

2023 Corporate Responsibility Report

Going beyond every day.™

ultragenyx



Dr. Michelle Fynan's Story

Dr. Michelle Fynan, diagnosed with Osteogenesis Imperfecta (OI) in childhood, exemplifies resilience by having overcome numerous fractures and surgeries to become a psychotherapist and an advocate, raising awareness and supporting others with OI, including her own daughters.

[Learn more about Dr. Fynan](#)



Forward-Looking Statements and Other Important Legal Information

This document and the materials or websites cross-referenced contain statements that are aspirational or reflective of our views about our future performance that constitute “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are generally identified through the inclusion of words such as “aim,” “anticipate,” “aspire,” “believe,” “commit,” “endeavor,” “estimate,” “expect,” “goal,” “intend,” “may,” “plan,” “seek,” “strive,” “target,” “will,” vision, “mission,” “strategy,” “commitment” and “work,” or similar statements or variations of such terms and other similar expressions. The forward-looking statements in this document and the materials or websites cross-referenced concern Ultragenyx’s goals, progress or expectations with respect to corporate responsibility, sustainability, patients, products, product candidates, employees, environmental matters, policy and business risks and opportunities. Forward-looking statements inherently involve risks and uncertainties that could cause actual results to differ materially from those predicted in such statements. These statements are based on numerous assumptions that we believe are reasonable but are open to a wide range of uncertainties and business risks. In addition, these statements may be based on standards for measuring progress that are still developing, controls and processes that continue to evolve, and assumptions that are subject to

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About this Report

Ultragenyx Pharmaceutical Inc. (Ultragenyx) is a rare disease drug development company.

Ultragenyx Pharmaceutical Inc. (Ultragenyx) is headquartered in Novato, California. We have offices and laboratories in 12 countries across North America, Europe, Asia and Latin America. In the U.S., we have offices and/or laboratories in California, Florida, Massachusetts, Texas and Utah.

Ultragenyx prepares an annual corporate responsibility report. This 2023 report contains disclosures for the period January 1 through December 31, 2023, unless otherwise noted. Data may be restated from previous years of reporting for several reasons (e.g., information has been updated or was not available at the time of a previous report, improvements in data collection or methodology or data errors). In the case of changes in data or information that results in a material restatement, a note is provided with the restated data or information.

The scope of this report is Ultragenyx’s wholly-owned operations globally. Third-party manufacturing is not included.

The term “employees” refers to our full-time employees, while the term “workforce” is used to refer to the wider groups of people working for and with us, including full- and part-time employees and contingent staff.

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Contact

We welcome your feedback. Please contact us at cr@ultragenyx.com with your comments and suggestions about this report.



Table of Contents

A Letter From Our CEO	4	People	46
About Us	7	Communities	66
Corporate Responsibility Approach	10	Planet	77
Innovation	14	Governance	84
Patients	33	SASB Index	97
		GRI Index	100



A Letter From Our CEO

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

Founder, President and CEO

Dear Stakeholders,

I feel it is a privilege to work in rare disease, yet our job comes with a great deal of responsibility. We must do whatever we can, as quickly as we can, to help the many families and individuals struggling every day to get access to the treatments they need. We're approaching this in multiple ways — through our own innovation, our initiatives to influence rare disease policy, our support for the parents and foundations working to develop new therapies, and our commitment to making our therapies accessible to all who can benefit.

In 2023, we made significant progress on multiple of our key programs in diseases with no approved therapies. We are particularly excited about potential treatments for osteogenesis imperfecta (OI) and Angelman syndrome, where we will have additional data to report later this year. In OI (“brittle bone disease”), our antibody has been shown to significantly reduce fractures and improve bone strength in a Phase 2 clinical trial and this has resulted in some patients in our study moving from wheelchairs to walking unaided. For Angelman syndrome, a developmental brain disorder, early Phase 1/2 clinical trial results with our antisense oligonucleotide demonstrate promising improvements in cognition, communication and motor skills in affected children.

We also opened our gene therapy manufacturing facility in Bedford, Massachusetts in 2023. It is equipped to handle both traditional Human Embryonic Kidney (HEK) manufacturing and our innovative Pinnacle PCL™ (Producer Cell Line) platform, enabling us to produce a wide range of gene therapies. This facility's unique ability to run multiple production cycles annually increases our capacity to develop

treatments. Moreover, the efficiencies we have achieved in the manufacturing process are crucial in reducing the cost of gene therapies. This reduction in cost is vital for making these therapies more accessible worldwide, especially in developing countries where such treatments can be life changing. Our goal is to set a new standard in gene therapy, making these advanced treatments more widely available for diseases that have no other treatment options.

This brings me to one of the big topics our industry has been grappling with as innovative therapeutic approaches are maturing and entering the market, and that is healthcare equity and accessibility. We're at an unprecedented time in drug development where scientific discoveries have given us an array of tools to identify and target previously intractable diseases. It is important that our healthcare systems support getting effective treatments to the individuals and families that will benefit from them, and this has long been a crucial part of our mission at Ultragenyx.

A Letter From Our CEO (cont.)

A key lever for this is rare disease policy. Developing therapies in rare and ultrarare diseases is challenging for myriad reasons: patient populations are small, the progression of these diseases is often slow and varies from person to person and clinical symptoms are often irreversible. We have been pushing for the U.S. Food and Drug Administration (FDA) to use the accelerated approval pathway in those areas where the science for using biomarkers is clear and may be the only route to developing new drugs. I've written a number of editorials on this topic, and in February 2024 we supported a workshop with the Reagan-Udall Foundation for the FDA entitled, "Qualifying Biomarkers to Support Rare Disease Regulatory Pathways." We are hopeful that public forums like this will pave the way to clear and consistent regulatory guidance that supports the use of accelerated approval for ultrarare diseases.

Another critical area to support health equity and access is through improved diagnosis. Accurate and timely diagnosis of disease is imperative to patients receiving optimal care and it should be a responsibility of the healthcare system. That said, one of the greatest challenges in rare disease is getting diseases properly diagnosed. Delays in diagnosis can result in patients receiving ineffective treatments and unnecessary testing, as well as the possibility of missing their opportunity for effective treatment altogether.

Unfortunately, we live in an environment where payers often decline reimbursement of genetic testing, which is critical to a proper diagnosis in rare and ultrarare disease areas. This clearly needs to change, and meanwhile it is imperative that companies like ours step up to fill this gap through sponsored testing programs, which are often the only way that individuals will receive an accurate, timely diagnosis and treatment.

I'm proud of these efforts and others at the company to continue to do what's right for patients and for the communities where we live and work. In 2023, we continued to run our Rare Bootcamp™ for parents and other patient advocates seeking to develop rare disease therapies, and we launched an inaugural global "Days of Service" initiative at Ultragenyx.

This year we also have worked to improve our disclosures in areas such as quality, clinical trials, cybersecurity, supply chain, and business continuity, reflecting feedback received from our stakeholders. We're pleased that our efforts are being recognized in our ratings. Last year, S&P ranked us 5th in North America and 8th globally across the biotech industry and our Sustainability Environmental, Social and Governance (ESG) risk rating improved to a "Low Risk" category. I welcome our communities to continue to engage with us on our corporate responsibility initiatives.

"Generosity" is a core value at Ultragenyx. It is fundamental to our purpose as a rare disease company, and I'm inspired every day by the commitment and drive our team members have to give back. Together, we will continue to push for progress and change to support rare disease drug development for the industry and the communities of patients waiting for proper care.

Sincerely,



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

Founder, President and CEO

2023 Awards



*for the second consecutive year



Listed among the fast-growing companies in the Life Sciences industry on the **2023 Deloitte Technology Fast 500™**, showcasing 250% revenue growth over the last 3 years.

Top Workplaces Culture Excellence Awards



About Us

Vision: Lead the future of rare disease medicine

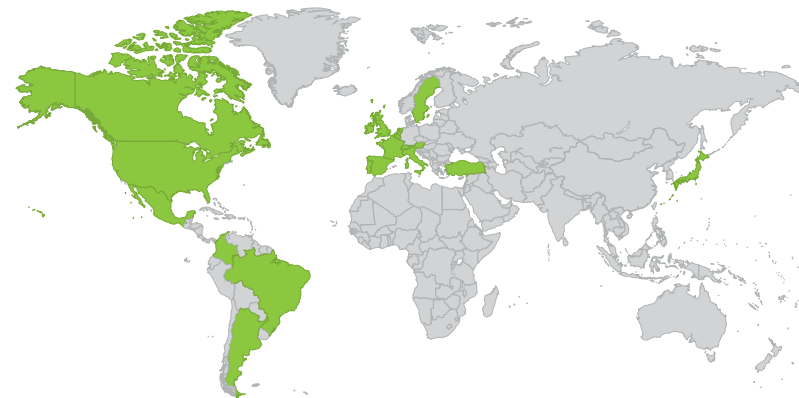
Mission: Transform the lives of people with rare disease



One Ultra Team

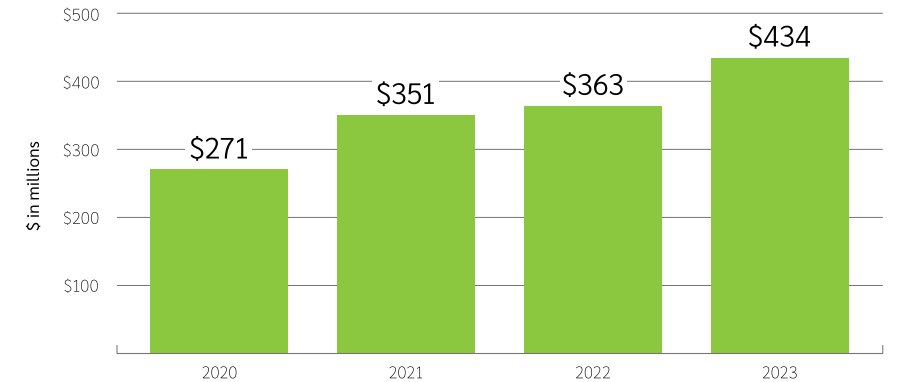
Ultragenyx Pharmaceutical Inc. (Ultragenyx) is a biopharmaceutical company headquartered in Novato, California, that is committed to bringing novel products to patients for the treatment of rare and ultrarare diseases, with a focus on serious, debilitating genetic diseases. Our purpose is to lead the 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.

1,270+ employees worldwide in 20 countries



More than 450,000 square feet of office and laboratory space in 12 countries

Revenue



In 2023, total revenue increased by **20%** from the previous year, with Crysvisa revenue growing by **17%** to **\$328 million**, including a **77%** increase in sales from Latin America and Turkey. Dojolvi revenue grew by **27%** in 2023 from the previous year to **\$71 million**.

\$648.5

million in R&D investment

>4,700

patients treated through commercial or expanded access in more than 50 countries

>130

clinical trial sites in operation in 19 countries

Provided updates on key development programs at the Ultragenyx 2023 Analyst Day

Opened our 112,500 square foot Gene Therapy Manufacturing Facility (GTMF) in Bedford, Massachusetts

Four Approved Treatments for Five Rare Diseases



Crysvita

X-Linked Hypophosphatemia (XLH) & Tumor-Induced Osteomalacia (TIO)

Mepsevii
(vestronidase alfa-vjbk)
injection, for intravenous use
10 mg/5 mL (2 mg/mL)

Mepsevii

Mucopolysaccharidosis Type VII (MPS VII)



Dojolvi

Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)



Evkeeza

Homozygous Familial Hypercholesterolemia (HoFH)

2023 UPDATES

Evkeeza:

Successfully filed reimbursement dossiers in 11 markets, with access achieved in 8 markets. Received marketing approval in Canada and Japan, and an expanded indication in children aged 5 years and older from the European Commission.

Crysvita:

Achieved marketing authorization in Argentina. Received expanded reimbursement for pediatric patients in Mexico and Brazil, with Brazil moving to full reimbursement.

Corporate Responsibility Approach

Ultragenyx's corporate responsibility approach, guided by a materiality assessment, focuses on six key pillars to prioritize efforts and resources towards initiatives that significantly impact our business and stakeholders.

Strategic Framework

Ultragenyx is committed to ethics, integrity and corporate responsibility, integrating these principles across all levels of our business. Our leadership sets a powerful example, passionately focused on enhancing the lives of individuals with rare diseases, empowering our employees, and contributing positively to the communities we serve.

Guided by the principle of doing the right thing, we continue to strive for excellence and improvement. This deliberate approach supports our efforts to consistently consider the sustainability and social impact implications of our actions and decisions.

In 2021, we formalized our corporate responsibility program and based on a materiality assessment, we developed a strategic framework with six pillars: **Innovation, Patients, People, Communities, Planet and Governance**. These pillars help focus our efforts and our communications with both internal and external audiences, and allocation of our resources towards initiatives that support the creation of long-term value and positive impact for our shareholders and society. They also inform the structure of this report and guide the development

of our corporate responsibility strategy, starting with the identification of broad aspirations and objectives, highlighted at the beginning of each chapter.

This report covers a wide range of business practices within our company and is meant to convey a clear and authentic story about Ultragenyx. We are committed to engaging meaningfully with our stakeholders. We have ongoing engagements with a wide range of internal and external stakeholders, which help inform and drive various aspects of our corporate responsibility program from enhancing disclosure strategies to improving information transparency.

This year, we have broadened our disclosures on several key topics, including clinical trials, quality and safety, public policy participation, business continuity, cybersecurity, and supply chain management.

Innovation



Patients



People



Communities



Planet



Governance



Materiality Assessment

In 2021, we conducted a materiality analysis to determine our priorities. This process took into account both internal and external perspectives and focused on identifying the topics that could have the greatest impact on our business and that matter most to our stakeholders.



Material topics are listed below each pillar; **bolded** topics have been identified as higher priority

Innovation

- R&D
- **Clinical Trial Practices**
- **Patient Safety**
- **Product Quality**

Patients

- **Access & Affordability**
- Patient Advocacy

People

- **Employee Equity, Diversity & Inclusion**
- **Employee Health & Safety**
- **Workforce Management**

Communities

- Community Relations

Planet




- Climate Change Risks & Management
- Energy Management
- Product Stewardship
- Waste Management
- Water Management

Governance

- Ethical Practices & Corporate Behavior
- Governance Structures & Mechanisms
- **Human Rights**
- Management of the Legal & Regulatory Environment
- **Privacy & Data Protection**
- **Risk Management & Business Continuity**
- Transparency

United Nations (UN) Sustainable Development Goals (SDGs)

In 2015, the UN introduced 17 SDGs as a “shared blueprint for peace and prosperity for people and the planet now and into the future.” Ultragenyx’s mission, vision and values align with several of the UN SDGs.

UN SDG	Contribution
<p data-bbox="936 531 1164 597">3 GOOD HEALTH AND WELL-BEING</p> 	<p data-bbox="1290 522 2882 591">Ultragenyx is focused on advancing health equity by developing treatments for rare and ultrarare diseases that improve the health and quality of life of individuals living with rare disease, and by broadly supporting rare disease communities.</p> <p data-bbox="1290 618 2845 687">We advocate for broad access to screening and treatment and strive to make our treatments accessible and affordable to as many patients as we can.</p> <p data-bbox="1290 713 2836 782">We are also committed to advancing employee health, safety and wellness. We continue to provide our employees with wellness offerings and comprehensive benefits to support their financial, familial, physical and mental health.</p> <p data-bbox="1290 808 2772 878">Ultragenyx provides the majority of its grants and philanthropic funding to organizations and initiatives that contribute meaningfully to the health and well-being of local and at-risk communities, including our rare disease communities.</p>
<p data-bbox="936 927 1108 992">4 QUALITY EDUCATION</p> 	<p data-bbox="1290 921 2867 1025">Ultragenyx promotes rare disease education and awareness among healthcare professionals and the rare disease community, and offers employee education and training to support career advancement for our employees. We are also committed to expanding access to Science, Technology, Engineering, Arts, and Mathematics (STEAM) education opportunities, particularly in economically disadvantaged communities.</p>
<p data-bbox="936 1239 1090 1305">5 GENDER EQUALITY</p> 	<p data-bbox="1290 1234 2781 1303">Ultragenyx promotes gender equality in the workplace by fostering diversity, equity, inclusion and belonging (DEIB) at all levels of the organization and is committed to equitable compensation practices.</p>

Innovation

Pioneering new approaches to drug development for rare and ultrarare diseases.

We are **committed** to delivering novel, disease-modifying treatments with speed and urgency to rare disease communities with limited or no treatment options.

Aspiration

To optimize and accelerate rare disease drug research and development (R&D), whether by us or others.

Our Objectives	2023 Progress
<p>Develop industry-leading clinical pipeline in rare and ultrarare diseases that have limited or no treatment options</p>	<ul style="list-style-type: none"> Initiated Phase 3 <i>Cosmic</i> study in patients aged 2 to <5 years and Phase 3 portion of Phase 2/3 <i>Orbit</i> study in patients aged 5 to <26 years for UX143 in osteogenesis imperfecta (OI) Completed enrollment in Phase 1/2 study evaluating GTX-102 for Angelman syndrome Completed dosing of all patients in the Stage 1 portion of the seamless <i>Cyprus</i>²⁺ Phase 1/2/3 study evaluating UX701 in Wilson disease Presented positive data from the pivotal <i>Transpher A</i> and long-term follow-up studies of UX111 for Sanfilippo syndrome type A (MPS IIIA) Completed enrollment in Phase 3 <i>GlucoGene</i> study of DTX401 for glycogen storage disease type Ia (GSDIa)
<p>Foster industry-wide and community funded development efforts in rare and ultrarare diseases</p>	<ul style="list-style-type: none"> Hosted two Rare Bootcamps with 40 participants representing more than 30 organizations in attendance Contributed to collaborative drug development by participating in multiple industry consortia, including the Angelman Biomarker and Outcome Measure Consortium (ABOM) and the Accelerating Medicines Partnership (AMP) Bespoke Gene Therapy Consortium, among others Supported more than 50 investigator-sponsored trials (ISTs) globally, fostering community and investigator-led research initiatives Opened a state-of-the-art Gene Therapy Manufacturing Facility (GTMF) in Bedford, Massachusetts

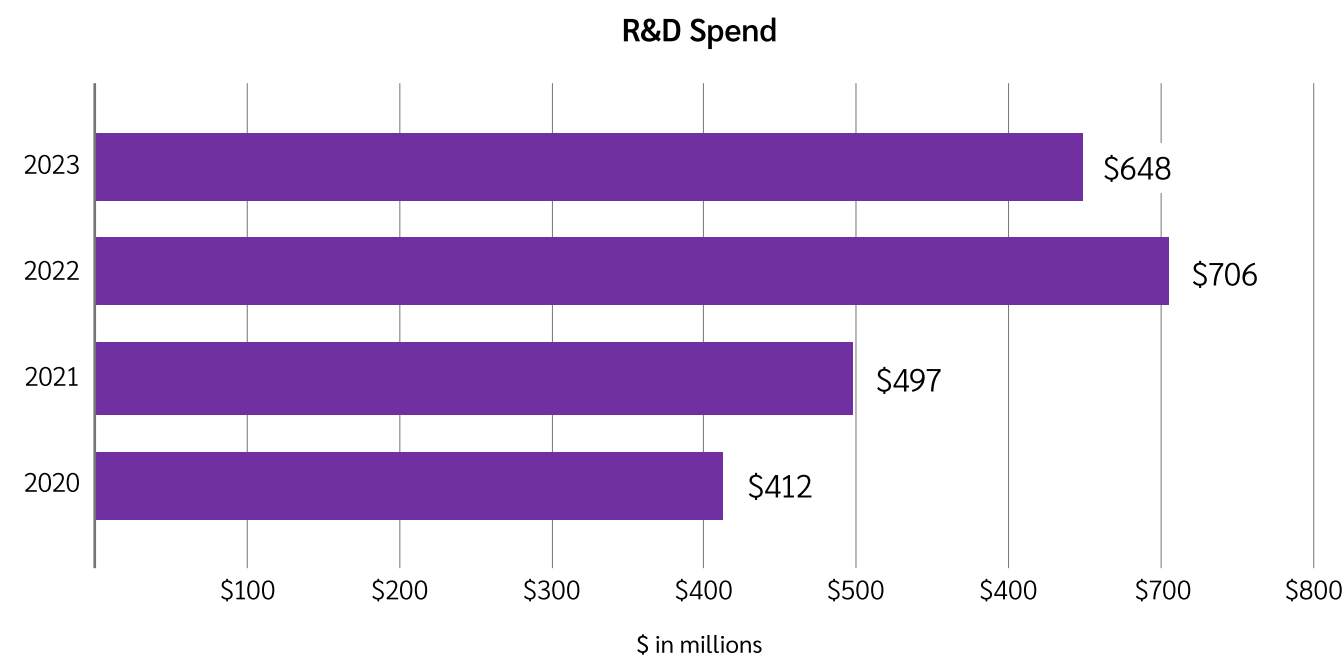
R&D

Innovation refers to our processes, initiatives, activities and investments aimed at the efficient and rapid advancement of investigational therapies that maximize patient health outcomes. Our patient-focused drug development model and cross-industry collaborations support our mission to transform the lives of people with rare disease.

We believe innovation in healthcare is critical for the many individuals living with rare diseases who are waiting for an approved treatment. We make significant investments in R&D to innovate and develop new therapies. In 2023, we spent approximately **65%** of our operating expenses on R&D.

We rely on patent protection, trade secrets, know-how and continuing innovation to develop and maintain our competitive position. Our policy is not to tolerate any unlawful use or activity that violates the intellectual property rights of others, as highlighted in our [Global Code of Conduct](#). We also expect suppliers with which we conduct business to respect the intellectual property rights of others. We seek patent protection in the U.S. and internationally for our products, investigational therapies and processes. Our policy is to patent or in-license the technologies, inventions and improvements that we consider important to the development of our business. As of December 31, 2023, we own, jointly own or have exclusive rights to more than **250** issued and in-force patents and more than **350** pending patent applications, more than **50** of which are in the U.S.

Our research aims to advance a new program into clinical development every one to two years. At the end of 2023, we had **four** rare disease treatments approved by the U.S. Food and Drug Administration (FDA) in **five** indications and **six** programs in clinical trials.



Total R&D spending decreased by **\$57.3 million**, reflecting changes in various program costs, including a decrease in upfront license, acquisition and milestone fees, and shifts in spending across gene therapy, biologic, nucleic acid programs and approved products. For more details, please see our [2023 Annual Report](#).

Patient-Focused Drug Development

More than 90% of rare diseases do not have available treatments, so we dedicate ourselves every day to the goal of developing and delivering new therapies with urgency. Three of our four approved medicines are the only FDA-approved therapy for their respective diseases.

