditioned upon stockholder approval only to the extent, if any, such approval is required by law (including the Code and applicable stock exchange requirements), as determined by the Administrator.

## 10. Other Compensation Arrangements

The existence of the Plan or the grant of any Award will not in any way affect the Company's right to Award a person bonuses or other compensation in addition to Awards under the Plan.

## 11. Miscellaneous

- (a) Waiver of Jury Trial. By accepting an Award under the Plan and to the extent permitted under applicable law, each Participant waives any right to a trial by jury in any action, proceeding or counterclaim concerning any rights under the Plan and any Award, or under any amendment, waiver, consent, instrument, document or other agreement delivered or which in the future may be delivered in connection therewith, and agrees that any such action, proceedings or counterclaim will be tried before a court and not before a jury. By accepting an Award under the Plan, each Participant certifies that no officer, representative, or attorney of the Company has represented, expressly or otherwise, that the Company would not, in the event of any action, proceeding or counterclaim, seek to enforce the foregoing waivers. Notwithstanding anything to the contrary in the Plan, nothing herein is to be construed as limiting the ability of the Company and a Participant to agree to submit disputes arising under the terms of the Plan or any Award made hereunder to binding arbitration or as limiting the ability of the Company to require any eligible individual to agree to submit such disputes to binding arbitration as a condition of receiving an Award hereunder.
- (b) Limitation of Liability. Notwithstanding anything to the contrary in the Plan, neither the Company, nor any Affiliate, nor the Administrator, nor any person acting on behalf of the Company, any Affiliate, or the Administrator, will be liable to any Participant or to the estate or beneficiary of any Participant or to any other holder of an Award by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of an Award to satisfy the requirements of Section 422 or Section 409A or by reason of Section 4999 of the Code, or otherwise asserted with respect to the Award.

## 12. Establishment of Sub-Plans

The Administrator may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable blue sky, securities or tax laws of various jurisdictions. The Administrator will establish such sub-plans by adopting supplements to the Plan setting forth (a) such limitations on the Administrator's discretion under the Plan as it deems necessary or desirable and (b) such additional terms and conditions not otherwise inconsistent with the Plan as it deems necessary or desirable. All supplements so established will be deemed to be part of the Plan, but each supplement will apply only to Participants within the affected jurisdiction (as determined by the Administrator).

## 13. Governing Law

- (a) Certain Requirements of Corporate Law. Awards will be granted and administered consistent with the requirements of applicable Delaware law relating to the issuance of stock and the consideration to be received therefor, and with the applicable requirements of the stock exchanges or other trading systems on which the Stock is listed or entered for trading, in each case as determined by the Administrator.
- (b) Other Matters. Except as otherwise provided by the express terms of an Award agreement, under a sub-plan described in Section 12 or as provided in Section 13(a) above, the provisions of the Plan and of Awards under the Plan and all claims or disputes arising out of or based upon the Plan or any Award under the Plan or relating to the subject matter hereof or thereof will be governed by and construed in accordance with the domestic substantive laws of the State of Delaware without giving effect to any choice or conflict of laws provision or rule that would cause the application of the domestic substantive laws of any other jurisdiction.
- (c) Jurisdiction. By accepting an Award, each Participant will be deemed to (a) have submitted irrevocably and unconditionally to the jurisdiction of the federal and state courts located within the geographic boundaries of the United States District Court for the Northern District of California for the purpose of any suit, action or other proceeding arising out of or based upon the Plan or any Award; (b) agree not to commence any suit, action or other proceeding arising out of or based upon the Plan or an Award, except in the federal and state courts located within the geographic boundaries of the United States District Court for the Northern District of California; and (c) waive, and agree not to assert, by way of motion as a defense or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that the Plan or an Award or the subject matter thereof may not be enforced in or by such court.



## EXHIBIT A

# Definition of Terms

The following terms, when used in the Plan, will have the meanings and be subject to the provisions set forth below:

“Administrator”: The Compensation Committee, except that the Compensation Committee may, subject to applicable law, delegate (i) to one or more of its members (or one or more other members of the Board (including the full Board)) such of its duties, powers and responsibilities as it may determine; (ii) to one or more officers or Employees of the Company the power to grant Awards to the extent permitted by Section 157(c) of the Delaware General Corporation Law; and (iii) to such Employees or other persons as it determines such ministerial tasks as it deems appropriate. In the event of any delegation described in the preceding sentence, the term “Administrator” will include the person or persons so delegated to the extent of such delegation.

“Affiliate”: Any corporation or other entity that stands in a relationship to the Company that would result in the Company and such corporation or other entity being treated as one employer under Section 414(b) and Section 414(c) of the Code.

“Award”: Any or a combination of the following:

- (i) Stock Options.
- (ii) SARs.
- (iii) Restricted Stock.
- (iv) Unrestricted Stock.
- (v) Stock Units, including Restricted Stock Units.
- (vi) Performance Awards.
- (vii) Cash Awards.
- (viii) Awards (other than Awards described in (i) through (vii) above) that are convertible into or otherwise based on Stock.

“Board”: The Board of Directors of the Company.

“Cash Award”: An Award denominated in cash.

“Cause”: In the case of any Participant who is party to an employment or severance-benefit agreement that contains a definition of “Cause,” the definition set forth in such agreement will apply with respect to such Participant under the Plan. In the case of any other Participant, “Cause” will mean, as determined by the Administrator in its reasonable judgment, (i) a substantial failure of the Participant to perform the Participant's duties and responsibilities to the Company or subsidiaries or substantial negligence in the performance of such duties and responsibilities; (ii) the commission by the Participant of a felony or a crime involving moral turpitude; (iii) the commission by the Participant of theft, fraud, embezzlement, material breach of trust or any material act of dishonesty involving the Company or any of its subsidiaries; (iv) a significant violation by the Participant of the code of conduct of the Company or its subsidiaries of any material policy of the Company or its subsidiaries, or of any statutory or common law duty of loyalty to the Company or its subsidiaries; (v) material breach of any of the terms of the Plan or any Award made under the Plan, or of the terms of any other agreement between the Company or subsidiaries and the Participant; or (vi) other conduct by the Participant that could be expected to be harmful to the business, interests or reputation of the Company.

“Code”: The U.S. Internal Revenue Code of 1986 as from time to time amended and in effect, or any successor statute as from time to time in effect.

“Company”: Ultragenyx Pharmaceutical Inc., and except as utilized in the definition of Covered Transaction, any successor corporation.

“Compensation Committee”: The Compensation Committee of the Board.

“Covered Transaction”: The occurrence of any one of the following events:

- (i) a consolidation, merger, or similar transaction or series of related transactions in which the Company is not the surviving corporation or which results in the acquisition of all or substantially all of the Company's then outstanding Stock by a single person or entity or by a group of persons and/or entities acting in concert (a “**Transaction**”), excluding a Transaction which would result in the holders of the voting securities of the Company outstanding immediately prior to such merger or consolidation continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or any parent thereof) at least 50% of the combined voting power of the securities of the Company or such surviving entity or any parent thereof outstanding immediately after such merger or consolidation;
- (ii) a sale or transfer of all or substantially all the Company's assets, excluding a sale or transfer to an entity, at least 50% of the combined voting power of the voting securities of which is owned by stockholders of the Company in substantially the same proportions as their ownership of the Company immediately prior to such sale; or
- (iii) a dissolution or liquidation of the Company.

Where a Covered Transaction involves a tender offer that is reasonably expected to be followed by a merger described in clause (i) (as determined by the Administrator), the Covered Transaction will be deemed to have occurred upon consummation of the tender offer. Notwithstanding the foregoing, for each Award that constitutes deferred compensation under Section 409A, and to the extent required to avoid accelerated taxation and/or tax penalties under Section 409A, a Covered Transaction shall be deemed to have occurred under the Plan with respect to such Award only if a change in the ownership or effective control of the Company or a change in ownership of a substantial portion of the assets of the Company shall also be deemed to have occurred under Section 409A.