Ultragenyx's Dynamic Development Model (DDM) supports effective decision-making in rare disease drug development through a patient-focused approach that involves collecting direct information and insights from patients. We engage in various activities to help align our programs with the unmet medical needs and expectations of the rare disease community. These activities include informal and structured patient interviews, clinical survey studies, [disease monitoring programs](#), natural history studies and [patient engagement plans \(PEPs\)](#). Engaging early with patients and their caregivers provides important insights that inform clinical trial design, endpoint selection and treatment expectations, potentially resulting in clinically meaningful endpoints that improve the lives of individuals living with rare diseases. The DDM strategy also encourages inventing new approaches and having backup plans to help address unexpected challenges.

For more information on how we partner with the rare disease community and leverage insights, see [Patient Advocacy and Engagement](#).

~70% historical success rate to date in developing therapies from clinical study initiation to receiving commercial approval from regulatory authorities

Our specialized approach to drug development

Match deep understanding of disease biology

With the right drug modality and tools

Informed by patient experience





A family affected by a rare disease, alongside Ultragenyx leadership and Middlesex County representatives, officially unveiled the new GTMF in June 2023.

Advancing Gene Therapy for Rare Diseases

In 2023, we opened our state-of-the-art **112,500-square-foot** Gene Therapy Manufacturing Facility (GTMF) in Bedford, Massachusetts, providing us with end-to-end gene therapy R&D and manufacturing capabilities. This fit-for-purpose facility can enable future process innovation and multi-modal process configurations, supporting our goal of delivering new treatments to patients as quickly as possible.

PINNACLE PCL™

The decision to build our own GTMF was driven by a strategic goal to enhance flexibility and control over our processes. Our Pinnacle PCL™ (Producer Cell Line) platform is a proprietary manufacturing system that is designed to produce adeno-associated virus (AAV) vectors more efficiently and cost-effectively than traditional methods to meet the increasing demand in today's gene therapy market. This high-titer, commercial-grade platform is designed to enable us to address large and extrahepatic indications with greater efficiency and lower manufacturing costs.

Our goals with the GTMF are to support the optimization of our Pinnacle PCL platform and expand our opportunities for future partnerships, maintain a stable and secure supply of gene therapies, and provide the agility needed to support our gene therapy pipeline. Given it enables the swift transitions between products, we expect to be able to accelerate the delivery of treatments to patients who need them urgently.



A Fusion of Innovation and Flexibility

The GTMF transcends the traditional role of a manufacturing site; it is a hub where pioneering research converges with cutting-edge technology. The facility's versatile multi-product design, complemented by 50L, 250L, 2x500L and 2000L single-use bioreactors, marks a significant advancement in gene therapy production, emphasizing flexibility, efficiency and scalability.

The facility is equipped with cGMP-compliant spaces for both drug substance (DS) and drug product (DP) manufacturing, as well as warehouse and office spaces. Its design is both flexible and purpose-driven, employing single-use mobile equipment and versatile utility panels to support ongoing process innovation and enable various configuration modes.

Moreover, the facility was designed to maximize both efficiency and effectiveness in our manufacturing operations. Having both DS and DP manufacturing under one roof is expected to streamline our production process, especially for small batches.

In addition, in the Greater Boston area, we operate a 500L production scale Pilot Plant and advanced GMP QC laboratories. These facilities, located near the GTMF, support our fully integrated end-to-end gene therapy R&D capabilities and position us at the forefront of industry developments in gene therapy.

Overcoming Challenges Together During COVID-19

The GTMF was completed both on time and under budget, despite the unique challenges posed by the COVID-19 pandemic. Our collaborative culture and integrated project delivery method helped us navigate these challenges effectively by taking strategic pauses to enable right-first-time solutions. Our team's emphasis on safety, problem-solving and a no-blame culture highlights our commitment to not just building a facility but fostering a community dedicated to transforming lives.

As part of our strategic initiatives outlined in our quality 5-year strategy planning, we have established a state-of-the-art gene therapy quality control (QC) laboratory. This lab represents a significant investment in our goal of achieving the highest quality of our gene therapy research. The QC lab is equipped with advanced technologies for rigorous testing and validation of gene therapies, aligning with our focus on quality and safety.

Our Pipeline

Our approved therapies and clinical-stage pipeline consist of four modalities: biologics, small molecules, gene therapies and nucleic acid therapies (antisense oligonucleotide [ASO] and messenger RNA [mRNA]). We have a broad translational research effort that works to turn observations in the laboratory and clinic into interventions that improve the health of individuals with rare and ultrarare diseases. We are advancing clinical and preclinical development programs across multiple rare disease therapeutic areas. Currently, we are developing **six** investigational therapies in pivotal clinical programs with the potential to reach more than 150,000 patients. In addition to our clinical efforts, we are actively working on advancing a number of preclinical programs, including UX055 for CDKL5 Deficiency Disorder (CDD) and UX810 for Duchenne Muscular Dystrophy (DMD).

As of March 2024:



Key: ● Protein Biologic ● Gene Therapy ● ASO/mRNA

Update to Our Pipeline

UX143 (setrusumab) for osteogenesis imperfecta (OI)

In July 2023, we initiated two late-stage clinical trials on setrusumab for OI types I, III, and IV. The Phase 3 part of the Phase 2/3 *Orbit* study assesses setrusumab's impact on fracture rates versus placebo in patients aged 5 to <26. The Phase 3 *Cosmic* study, an active-controlled trial, compares setrusumab to IV bisphosphonates in patients aged 2 to <5, focusing on total fracture rates. At the American Society for Bone and Mineral Research (ASBMR) 2023 annual meeting, we presented interim Phase 2 *Orbit* study data that showed a significant (~67%) fracture rate reduction and bone density improvements after 6 months, with no serious adverse events reported as of the data cut-off date. UX143 has gained Rare Pediatric Disease and Orphan Drug designations in the U.S., and PRIME and Orphan product designations in the EU.

GTX-102 for Angelman syndrome

The Phase 1/2 study evaluating the safety and tolerability of GTX-102 in pediatric patients with Angelman syndrome completed enrollment in December 2023. Interim data presented in April 2024 demonstrated clinically meaningful improvements across multiple clinical domains, compared to natural history data, where available. GTX-102 has received Rare Pediatric Disease, Orphan Drug, and Fast Track designations in the U.S., plus PRIME designation in the EU.

UX701 for Wilson disease

The seamless *Cyprus²⁺* Phase 1/2/3 study of UX701 for Wilson disease completed dosing the three dose-escalation cohorts of Stage 1 in January 2024 and is evaluating safety, efficacy, and dose selection for the pivotal, randomized, placebo-controlled Stage 2. Initial data showed four out of five patients in the lowest-dose cohort experienced reductions in urinary copper and were tapering off chelators and/or zinc therapy, with no serious adverse events reported as of the data cut-off date. UX701 has received Orphan Drug and Fast Track designations in the U.S., and Orphan medicinal product designation in the EU.

UX111 for Sanfilippo syndrome type A (MPS IIIA)

Positive data from the pivotal *Transpher A* study, which is evaluating the efficacy and safety of UX111 in children with Sanfilippo syndrome type A, along with results from long-term follow-up studies, were presented at the WORLDSymposium 2024 20th annual research meeting. These findings showed rapid and sustained decreased levels of heparan sulfate (HS) levels in cerebrospinal fluid (CSF), correlating with improved long-term cognitive development. UX111 has received Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Rare Pediatric Disease, and Orphan Drug designations in the U.S., and PRIME and Orphan medicinal product designations in the EU.

DTX401 for glycogen storage disease type Ia (GSDIa)

The Phase 3 *Glucogene* study completed enrollment in May 2023. The 48-week study is evaluating patients aged 8 years and older randomized 1:1 to DTX401 or placebo. The primary endpoint is the reduction in oral glucose replacement with cornstarch while maintaining glucose control. DTX401 has received Orphan Drug designation, Regenerative Medicine Advanced Therapy (RMAT) designation and Fast Track designation in the U.S., and PRIME and Orphan medicinal product designations in the EU.

DTX301 for ornithine transcarbamylase (OTC) deficiency

The Phase 3 *Enh₃ance* study is expected to complete enrollment in the first half of 2024. The pivotal, 64-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by removal of ammonia-scavenger medications and protein-restricted diet and change in 24-hour ammonia levels. DTX301 has been granted Orphan Drug designation and Fast Track designation in the U.S., and Orphan medicinal product designation in the EU.

Clinical Trials

Prior to receiving marketing approval from regulatory authorities for our investigational therapies, we must evaluate and demonstrate their safety and efficacy in clinical trials.



We are committed to conducting trials in an ethical manner and to adhering to our internal procedures and policies, the International Council for Harmonisation (ICH) of Technical Requirements for Pharmaceuticals for Human Use E6 Guideline for Good Clinical Practice (GCP), and applicable national and local regulations for trial design and conduct. We seek to protect patient safety and well-being by adhering to appropriate informed consent procedures and good clinical practices. We work to maintain clinical trial safety through controlled processes, policies and management systems. We also document and report relevant safety information and adverse events (AEs), such as any new or worsening conditions, to the relevant regulatory authorities and participating investigators.

Regular monitoring of patient safety throughout our trials aims to prevent harm and maintain a positive benefit-risk profile for our investigational therapies. To support clinical trial efforts, we partner with contract research organizations (CROs) and other vendors, requiring them to undergo a vendor qualification audit, adhere to our policies as well as applicable laws and regulations, and participate in our monitoring oversight and auditing program to sustain compliance and alignment with our standards. By working closely with our partners, we aim to confirm that study conduct aligns with the clinical trial protocol, our internal policies and procedures, and that participants' rights, safety and well-being are upheld, in accordance with ethical and regulatory standards.

For more information on the risks related to the discovery and development of our investigational therapies, please see our [2023 Annual Report](#).

Clinical Site Assessment and Compliance

Our approach to clinical site selection and routine site monitoring is essential to our goal for safeguarding the safety and reliability of our clinical operations. Before beginning operations at any clinical site, we carefully assess available resources, clinical trial experience and the suitability of its facilities, staff and equipment.

Every clinical trial site is subject to a thorough vetting process designed to confirm both scientific quality and regulatory compliance. During the trials, routine monitoring at each study site is conducted with the goal of confirming adherence to protocols and Good Clinical Practice (GCP) standards, while also focusing on site quality and the safety and rights of patients.

Our quality team is responsible for conducting independent site audits for each study, based on a risk-based approach. Through our GCP audit program, we evaluate each site for compliance risks. The frequency of these audits is adjusted according to the specific risk factors identified. We consistently monitor for any deviations from GCP standards, misconduct or violations of patient rights or safety. In cases where such issues are identified, we require corrective measures to be implemented, conduct severity impact assessments and, if necessary, report these findings to the FDA and comparable regulatory authorities in other countries.

In 2023

*we had more than **130** clinical trial sites in operation across **19** countries.*

Clinical Trials (cont.)

Data Transparency

Data transparency is essential to fostering trust with patients, healthcare professionals, regulatory agencies and medical researchers. We recognize this importance and support the overall principles of greater clinical trial data transparency as part of our patient-focused drug development model.

We follow the standards and principles for clinical trial data transparency set forth by international industry organizations such as:

- International Federation of Pharmaceutical Manufacturers & Associations (IFPMA)
- European Federation of Pharmaceutical Industries and Associations (EFPIA)
- Japan Pharmaceutical Manufacturers Association (JPMA)
- Pharmaceutical Research and Manufacturers of America (PhRMA)

Additionally, Ultragenyx is a member of the Biotechnology Innovation Organization (BIO) and follows the BIO Position Statement on Clinical Trial Registries and Dissemination of Clinical Trial Results.

We endeavor to make clinical trial information and results public in a timely manner while protecting essential proprietary information and patient privacy. Ultragenyx registers protocol information for company-sponsored clinical trials of investigational therapies and marketed medicines in accordance with applicable laws and regulations. In the U.S., protocol information is registered at www.clinicaltrials.gov.

Ultragenyx discloses the results of company-sponsored clinical trials in accordance with applicable laws and regulations. We also seek to publish results – regardless of outcome – in peer-reviewed journals or at medical and scientific meetings. We publish the results of both successful and failed trials to advance scientific learning. Our medical writing follows industry standards, such as the Good Publication Practice guidelines (GPP3) published by the International Society for Medical Publication Professionals, and Recommendations for the Conduct, Reporting, Editing and Publication of Scholarly work in Medical Journals published by the International Committee of Medical Journal Editors (ICMJE). We also prepare plain language summaries of results to share with patients and the public. Additionally, we make reasonable efforts to address clinical data requests

for legitimate medical/scientific research purposes from qualified researchers in the interest of improving patient care and helping advance medical science.

These collective efforts help patients access trial result information and facilitate compliant data sharing with interested parties. For more information, see [Our Clinical Trial Transparency Commitment](#) and [Ultragenyx Clinical Trial Results](#).

Data Protection, Anonymization and Security

Ultragenyx is committed to high standards of integrity and compliance with applicable laws and regulations when handling patient data. Clinical trial data that is transferred to Ultragenyx is required to be anonymized, meaning no names or other personally identifiable information (PII) is provided. Trial participants must be informed if Ultragenyx examines their medical records. We require vendors supporting our trials to comply with applicable data protection laws and to have a data breach response plan in place. These measures are some of the ways that Ultragenyx works to protect the privacy and rights of our clinical trial participants.

See [Data Privacy](#) in this report for more information.

In 2023

Registered **2** new protocols on www.clinicaltrials.gov

Published **11** peer-reviewed manuscripts

Submitted **99** abstracts to congresses

Made **135** oral and poster presentations at medical and scientific meetings

Diversity in Clinical Trials

We are committed to ethical, responsible and inclusive clinical research. We advocate and lobby for people living with a rare disease to receive an accurate diagnosis, quality care and available therapy as quickly as possible.

*More than **50** clinical ISTs approved and **495+** clinical participants enrolled globally since 2012*

Individuals and families impacted by rare and ultrarare diseases face challenges in securing accurate diagnoses and accessing optimal care, particularly when there is no approved therapy for a particular condition. This is further compounded by the fact that many physicians lack experience with these diseases and are often unaware of the options available for enrolling in clinical trials.

Given these challenges, we design our clinical trials with the goal of maximizing diversity and heterogeneity in our study populations. We strive to limit exclusions as much as possible, weighing the benefit/risk balance in an effort to promote broad inclusivity.

We recognize that a significant number of racial and ethnic groups in the U.S. are not adequately represented in clinical research conducted within our sector and that building and rebuilding trust with these groups is a critical step. This lack of representation may compromise a research program's effectiveness. Emphasizing inclusive designs in clinical trials is a crucial step toward enhancing both the quality and fairness of treatments for the populations most impacted.

As we continue to monitor and strive to improve participants' diversity metrics within the U.S., our global expansion further amplifies our commitment to inclusivity across diverse regions. Our dedicated Patient Find team works with clinical operations and development teams

to identify potential participants, and we have clinical trial sites in both developed and developing countries.

We endeavor to make our clinical trials inclusive and diverse – available in multiple countries to individuals living with rare and ultrarare diseases, regardless of gender, ethnicity or socioeconomic status. As many of the diseases we are studying have no approved treatments, we strive to make these opportunities accessible to a broad range of patients. To help minimize the financial burden on patients, we cover expenses necessary for clinical trial participation, including travel. We also prioritize reducing the burden of participation through home visits and phone calls when possible.

Building on our global initiatives and the insights gained from diverse patient populations, we have implemented several key measures in our endeavor to foster greater inclusivity and diversity in our clinical trials. Examples of these measures include:

- Preparing multilingual materials to educate patients on each clinical trial
- Utilizing additional techniques to further outreach to patient communities, including social media and other digital marketing channels
- Engaging and building partnerships with the rare disease community through advisory panels to adequately represent their experiences and needs

- Investing in employee training programs for cultural sensitivity and Diversity, Equity, Inclusion and Belonging (DEIB) to enhance our employees' ability to engage effectively with diverse populations
- Regularly collecting feedback from participants for continued improvement

We also provide support for investigator-sponsored trials (ISTs) for our investigational or approved products worldwide. We encourage proposals that align with our scientific areas of interest and include integrated evidence plans for our products and programs.

Looking ahead to 2024, we are focusing on supporting economically and racially marginalized populations in entering the sciences. To that end, we are initiating a fellowship program that aims to bring in research fellows, to conduct, lead and publish research with a particular emphasis on attracting a diverse pool of candidates. This program is a step toward broadening our impact and fostering a greater diversity of physicians, and benefits from a wide range of perspectives and expertise.

Disease Monitoring Programs and Post-Marketing Commitments

We utilize disease monitoring programs (DMPs) to evaluate long-term outcomes for newly approved therapies, facilitate knowledge sharing with the rare disease community, and fulfill any post-marketing regulatory requirements.

Disease registries and other post-marketing studies can provide an organized way to collect patient data and allow for post-marketing surveillance of approved medicines. However, rare disease registries are costly, may have incomplete or missing data, and seldom provide compelling publishable data, due to small patient numbers and/or patient attrition. Traditional registries often fail to provide patients with their own collected data, losing an opportunity to share useful information for medical care with patients and physicians.

To address these challenges, Ultragenyx developed the novel concept of a DMP. This is a global study that assembles regulatory-quality data on a broad population of individuals living with a rare disease, whether treated – via commercial access to a medicine – or not. DMPs go beyond clinical trials by enhancing the understanding of the disease and its treatments for the benefit of patients, physicians, payers and the company. DMPs provide progress reports, broader patient population data analysis, high-quality disease information, and supportive long-term outcome data, and promote therapy science advancement and research.

As part of a DMP, the pharmaceutical sponsor may partner with an academic institution and a patient advocacy group, if applicable, on the ownership and management of data.



DMPs can also support the generation and transparent sharing of high-quality, Good Clinical Practice (GCP)-compliant data with patients, physicians, sponsors and the rare disease community. We provide participants in our DMPs with the opportunity to receive their own data in easy-to-understand language.

To date, we have initiated **eight** DMPs, including several for Crysvita (burosumab-twza) for the treatment of X-linked hypophosphatemia (XLH). Our in-clinic DMP for XLH is our largest study, with more than **780** patients in its **sixth** year. In 2022, we enrolled the first patient in our most recent DMP, for Dojolvi (triheptanoin).

DMP Highlights

*First **DMP** launched in 2012*

***1,380+** patients enrolled*
at nearly **57** sites in **12** countries*

*Enrollment as of December 31, 2023

Quality

Our commitment is to deliver quality medicines to the rare disease community. We strive to uphold an engaged quality culture that emphasizes the safety, efficacy and reliable quality of our medicines.

We have implemented a company-wide quality program to manage product and safety risks, with the goal of full compliance with applicable laws, regulations and international standards. Our quality program, built on a foundation of safety, efficacy, product quality and network reliability, embodies our core values in research, development and manufacturing. This program is spearheaded by our chief quality operations officer. Our leadership is committed to fostering a responsible quality mindset, supported by a robust quality management system (QMS). This system is designed to promote sound science and appropriate behaviors across all levels of operations and across all stages of development, manufacturing and distribution of our medicines. Our quality system continues to evolve to meet business and quality requirements, as well as growth and scaling needs.

Our approach to quality is centered around three focus areas that guide our performance:

- **Regulatory Compliance and Data-Driven Innovation**
We design our programs to adhere to regulatory standards and strive to use data to drive continued quality improvement.

- **Integrated Quality and Risk Management**

We are committed to integrating quality throughout our operations and employ proactive risk management strategies.

- **Quality Culture**

We combine a leadership-driven quality culture with a strong focus on safety and efficacy.

Ultragenyx works to adhere to “good practice” quality guidelines, regulations and international standards, collectively referred to as GxP. This extends to our suppliers and business partners, facilitating a unified compliance framework. We require our suppliers to comply with the Drug Supply Chain Security Act (DSCSA). We continue to update our practices to align with industry trends and regulatory expectations.

Our QMS integrates people, processes and systems. It is a principles-based framework designed for quality assurance, continuous improvement and compliance. The system includes organizational structure, responsibilities, procedures and resources, detailed in our Quality Manual, Standards and SOPs, which are subject to regular review.

Continued improvement is a cornerstone of our QMS, which evolves to meet business growth, scale and quality requirements. Quality risk management (QRM) is an integral part of our QMS, extending beyond ICH Q9 guidelines. It involves systematic assessment, control, communication and review of risks throughout the product lifecycle. This approach is intended to foster a risk-curious mindset across the company, encouraging a proactive stance toward risk management and to facilitate a culture of learning and growth.

We aspire to become an industry leader in cultivating a quality learning culture, going beyond traditional training methods. We focus on learner success and development and strive to continue enhancing our learning modules and materials. This approach is key to our goal of embedding a quality mindset across the company, promoting open communication, employee ownership and data-driven performance monitoring.

Quality in Supply Chain

To maintain high standards, we have a robust supplier oversight program. This program includes risk-based auditing and monitoring of our supply chain partners, with the goal of ensuring compliance with regulatory and internal requirements.

Our third-party audits, essential for product quality and safety, involve reviewing records, conducting interviews, analyzing third-party reports such as RX-360 of The International Pharmaceutical Supply Chain Consortium, and performing onsite inspections. In 2023, we licensed 6 RX-360 audit reports to satisfy the audit requirements for 6 supplier sites (3 in the U.S. and 3 international). Additionally, we assess compliance with standards, contract adherence, and quality benchmarks. This proactive approach is designed to help us identify improvement areas and uphold our supply chain's integrity.

Our Corrective and Preventative Actions (CAPA) Management System is central to our QMS, and we take a risk-based approach to managing incidents and quality issues internally or with contract manufacturing organizations (CMOs). We focus on identifying root causes of quality events, with the goal of implementing effective corrective and preventive measures to avoid recurrence. We continue to assess and advance the maturity of our CAPA practices, aimed at optimizing our systems and enhancing training, with a cross-functional Community of Practice driving these improvements.

Counterfeit Products

We take the safety and effectiveness of our medicines seriously and have implemented processes designed to identify and address potential or known risks associated with counterfeit products. Our field action procedure allows for cross-functional collaboration to address counterfeit or quality issues and communicate with stakeholders as necessary.

In our endeavor to further mitigate the risk of counterfeit products entering our supply chain, we have implemented security features, such as tamper-evident seals and serialization of product labeling. Each commercial drug product is tracked through a serialization process, which assigns a unique identifier to each package. This is designed to allow our partners in the distribution network to verify that a given package is a legitimate product of our company, providing added protection to patients against the risk of counterfeiting.

Our commitment to transparency and proactive measures to identify and address risks associated with counterfeit products is aimed at maintaining the trust and confidence of our stakeholders in the safety and effectiveness of our products. We expect to continue to implement rigorous procedures and security features designed to ensure the authenticity and quality of our products

In 2023

*Initiated over **100** GxP audits with plans to address all findings*

***Zero** product recalls; no units recalled*

Safety

Our reputation is dependent on the trust that patients, healthcare professionals, regulatory authorities and the general public place in us.

Our reputation is dependent on the trust that patients, healthcare professionals, regulatory authorities and the general public place in us. We work to uphold this trust by striving to comply with all applicable laws, regulations and international standards, such as International Conference on Harmonization (ICH), Good Clinical Practices (GCP), Good Distribution Practices (GDP), Good Laboratory Practices (GLP), Good Manufacturing Practices (GMP) and Good Pharmacovigilance Practices (GVP), collectively known as GxP, with all Ultragenyx employees receiving annual GxP training.

At the heart of our mission to be the leader in rare disease medicine is our commitment to patient safety, upheld through a pharmacovigilance (PV) system designed with comprehensive, inspection ready systems and procedures designed to be in compliance with laws, regulations, standards and industry best practices. Our Global Drug Safety & Pharmacovigilance (DSPV) department maintains a robust medical and safety surveillance.

Safety information is received, collated and analyzed throughout a product's lifecycle, from early development phase and throughout the post-approval phase.



This information comes from many potential sources.

These include:

- Clinical trials
- Disease monitoring programs
- Patient access programs such as compassionate and named patient use
- Patients and their families and caregivers
- Healthcare providers
- Scientific literature
- Regulatory agencies and their databases
- Social media
- Vendors or business partners with whom we have legal agreements that include requirements to report relevant safety information they become aware of

Our pharmacovigilance (PV) system, supported by our quality system, is designed to efficiently and accurately process and evaluate available safety information for our products to confirm the benefits outweigh the risks. When there is a change that impacts the benefit/risk profile, we are committed to promptly communicating with regulatory authorities, patients and healthcare providers. Our diligent approach to patient safety and swift communication of safety data that informs benefit-risk of our medicines aims to empower patients and healthcare providers to make informed treatment decisions, consistent with our goal of contributing to safer and healthier lives.

All Ultragenyx workforce, representatives or agents working on behalf of Ultragenyx play an important role in promoting the safety of our products. Web-based training is required of our workforce on how to report safety information including AEs and product quality complaints (PQCs) associated with any Ultragenyx medicine.

Recognizing the value of proactive pharmacovigilance, we assess industry standards and best practices. Through continual learning from our data and the broader research community, we strive to enhance patient safety and uphold our commitment to public trust.

Strategic Collaborations

We are working to develop a robust and diverse pipeline of investigational therapies to support our mission *to transform the lives of people with rare disease.*

In addition to our translational research efforts, we have a partnership strategy that combines Ultragenyx's expertise and know-how in developing and commercializing rare and ultrarare therapeutics with R&D efforts at other companies and institutions. Ultragenyx seeks partners aligned with the company's core values and vision for developing and bringing transformative therapies to those living with rare and ultrarare diseases worldwide. Our flexible partnering model is intended to allow us to collaborate broadly with academics and biopharma industry partners in developing rare disease therapeutics or platform technologies.

Ultragenyx has a successful history of partnering with other biotech companies to develop and commercialize rare disease therapeutics. Since 2013, Ultragenyx and Kyowa Kirin have been partners in the successful development and worldwide commercialization of Crysvida across two indications. Ultragenyx also has license and collaboration agreements with Mereo BioPharma on the development of setrusumab globally and with Regeneron to clinically develop, commercialize and distribute Evkeeza (evinacumab) in countries outside of the U.S.

Please see our [2023 Annual Report](#) and our [website](#) for a list of our collaborations.



Research Collaborations

We participate in multiple industry consortia and partnerships to foster and support collaborative, industry-wide drug development in rare and ultrarare diseases.

We're a member of the **Angelman Biomarker and Outcome Measure Consortium (ABOM)**, launched to drive information sharing, agree on the most important disease domains in Angelman syndrome, and develop or modify existing endpoints or biomarkers in order to know how to measure change in clinical studies.

We're a founding sponsor of **BeginNGS™** (newborn genomic sequencing), a public-private coalition led by Rady Children's Institute that is piloting a program to use genetic testing technology to screen newborns for rare genetic diseases. The ultimate goal is to test for up to 1,000 disorders and conduct genomic screening on 3.7 million newborns in the U.S. annually. See [Public Policy Participation](#) for more information on our support of newborn screening (NBS).

We're a member of the Accelerating Medicines Partnership® (AMP®) **Bespoke Gene Therapy Consortium**, a public-private partnership with the National Institutes of Health (NIH), the FDA, and multiple public and private organizations to create a standardized approach to help reduce upfront costs and lower barriers to developing new gene therapies for rare and ultrarare diseases.

We're a member of the **LouLou Foundation CDKL5 Deficiency Disorder Consortium**, which is directing the Clinical Assessment of NeuroDevelopmental measures In CDD (CANDID), a three-year observational study for the development of disease-modifying therapeutics for CDD. The CANDID study is anticipated to enroll more than 100 participants, with sites in the U.S., Canada, U.K., Germany, France, Spain, Italy and UAE. CANDID study results will be shared with the entire community to aid CDD clinical trial design and inform therapeutic development for related neurodevelopmental disorders.

We're a supporter of the **"Living with Osteogenesis Imperfecta: Understanding Experiences Based on Community Insight and Experience" (IMPACT) Survey**, the largest collection of data about osteogenesis imperfecta (OI) and its impact on patients, their families and care givers. OI is a rare genetic condition that leads to abnormal bone structure, decreased bone mass, bone fragility and weakness. The IMPACT Survey is a joint research project between the Osteogenesis Imperfecta Foundation (OIF) and the umbrella organization Osteogenesis Imperfecta Federation Europe (OIFE), with support from Mereo BioPharma, Ultragenyx's

partner in the development of UX143 (setrusumab) for the potential treatment of OI. The first article based on data from the IMPACT Survey, "The patient clinical journey and socioeconomic impact of osteogenesis imperfecta: a systematic scoping review," was published in Orphanet Journal of Rare Diseases in February 2023.

We're a partner in the **n-Lorem Foundation**, whose mission is to discover, develop and provide experimental antisense oligonucleotide (ASO) medicines for diseases that affect nano-rare patients (one to 30 patients worldwide) for free, for life. To date, more than 100 patients have been accepted into n-Lorem's drug development program, six patients are on treatment, and nine Investigational New Drug (IND) applications have been submitted to the FDA.



Zoe's Inspiring Journey with Sanfilippo Syndrome Type A

Zoe's life story is a testament to resilience and hope in the face of Sanfilippo syndrome type A, an ultrarare neurodegenerative disorder. Diagnosed at just three months old, Zoe's journey was marked by early developmental delays. Unlike typical language learning challenges in bilingual households, Zoe's late walking and mild to moderate hearing loss were early signs of a deeper, more complex condition.

The journey to diagnosis was fraught with uncertainty and lack of support, leaving Zoe's family to navigate the complexities of an ultrarare disease affecting one in 70,000 newborns. Her family initially assumed her bilingual environment was the cause of her developmental delays and was shocked when she was diagnosed with Sanfilippo syndrome type A. They desperately needed resources and emotional support and found it challenging to appear strong, but they found solace and community through the Cure Sanfilippo Foundation and the National MPS Society.

Now 11 years old, Zoe is a vibrant child who loves Disney movies, baking cookies and music. Despite the progression of her condition, which has impacted her ability to speak, Zoe's joy for life remains undiminished. Her story is not just about the difficulties of living with a rare disease, but about the strength and love of a family adjusting their lives to support their daughter's needs.

Zoe's journey highlights the importance of early diagnosis, the need for a supportive community, and the relentless spirit of families dealing with rare diseases. Her smile continues to inspire hope and resilience, serving as a beacon for others on a similar path.

Zoe's story is not just about the difficulties of living with a rare disease, but about the strength and love of a family adjusting their lives to support their daughter's needs.

Rare Bootcamp™

We host a recurring Rare Bootcamp™, where we share our knowledge, expertise, insights and connections to help patient families, foundations and organizations seeking to develop novel treatments for rare diseases.

Our Rare 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.

What began as a half day meeting in 2017 is now a multi-day event and takes place twice a year. Topics include therapeutic modalities, manufacturing strategy, development strategy (including clinical endpoints and diagnostics), regulatory considerations, raising capital and partnering. We also allow time for one-on-one meetings so experts can share their advice.

The event has evolved based on participant feedback. For example, on the research side, we now conduct a working group to assess scientific gaps in knowledge associated with each disease and the appropriate therapeutic modality to prioritize. In addition, we have added a panel discussion and lecture topics focused on selecting an academic partner to conduct this research as well as how to define contract terms governing data ownership and research progress.



Rare Bootcamp Highlights

*In 2023, we hosted **2** Rare Bootcamps with **40** participants from more than **30** organizations attending.*

*Since 2017, we have held **8** rare Bootcamps with more than **135** participants from more than **100** organizations in attendance.*

Patients

Propelling the entire rare disease community forward to transform as many lives as possible

We are **committed** to supporting the 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 policymakers for meaningful change.



Sadie and her mother, Ashley.

Sadie is a very happy girl who lights up a room with her beautiful smile and her eyes full of joy. She lives with Sanfilippo Syndrome, also known as MPS III.

Aspiration

To achieve meaningful benefit for people living with rare diseases by delivering on the promise of our science and our therapies.

Our Objectives	2023 Progress
<p>Achieve majority access through responsible pricing and support services</p>	<ul style="list-style-type: none"> • We continue to align U.S. price increases consistent with the Consumer Price Index • More than 600 patients in over 45 countries have been approved for access to Ultragenyx treatments through various global expanded access and patient assistance programs since 2013 • As of December 31, 2023, more than 4,700 patients have received Ultragenyx treatments through commercial or expanded access in more than 50 countries
<p>Use our expertise to amplify the voices of the rare disease community to inform and influence key decision-makers in the field of rare disease</p>	<ul style="list-style-type: none"> • Successfully advocated for the inclusion of more rare diseases in newborn screening (NBS) programs across several U.S. states • Contributed to policy discussions and advocated for legislation that supports the development of treatments for rare diseases, with the goal of ensuring that the voices of the rare disease community are heard at the legislative level
<p>Incorporate the perspectives and experiences of patients and caregivers in our decision-making, with a focus on addressing unmet needs for basic necessities and improving quality of life</p>	<ul style="list-style-type: none"> • Attended over 25 patient advocacy conferences, meetings, and town halls worldwide, engaging with advocacy leaders and rare disease community members, fostering awareness and collaboration • Hosted regular patient and community leadership councils, including new councils for emerging therapeutic areas, to gather insights and feedback on the company’s strategies, research priorities and patient support services • Co-authored and presented research findings, including patient advocacy perspectives on gene therapy, at key industry conferences, highlighting the challenges and opportunities within the rare disease field to a broad audience of stakeholders, including healthcare professionals, researchers and policymakers

Access and Affordability

We believe the greatest impact we can have on the lives of individuals with rare disease is to make our treatments accessible and affordable to anyone who can benefit from them and to engage and support rare disease families along every step of their journey. To date, more than **4,700 patients** have received Ultragenyx treatments via commercial or expanded access in more than **50** countries.

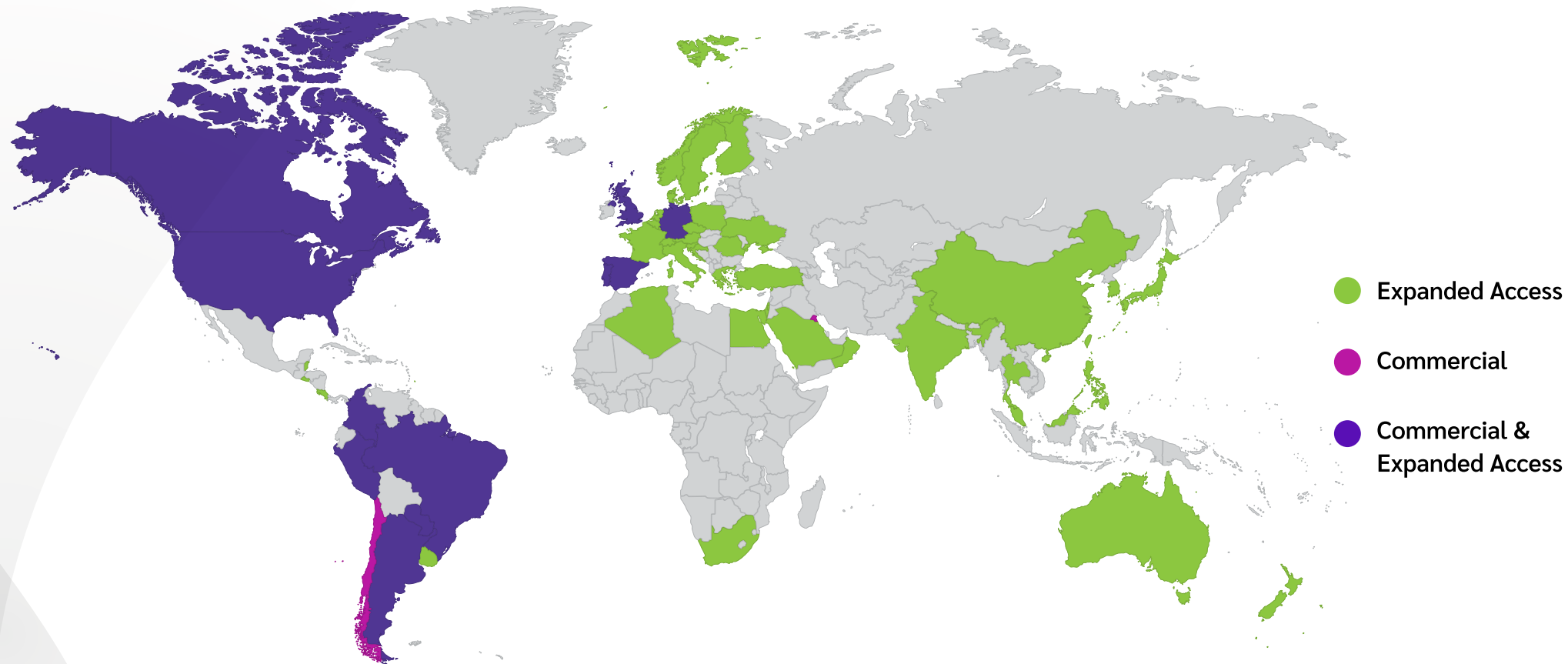
We have taken a responsible approach to pricing our therapies from the start with the goal of enabling as many appropriate patients as possible to access treatment. Our focus is on expanding patient access while maintaining investment in innovation. We evaluate each medicine's value based on healthcare economics, clinical data and comparisons to similar therapies. Additionally, we consider the costs of producing high-quality medicines and sustaining a robust global supply chain. We set our medicine prices with global pricing in mind. In the U.S., we leverage effective reimbursement strategies and use our best efforts to prevent patients from foregoing Ultragenyx therapy for financial reasons. Our patient assistance programs include co-pay support and free access where necessary. In 2023, U.S. price increases for the commercial products for which we lead promotion were guided by the Consumer Price Index. As we look ahead to our next-generation gene therapies, we have invested in our proprietary Pinnacle PCL gene therapy technology platform and our GTMF in Bedford, Massachusetts. Gene therapy manufacturing issues have been a known barrier to treatment access, and our goal in establishing end-to-end gene therapy R&D and manufacturing capabilities is to improve our control over costs, production and our ability to scale.

In the last five years, Health Technology Assessment (HTA) Agencies around the world have evaluated and recommended our therapies for reimbursement, which is a testament to the potential for these treatments to enhance healthcare outcomes globally.

- Mepsevii (vestronidase alfa-vjkb) was recommended by Brazil's CONITEC, England's NICE, Germany's G-BA, Italy's AIFA, Spain's AEMPS and Portugal's INFARMED.
- Crysvita was recommended by Canada's CADTH and INESSS, Brazil's CONITEC and Mexico's Federal Reimbursement List.
- Dojolvi was recommended by Canada's CADTH and INESSS and Mexico's Federal Reimbursement List.
- Evkeeza was recommended by France's HAS, Italy's AIFA and Canada's CADTH.

Access and Affordability (cont.)

Reaching patients around the world with Ultragenyx medicines



Timely Care for Rare Disease Patients Anywhere

Our product supply team’s strategy is designed to address the unique challenges of the rare disease community, which is characterized by low volume and high complexity demands and urgent needs. We have enhanced our supply network and implemented a strategic logistical approach, with the goal of providing swift deliveries for rare disease patients globally. To optimize operational efficiency and minimize extensive travel, we have set up regional hubs staffed with local experts. In collaboration with our managed access partner, we streamline processes by consolidating smaller orders. This is designed to reduce shipment frequency and focus on supplying therapies for three to six months at a time. Where possible, we also offer direct-to-patient deliveries. This strategy is intended to maintain continuous access and a better delivery experience while enhancing cost efficiency.

Access and Affordability (cont.)

UltraCare® Programs

We created our UltraCare programs to help patients and caregivers understand their insurance coverage, determine eligibility for our financial assistance programs, navigate access to treatment and find patient support programs. UltraCare programs, based on local regulations, are currently in place in the U.S., Canada, Argentina, Brazil, Colombia and Mexico, and programs are in development in additional countries. Visit [Ultracaresupport.com](https://ultracaresupport.com) for more information about our U.S. programs.

Expanded Access

We are committed to supporting individuals with rare disease and their families in receiving proper diagnoses and optimal care following a rare disease diagnosis. Our commitment to developing new medicines for both children and adults with rare and ultrarare diseases is matched by our efforts to make our commercial therapies accessible to patients via appropriate mechanisms, particularly in countries where regulatory authorities have yet to approve such treatments.

Although clinical trials offer access to our investigational therapies, some patients who suffer from serious or life-threatening diseases may be ineligible to participate in such studies and may not have other viable treatment options. In such cases, where able, we offer our investigational therapies on a compassionate use basis to qualified patients worldwide via our early access program. Our evaluation of requests for individual patients to receive investigational therapies outside of a clinical study is conducted on a case-by-case basis. Given the urgency associated with treating patients with rare diseases, we aim to respond to compassionate use requests within 24 hours and to ship therapies within 48 hours wherever feasible. We are committed to supporting physicians in the timely and excellent care of their patients and patient families.

*Over **40** patients were treated with Ultragenyx medicines through compassionate use in 2023*

*More than **600** patients in over **45** countries have been approved for access to Ultragenyx medicines through various global expanded access and patient assistance programs since 2013*

Sponsored testing programs shorten the diagnostic odyssey

A typical rare disease patient requires 6 years and an average of 17 clinical encounters in the quest for an accurate diagnosis after symptom onset. Genetic testing is often necessary to confirm a rare disease diagnosis and for a patient to qualify for reimbursement of optimal therapy. However, genetic testing itself is often not reimbursed. Ultragenyx is focused on differentially diagnosing 9 different rare diseases to give physicians information that will support clinically-appropriate decision making and disease management. These sponsored testing programs can reduce the time to diagnosis for patients and help provide patients with timely care. Ultragenyx sponsored testing programs have specific eligibility requirements and tests are only offered to appropriate, clinically-suspected patients who are already embedded in the medical system on a diagnostic odyssey. At Ultragenyx, we are committed to sharing the learnings from our sponsored testing programs with the public. This includes information about what gene changes (or variants) are associated with a given disease. We have created a website that makes this data available to the public and we encourage users of the [website](#) to not only explore our data but also contribute their own new variant data.

Patient Advocacy and Engagement

Ultragenyx was built hand-in-hand with the rare disease communities we serve. Our priority is to partner with patients and their families from the earliest stage of drug development through clinical research and support patients and their families throughout the treatment life cycle.

We also have the opportunity to listen to first-hand experiences from invited speakers living with rare diseases, which allows us to see the real-world impact of our medicines on patients and families.

Our patient advocacy and engagement work includes the following:

- **Establishing** partnerships with patient organizations to best support patients and patient communities and incorporate community insights and priorities in the development of therapies to address unmet needs.
- **Educating** the broader rare disease community by sharing information about rare and ultrarare diseases and their impact.
- **Engaging** with key patient community members to amplify the voices of the rare disease community.
- **Supporting** patients, their families and the rare disease community through medical education and health-related grants. Please see the [Grants](#) section of this report for information on our support of medical education grants and health-related grants to patient advocacy groups and our [website](#) for a list of patient advocacy groups we partner with to provide education, support and periodic updates of our clinical programs.

Patient Engagement Plans (PEPs)

PEPs are collaboratively developed by a cross-functional group led by our patient advocacy department to include the patient and patient community perspectives and lived experiences across all functional areas of product development. Multiple teams explore and address various aspects of a disease with patients, caregivers and the affected community, including specific care landscapes and the associated healthcare environment and services. Since physicians and disease experts may not have comprehensive insight into how patients perceive the disease's impact on their quality of life, the PEP process involves regularly reassessing existing sources of patient experience data. Patient involvement is critical to making informed decisions in drug development and PEPs provide a framework for cross-functional leaders to seek out insights and incorporate patient feedback into their work.

A real-life example of an effective PEP is the one we used with our UX143 program for osteogenesis imperfecta (OI). The cross-functional team met early in product development to gather patient insights and perspectives, and the work with the patient community has evolved simultaneously with the overall program. Patient insights from the OI community were included in the work done by our Clinical Operations, Clinical Development, Regulatory and Global Brand Strategy departments.

Partnering with Patient Organizations

The Ultragenyx Patient Advocacy and Patient Engagement team is dedicated to advancing global rare disease advocacy through inclusive patient engagement and partnerships.

To further this purpose, in 2023, we attended more than **25** patient advocacy conferences, meetings and town halls across the globe. We engaged with advocacy leaders and rare disease community members, sponsored educational events, and shared information on our pipeline research programs.

Ultragenyx also hosts regular patient and community leadership councils with the goal of having a consistent and structured way to gain insights from the patient community on our work. These leadership councils are a forum for Ultragenyx to provide updates on our work and to receive feedback from council members on areas of interest to them. The insights from these efforts help inform our strategies and decision-making throughout the product lifecycle and place patient and community perspectives at the center of our work.

We currently host the following community leadership councils:

- LC-FAOD Patient Leadership Council
- XLH Patient Education Advisory Council
- CTD Caregiver Leadership Council
- Angelman Caregiver Leadership Council
- OI Leadership Council
- Sanfilippo Caregiver Council
- Global Gene Therapy Advisory Council

Global Gene Therapy Advisory Council

In 2021, Ultragenyx founded our Global Gene Therapy Advisory Council, which includes nine patient advocacy leaders from the U.S., Mexico, Netherlands, U.K. and Sweden, representing various rare disease communities. The goals of the council are to garner insight from council members on priority challenges, knowledge gaps and unmet needs related to gene therapy within rare disease communities, increase community awareness, knowledge and understanding of gene therapy, and identify potential collaboration opportunities to address unmet community needs regarding gene therapy.