“Date of Adoption”: April 20, 2024.

“Employee”: Any person who is employed by the Company or an Affiliate.

“Employment”: A Participant's employment or other service relationship with the Company and its Affiliates, which may include service as a director, consultant or independent contractor. Employment will be deemed to continue, unless the Administrator expressly provides otherwise, so long as the Participant is employed

by, or otherwise is providing services in a capacity described in Section 5 to the Company or an Affiliate. If a Participant's employment or other service relationship is with an Affiliate and that entity ceases to be an Affiliate, the Participant's Employment will be deemed to have terminated when the entity ceases to be an Affiliate unless the Participant transfers Employment to the Company or its remaining Affiliates. Notwithstanding the foregoing and the definition of "Affiliate" above, in construing the provisions of any Award relating to the payment of "nonqualified deferred compensation" (subject to Section 409A) upon a termination or cessation of Employment, references to termination or cessation of employment, separation from service, retirement or similar or correlative terms will be construed to require a "separation from service" (as that term is defined in Section 1.409A-1(h) of the Treasury Regulations) from the Company and from all other corporations and trades or businesses, if any, that would be treated as a single "service recipient" with the Company under Section 1.409A-1(h)(3) of the Treasury Regulations. The Company may, but need not, elect in writing, subject to the applicable limitations under Section 409A, any of the special elective rules prescribed in Section 1.409A-1(h) of the Treasury Regulations for purposes of determining whether a "separation from service" has occurred. Any such written election will be deemed a part of the Plan.

"Fair Market Value":

- (a) If the Stock is readily traded on an established national exchange or trading system (including the Nasdaq Global Market), the closing price of the Stock as reported by the principal exchange on which such Stock is traded; provided, however, that if such day is not a trading day, Fair Market Value will mean the reported closing price of the Stock for the immediately preceding day that is a trading day.
- (b) If the Stock is not traded on an established national exchange or trading system, the average of the bid and ask prices for such Stock where the bid and ask prices are quoted.
- (c) If the Stock cannot be valued pursuant to clauses (a) or (b), the value as determined in good faith by the Board in its sole discretion consistent with the rules of Section 422 and Section 409A to the extent applicable.

"ISO": A Stock Option intended to be an "incentive stock option" within the meaning of Section 422. Each Stock Option granted pursuant to the Plan will be treated as providing by its terms that it is to be an NSO unless, as of the date of grant, it is expressly designated as an ISO.

"NSO": A Stock Option that is not intended to be an "incentive stock option" within the meaning of Section 422.

"Participant": A person who is granted an Award under the Plan.

"Original Effective Date": June 7, 2023.

"Performance Award": An Award subject to Performance Criteria.

"Performance Criteria": Specified criteria, other than the mere continuation of Employment or the mere passage of time, the satisfaction of which is a condition for the grant, exercisability, vesting or full enjoyment of an Award. Performance Criteria will include but not be limited to any objectively determinable measure of performance relating to any, or any combination, of the following (measured either absolutely or by reference to an index or indices and determined either on a consolidated basis or, as the context permits, on a divisional, subsidiary, line of business, project or geographical basis or in combinations thereof): sales; revenues; assets; expenses; earnings from operations; earnings before or after deduction for all or any portion of interest, taxes, depreciation, amortization, incentives, service fees or extraordinary or special items, whether or not on a continuing operations or an aggregate or per share basis; net income or net income per common share (basic or diluted); return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow, free cash flow, cash flow return on investment, or net cash provided by operations; stock price, dividends or total stockholder return; development of new technologies or products; sales of particular products or services; economic value created or added; operating margin or profit margin; customer acquisition or retention; raising or refinancing of capital; successful hiring of key individuals; resolution of significant litigation; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings; or strategic business criteria, consisting of one or more objectives based on the following goals: meeting specified market penetration or value added, product development or introduction (including, without limitation, any clinical trial accomplishments, regulatory or other filings or approvals, or other product development milestones), geographic business expansion, cost targets, cost reductions or savings, customer satisfaction, operating efficiency, acquisition or retention, employee satisfaction, information technology, corporate development (including, without limitation, licenses, innovation, research or establishment of third party collaborations), manufacturing or process development, legal compliance or risk reduction, patent application or issuance goals, or goals relating to acquisitions or divestitures (in whole or in part), joint ventures or strategic alliances. Performance Criteria and any targets with respect thereto determined by the Administrator need not be based upon an increase, a positive or improved result or avoidance of loss.