The council met several times in 2023. At the end of the year, we developed a framework and messaging for educational resources on gene therapy based on the diverse feedback we received from council members. The council also identified a series of strategic issues that can be addressed across communities to increase patient understanding and acceptance of gene therapies on a global scale. In conjunction with Ultragenyx, several members of the council co-authored and presented a poster titled “Rare Disease Patient Advocacy Perspectives on the Promise and Challenges of Gene Therapy” at the 2023 American Society of Gene & Cell Therapy Annual Meeting.

Providing Educational Resources

Our patient-focused websites provide customized education and rare disease awareness to patients and their families. We also prepare plain language summaries of clinical trial and DMP results.

Our Educational Resources:

www.mpsviiifocus.com

A disease education website about mucopolysaccharidoses (MPS) VII available to the global community in English, Spanish, Portuguese, Italian, Polish, Romanian, Hungarian and Croatian

www.OneXLHvoice.pt

A disease education website about X-linked hypophosphatemia (XLH) available to the community in Mexico and Brazil

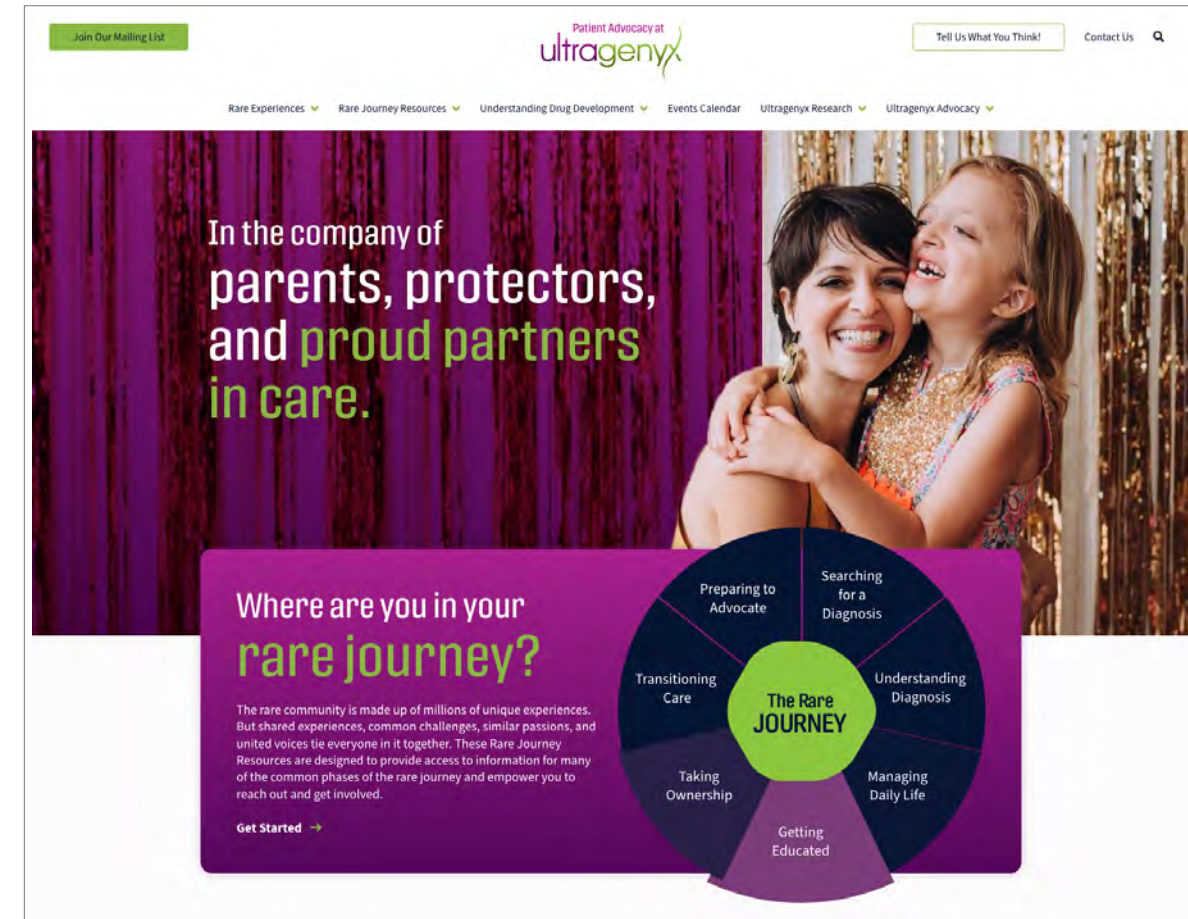
www.faodinfocus.com

A disease education website about long-chain fatty acid oxidation disorder (LC-FAOD) available in English and Spanish

ultraclinicaltrials.com

A global clinical trial recruitment website for caregivers and people living with rare diseases

Our Rare Journey Resources, at [Ultrareadvocacy.com](https://ultrarareadvocacy.com), are designed to provide access to information for many of the common phases of the “rare” journey and empower patients and their caregivers to connect and get involved.





Resilience and Advocacy: Dr. Michelle Fynan’s Journey with Osteogenesis Imperfecta (OI)

Meet Dr. Michelle Fynan, a distinguished psychotherapist and advocate, whose life is a testament to resilience and strength in the face of OI, a genetic disorder characterized by fragile bones. Diagnosed in early childhood, Michelle has not only overcome immense physical challenges but also become a beacon of hope and change for others facing similar struggles.

From her earliest years, Michelle faced the daunting challenges of OI. Her life has been marked by more than 30 fractures and numerous surgeries. Despite facing both physical and psychological impacts, including post-traumatic stress disorder, Michelle has achieved remarkable milestones, showcasing her relentless spirit and adaptability.

Michelle’s family, initially unfamiliar with OI, quickly became her bedrock of support. Their unwavering determination and love have been pivotal in shaping her journey. Today, as a mother to Addison and Avery, both diagnosed with OI, Michelle instills the same spirit of resilience and empowerment in her daughters.

Moreover, Michelle’s advocacy extends beyond her personal experiences. She actively supports Ultragenyx’s mission in rare genetic disorder research, championing awareness for OI and underscoring the importance of community support, self-advocacy and innovative treatments.

“For me, resilience means more than just overcoming; it’s about thriving in the face of challenges. It’s about transforming obstacles into opportunities for growth and personal evolution.”

— Dr. Michelle Fynan

Public Policy Participation

Ultragenyx recognizes the vital importance of public policy engagement in our mission to serve individuals living with rare diseases.

We understand that policy and regulations are essential for the cost-effective and timely development and commercialization of treatments for the 1 in 10 Americans living with rare diseases. They also play a critical role in helping people with rare diseases receive accurate diagnoses, quality care and timely access to therapies. Our commitment to public health and medical innovation is reflected in our advocacy for patient access to FDA-approved medicines, awareness of these treatments, and reforms to foster patient-centered care.

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.

The committee sets our positions and defines our priority policy areas, focusing on making a significant impact on public health policies. These priorities encompass enhancing the patient experience in drug development, providing access to innovative therapies through responsible

pricing, and accelerating the development timelines for new medicines. Efforts also include improving diagnostic processes through newborn screening (NBS), contributing to gene therapy development, and increasing awareness of ultrarare diseases in emerging markets. We establish these priorities and positions in consultation with our management team, who are updated annually on our advocacy efforts.

Our activities in public policy are guided by our [Global Code of Conduct](#), with the goal of acting in compliance with all relevant laws in our engagements with public and governmental entities. Our Government Affairs and Public Policy team is at the forefront of our interactions with legislative and regulatory bodies, committed to contributing responsibly and with a civic-minded approach to the science of rare disease medicine. At the federal and state levels, members of the Ultragenyx team engage in policy discussions with governments, trade associations, patient groups and other stakeholders. By sharing our unique experiences and insights as a biopharmaceutical company dedicated to developing therapies for patients with unmet medical needs, we believe we add valuable perspectives to the ongoing dialogue about tackling rare diseases.

As part of our commitment to transparency, we [disclose](#) in a timely fashion our limited corporate contributions to several California candidates, and the Ultragenyx Political Action Committee (PAC) makes contributions to various lawmakers and committees. The Ultragenyx PAC is registered with the Federal Election Commission (FEC) and works to adhere to all reporting requirements. Information about our contributions is publicly available on the FEC's [website](#).

Additionally, we are a founding member of the Rare Disease Company Coalition (RDCC), a coalition of **23** companies that are collectively investing more than **\$15 billion** annually in R&D and that have over 200 treatments either approved or in development. Betsy Ricketts, our vice president of Policy, Government and Public Affairs, was elected in 2023 to serve as secretary of RDCC's Executive Committee. RDCC educates policymakers on the distinct considerations of life science companies operating in the rare disease field and focuses on three priorities:

- supporting robust development and innovation
- promoting accessibility
- enabling earlier diagnosis

Public Policy Participation (cont.)

We amplify the voices of the rare disease community by supporting the following policy priorities:

Patient Experience	Prioritizing patients in development and commercialization, including patient experience data in drug applications and labels
Market Access	Helping make rare diseases a priority in global markets
Pricing and Reimbursement	Proactively engaging with payers to achieve majority access to treatments while advocating for responsible pricing and flexible payment models for innovative therapies
Diagnostic Odyssey	Shortening the timeline from diagnosis to treatment access through initiatives such as NBS and genetic testing
Gene Therapy	Engaging policymakers in innovative approaches to clinical and manufacturing development while characterizing unmet needs and burden of illness in peer-reviewed literature
Accelerated Development	Streamlining drug development timelines, including biomarker and endpoint development, to speed up assessment and approval by global health authorities
Emerging Markets	Raising awareness of ultrarare diseases, encouraging local definitions, and educating on advanced therapies, including gene therapy and mRNA



Our CEO alongside members of the public affairs team during a public policy advocacy visit to Washington, D.C.

Public Policy Participation (cont.)

Advocating for Newborn Screening (NBS)

NBS is a vital part of treating rare diseases. With early detection, affected infants can receive prompt treatment that can help prevent permanent disability, developmental delay and death. NBS programs in the U.S. are state-run public health programs that identify newborns with certain genetic, metabolic, hormonal or functional disorders. Because NBS programs are state run, there are major discrepancies regarding the diseases each state screens. The total number of conditions included in screening ranges from 34 to 67, and no state screens for all 37 core conditions and all 26 secondary conditions on the Recommended Uniform Screening Panel (RUSP). Ultragenyx advocates for these state-run programs to screen for all conditions on the RUSP so that infants with rare diseases can receive prompt access to treatment no matter where they are born in this country. In 2023, Ultragenyx advocated for the passage of legislation in Texas so that when new conditions are added to the RUSP, Texas will add those conditions to its state panel within a specific period of time and that it has the requisite funding to do so (RUSP-alignment legislation).

In 2024, Ultragenyx will be working to pass RUSP-alignment legislation in Alabama, Colorado, Tennessee and Washington. See also [Research Collaboration](#) for information on Ultragenyx joining BeginNGS consortium, a Rady Children's initiative to develop genomic analysis tools for hospitals to significantly increase NBS and early diagnosis of rare diseases.



Advocating for an UltraOrphan Drug Act

January 4, 2023 – 40 Years of the Orphan Drug Act (ODA): This landmark legislation has been instrumental in fostering the development of treatments for rare diseases, leading to over 600 treatments for more than 1,000 rare diseases and significantly enhancing the lives of those with rare conditions.

Challenges in Ultrarare Disease Drug Development: Despite the success of the ODA, there are substantial regulatory and commercial hurdles in creating treatments for ultrarare diseases, which affect extremely small patient populations. This has led to the cessation of development of multiple investigational therapies by many companies, prompting nonprofits, often spearheaded by parents of children with ultrarare diseases, to take charge of therapy development.

Ultragenyx's Advocacy for the UltraOrphan Drug Act: In response to these challenges, Ultragenyx is proposing the UltraOrphan Drug Act, legislation intended to implement a new framework for incentivizing development of therapies for diseases with very small patient populations that will be a supplement to the existing ODA. This new act aims to facilitate the development of therapies for diseases affecting fewer than 2,000 people in the U.S., defined as ultrarare diseases.

Key elements of the proposed framework include:

- Recognition of qualifying biomarkers as primary endpoints in pivotal clinical trials
- Acceptance of alternative study designs and analyses for ultrarare diseases
- A 50% tax credit for clinical trial costs (capped at \$100 million per program), 10 years of marketing exclusivity, and waiver of application user fees
- Implementation of a "sufficient efficacy standard" akin to the emergency use authorization standard, replacing the "substantial efficacy standard"
- Assurance that approvals under this framework are full, not experimental, and are recognized as such by payers



Gabrielle's Journey with Angelman Syndrome (AS)

When Gabrielle was 7 months old, her parents noticed that she was behind on certain developmental milestones. Pediatric testing resulted in a general categorization of “gross developmental delay.” At 18 months, she was given a blood test for AS following a health scare and the results were negative. Gabrielle was misdiagnosed several times before finally being given whole-exome sequencing, which finally resulted in a definitive diagnosis of AS at age 2. She has a rarer subtype of AS that didn't show up with a blood test.

AS is a condition characterized by developmental delays and neurological challenges and affects roughly 1 in 15,000 births. With no therapy available, Gabrielle's diagnosis catalyzed her family's commitment to advocacy, research support and community engagement. Her mother Anna's advice for other families walking the same path is to know that AS is part of your child, but it does not define who they are.

Gabrielle loves to give hugs and finds joy in many things. Now at school, she is known as “Mayor Gabby” because she takes the time to observe if people are doing their jobs. Gabrielle's family motto is to always BELIEVE. Anna says that while life will be different, you shouldn't give up on your dreams for your child.

Gabrielle's diagnosis catalyzed her family's commitment to advocacy, research support and community engagement.

People

Sustaining and strengthening our generous and inclusive culture while enhancing our health and safety practices

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.



Aspiration

To be an inclusive, sought-after company where employees come first and feel motivated to bring the best versions of themselves to work each day, knowing they are making a difference in the lives of the rare disease community.

Our Objectives	2023 Progress
<p>Maintain a positive workforce culture by achieving a total turnover rate below the industry average and continuing to have high employee engagement</p>	<ul style="list-style-type: none"> Total turnover was 15.5%, which is below the U.S. and global averages for our industry (according to Aon Radford’s Salary Increase and Turnover Studies) Maintained a high engagement score of 86% in our employee engagement survey
<p>Support internal career and leadership development through our significant investment in customized employee programs that build core competencies and bring our company values and culture to life</p>	<ul style="list-style-type: none"> Successfully implemented an enhanced and bespoke framework for evaluating and rewarding performance Nearly 22% of our global workforce participated in the on-demand career coaching services we offer Hosted 24 interns across our emerging talent programs
<p>Continue to strengthen and expand diversity and inclusion through intentional talent acquisition and management efforts, including candidate pipelining, interview processes, ongoing education, awards, promotions and succession planning</p>	<ul style="list-style-type: none"> Women accounted for 60% of promotions globally, while U.S. employees who self-identified as racially or ethnically diverse represented 46% of U.S. promotions Broadened our mission and evolved from Inclusion & Diversity (I&D) to Diversity, Equity, Inclusion and Belonging (DEIB) Conducted DEIB Leaders’ Workshops to unite DEIB Action Team and ERG leaders, enhancing our 2020-2025 DEIB Roadmap
<p>Implement a robust and comprehensive health and safety management system framework and audit process</p>	<ul style="list-style-type: none"> Introduced a New Hire EHS Checklist across all sites, completing 315 checklists to enhance safety awareness among employees and contractors regarding evacuation routes and emergency equipment Launched a safety observation system, engaging employees in safety improvements, leading to over 300 reported and addressed hazards Completed more than 140 ergonomic hazard assessments

Culture and Values

We have intentionally built and consistently nourished our company culture so our employees can experience a sense of purpose and fulfillment in their work – while feeling connected each day to the bigger impact we have on the rare disease community.

We aspire to be a company where our family, friends and children are proud to work. This means having a steadfast commitment to working to create and sustain a healthy, inclusive company culture where our people feel genuinely cared for and supported so they can thrive in all areas of their lives. As foundational to our culture, we encourage generosity, curiosity and humility so we can continue to learn together while fostering an environment that supports profound growth.

Our people are

Ultra-focused	Our team works together fearlessly to uncover new possibilities.
Ultra-curious	Our team applies their biggest ideas in courageous ways. Instead of asking “Why?” they ask “Why not?”
Ultra-impactful	Our team works hard to make a difference for those who need it most.
Ultra-dedicated	Our team recognizes their biggest challenges yield rare possibilities.
Ultra-innovative	Our team takes rare and dynamic challenges head on.

We carefully crafted our cultural values to empower our team members, allow for their voices to be heard, and encourage them to strive to make a difference.

GENEROUS

We are committed to helping – sharing our knowledge and skills with our patients, our field and each other.

DYNAMIC

We learn and adapt – constantly searching for deeper understanding and rapidly evolving our plans based on our insights.

COURAGEOUS

We go where others won’t – targeting untreated diseases and taking on the challenges that move our field forward.

POSSIBILITY

We seek the undiscovered discoveries – we’re committed to finding options for those who don’t have any.

RELENTLESS

We won’t give up fighting for the rare disease community – together always searching for solutions.

Human Capital Development

Ultragenyx has more than **1,270** employees globally. We are dedicated to building a global and diverse team; maintaining a healthy, inclusive company culture where employees feel respected and valued; and providing opportunities for learning, personal growth and career advancement.

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 turnover was **10.5%** in 2023, which is below the U.S. and global averages for our industry (according to Aon Radford’s Salary Increase and Turnover Studies).

We push each other to perform at our very best to support our mission to transform the lives of people with rare disease. In an effort to enhance camaraderie and embed recognition into our culture, we have an internal platform called “U Earned It,” where employees at all levels can recognize colleagues for their contributions and award them with points that they can redeem for rewards.

We actively nurture and develop our internal talent pipeline, providing opportunities for employees to grow within their roles and beyond. This includes our Mentorship Program, which pairs employees across the business to accelerate growth and development, foster cross-functional relationships, and strengthen our teaching, learning and networking skills. In 2023, more than **110** employees participated in the program. Our executive leadership team plays an important role in workforce planning by periodically assessing the company’s overall organizational design

and structure. The goal is to support the development of future leaders, identify skills and capability needs, update leadership succession plans, and refine DEIB strategies. Our Executive Team meets annually to discuss the succession plan for the company, and each Executive Team member updates the plan for their employees.

We also support and encourage team building with team and department offsites, weekly company-sponsored lunches, UltraTalk Speaker series, group exercise, happy hours, milestone celebrations, summer and holiday events, and more. These events are held both virtually and in-person.

In 2023

*More than **35%** of new hires came through our employee referral program*

Turnover in 2023	
Total Turnover Rate	15.5%
Voluntary Turnover Rate	10.5%
Executive/Senior-Level Officials and Managers	0.5%
First/Mid-Level Officials and Managers	6.7%
Others (Administrative Support Workers, Professionals, Technicians, and Laborers and Helpers)	3.3%

UltraPerformance Management

We refer to our performance management system as UltraPerformance, which represents a holistic approach to management by objectives, aligning individual efforts with the company’s overarching goals. It plays a crucial role in empowering team members to make significant contributions toward shared objectives while offering opportunities to enhance skills and advance within the company.

The core of UltraPerformance involves establishing and periodically reviewing annual goals to help keep them aligned with the company’s strategic direction. We conduct formal employee reviews twice a year where performance is assessed against an employee’s objectives. We expect our managers to have regular check-ins with their direct reports throughout the year and provide real-time feedback and recognition. These interactions provide a broader understanding of each employee’s strengths, career aspirations and performance contributions. They also help identify opportunities to accelerate career development and align individual achievements with compensation and rewards. This comprehensive approach helps to cultivate a culture of continued improvement and open communication and is integral to our ongoing success, with the goal that individual contributions are not only recognized but also directly linked to both personal and company-wide achievements.

See also [Employee Compensation and Benefits](#).

In 2023

Women accounted for **60%** of promotions globally, while U.S. employees who self-identified as racially or ethnically diverse represented **46%** of U.S. promotions

Enhancing Performance Management

After an extensive three-year planning process, incorporating stakeholder feedback from employee and manager focus groups, we have moved away from our prior five-point rating system and successfully implemented an enhanced framework for evaluating and rewarding performance. This new and bespoke framework, applicable to all employees, is designed to empower discussions and evaluation of individual performance and development in a comprehensive and meaningful way. This new framework formally integrates three equally weighted categories, historically fundamental to evaluating performance, that form the basis for calculating the annual bonus payout:

Goal Attainment

Outcomes achieved against individual objectives.

Role Performance

Performance against key job priorities and expectations.

Core Value Alignment

Behaviors consistent with integrity and our core values at Ultragenyx.

This transition has delivered several benefits for our workforce:

Clarity: Employees now have a clearer understanding of their goals, job expectations, and how their actions reflect our values on a day-to-day basis.

Empowerment: People managers now have greater flexibility and nuance in recommending year-end bonuses, enabling them to recognize and address both successes and areas for improvement.

Alignment: A stronger correlation between company, individual performance, and actual bonus payout, reinforcing our commitment to the pay-for-performance framework.

Transparency: The new framework provides a transparent structure for offering feedback, encompassing both celebratory achievements and opportunities for growth.

To facilitate the transition, we employed a thoughtful change management strategy. This included the development of guides, FAQs, and training videos to assist our employees in navigating the changes.

Employee Learning and Development

Our culture continues to grow and evolve, and we believe that each employee plays an important role in shaping and sustaining it. That is why we are deeply invested in the personal and professional growth of our employees, making it a strategic focus of our company.

We have instituted a unique approach to employee development, led by an in-house team of skilled learning experience designers and facilitators. This team develops and customizes the majority of our programs that are designed to align with our culture and meet our community's specific needs. These needs are identified through a thorough process that includes behaviors and attitudes measured by our annual engagement and pulse surveys, performance management processes, ongoing dialogues with business leaders and teams, and assessment of alignment with our company values, vision and strategy. We also rigorously track the impact of our programs. Each course undergoes real-time evaluation for relevance, application potential, and overall enjoyment. We aim to continue to refine our programs based on feedback so that even the highest-rated courses are regularly updated.

The goals of our employee development programs are comprehensive, aiming to deepen self-awareness, encourage curiosity and humility, teach effective feedback, reinforce an empowered mindset, strengthen compassion of/for self and others, build a diverse and inclusive community, and support our company vision and strategy. These objectives not only relate to the ability of our employees to excel in their roles and collaborate effectively but also are designed to contribute to crucial business outcomes such as increased engagement, high retention rates, career growth opportunities, talent attraction, brand enhancement and leadership in corporate culture.

Impact

Our employee development initiatives have significantly boosted our engagement and retention key performance indicators (KPIs) above industry benchmarks over the past six years:

- Raised our annual engagement index and DEIB engagement scores
- Maintained high retention and job satisfaction rates
- Attained a high rate of manager effectiveness

Employee Learning and Development (cont.)

We offer both required and optional workshops that extend beyond conventional models, combining high engagement and impactful content with a mix of in-person and digital formats. Required workshops include:

Dynamic Feedback

Seek, Offer, Receive: Encourages a culture of ongoing improvement by teaching employees to effectively seek, give and accept feedback

Empowered Mindset

Helps employees link their mindset with actions and outcomes, promoting better decision-making

Managing at Ultragenyx

A comprehensive workshop series providing managers with communication tools, management strategies and online career development resources

How to Effectively Manage Declining Performance

A focused 90-minute training for managers to address team underperformance

Leadership Development Program (LDP)

Helps enhance understanding of personal leadership styles, improves work relationships, and reinforces company culture commitment

Some of our other popular programs to support employees in their development include:

High Performing Teams

Our customized model and approach to help teams move quickly from forming and storming to performing

People Manager Tools

Comprehensive support for people management, including a resource library, monthly newsletters, essential workshops and topical lunch series

Cultivating Resilience in Everyday Life

Provides research-based tools to stay strong and promote well-being

Energy Management Wins – PeopleFuel

Helps employees use energy effectively, assess life areas and manage emotions for resilience and well-being

Technical training, particularly in areas such as quality and compliance, is managed separately by a specialized technical group. This approach maintains focused expertise and provides tailored training for specific technical needs, thereby complementing our broader employee development efforts.

Additionally, we sponsor programs such as **UltraTalks**, our version of TED Talks, to bring new perspectives and insights to the company. These experiences are designed to build employee morale, stimulate innovation and invest employees in company improvement. Some of our past speakers have included **Dr. Cal Newport, Abby Wambach, Patrisse Cullors, Dr. Laurie Santos and Paul Hawken.**

Insights 101: Introduction

Promotes enhanced self-awareness and ability to understand others by learning about the insights working style assessment and the four-color energies

Mindfulness for Stress Relief

Provides simple stress relief tools, online resources and insights into the scientific benefits of mindfulness

Presenting with Impact

Teaches basic skills for developing clear messaging, memorable content and effective delivery style to business presenters who do not have extensive presentation experience

In 2023

*We offered **39** employee development workshops, of which **29** were provided by our in-house Organizational Development team.*

*The average rating for in-house delivered workshops in terms of relevance, application potential and overall enjoyment was **92** out of 100.*

The average training and development time per employee across the **39** development workshops we offered was over one hour. The attendance breakdown is representative of our employees' gender and racial/ethnic diversity.*

*Our average training hours per person exclude time for technical training, required document reviews, and continuing education/accreditation from third parties, which certain employees must complete for their roles

Improving How We Work

We believe that by embedding the tools and principles of continuous improvement and **Lean Six Sigma** into the way we work at Ultragenyx, we can achieve our near-term goals and advance our vision and strategy. We aspire for our employees to adopt a continuous improvement mindset, focusing on efficiency, effectiveness, and working smarter, not harder.

In 2023, we invested in Lean Six Sigma by partnering with Acuity Institute to bring this high impact methodology to help solve problems and improve efficiency across our company. The objective is to identify and deliver operational improvements in the form of speed, quality and cost, enabling us to refine our processes, increase capacity and advance our strategic goals. Additionally, we curated and customized a set of Lean Six Sigma tools and resources for projects, so that our teams are well-equipped to drive improvements. We also increased awareness through company-wide meetings, internal websites and various communications campaigns, embedding these concepts into our corporate culture.



In 2023

*Certified **29** Lean Six Sigma Champions.*

*Trained **18** Green Belts, **154** White Belts, and **5** in Lean Foundations.*

*Kicked off **11** Lean Six Sigma projects.*

Career Development

We strive to not only be a place where employees can do the best work of their career but also where they can experience profound professional and personal growth. In addition to our set of employee development offerings, we have career development tools and programs designed to support employees in growing in their careers.

Ultra-Orbit

The Ultra-Orbit program aims to provide an opportunity for employees at all levels who are interested in developing or enhancing their program and project management skills while remaining in their current role. Each participant engages with a program manager mentor, focusing on measurable outcomes and timelines. Since the program's pilot in 2022, **15** employees have benefited from participating in Ultra-Orbit rotations, and a significant number of employees have inquired about these rotations. In 2024, we plan to expand the program more broadly within the company.

Career Coaching

To enhance career growth and development, we offer on-demand career coaching services through an external network of professional executive coaches. Coaching session topics include working relationships, performance and role, growth and development, and general stress.

In 2023, nearly **22%** of our global workforce participated in the coaching program. Participants rated the quality of sessions **4.9** out of 5 stars. Initial feedback showed:

97% of participants feel more positive about their issue.

98% of participants are more likely to address their situation.

94% of participants learned a new skill or strategy.

High-Potential Program: LEAD (Leadership Expansion And Development)

We invest in various training and development programs that are designed to build and strengthen our employees' leadership and professional skills. The goal of our LEAD program, which started in 2018, is to build the next level of leaders that will help us evolve our company and support our culture as we grow.

Each of the five LEAD cohorts since the program began have been cross-functional and diverse as well as balanced geographically, with the aim of being inclusive of remote employees. The program provides workshops, business simulations, mentorship and executive leadership talks to help equip participants with the skills to activate change throughout the company. Among the first four LEAD cohorts, **72%** of participants were promoted within one year of completing the program.

2023 LEAD Cohort Highlights

43% were women

13% were based outside the U.S.

36% self-reported as members of ethnic/racial minorities

In 2023

More than **10%** of total open positions were filled by internal candidates

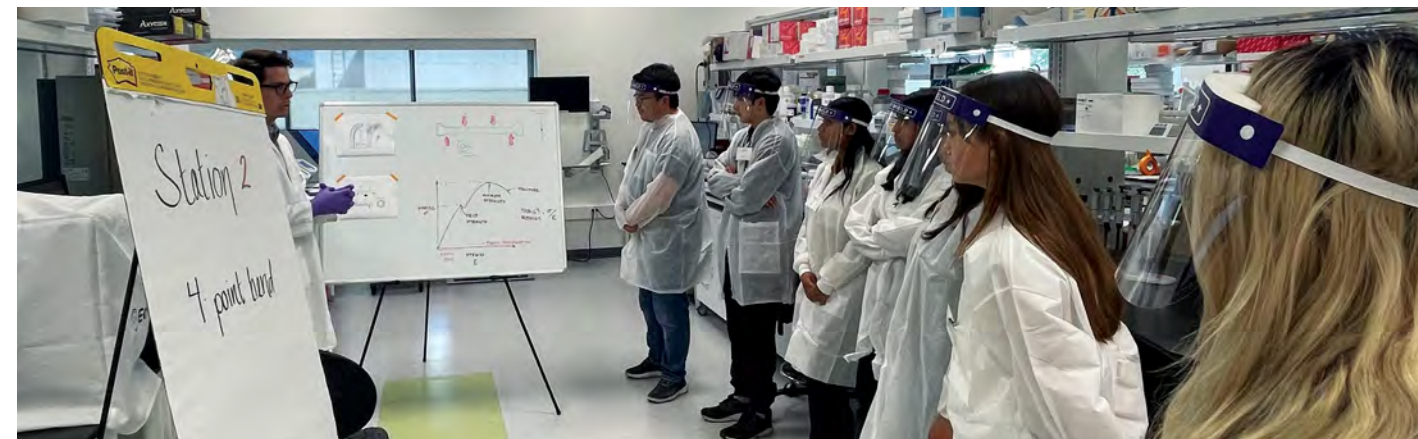
Emerging Talent Programs

Ultragenyx is committed to providing emerging talent with a range of opportunities to learn from a diverse team of world-leading experts at the cutting edge of research.

Through mentorship, hands-on experiences and exposure to cutting-edge methodologies, Ultragenyx aims to empower the next generation while building a sustainable pipeline of skilled professionals poised to make significant contributions to the field.

In 2023, Ultragenyx focused on strengthening relationships with local schools and universities. We have invested in high school outreach through biotech partners and continued partnership with the Japanese Community Youth Council (JCYC) where we support professional and technical development with juniors and seniors at Oakland Tech in Oakland and Galileo High School in San Francisco. Further, we have relationships with the University of California, San Francisco, to provide advanced pharmacy practice experience (APPE) rotations to prepare students for careers in medical affairs and with Middlesex Community College in Massachusetts as a pathway for careers in manufacturing.

Ultragenyx Emerging Talent programs include high school internships and outreach, college internships, co-ops, Advanced Pharmacy Practice Experiences (APPE) rotations and fellowships. In 2023, we hosted **24** interns.



Internships and seasonal co-ops

Offers students practical training in life science/ biotechnology, integrating them into meaningful projects at Ultragenyx to enhance their professional development.

Fellowships and post-doctoral programs

Offers a chance to explore key scientific questions affecting the rare disease community, develop investigative skills, and grow in a collaborative environment under expert mentorship in life sciences/biotechnology. In 2024, we plan to expand our fellowship offering to engage with underrepresented groups in the sciences, aiming to enhance physician diversity and integrate various perspectives in rare disease research.

Hosted lab tours experience

Immerses students in a biopharma environment, offering hands-on lab experiences and interactions with scientists to inspire Science, Technology, Engineering, and Mathematics (STEM) careers. In 2023, we hosted more than **60** students and their instructors from San Marin High School and the University of California, Davis, Graduate School of Management. Additionally, Ultragenyx summer interns, along with juniors and seniors from six local schools – including Balboa and Galileo high schools in San Francisco, and San Marin, Novato, Terra Linda and Tomales high schools in the greater Bay Area – visited our Novato campus for a lab tour and various STEM-related events and talks.

Employee Engagement

We believe active listening and employee engagement are essential to maintaining a healthy and thriving workplace environment.

We use the results of the “Your Voice” employee engagement survey to pinpoint key areas of focus and to take action on opportunities that will improve employee experience and engagement. To implement feedback more effectively and hear from our employees more frequently, we restructured our approach so our most robust “Census” survey with 65 questions happens every 18 months instead of every 12 months, and we added two new “Pulse” surveys within those 18 months that focus on a limited set of topics, including our corporate goals and key strategic initiatives and efforts. We made this change to create a timely listening platform so we can more quickly develop and deploy solutions that address opportunity areas.

We use our survey consistently to measure three people-related corporate goals that are assessed using an index of four to six survey questions for each goal. The areas we measure are focused on:

- Enhancing employee engagement at Ultragenyx
- Strengthening how our managers and leaders support DEIB in the workplace
- Improving Health & Well-being in the workplace to reduce stress and balance work and personal life

“YourVoice” results continue to be discussed with our board of directors as part of its oversight of the general organizational health of the company. In addition, the results are routinely shared in a timely manner with our senior leaders, managers and employees through team and company-wide meetings to increase shared accountability for the health of the culture and levels of engagement.



Employee Engagement (cont.)

Results from our 2023 Survey

We conducted our most recent 18-month survey in September 2023 and included a set of 65 scaled items and four open-ended questions.

Employees who have worked for Ultragenyx for at least 90 days were eligible to participate in the survey. We continued to have strong participation in our surveys with **90%** of eligible employees participating.

In our most recent survey, we achieved or outperformed on all three of our Thriving Culture 2023 corporate goals, including Employee Engagement, DEIB and Health & Well-being.

The following highlights some of our company-wide strengths:

- Employees continue to have a strong sense of belonging and pride in working for Ultragenyx, suggesting that they feel connected to something bigger than themselves.
- Manager effectiveness is a key strength for us as a company as demonstrated by employees having a positive perception of their direct manager's support of inclusion and diversity, and managers creating an environment where people can grow and develop.
- Our rewards and recognition category continues to be a strength, and we outperform benchmark data.

Company-wide actions on improvement opportunities identified from the most recent employee engagement survey include:

- Continue to embed principles and methodologies of Continuous Improvement including Lean Six Sigma into the way we work at Ultragenyx.
- Continue to increase connectivity and collaboration across different functions, teams and the broader company.
- Continue to empower leaders and employees to improve the effectiveness of their meetings* and embrace our shared best practices.

* This year, we added a set of questions to better understand the effectiveness of our meeting practices as this has been a hot topic for our leaders.

Employee Engagement Score

Enhancing employee engagement is one of our corporate goals. We measure this with a set of questions focused on employee pride in Ultragenyx, whether an employee would recommend Ultragenyx as a good place to work, if employees feel a personal sense of accomplishment from their work, and their intent to remain with us. Over the last five years, we have continued to maintain a high employee engagement score, scoring **86%** overall in 2023, with **92%** of our employees responding that they are proud to work at Ultragenyx, **88%** feel a personal sense of accomplishment from their work, and **88%** feel like they can build and maintain strong working relationships with their colleagues no matter where they are working.

External Recognition

In addition to strong employee engagement results, our achievements over the past year have consistently been recognized. Notably, we were named one of the **Top Places to Work** by the Boston Globe for the second consecutive year and also received the **Cultural Excellence Awards** from Top Work Places for Diversity, Equity & Inclusion Practices, Appreciation, Employee Well-being, and Professional Development. Furthermore, U.S. News & World Report named us as one of Healthcare's **Best Companies to Work For**, and we were recognized as one of the **Best Places for Working Families**®.

Employee Recognition

Our recognition programs are designed to contribute to fostering a supportive, dynamic and inclusive workplace, celebrating the exceptional contributions of our team members who embody our mission, vision and values. **The Ultra Leadership Recognition Program, Ultra Sharp Award Program, and the Rare Pearl Spotlight Award** – presented by the Empower Women in Biotech employee resource group (ERG) – honor individuals who demonstrate exemplary leadership, enrich our culture of inclusion, and go above and beyond in their roles. Additionally, our **“U Earned It”** program and **UltraRare Award** celebrate achievements and everyday successes, fostering a culture of appreciation and excellence.



Diversity, Equity, Inclusion and Belonging (DEIB)

We are committed to fostering a healthy, inclusive environment while nurturing a culture of belonging where all employees have equal opportunities. We strive to create an environment where everyone we work with, serve and engage with feels valued, respected and empowered.

Our evolution from Inclusion & Diversity (I&D) to Diversity, Equity, Inclusion & Belonging (DEIB) broadens our mission. This change, initiated by our DEIB leaders during a focused workshop, reflects lessons learned from our ongoing journey and a commitment to addressing challenges, uplifting the voices of all groups, and driving meaningful change.

Our multi-year vision for DEIB aims at going beyond words, with meaningful, tangible actions and results that reflect our values. To realize this vision, we have identified four strategic DEIB pillars:

- People and Communities**
 We prioritize creating a safe, inclusive culture for our people and the communities we serve.
- Inclusive Leadership**
 We lead by example, aimed at integrating DEIB into our activities.
- Organizational Diversity**
 We focus on attracting, developing and promoting diverse talent.
- Accountability and Transparency**
 We commit to working to drive DEIB progress, integrate it into our operations, and transparently share our progress.

Highlights of our recent DEIB accomplishments include the following:

Hosted Pivotal DEIB Leaders' Workshops

Our offsite DEIB workshops/retreats brought together members of our DEIB Action Team and leaders of our ERGs. These sessions were crucial for team building, developing our leadership skills, critically assessing and enhancing our 2020-2025 DEIB Roadmap, aligning our efforts, and executing the roadmap's action items. Participants reported that these workshops ended with a renewed commitment and focused energy toward our DEIB initiatives.

UltraTalks 2023 – Engaging in Neuroaesthetics and Intersectional Media

The 2023 UltraTalks featured Susan Magsamen and Ivy Ross discussing the science of neuroaesthetics, and the CIME team (Joy Donnell, Munika Lay and Nicole Haggard) sharing insights on intersectional representation in movies and media. These talks provided unique perspectives on how art and media influence community strength and individual well-being.

Interview Process Enhancement

We revamped our Interview Guide and tools to increase consistency and raise awareness of unconscious bias and how it can show up in the interview process.

Launched Internal DEIB webpage

Our internal DEIB webpage centralizes our dedication to diversity, equity, inclusion, and belonging. It serves as a comprehensive resource detailing our DEIB strategy, showcasing DEI metrics and highlighting our ERGs and community group initiatives, along with engagement opportunities.

Conducted Representation Audit

We shared department DEIB demographic data with each functional area leader, highlighting potential gaps and opportunities to improve diversity and representation.

Workforce Data

All data as of December 31, 2023*

Number of Total Employees	1,276
% of employees who are women	56.2%
% of employees with a disability	3.8%
Age Breakdown (Total Employees)	
<30 years of age	6.3%
30-50 years of age	63.0%
>50 years of age	30.6%
Number of Employees on the Executive Leadership Team (XLT)	
Number of XLT members who are women	1
Number of XLT members who self-reported and identified as racially or ethnically diverse	2
Number of Women in Management Positions	
% of women in all management positions (as % of total management positions**)	50.1%
% of women VP and above (as % of total management positions**)	43.9%
% of women in STEM-related positions (as % of total STEM positions)	55.5%

Number of U.S. Employees	1,089
% of U.S. employees who are women	56.6%
% of U.S. employees who self-reported as veterans	1.0%
% of U.S. employees who self-reported and identified as racially or ethnically diverse	45.8%***
Asian	27.8%
Black/African American	5.6%
Hispanic or Latino	8.5%
White	54.0%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	3.7%
Not Specified	0.4%
Number of U.S. employees in management positions	
Asian	25.6%
Black/African American	3.3%
Hispanic or Latino	6.2%
White	62.8%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	2.1%

*U.S. employee data is consistent with the company's submission on the U.S. Federal Employer Information Report Equal Opportunity Form (EEO-1).

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

***Due to rounding, the percent breakdowns for racial/ethnic minorities add up to <100%.

For information on diversity in our board of directors, see [Corporate Governance](#).

According to our 2023 internal employee* engagement survey, the perceptions of our managers' commitment to creating an inclusive and diverse environment improved compared to the 2022 results.

92% of our employees* feel that their managers support I&D in the workplace, up two percentage points from 2022.

90% feel that their managers model inclusive behaviors, up two percentage points from 2022.

87% feel that their managers create an environment where people feel they belong, consistent with 2022.

*Refers to employees that participated in the survey.

Employee Resource and Community Groups

Employee Resource Groups (ERGs) and Community Groups (CGs) are voluntary, employee-led groups that have a distinct impact on the culture at Ultragenyx and are equipped with a charter, mission and support team.

ERGs are created with the purpose of building inclusive spaces for individuals who share common aspects of identity. ERGs are an integral part of our DEIB strategy, as they are structured with the objective of influencing and advancing our business’s DEIB goals.

CGs are designed to foster connections among employees with shared interests. These groups offer a platform for like-minded individuals to engage in activities and discussions related to their common interests.



Seeks to empower the LatinX community at Ultragenyx to realize its fullest potential and encourages the next generation of LatinX talent to pursue careers in science and biotechnology



Works to promote a culture of equality and belonging to enable LGBTQIA+ employees to thrive in their careers and lives and achieve greater impact in the world



Connects employees with opportunities to support nonprofit organizations assisting underserved communities



Creates a forum that celebrates the diverse cultures of Asian and Pacific Islanders (APAC), promotes dialogue about the APAC experience, and supports the career growth and development of APAC employees at Ultragenyx



Seeks to create a supportive culture to encourage women, diversity and equity in leadership and science



Supports employees in taking a holistic approach to their well-being and work-life balance through a variety of events, initiatives and classes



A mix of ethnic groups and cultures that coexist with society and here at Ultragenyx, with the aim of recognizing, celebrating and using our gifts, abilities and resources to support one another and the company



Seeks to enhance Ultragenyx’s remote working experience to provide employees with a sense of belonging even while working from home



Plans events, including fun pop-ups, happy hours, watch parties and annual company parties

The UltraProud ERG hosted its third Annual Coming Out Day, featuring Alicia Roth Weigel and Hope Giselle, who led discussions on allyship.

DEIB in Recruitment

We have an intentional approach to recruitment that focuses on creating a welcoming and positive experience for each candidate. We aim to create a sense of belonging from the very start to foster an inclusive workforce.

Our intention is to create a diverse pipeline of candidates applying for jobs at Ultragenyx by using multiple strategies, such as diverse interview teams to “screen in” instead of “out” to mitigate bias, and hosting virtual open houses and career fairs to support our broad outreach efforts. We also provide interview skills training to our employees to support inclusive interviewing and a deepened understanding of how unconscious bias shows up in the interview process and can create barriers for those who are underrepresented in the workforce. This training supports our ultimate company goals of being an employer of choice for diverse talent and to have candidates and new employees feel a sense of belonging from the very start of their relationship with Ultragenyx.

Additionally, we continued our partnership with Disability Solutions (DS) on outcomes for talent with disabilities, and Ultragenyx’s jobs became live on the Disability Solutions’ Career Center. The DS Career Center works with Ultragenyx to drive traffic to our job postings by actively recruiting candidates with disabilities from their large network of local and national workforce community partners.



Through our intentional approach, we have seen an increase in the number of diverse candidates interviewing for potential employment as well as the number of new hires who self-report as members of diverse populations.

In 2023

More than **35%** of new hires came through our employee referral program.

More than **half** of our **120** new U.S. hires self-reported as members of diverse populations.

Occupational Health, Safety and Wellness

The health and safety of our workforce is a key priority.

Our health and safety management system is vital to a safe and healthy work environment and includes several elements, such as Global Environmental, Health, Safety and Sustainability (EHSS) standards, site-specific standard operating procedures, incident and safety observation reporting, hazard identification and risk assessments, job safety analyses, ergonomic assessments and industrial hygiene evaluations. The system is based on the principles of ISO 45001:2018, the International Organization for Standardization (ISO) standard for occupational health and safety management, and is designed to help us comply with applicable statutory and regulatory requirements and Ultragenyx policies, proactively identify and prioritize occupational health and safety risks and potential mitigation options for our operations, and drive continued improvement across our global operations. We continue to search for opportunities to improve. In 2023, as part of our efforts to advance health and safety management across our business, we focused on the ongoing development, improvement and effective implementation of our health and safety management system and reporting processes. In 2023, we rolled out the high hazard work standard and developed a contractor safety management standard, which is expected to be rolled out in 2024.

2023 Highlights:

Developed and published monthly EHSS Performance Reports to provide an enterprise-wide view on incidents, compliance, targets and training to stakeholders and the executive sponsor.