"Plan": The Ultragenyx Pharmaceutical Inc. 2023 Incentive Plan as amended and restated effective as of the Effective Date, and as from time to time amended and in effect.

"Prior Plans": The Ultragenyx Pharmaceutical Inc. 2011 Incentive Plan and the Ultragenyx Pharmaceutical Inc. 2014 Incentive Plan, in each case as amended from time to time.

"Restricted Stock": Stock subject to restrictions requiring that it be redelivered or offered for sale to the Company if specified conditions are not satisfied.

"Restricted Stock Unit": A Stock Unit that is, or as to which the delivery of Stock or cash in lieu of Stock is, subject to the satisfaction of specified performance or other vesting conditions.

"SAR": A right entitling the holder upon exercise to receive an amount (payable in cash or in shares of Stock of equivalent value) equal to the excess of the Fair Market Value of the shares of Stock subject to the right over the base value from which appreciation under the SAR is to be measured.

"Section 409A": Section 409A of the Code.

"Section 422": Section 422 of the Code.

"Securities Act": The U.S. Securities Act of 1933, as amended.

"Stock": Common stock of the Company, par value \$0.001 per share.

**“Stock Option”:** An option entitling the holder to acquire shares of Stock upon payment of the exercise price.

**“Stock Unit”:** An unfunded and unsecured promise, denominated in shares of Stock, to deliver Stock or cash measured by the value of Stock in the future.

**“Unrestricted Stock”:** Stock not subject to any restrictions under the terms of the Award.



**60 Leveroni Court**  
**Novato, California 94949**  
*<https://www.ultragenyx.com/>*





ULTRAGENYX PHARMACEUTICAL INC.  
60 LEVERONI COURT  
NOVATO, CA 94949



**SCAN TO**  
VIEW MATERIALS & VOTE

**VOTE BY INTERNET**  
*Before The Meeting* - Go to [www.proxyvote.com](http://www.proxyvote.com) or scan the QR Barcode above

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/RARE2024](http://www.virtualshareholdermeeting.com/RARE2024)

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:

V46219-P10936

KEEP THIS PORTION FOR YOUR RECORDS  
DETACH AND RETURN THIS PORTION ONLY

THIS PROXY CARD IS VALID ONLY WHEN SIGNED AND DATED.

**ULTRAGENYX PHARMACEUTICAL INC.**

**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. Deborah Dunsire, M.D.	<input type="checkbox"/>	<input type="checkbox"/>
1b. Michael Narachi	<input type="checkbox"/>	<input type="checkbox"/>
1c. Corsee D. Sanders, Ph.D.	<input type="checkbox"/>	<input type="checkbox"/>

The Board of Directors recommends you vote FOR proposals 2, 3 and 4:

	For	Against	Abstain
2. Approval of the Amended and Restated 2023 Incentive Plan.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3. Ratification of the selection of Ernst & Young LLP as our independent registered public accounting firm for the fiscal year ending December 31, 2024.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4. Advisory (non-binding) vote to approve the compensation of our named executive officers.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Please sign exactly as your name(s) appears 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.

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Signature [PLEASE SIGN WITHIN BOX]

Date

--	--

Signature (Joint Owners)

Date

**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, 2023  
are available at [www.proxyvote.com](http://www.proxyvote.com).

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**ULTRAGENYX PHARMACEUTICAL INC.**

**Annual Meeting of Stockholders**

**June 18, 2024 at 9:30 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 2024 Annual Meeting of Stockholders to be held at 9:30 a.m., Pacific Time, on June 18, 2024, 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).**

Continued and to be signed on reverse side