Strengthened key Global EHSS Standards for Waste Management, Occupational Health, Office Safety, Contractor Safety and High Hazard Work Activities.

Enhanced our safety culture by establishing and communicating targets to increase safety observation reporting, expand preventative ergonomic assessments, and complete hazard assessments for our top chemicals of concern.

Implemented a **New Hire EHS Checklist** to familiarize employees and contractors with site-specific safety procedures such as evacuation routes and emergency equipment. A total of **315** new hire checklists were completed in 2023.

Rolled out **laboratory safety refresher training** to more than **200** employees to reinforce hazard identification and safe work practices in our laboratories.

Rolled out **comprehensive EHS training for manufacturing** employees at the new GTMF, including such topics as site-specific EHS training, biological safety, hazardous waste management, respiratory protection,

warehouse safety and lockout tagout procedures. We provided more than **50** training courses to **860** employees in 2023.

Established a safety observation system to engage our front-line employees in the safety improvement process, which resulted in the proactive reporting and addressing of over **300** safety hazards.

Global EHSS conducted industrial hygiene surveys at our manufacturing and R&D sites, collecting **70** samples for chemicals such as formaldehyde, potent pharmaceutical compounds, anesthetic gases, disinfectants and solvents. We also conducted surveys for noise and indoor air quality.

Completed more than 140 ergonomic hazard assessments for employees to identify and mitigate potential muscular skeletal injuries. In 2023, we emphasized promoting ergonomic assessments, with the majority being proactive rather than due to discomfort.

Improved our internal hazardous waste management program by establishing membership with CHWMEG, a non-profit association that promotes responsible waste stewardship, and performing due diligence reviews of **6** treatment, storage and disposal facility audit reports to proactively assess and mitigate environmental liability.

Occupational Health, Safety and Wellness (cont.)

Wellness

In addition to formalizing our new flexible and remote work models, we continue to provide our employees with wellness offerings to support their physical and mental health.

Spring Health offers mental wellness screening, stress management, coaching and up to four free virtual therapy sessions and two free psychiatry sessions each year to employees globally and covered dependents.

Mindfulness and Meditation programs as well as informal support groups are available to employees struggling with caregiving, isolation and stress.

Annual Flu Shot Clinics are available in offices. Flu shots are covered for free in the U.S. for employees plus family members and are reimbursed for employees and their family members outside the U.S. Additionally, COVID-19 rapid antigen tests are available globally in offices at no charge.

Caring for U is a global reimbursement program offering employees up to **\$1,200** annually (in local currency) for wellness and caregiving activities. This includes fitness classes, gym memberships, childcare, eldercare, meal delivery for dependents, and pet walking, supporting a well-rounded, healthy lifestyle and caregiving responsibilities.

Access to a Personal Health Advocate is available to assist our U.S. employees and their families with navigating the healthcare system and maximizing benefits.

Ergonomics Assessments are offered to help employees adjust their workstations and equipment for long-term comfort and physical health.

Emergency Response and Preparedness

Our Global EHSS standard establishes the requirements for emergency preparedness and response planning and our site-specific Emergency Response and Preparedness Plans (ERPP) are implemented to protect our workforce in the event of an emergency. Site-specific ERPPs include procedures for reporting emergencies, a clearly defined chain of command, identification of resources to provide rescue and medical services, and evacuation and shelter-in-place procedures.

We conduct emergency evacuation drills at least annually, designed to familiarize our workforce with site emergency procedures, establish proficiency in executing an orderly evacuation, and determine accurate occupant accountability. Reviews are carried out after each drill to identify the effectiveness and opportunities for ERPP improvement.

In 2023, we implemented a mass notification system to rapidly alert our workforce of emergency situations or potential threats and direct them on how to respond.

2023 Safety Data*

Lost Time Incident Frequency Rate (LTIFR)**	0.83
Total Recordable Incident Rate (TRIR)***	0.58
Number of Fatalities – Employees	0
Number of Fatalities – Contractors	0

* Data covers employees and contractors that are directly supervised on a day-to-day basis

** LTIFR = (Number of lost-time injuries) / (Total hours worked) x 1,000,000

*** TRIR = (Number of recordable incidents) / (Total number of hours worked) x 200,000

Return to Office Update

In 2023, as COVID-19 transitioned to an endemic state and more treatments became readily available, we sunsetted our vaccine requirement for employees, contractors and temporary workers and relaxed our COVID-19 Health and Safety guidelines. These decisions were made after careful consideration of the most recent medical, scientific and government data, analyzed by the COVID-19 Task Force and senior leadership.

Employee Compensation and Benefits

We recognize that our employees are our greatest asset, and their dedication and talent are the driving forces behind our success. To honor this commitment, we offer competitive compensation and benefits packages aimed at attracting, retaining and motivating top industry talent.

Our approach to compensation is rooted in fairness and equity, operating within a pay-for-performance framework. This strategy aligns our organizational culture and mission with our goal of providing equitable, transparent and unbiased remuneration for our employees. Our compensation and benefits package offers extensive support for health, family and financial well-being. It includes health, life and disability insurance, 401(k) matching, cash bonuses, equity awards, paid time off for volunteering, wellness programs and tuition reimbursement.

We base compensation decisions on multiple factors, including role, performance, location, relevant experience, external and internal peer data, and professional contributions. These decisions are thoroughly reviewed by senior leadership to help promote fairness and consistency across the company, align with industry benchmarks and maintain competitiveness. This approach not only helps us attract and retain top talent but also fosters a culture of inclusivity and meritocracy.

We are firmly committed to maintaining pay equity for all our employees worldwide, an integral component of our broader focus on DEIB. 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 advanced software that enhances our capacity to effectively monitor and manage pay equity practices. This includes utilizing sophisticated data analytics, which enable a 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 resolution of any emerging issues.

Key compensation practices include:

- **Benchmarking process:** We review each position for an appropriate pay range by referencing external talent markets, utilizing third-party benchmark data, and considering internal equity. Our aim is to maintain equitable and competitive compensation within these established ranges.
- **Transparency:** We embrace transparent pay practices by clearly defining salary ranges for every role and promptly share this information with employees upon request. We offer extensive training and resources to help employees understand how their compensation is assessed and calculated.
- **Regular reviews:** Regular reviews of our compensation policies and practices are integral to our strategy. These reviews help in addressing any disparities or inequities that may emerge.
- **Customized compensation structures:** Employees commuting to an office in higher cost of living areas receive higher salary increase budgets and are benchmarked to salary ranges tailored to enable them to support themselves and their families in their locale. Additionally, employees in our manufacturing facilities are eligible for competitive shift differentials and overtime to supplement base earnings. These practices are aimed to provide that our workforce is paid with fair and livable wages, incorporating considerations of local economic landscapes, cost-of-living/labor metrics and industry standards.
- **Performance-based rewards:** Our compensation structure includes performance-based cash and stock-based elements to recognize and appropriately reward exceptional performance.

For information on median compensation and CEO pay ratio, see [the Proxy](#).

Employee Compensation and Benefits (cont.)

Our Benefit Programs

Our benefit programs provide employees and their families with access to a suite of innovative programs that are designed to enhance their physical, emotional, familial, financial and social well-being, plus additional perks to support employees both in and outside the office. Our programs include a comprehensive selection of medical, dental and vision plans; retirement savings options; competitive paid time off; and other initiatives that support balancing work with life. Eligible employees participate in our annual short-term and long-term, equity-based incentive programs, which provide opportunities to share in our company’s success.



A flexible work model



12 weeks of paid family care leave with no waiting period



\$1,200 per year in wellness credits



Paid holiday weeks in August and December



Paid volunteer time: up to 16 hours per year



Employee stock purchase plan with shares discounted 15%



Regularly hosted UltraTalks from a variety of guest speakers, designed to spark new ways of thinking



Robust employee inclusion and development programs

More information can be found on our [Career webpage](#).

2023 Compensation and Benefits Enhancements

Increased the employer contribution to the Health Savings Account (HSA) for employees enrolled in the High Deductible Health Plan.

Improved our company-paid disability coverage, providing additional income protection.

Launched a voluntary Long Term Care insurance program for U.S. employees.

Introduced a new performance and bonus evaluation framework in 2023, focusing on goal achievement, job performance, and value alignment to enhance clarity, alignment, empowerment, and transparency.

Communities

Aligning our corporate philanthropic efforts with our mission and purpose

We are **committed** to supporting initiatives that provide meaningful impact for the rare disease community; public health and access to care; Science, Technology, Engineering, Arts and Mathematics (STEAM) education; and local, at-risk communities.



Aspiration

To make a positive impact in the communities where we operate and beyond.

Our Objectives	2023 Progress
Support charitable organizations that contribute meaningfully to the health and well-being of the communities where we operate and beyond	<ul style="list-style-type: none"> • Approved more than \$2.5 million in charitable donations, independent medical education and health-related grants across 21 countries • Provided support to organizations focused on emergency and humanitarian relief, including contributions to earthquake efforts in Turkey and Syria, storm relief in Pajaro, California, wildfire assistance in Maui, Hawaii, and hurricane relief in Acapulco, Mexico
Expand opportunities for employee volunteerism	<ul style="list-style-type: none"> • Partnered with Life Science Cares and other organizations to launch the inaugural Ultragenyx Days of Service in the U.S., Canada, EMEA (Europe, Middle East and Africa) and LatAm (Latin America) regions, with employees completing more than 30 service projects • More than 50% of employees signed up to participate in Days of Service projects

Charitable Giving

Our charitable giving mission builds on Ultragenyx’s broader mission to transform the lives of people living with rare disease.

We direct our charitable giving to our local communities and beyond, and we recognize that healthy lives are supported by healthy communities and environments, including education and access to healthcare, and emergency relief aid during times of crisis. Our corporate philanthropy is focused on the following priorities:

- Rare disease community support
- Equitable healthcare
- STEAM education
- Local, at-risk communities
- Emergency relief aid

We are committed to supporting initiatives that we believe provide impactful resources for each of these priorities.

Employee Giving and Volunteering

Ultragenyx offers employees the opportunity to take two paid volunteer days each year (16 hours), so they can spend time giving back to our communities and contribute to local initiatives. In 2023, more than **450** full-time and part-time employees recorded donating more than **2,900** hours for numerous volunteer activities, including supporting STEAM events, raising money for unhoused communities, working at local food banks, participating in holiday gift drives, building hygiene kits, and working with family-to-family programs. Additionally, we have an employee resource group

(ERG) called UltraGiving that is committed to connecting employees with opportunities to support nonprofit organizations assisting underserved communities. Once the connections are made, employees can choose to use their paid volunteer time, volunteer on their own, or make donations. In some cases, the organizations are the same as those supported by corporate giving; in others, employees identify different organizations they wish to support. UltraGiving has chapters in the San Francisco Bay and Greater Boston areas, Utah and Latin America.

In 2023

*approved more than **\$2.5 million** in charitable donations, independent medical education and health-related grants across **21** countries.*



Inaugural Days of Service

During our inaugural Days of Service, more than 50% of our employees worldwide registered to participate in numerous impactful volunteer activities, embodying our commitment to social impact and underscoring the power of collective action in making a tangible difference in our local communities. Throughout the campaign, employees completed more than 30 service projects around the world.

KEY HIGHLIGHTS FROM THIS INITIATIVE INCLUDE:

Enjoyable and Rewarding Experiences

Employees across various locations actively participated in diverse volunteer activities, finding joy and fulfillment in stepping away from routine work to serve others.

Strengthening Community Ties

Whether volunteering at food banks, schools or nature preserves, our team members deepened their connections to local communities.

Significant Impact and Outreach

- **5,100** care packages assembled in California, including STEM and hygiene kits, benefiting various nonprofit organizations
- **800** humanitarian kits provided for Syrian war refugees by our Canada team
- **880** care packages assembled in Massachusetts, including STEM kits and school supplies, supporting local education and foster care
- **750** emergency necessity kits distributed by our Miami LATAM team, aiding earthquake and war victims

- Environmental conservation efforts across LATAM, EMEA and the U.S., including:
 - Cleaning up the Xochimilco Lake area in Mexico as part of a nature conservation effort
 - Planting **30** native species in Colombia
 - Removing more than **300 lbs (138 kg)** of waste from the Llobregat River in Spain
- Over **120** to-go meals donated in Salt Lake City to aid the unhoused community
- Many additional hours volunteered across various programs, focusing on education, health, environmental conservation, and emergency relief

*Employees completed more than **30** service projects around the world during our inaugural Days of Service campaign.*

Rare Disease Community Support

We partner with organizations that share in our mission to transform the lives of people living with rare disease. Our rare disease community support includes patient and sibling programs, respite care and support programs for care partners, and awareness initiatives.

In 2023, Ultragenyx proudly supported:



Camp Korey is a medically safe camp experience that offers children and families an escape from the endless medical treatments that often overshadow childhood. The objective is to create empowering and adaptive year-round programs for children and their families living with life-altering medical conditions, free of charge. Ultragenyx funded the overnight summer program where more than **270** children aged 7 to 17 with complex medical conditions and their family members spent four to five nights at camp enjoying activities such as Archery, Arts & Crafts, Silly Olympics, Fishing & Boating, Swimming, Pet Therapy and Stage Night.



Ultragenyx made a contribution to **Parent to Parent USA**, a nonprofit organization dedicated to offering emotional and informational support to families with children who have special needs. The donation was directed toward supporting the P2P USA's Leadership Institute, a live online event that brought together nearly **200** parents and staff for three and a half days of professional development and topics that affect parents of individuals with disabilities.



A Rare Affair

Ultragenyx hosted the 2023 Rare Affair fundraising event, now in its 11th year. Each year the Rare Affair has focused on raising money and visibility for a different rare disease advocacy organization. The 2023 event gathered more than **250** biotech executives, venture capitalists and rare disease advocates and successfully generated donations to the Osteogenesis Imperfecta Foundation (OI Foundation) to support its mission in OI research and advocacy.

Grants

In addition to our philanthropic efforts, we support the rare disease community through educational initiatives, patient advocacy, research and access to information.

Our grant support aims to enhance awareness, advance the medical and scientific understanding of rare and ultrarare diseases, and empower healthcare professionals to bridge clinical, research and practice gaps. Our specific focus is on patient and professional organizations that help improve awareness, care and access, and provide vital support to the rare disease community.

OUR GRANT SUPPORT INCLUDES:

Independent Medical Education

Supporting both accredited and nonaccredited clinical, technical and scientific programs, as well as continuing medical education (CME) activities focused on rare diseases for healthcare providers.

Health-Related Grant Funding

Sponsorships and grants for nonprofit patient organizations and for-profit health institutions for multiple initiatives. These include patient advocacy-focused initiatives, non-accredited scientific meetings such as conferences, summits and forums, fundraising and disease awareness events, research and educational programs, strategic partnerships, medical publications in Europe and Latin America, and fellowships.

To promote compliance with applicable standards and guidelines, our funding undergoes rigorous evaluation, that is based on:

- The Accreditation Council for Continuing Medical Education (ACCME) Standards for Commercial Support (SCS)
- The American Medical Association (AMA) Ethical Guidelines for Gifts to Physicians from Industry
- The FDA Guidance for Industry: Industry Supported Scientific and Educational Activities
- PhRMA Code on Interactions with Healthcare Professionals

Please see our [Grants webpage](#) for the latest list of areas currently being considered for funding.

In 2023

*More than **100** organizations focused on medical research and treatment, and rare disease awareness, education and advocacy located in **20** countries received health-related grants totaling nearly **\$2 million***

*More than **20** organizations in **10** countries received independent medical grants totaling approximately **\$0.4 million**, with over **2,000** healthcare professionals educated through these supported independent medical education programs*



Empowering Patient Advocates

Elsie Evans has had a profound journey living with homozygous familial hypercholesterolemia (HoFH), a rare lipid condition, and becoming a passionate advocate for patient education and support. Diagnosed with HoFH when she was 3.5 years old, Elsie faced numerous health challenges, including heart attacks in her early 20s and the need for regular apheresis treatment.

Her transition from a career in education to a role in patient advocacy is a testament to her dedication to making a difference. Elsie now contributes significantly to the EFFORT Europe Foundation and Heart UK, focusing on educating and supporting patients living with rare conditions such as HoFH. Her work emphasizes the importance of informed treatment choices and a deeper understanding of rare conditions. Elsie's experience with treatments, particularly apheresis, highlights the varied and complex nature of managing HoFH and underscores the crucial need for treatments that do not adversely impact the patient's quality of life.

Elsie's story underscores the significance of community and connection for individuals battling rare diseases.

Elsie's story also underscores the significance of community and connection for individuals battling rare diseases. By sharing her experiences and challenges, Elsie has fostered a network of support and shared knowledge, strengthening the bond among patients facing similar struggles. This sense of community is vital for emotional support and collective growth, helping patients navigate their journeys with greater confidence and understanding.

We are committed to supporting remarkable individuals such as Elsie and patient advocacy groups that play a crucial role in enhancing the lives of those affected by rare diseases. We strive to bridge the gaps between patients, healthcare providers and researchers, raising awareness about rare conditions and advocating for equitable healthcare access. Elsie's journey and her advocacy work shed light on the disparities in treatment access and healthcare, reinforcing our dedication to creating a more inclusive and supportive healthcare environment for all.

Elsie embodies the resilience and transformative power of patient advocacy in the realm of rare diseases. Her dedication to education and advocacy has not only improved her own life but also promises to make a lasting impact on the rare disease community worldwide.

Equitable Healthcare

We are committed to working to foster a society where healthcare is accessible to all, recognizing this as a fundamental element of a thriving and sustainable community. Our efforts are focused on reducing healthcare disparities, with the goal of everyone, regardless of background, gaining equal access to the health services they need.

We support organizations working to enhance public health and expand access to care for local and underserved communities. Our equitable healthcare contributions focus on public health initiatives, improving access to services, and promoting wellness in these communities, all with the goal of reducing healthcare disparities.

2023 HIGHLIGHTS

Transforming Lives in Colombia

Donated to **Fundacion Operacion Sonrisa** that provide more than 23,000 surgeries for children with cleft conditions, leveraging a network of 200 specialist volunteers for comprehensive care.

Promoting Public Health and Wellness

Partnered with Life Science Cares, offering Ultragenyx employees volunteer opportunities to support health initiatives in the San Francisco Bay and Greater Boston areas.

Supporting Pediatric Healthcare

Our UltraGiving team joined the Intermountain Foundation at The Festival of Trees fundraiser, benefiting Primary Children's Hospital through a silent auction of holiday decorations to aid needy families and enhance pediatric care.

Inaugural Days of Service

- In California, team members assembled **1,600** hygiene kits benefiting California's underserved populations. This effort benefitted a variety of Bay Area nonprofit organizations and programs, including **650** hygiene kits for Ritter Center, **150** for North Marin Community Services, **300** for HealthRIGHT360, and **500** for Ayudando Latinos A Soñar (ALAS).
- The Brazil LATAM team members volunteered at Lar da Criança Ninho de Paz in São Paulo, an organization caring for individuals aged 15-41 with severe cerebral palsy. Our team supported the **13** residents of Ninho de Paz with both in-kind and monetary donations.



Employees in California packing hygiene kits for Ritter Center during Ultragenyx's inaugural Days of Service.

STEAM Education

We support locally-implemented initiatives and organizations that inspire and advance the development of the next generation of leaders in Science, Technology, Engineering, the Arts, and Mathematics (STEAM), thereby empowering young minds irrespective of their backgrounds.

Our STEAM education giving targets include diversity, equity and inclusion (DEI) initiatives, scholarships and science events or programs.

2023 HIGHLIGHTS

Partnership with Biotech Partners

We provided financial support to Biotech Partners, enhancing educational opportunities for students pursuing careers in STEAM fields.

Empowering Young Women

We sponsored more than **50** seventh and eighth-grade girls from Sonoma County and nearby areas, enabling their participation in 10 mini workshops. These workshops, led by female scientists and engineers, were organized by Expanding Your Horizons to inspire young women to explore STEAM careers.

Promoting Creativity in STEAM

In Massachusetts, we supported students chosen by Bedford Creativity to compete in Destination Imagination's global finals. This competition encourages students to apply their STEAM knowledge creatively to solve complex, real-world problems.

Investment in Scientific Adventure for Girls

Our donation of **\$25,000** to Scientific Adventure for Girls supported engaging and educational afterschool STEM programs for young girls in the San Francisco East Bay Area. Additionally, we extended our support to the Buck Institute and participated in the North Bay Science Discovery Day, hosting a student-centered exhibit that was visited by more than 8,000 visitors, mostly families with children under the age of 10.

Back-to-School Support

Our teams in Boston and San Francisco organized supply drives to assist local community organizations such as East End House and Homeward Bound. Our employees generously donated approximately **150** filled backpacks and volunteered their time to assemble them.

UltraMosaic ERG's Contribution

Demonstrating our commitment to higher education and diversity in STEAM, the UltraMosaic ERG donated **\$5,000** to the scholarship fund at Alabama A&M University, supporting students in their academic pursuits.

Inaugural Days of Service

- In Massachusetts, team members assembled **880** care packages, including STEM kits, school backpacks and duffle bag care kits. This effort benefited the School on Wheels and Foster Love organizations.
- In California, team members assembled **3,500** STEM and final exam care kits and school backpacks. This effort benefitted a variety of Bay Area nonprofit organizations and programs, including **1,000** STEM kits for WEAREPIEFEST, **1,000** STEM kits for Scientific Adventures for Girls, **500** backpacks and supplies for Ritter Center and North Marin Community Services. Upward Scholars, Breakthrough Collaborative SF, Guardian Scholar Program, and HARTS Program together received **1,000** final exam care kits.

Local and At-Risk Communities

We are committed to helping to nurture and improve local communities, foster closer-knit bonds, and create a positive impact that provides all individuals, especially those in challenging circumstances, access to essential resources and opportunities.

Beyond corporate giving, our employees also donate through the UltraGiving ERG to advance philanthropy through volunteer events, fundraising and other efforts that aim to benefit local and at-risk communities. Additionally, Ultragenyx employees volunteer their time to serve on the boards of directors for local nonprofits, such as North Marin Community Services and the Novato Chamber of Commerce.

Heroes and Helpers Grant

Ultragenyx provided a grant to the Heroes and Helpers Program, run by North Marin Community Services (NMCS) in partnership with the Novato Police Department and the Novato Fire Protection District. The grant allowed **10** children from the NMCS Child Development or Case Management Programs to shop for holiday gifts for their family members, assisted by a police officer or a firefighter, the heroes and helpers.

Support for Hunger Relief

Ultragenyx supported the Alameda County Food Bank in its hunger-relief efforts to provide meals to at-risk communities.

Spreading Joy with Toys

Our UltraGiving team in Salt Lake City collaborated with Tiny Tim's Toys to build wooden toy cars for children in several countries, including the U.S., Zimbabwe, Iraq, Afghanistan and others. In the U.S., the toys were distributed to hospitals, including Primary Children's and Shriners, for patient enjoyment. Additionally, our UltraGiving team in EMEA donated wrapped gifts for **30** refugee children aged 2 to 18 years.

Inaugural Days of Service

- In Salt Lake City, Ultragenyx collaborated with The Road Home to support unhoused individuals and families. More than **120** to-go meals were donated and delivered, and team members spent two days serving hot lunches at the organization's downtown facility.
- In California, employees volunteered to bag fruits and vegetables at Redwood Empire Foodbank and SF-Marin Food Bank and serve meals at Family House.
- Employees also took part in environmental conservation efforts across LATAM, EMEA and the U.S., including:
 - In Massachusetts, employees volunteered to remove trash from parks and local waterways via a partnership with the Mystic River Watershed Association and the Charles River Conservancy, mulch and weed at The Food Project farm, and assemble meals with the Community Servings organization.
 - In California, employees volunteered at Marin County Parks to plant monarch butterfly habitats in green space, and to mulch and weed at Florence Fang Community Farm.
 - The Miami LATAM team actively participated in tree planting and the cleanup of local lakes and rivers.
 - The Argentina LATAM team partnered with Fundación Manos Verdes for a river cleanup and collected **15** garden-sized bags of trash.
 - The Colombia LATAM team partnered with Fundación Red de Arboles, planting **30** different native species and eliminating invasive ones, as well as cleaning the area.

- The Mexico LATAM team collaborated with Voluntarios México in cleaning up the Xochimilco Lake area as part of a nature conservation effort.
- EMEA team members, in collaboration with Ecoherencia, volunteered by cleaning the banks of the Llobregat River in Barcelona, Spain, and successfully removed more than **300 lbs (138 kg)** of trash. Additionally, they constructed **25** birdhouses to support local bird species in the river's man-made areas.

Cross-ERG Gift Drives

The EmpowerX Women in Biotech ERG partnered with UltraGiving team members to organize year-end gift drives:

- Supported the work of Wonderfund, an organization that serves children engaged with the Massachusetts Department of Children and Families (DCF). Wonderfund works directly with social workers to meet the individual needs of children and helps create magical holiday moments for children throughout the state. Ultragenyx employees provided holiday gifts and clothing for **125** local families in Massachusetts.
- Sponsored **66** children supported by the Marin Foster Care Association. Each child provided a wishlist of three items. Ultragenyx employees fulfilled all of these wishes in addition to providing stocking stuffer gifts.

Emergency Aid Relief

We prioritize emergency relief aid as part of our mission, offering rapid assistance during humanitarian crises, especially in the realms of public health and medical services.

In 2023, we directed our support to organizations dedicated to offering emergency and humanitarian relief in the aftermath of several natural disasters:

- Contributed to the Turkey and Syria earthquake relief efforts through **Direct Relief & AHBAP**.
- Supported the Pajaro levee storm relief via the **Community Foundation for Monterey County**.
- Participated in wildfire relief efforts through the Maui Strong Fund of the **Hawai'i Community Foundation**.
- Assisted in hurricane relief in Acapulco, Mexico, through **Cruz Roja Mexicana**.
- Our UltraGiving team in Canada partnered with **Global Medic** and assembled **800** humanitarian kits to be delivered to victims of the Syrian civil war.
- **Inaugural Days of Service:**
 - Our Miami LATAM team assembled **750** emergency necessity kits for earthquake victims in Turkey and those impacted by the war in Ukraine.
 - In Canada, team members assembled **800** humanitarian kits for Syrian war refugees.



During Ultragenyx's Inaugural Days of Service, employees in Miami, FL packed humanitarian aid kits to support those impacted by the Turkey earthquake and the war in Ukraine.

Planet

Reducing our environmental impact and promoting sustainability

We are **committed** to developing an environmental strategy that minimizes our environmental footprint across our business.



Aspiration

To conduct business in an environmentally responsible manner and strive to continuously improve our performance to benefit our employees, customers, communities and the environment.

Our Objectives	2023 Progress
Continue to implement improvements to reduce our environmental footprint	<ul style="list-style-type: none"> • Purchased 1,955+ megawatt-hours (MWh) of renewable electricity, which avoided an estimated 473 metric tons (MT) of carbon dioxide equivalent (CO₂e); installed additional electric vehicle charging ports, bringing our total to 38 • Strengthened our waste vendor partnerships to expand diversion and circularization programs • Launched My Green Lab certification pilot program at our Translational Sciences lab in Novato, California
Develop an environmental strategy	<ul style="list-style-type: none"> • Continued and expanded the collection of environmental data across our facilities using our newly developed procedures and policies; enhanced our global EHS&S policy to cover additional health, safety and sustainability topics • Considered sustainability impacts and opportunities across our supply chain

Reducing Environmental Impacts

Ultragenyx is committed to working to reduce the environmental impact of our operations by enhancing and promoting sustainable practices throughout our office, laboratory and manufacturing spaces, both leased and owned. We design our facilities with sustainability in mind.

Over the past two years, we have developed policies and procedures for data collection in alignment with the Greenhouse Gas (GHG) Protocol. The policies set standards for tracking and reporting environmental information, facilitating the analysis of our environmental impacts by location. Additionally, the established procedures are designed to support the comparability and reliability of our key performance indicators. In 2023, we made progress in automating data collection and in implementing a dashboard designed to assist in monitoring our performance. Alongside our efforts in benchmarking and analytics, we anticipate these programmatic improvements will enable our teams to develop environmental initiatives with greater efficiency.

Ultragenyx acknowledges the impacts and risks posed by climate change on our business operations and stakeholders. Our strategy incorporates senior management level oversight to comply with environmental regulations as well as manage potential climate-related risks and opportunities to support business continuity. We are taking steps to implement governance processes designed to manage and mitigate climate-related risks and impacts.

New Space with Sustainability in Mind

During 2023, we relocated our team in Cambridge, Massachusetts to a brand-new space in Somerville, Massachusetts, to accommodate our need for additional space and modern facilities essential for the vital work being carried out by this team. The Real Estate and Capital Projects team carefully selected this new Somerville location, noting the energy efficiency and accessibility to the MBTA Green Line East Somerville train stop.

The building, located in the Brickbottom district of Somerville, is expected to achieve Platinum status under the U.S. Green Building Council's LEED (Leadership in Energy and Environmental Design) for Building Design + Construction (BD+C) Core and Shell standard.

Designed with a commitment to environmental sustainability, the building emphasizes clean indoor air quality and ample natural lighting. It also features a range of high-performance technologies aimed at reducing energy and water consumption, thereby lessening our impact on the city's resources.

Reducing Environmental Impacts (cont.)

We have strategically implemented a series of initiatives aimed at reducing our environmental footprint:

Renewable Energy

We purchase **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 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 CO₂e.



Sustainable Transportation

To reduce commuting emissions and encourage sustainable urban mobility, we offer **38** electric vehicle (EV) charging stations across our California and Massachusetts facilities, including **4** ADA-compliant connections, to support employees who drive electric vehicles. Additionally, we promote the use of public transportation through comprehensive commuter reimbursement benefits. Our new Somerville facility in Massachusetts was strategically selected for its proximity to the MBTA Green Line East Somerville stop.

Sustainability in Lab Operations

In 2022, the 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. A cross-functional team is set to implement eco-friendly changes in 2024 and aims to eventually extend the certification to more labs.

Eco-Efficient Facility Upgrades

Our facilities team has converted to LED lighting in all common spaces; replaced HVAC systems with more energy-efficient models and programmed these systems for setbacks during off hours; installed building management systems that have improved efficiency in use of lighting, heating and cooling; and replaced main passenger elevators with higher-efficiency models.

Improved Space Utilization

In 2023, our global facilities and engineering teams conducted a space utilization assessment. This led to the consolidation of office buildings on our Novato, California, campus and is expected to reduce energy consumption.

Engaging Our Workforce in Sustainability

Engaging our workforce in environmental sustainability is critical to our objective to continue to improve our performance and reduce our environmental footprint. We recognize the grassroots efforts to reduce our environmental impacts and are proud of our workforce, who we believe are environmentally conscious.

In 2023, we launched a My Green Lab certification pilot program at our Translational Sciences laboratory in Novato, California. The My Green Lab Certification is a benchmark for best practices in laboratory sustainability.

This initiative has gained strong support from both lab users and executive leadership, with more than **60%** of lab users completing the baseline survey assessment. A dedicated cross-functional working group plans to implement changes based on the assessment findings throughout 2024. Additionally, the group aims to extend the My Green Lab Certification to more labs in the coming years.

During Ultragenyx's inaugural Days of Service in May 2023, employees in California and Massachusetts supported several environment related volunteer projects. These included gardening, mulching and weeding at Florence Fang Community Farm in San Francisco, as well as waterway cleanup and park beautification in Cambridge and Woburn, Massachusetts.



GTMF Slash the Trash

In April 2023, in honor of Earth Day, members of our workforce at the Gene Therapy Manufacturing Facility (GTMF) hosted a trash cleanup volunteer project in the industrial park surrounding the building.

Managing Waste and Water

We work to comply with applicable federal, state and local requirements for the management of water and hazardous and non-hazardous wastes.

We have procedures in place, along with training and compliance audits, to promote appropriate handling and disposal of our waste streams. We partner with Polycarbin, a company dedicated to circular solutions designed to transform today's laboratory waste into tomorrow's laboratory products, to recycle pipette tip boxes. Polycarbin's mailbox program follows a closed-loop recycling approach, where it receives lab plastics that might otherwise go to landfill or downcycling and remanufactures the waste into new lab products. Through this partnership, Ultragenyx diverted more than **1,300** pounds of plastic waste from landfill since 2022. Furthermore, we have implemented a single-stream recycling program across our sites.

Since 2022

*More than **1,300** pounds of laboratory plastic waste diverted from landfill through our Polycarbin partnership.*

Managing Water

We continue to be conscious of our water footprint. We have upgraded common area toilets and sinks to low-flow models in an effort to conserve water. In the future, we plan to continue to enhance our water stewardship, particularly at facilities located in water-scarce areas in the U.S.

Advancing Sustainability in Our Wastewater Treatment

In 2023, our global Facilities, Engineering and Capital Projects teams, alongside external consultants and industry experts, conducted a review of our wastewater treatment at select East Coast facilities. As a result of the study, we introduced a new chemical decontamination method to treat process and laboratory waste effluent at our lab in Woburn, Massachusetts, and GTMF in Bedford, Massachusetts. These innovative processes replace thermal sanitization, an industry standard practice that is energy intensive. Choosing chemical decontamination allows us to bypass the additional energy consumption and carbon emissions associated with traditional lab practices.

Additionally, our wastewater pretreatment systems in Woburn and Bedford now use sodium hydroxide instead of bleach for decontamination. This switch mitigates the potential for harmful byproducts associated with bleach use and reduces the risk of adverse environmental impacts. We determined that lower volumes of a concentrated sodium hydroxide solution are as effective as bleach in wastewater treatment, thereby minimizing chemical volumes required for the process.



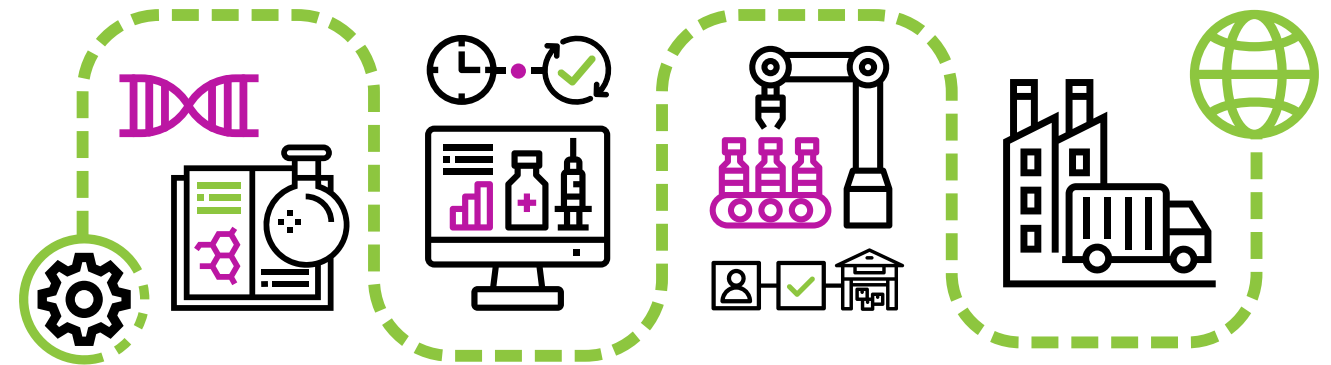
Supply Chain Sustainability

Our commitment to operating in an environmentally responsible manner extends to our supply chain.

We expect our suppliers to follow applicable environmental laws, regulations and standards, such as those concerning chemical and waste management, recycling, industrial wastewater treatment and discharge, air emissions controls, environmental permits and environmental reporting. We expect and encourage our suppliers, wherever possible, to support a proactive approach to environmental matters, undertake initiatives to promote greater environmental responsibility, and encourage environmentally preferable technologies and sound life-cycle practices. These expectations are laid out in our [Global Standard for Suppliers](#). Our teams work closely with internal and external stakeholders to evaluate whether best practices are followed across various steps of our supply chain and whether vendors are able to support our objectives.

Key examples of integrating sustainability within our supply chain include:

- Consolidating small volume orders to help reduce the number of shipments and prioritizing direct-to-patient shipments whenever feasible to eliminate the need for extra transportation and handling.
- Conducting long-term stability studies for our products with the goal to maximize shelf life for our products to provide a reliable supply for patients and reduce disposal needs and associated environmental impacts.
- Balancing shipping and packaging sustainability with patients' needs and industry requirements, such as utilizing sea freight in lieu of air freight whenever possible.



Governance

Maintaining robust corporate governance and risk management and upholding high standards of honest and ethical business conduct

The foundation of our purpose to lead the future of rare disease medicine is built upon our **commitments** to strong corporate governance, ethics and integrity, compliance, data protection and security, and responsible procurement.

Aspiration

Through strong corporate governance and a culture of integrity, we seek to prevent significant issues before they occur and foster an environment where issues can be disclosed without the threat of retaliation.

Our Objectives	2023 Progress
Act responsibly and with integrity and provide annual, targeted training to our workforce on Ultragenyx’s ethical standards	<ul style="list-style-type: none"> • ~90% of survey responders to the annual compliance culture survey stated that the annual compliance training is adequate for them to confidently execute their responsibilities
Maintain a high compliance culture and adherence to all applicable legal requirements	<ul style="list-style-type: none"> • All reported complaints related to potential breaches to our code of conduct and incidents of discrimination or harassment were investigated and promptly addressed, as appropriate
Maintain a high rate of third-party due diligence of our suppliers	<ul style="list-style-type: none"> • Published a formal Global Human Rights policy • Continued efforts to comply with the U.S. Department of Treasury’s OFAC regulations

Corporate Governance

We believe that good corporate governance promotes the long-term interests of our stockholders and other stakeholders. We are committed to maintaining good corporate governance practices and periodically reviewing our practices.

Our [Global Code of Conduct](#) establishes principles and expectations that apply globally to all employees, officers and directors regardless of position or tenure. Our corporate governance guidelines serve as a framework for conducting the board’s business and assist the board in the exercise of its duties and responsibilities to serve the best interests of Ultragenyx and its stockholders.

Board of Directors

Our board of directors provides us with strategic guidance as we work to advance our mission to transform the lives of people with rare disease. Our board comprises experienced leaders who represent a diversity of talents, skills, backgrounds and expertise.

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 setting forth the functions and responsibilities of the committee, a copy of which is available on our [website](#).

As of January 31, 2023, our board consisted of **eight** directors, **seven** of whom are independent.

For more information on our directors and corporate governance, please see our [Proxy Statement](#). See also [Corporate Responsibility Oversight](#).

Board Diversity

- **Three** are women
- **Three** self-identify as a racial or ethnic minority
- **One** self-identifies as LGBTQ+
- Average age of **60.4** years
- Average tenure of **7.4** years

Examples of our commitment to good corporate governance include the application of the following:

Best Practices

- Ongoing shareholder engagement program
- Diverse board, including adoption of “Rooney Rule” for director search process
- Board oversight of sustainability and corporate responsibility matters
- Minimum stock ownership requirements for directors and named executive officers
- More than 90% attendance of board and committee meetings in 2023 by our current directors
- Director Overboarding Policy

Independence

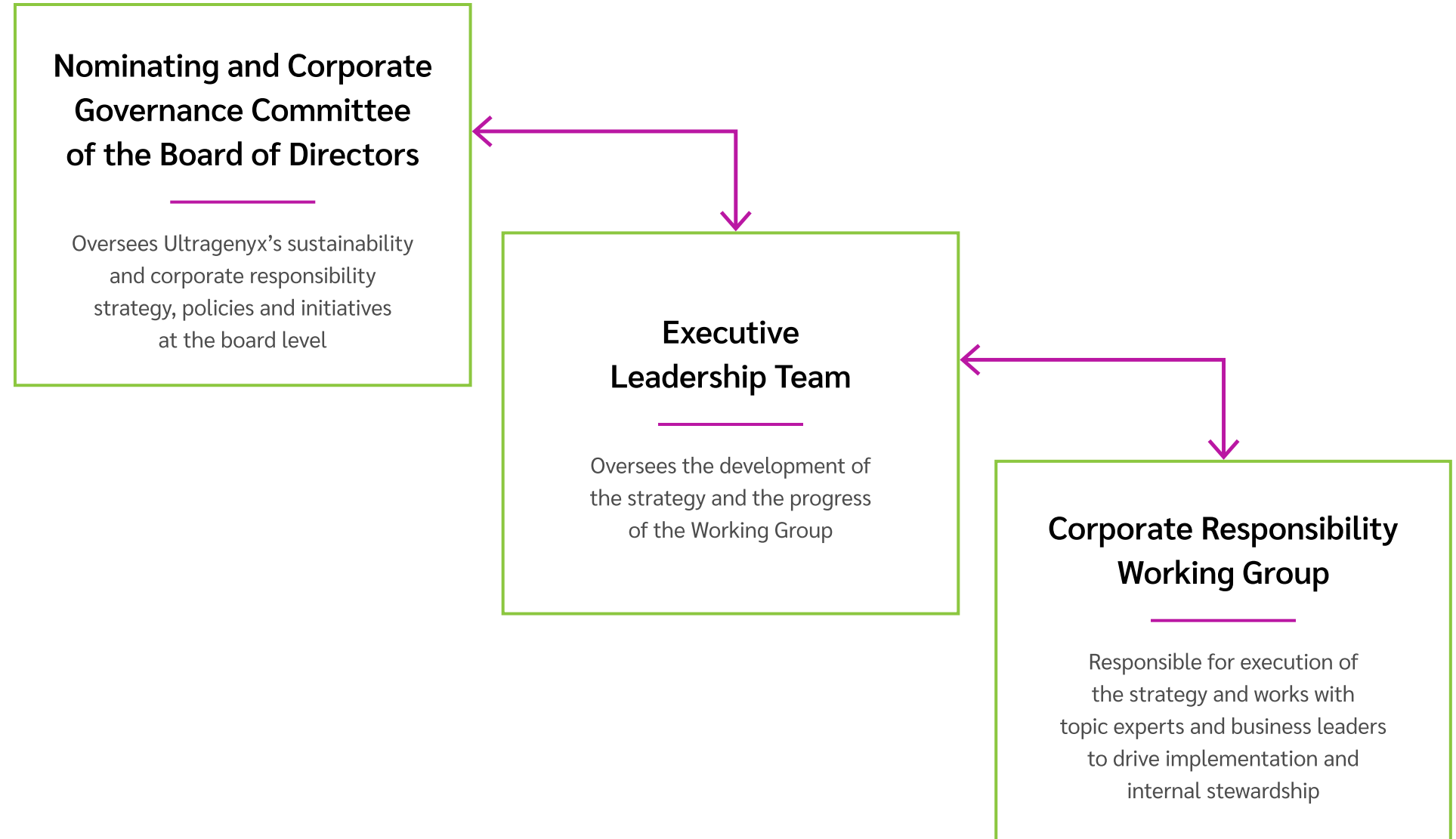
- Active independent board chairman
- All directors are independent except our president and CEO
- 100% independent directors on Audit Committee, Nominating and Corporate Governance Committee, and Compensation Committee

Accountability

- Director Resignation Policy for directors who receive less than majority support in uncontested elections
- Clawback Policy with discretionary recoupment provisions beyond basic legal requirements
- Annual board and committee self-evaluations
- Prohibition against hedging transactions

Corporate Responsibility Oversight

We are committed to integrating corporate responsibility with our overall corporate strategy. This commitment starts at the board of directors' level. The Nominating and Corporate Governance Committee of our board regularly reviews and makes recommendations on sustainability and corporate responsibility matters including policies and initiatives. Furthermore, the Corporate Responsibility Working Group, an executive-sponsored, cross-functionally represented group tasked with advancing our progress, reports to the executive leadership team on our progress.



Risk Management

Ultragenyx's board has overall responsibility for the oversight of the company's risk management process, which is designed to support the achievement of organizational and strategic objectives to improve long-term organizational performance and enhance stockholder value.

The board periodically reviews our business strategy and management's assessment of the related key and emerging risks and discusses with management the appropriate level of risk for the company. In 2023, the board and the committees reviewed with management various risks and mitigation strategies, including those related to:

- Implications from the macroeconomic climate
- The company's initiatives related to corporate responsibility and sustainability matters
- Cybersecurity and security programs related to our information technology systems
- Human capital management, such as employee retention and recruitment
- The continued appropriateness of the company's classified board and other structural elements of the company
- The company's approach to evaluating our clinical and preclinical programs

The board delegates oversight of certain risks to each board committee, and each member of the executive leadership team is responsible for certain risk areas. Executive leadership is responsible for establishing our business strategy, identifying and assessing the related risks, and implementing appropriate risk management practices. For a summary of risks and uncertainties related to our business and operations, see Item 1A Risk Factors in our [2023 Annual Report](#).

Business Continuity Management and Disaster Recovery

Ultragenyx has established the fundamentals of a Business Continuity Management (BCM) and Disaster Recovery (DR) program, guided by best practices from organizations such as the International Organization for Standardization (ISO), including ISO 22301. This program is aimed at supporting organizational resilience against disruptions in business operations. The overarching goal is to continually improve practices and foster the uninterrupted continuation of critical business functions in the face of disruptions.

This program integrates several key elements: program governance for executive oversight and decision-making, business impact and risk assessments for strategy development, and crisis management and communications for coordinating company-level responses. Responsibilities within Ultragenyx are clearly delineated. Leadership roles, including Functional Leaders and Critical Process Owners, are tasked with identifying and safeguarding critical processes under Business Continuity Plans (BCPs).

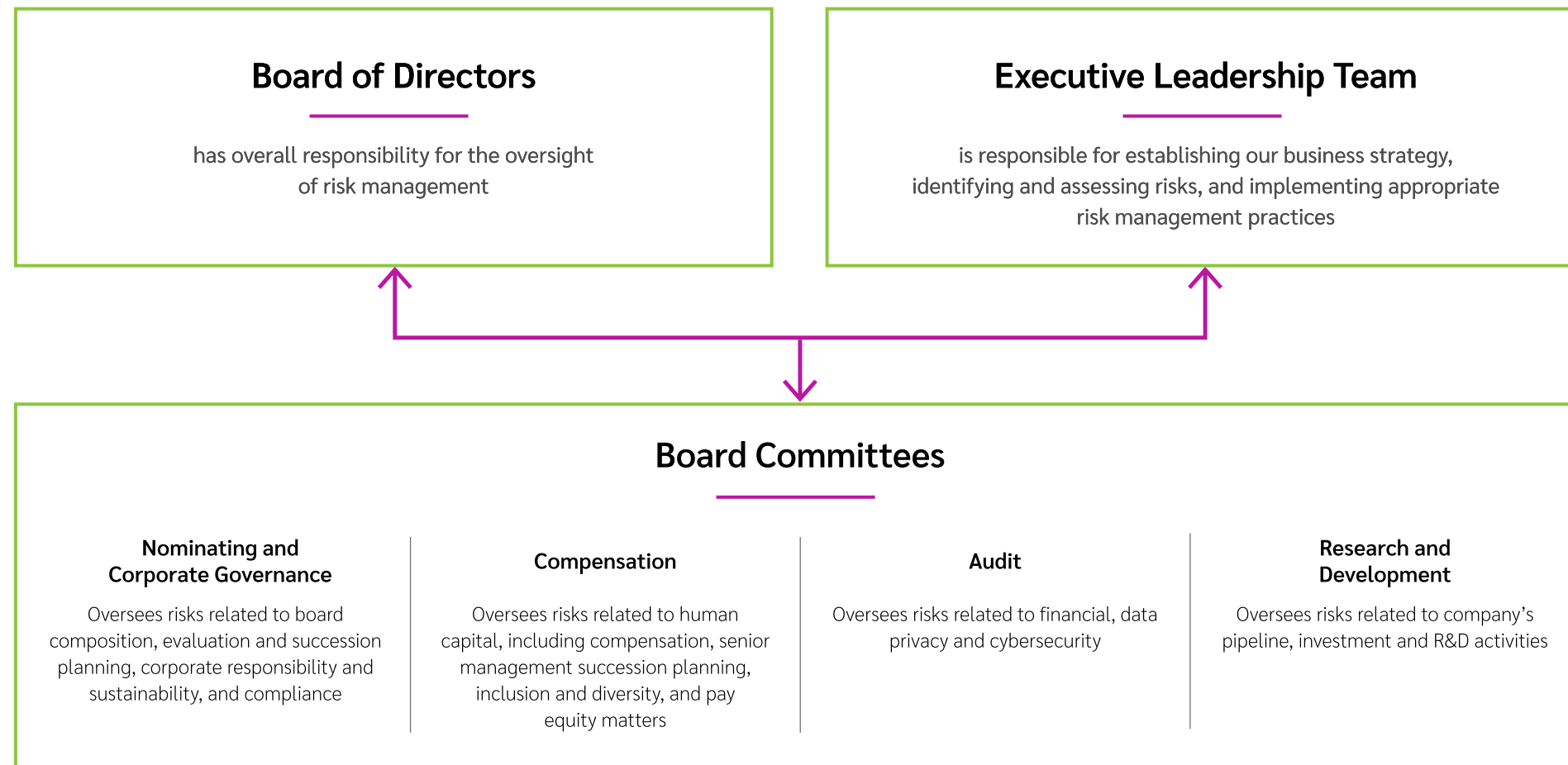
BCPs are annually reviewed and regularly updated to reflect the evolving business environment. The implementation of BCPs is centrally managed through cloud-based software.

Additionally, we have IT Disaster Recovery plans that focus on restoring critical networks and systems essential for maintaining uninterrupted business processes. To validate their effectiveness, we regularly perform and document various recovery tests.

In 2023, we undertook a third-party assessment designed to enhance the maturity of the program. Future potential enhancements under consideration include refining the company's impact analysis, further enhancing the company's recovery and crisis plans, and expanding testing activities.

Risk Management (cont.)

The board of directors, its committees and Ultragenyx’s executive leadership team oversee the company’s risk management program, which includes periodic reporting and open lines of communication.



Ethics and Integrity

Ultragenyx is committed to upholding high standards of honest and ethical business conduct as the foundation upon which we build our reputation.

We expect every director, officer, employee and supplier to adhere to high ethical standards in all business interactions, both within our company and with our customers, business partners, competitors and the communities we partner with and where we operate. We have cultivated a culture and established policies aimed at guiding our employees to do the right thing. The policies are specifically crafted to prevent, deter and identify instances of bribery, fraud and other unethical business practices.

Our code of conduct sets expectations on ethical decision-making and covers a variety of topics, such as equal employment opportunity, anti-discrimination and anti-harassment, anti-bribery and anti-corruption, and anti-trust and competition laws. It also makes clear when and how individuals should raise concerns and documents our no-retaliation policy. We enforce our policies and requirements with appropriate disciplinary actions, when necessary, and we take a zero-tolerance approach to any violation of law or policy.

We work to uphold a high compliance culture by requiring ethical behavior, holding each individual accountable for compliance, fostering effective communication and working together to make good decisions. We work to comply with applicable laws and regulations while maintaining patient safety and leadership accountability. We developed our compliance program in accordance with the laws applicable to our industry, the Program Guidance for Pharmaceutical Manufacturers published by the Office of the Inspector General of the U.S. Department of Health and Human Services, and the PhRMA Code of Interactions with Healthcare Professionals.

Ultragenyx's compliance program includes:

- A compliance officer and compliance committee responsible for developing, operating and monitoring the compliance program and with authority to report directly to the board and our CEO
- Written standards of conduct, policies and practices that document the company's commitment to compliance and requirements to strictly follow fraud and abuse laws
- Easy to understand, effective and readily available education and training programs for all employees
- Open lines of communication and partnership between the compliance officer and workforce to help us ethically achieve our company mission and goals
- Continued enhancement of an audit and monitoring program to identify and address risks
- Enforcement of compliance obligations through guidelines that include disciplinary action for noncompliance
- Mechanisms to investigate and respond promptly and properly to reports of noncompliance, including processes to initiate corrective measures

In 2023

*Ultragenyx did **not** have any material monetary losses as a result of legal proceedings associated with corruption and bribery*

(please refer to our [2023 Annual Report](#)).

Ethics and Integrity (cont.)

We consistently perform a range of monitoring and auditing activities across business operations, in collaboration with our internal audit, finance and legal teams. We use technology and automated tools to monitor and report on compliance matters. An annual survey is also distributed to assess our compliance culture. In 2023, approximately **90%** of survey respondents indicated that the annual compliance training sufficiently prepares them to confidently execute their responsibilities.

The company reinforces adherence to the code of conduct's expectations by providing employees with training on anti-bribery and anti-corruption, conflicts of interest, insider training, anti-harassment, and data protection and privacy, among other areas. These trainings, along with our policies and procedures, outline the expected conduct for day-to-

day responsibilities. All full-time, U.S.-based employees must acknowledge our anti-harassment and anti-discrimination policy and complete the company-offered training within the first several months of employment. All U.S. employees, including part-time and temporary employees, must take a harassment prevention training course every year. Additionally, on an annual basis, employees are expected to receive and acknowledge understanding of the code of conduct. Every year, an annual compliance culture survey is sent to all employees to gauge our compliance culture. In 2023, **99%** of full-time employees have received training on the code of conduct and other ethical standards within the last three years and **97%** of full-time employees provided written or digital acknowledgment of the code of conduct in 2023.

Employees have an obligation to report any conduct that they, in good faith, believe violates laws, corporate policies, and/or the code of conduct. Various avenues are available to seek advice on ethical behavior and report concerns related to violations of such behavior, and we have a strict no-retaliation policy for individuals who raise concerns in good faith.



Ethics and Integrity (cont.)

Raising A Concern

Ultragenyx's compliance hotline, which also serves as the Confidential and Anonymous Financial Concern Hotline, allows employees or anyone else to report any potential or actual violation of our code of conduct, company policies and procedures, and applicable laws and regulations. Any individual can provide comments using the hotline. Messages can be submitted anonymously using a secure web form, email or telephone. Additionally, a strict non-retaliation policy ensures individuals can report concerns without fear of reprisal or discrimination, promoting an environment of openness and trust.

Complaints or other messages left on the compliance hotline are anonymously sent to our chief legal officer, head of compliance and the chairperson of our Audit Committee, who then take the necessary next steps. All hotline reports

are required to be promptly handled, and any identified issues are required to be addressed. If an employee makes a complaint of discrimination or harassment, regardless of where the complaint is made, Ultragenyx's policy is to conduct a timely and thorough investigation and take appropriate action. Investigations are required to be assessed and conducted based on Ultragenyx's internal investigations protocol and are required to be conducted by the appropriate personnel depending on the issue. If and when issues arise, we are required to identify root causes and, in a timely and efficient manner, implement measures to stop repeat occurrences. In 2023, all reported complaints related to potential breaches to our code of conduct and incidents of discrimination or harassment were investigated and promptly addressed as appropriate.

Ethical Treatment of Animals

Ultragenyx is committed to the ethical treatment of animals used in the development of potential new and life-changing therapies for patients with rare disease.

Our company is committed to the "3Rs:"

Replace: Use non-animal methods for experiments whenever possible, such as simulations and computational tools or in vitro systems

Reduce: Use the minimum number of animals in each study in order to achieve valid results and objectives

Refine: Use procedures that decrease the potential for pain and limit distress for animals

We expect external service providers to meet or exceed all animal care and use standards that are applicable to local and national laws and regulations. Our external service providers are accredited by the American Association for Accreditation of Laboratory Animal Care (AAALAC), a private, nonprofit organization that promotes the humane treatment of animals in science through voluntary accreditation and assessment programs. Additionally, animal facilities are governed by an Institutional Animal Care and Use Committee (IACUC), which oversees all aspects of animal care, welfare and scientific programs for research. The IACUC reviews all animal use protocols, oversees compliance with federal regulations, inspects animal facilities, and manages animal handling/training and educational programs.

Interactions with Patients, Caregivers and Healthcare Professionals

We respect the doctor-patient relationship and the privacy rights of patients. We strive to interact with patients and caregivers in an appropriate manner and in compliance with applicable laws, regulations and our internal Healthcare Compliance Manual.

Ultragenyx has adopted policies and practices consistent with the PhRMA Code and other applicable industry standards that govern interactions with healthcare professionals. These policies encompass support for medical education and collaboration with healthcare professionals who provide services to our company as researchers, consultants and speakers. Additionally, the policies include provisions for business courtesies, grants and charitable contributions that state that such funds are not conditioned, either expressly or implicitly, on any agreements to prescribe, purchase, recommend, influence or provide favorable formulary status for any Ultragenyx medicine. The policies also cover provisions related to the promotion of Ultragenyx medicines in compliance with the FDA's regulatory framework, as well as with regulatory requirements in other jurisdictions regarding the promotion of pharmaceutical products.

Our Healthcare Compliance Manual outlines key principles related to our ethical marketing and sales practices:

- Interactions with customers focus on education about the benefits and risks of our products to promote their appropriate use. These interactions must occur in venues conducive to education, and Ultragenyx prohibits the provision of entertainment to its customers.
- Promotional communications must be truthful, not misleading, and fairly balanced with appropriate safety information. They must also be consistent with the medicine's label.
- Employees are prohibited from using items of value or in-kind services to reward or induce a healthcare professional to utilize, prescribe, purchase or recommend our medicines.
- The hiring of healthcare professionals as speakers or consultants must be based on a legitimate business need, free from inappropriate influences. Any fees paid to healthcare professionals must not exceed the fair market value of the service provided.

Our Healthcare Compliance Manual is available in **English, Spanish, Portuguese, French and German**. Our compliance program is overseen by a management-level compliance committee that provides guidance with respect to healthcare law compliance. The committee meets at least quarterly.

Ultragenyx is committed to meeting all U.S. state and federal reporting requirements including the Open Payments Report, commonly known as the Sunshine Act, as well as other applicable global transparency reporting requirements. Our disclosure of payments to the Sunshine Act can be found on the [Centers for Medicare & Medicaid Services \(CMS\) open payments website](#).

Data Privacy

The rightful collection and utilization of personal information from diverse sources – including patients, clinical trial participants, customers, healthcare providers and our employees – are integral to our operations.

We are dedicated to protecting the privacy and integrity of this information and related holders by adhering to global privacy laws. This commitment is upheld through our data privacy program, which encompasses global privacy policies, comprehensive training, and system operating procedures and programmatic controls. Our proactive approach to privacy demonstrates our unwavering commitment to high standards of data security and compliance, thereby safeguarding the trust of our stakeholders and maintaining the integrity of our business practices.

Our chief information officer (CIO), in partnership with our data privacy officer (DPO) and the rest of our Legal and Compliance departments, oversee and manage our approach to privacy-related matters, as they relate to Ultragenyx, third party data and technological and cyber security platforms.

We follow all applicable data protection laws, regulations and best practices, including the General Data Protection Regulation (GDPR), California Consumer Privacy Act (CCPA) and Lei Geral de Proteção de Dados (LGPD), among others.

- Our [privacy policies](#) set forth the practices of Ultragenyx regarding the collection, use, retention and disclosure of personal information in connection with all corporate activities.
- Our DPO collaborates across the business with the goal of ensuring that any data shared internally is with the right functions and for the right reasons.
- Our information technology team, in collaboration with the DPO, and their respective teams are responsible for evaluating the company's software programs and applications for data privacy compliance. This review includes, among other considerations, a determination of whether personal information will be transmitted with the goal of ensuring that all new and existing critical applications are managed in accordance with applicable laws.
- We have a standardized process for responding to data subject requests regarding their data that Ultragenyx processes (see [Data Protection, Anonymization and Security](#) in this report for more information).
- We expect our workforce to be accountable, to protect personal data – which we may have access to during the ordinary course of our business operations – and to process such data responsibly in accordance with company policies and any applicable laws. All employees receive periodic training on privacy as part of our annual code of conduct training.

In 2023

*Ultragenyx did **not** have any material data privacy breaches.*

Cybersecurity

Ultragenyx has an information security program with policies and procedures designed to guide our security and data protection decision-making process.

In 2023

98% of employees received IT-related training

Governance	Training and Awareness	Business Resilience and Compliance	Identifying and Mitigating Cybersecurity Risks
<ul style="list-style-type: none"> Board-level oversight is assigned to our Audit Committee. The CIO regularly reports to the Audit Committee to provide an overview of the risks, processes, procedures, recommendations and an overall assessment of our cybersecurity program. Strategic and tactical oversight and direction for cybersecurity-related matters (cyber program strategy, program deficiencies and maturation, program and process review and testing, training and awareness, and reporting) are led by our head of information security, who reports directly to the CIO. Our cybersecurity approach is informed by the NIST 800-53 standard and compliance framework. Our team includes members who are certified with qualifications including CISSP, CRISC and CISA, and are proficient in the management of cybersecurity protocols and practices. We have established a partnership between the DPO and CIO to implement proper controls for data protection, use, storage and retention that are designed to confirm our technological solutions meet legal and regulatory requirements. 	<ul style="list-style-type: none"> We provide information security training to employees at a minimum of twice per year. The same training is provided to members of our contingent workforce who have access to our internal systems and can be a risk to our information technology infrastructure. We implement additional training, as needed. We conduct phishing exercises at least three times per year, focusing on users with repeated simulation failures and implementing corrective actions. We perform additional testing, as needed. Our Acceptable Use Policy (AUP) was updated to include generative AI usage and included in our training curriculum. We issue regular employee awareness communications, including email newsletters. These communications are intended to consistently inform users about the escalation process to follow if they notice anything suspicious and remind them to report any security incidents to our security email address. 	<ul style="list-style-type: none"> We have documented the appropriate disaster recovery plans for all Ultragenyx critical systems. We also have documented our incident response plan’s process and have performed tabletop exercises annually. We updated our critical vendors disaster recovery plans for our information technology systems. We purchase a fixed amount of cybersecurity and crime insurance coverage to help mitigate some of the risk and potential liability from cybersecurity breaches, including claims related to data privacy regulatory matters, third-party lawsuits and the costs associated with data breach events. We require that new and existing systems are built to comply, and remain in compliance with regulatory requirements, including SOX and GxP programs. 	<ul style="list-style-type: none"> We have ongoing managed vulnerability scanning and patching through our vulnerability management program. We monitor internal and external cybersecurity threats and review and revise our cybersecurity defenses on a regular cadence. Targeted audits and penetration tests are conducted throughout the year by internal and external entities. In 2023, we conducted our annual third-party internal and external penetration testing. The third-party also performed vulnerability testing and simulated various types of attacks to exploit any weakness discovered. We document findings and execute remediation plans. Our security team also performs various vulnerability scanning throughout the year. In 2023, we continued to onboard and inventoried all new systems in a central asset management tool and monitor and update operating procedures. We operationalized cloud security, endpoint data loss prevention, and privileged access management solutions. We onboarded a governance, risk and compliance solution as well as a risk monitoring service to monitor Ultragenyx security performance, as well as that of our vendors.

Responsible Supply Chain Management

We understand the critical role of responsible supply chain management in achieving our mission.

In 2022, we introduced the [Global Standard for Suppliers](#) to complement and supplement the [Global Code of Conduct](#). This standard sets forth expectations for our suppliers in areas including integrity, legal compliance, labor standards, human rights and environmental stewardship. In 2023, we published a formal [Global Human Rights policy](#), which covers extensive areas, such as labor practices, clinical trials, product governance and supply chain ethics, and aims to address potential human rights issues.

Our approach to responsible procurement includes our expectation that suppliers uphold high ethical standards. Ultragenyx maintains a zero-tolerance stance on modern slavery in any form. We expect all suppliers to comply with all relevant laws. We transparently communicate our efforts against modern slavery in our statement on [Transparency in our Supply Chain and Modern Slavery](#).

Central to our success in developing therapies to treat rare and ultrarare diseases is the establishment of strategic, long-term partnerships with suppliers that share our values. Our supplier engagement process encompasses legal and compliance risk screening, including screening for economic sanctions and anti-bribery and anti-corruption activities, and is designed to align with our requirements and company policy. All of our strategic manufacturers are currently located in North America, Western Europe and Japan. Part of our audit process involves the integration of corporate responsibility criteria, which allows us to consider the third-party's commitments in our evaluation of potential partners.

Our supplier quality management system plays a pivotal role in our supply chain management. It allows us to more effectively monitor supplier quality and supports integration with future partners. High-risk suppliers undergo formal

assessments and audits designed to verify compliance with applicable laws and regulations.

Finally, our key contracts stipulate that suppliers be open to audits assessing compliance. Post-audit, we endeavor to engage collaboratively with suppliers to develop and follow through on action plans. Additionally, we require compliance with the U.S. Department of Treasury's OFAC regulations before financially transacting with any contracted third party.

These measures reflect Ultragenyx's commitment to fostering a supply chain that is aligned with our core values and the unique needs of the rare disease community.

SASB Index

The following index lists the activity and accounting metrics from the Sustainability Accounting Standards Board (SASB) Biotechnology and Pharmaceuticals Industry Standard (2018) with associated response, reference or report location.

Accounting Metric	Code	Response / Reference / Report Location
Number of patients treated	HC-BP-000.A	See our 2023 Corporate Responsibility Report, Access and Affordability .
Number of drugs 1) in portfolio and 2) in research and development (Phases 1-3)	HC-BP-000.B	See our current Pipeline .

Topic	Accounting Metric	Code	Response / Reference / Report Location
Safety of Clinical Trial Participants	Discussion, by world region, of management process for ensuring quality and patient safety during clinical trials	HC-BP-210a.1	See our 2023 Corporate Responsibility Report, Clinical Trials , Quality and Safety .
	Number of FDA Sponsor Inspections related to clinical trial management and pharmacovigilance that resulted in: (1) Voluntary Action Indicated (VAI) and (2) Official Action Indicated (OAI)	HC-BP-210a.2	There were no FDA Sponsor Inspections related to clinical trial management and pharmacovigilance that resulted in either Voluntary Action Indicated (VAI) or Official Action Indicated (OAI) in 2023. See the FDA Compliance Dashboard for more information.
	Total amount of monetary losses as a result of legal proceedings associated with clinical trials in developing countries	HC-BP-210a.3	There have been no reported monetary losses as a result of legal proceedings associated with clinical trials in developing countries for the year 2023. See our 2023 Annual Report , page 67 (Item 3. Legal Proceedings).
Access to Medicines	Description of actions and initiatives to promote access to health care products for priority diseases and in priority countries as defined by the Access to Medicine Index	HC-BP-240a.1	Our products and clinical research primarily target rare and ultrarare diseases, which fall outside the 2023 Access to Medicine Index's main focus areas. Our investments in R&D, strategic partnerships, and innovative technologies are all aimed at enhancing treatment accessibility and affordability. Additionally, through patient assistance programs and expanded use initiatives, we support patients globally, especially in economically disadvantaged countries. We also provide health-related grants that support organizations focused on medical research and treatment, and rare disease awareness, education and advocacy globally. See our 2023 Corporate Responsibility Report, Access and Affordability , Rare Disease Community Support and Grants .
	List of products on the WHO List of Prequalified Medicinal Products as part of its Prequalification of Medicines Programme (PQP)	HC-BP-240a.2	Ultragenyx products are not on the WHO list due to our focus on rare and ultra-rare diseases, while the WHO prioritizes conditions such as HIV/AIDS, tuberculosis, malaria, and reproductive health. See the WHO Prequalification of Medical Products for more information.

SASB Index (cont.)

Topic	Accounting Metric	Code	Response / Reference / Report Location
Affordability & Pricing	Number of settlements of Abbreviated New Drug Application (ANDA) litigation that involved payments and/or provisions to delay bringing an authorized generic product to market for a defined time period	HC-BP-240b.1	See our 2023 Annual Report , pages 19-23 (Item 1. Business - Patents and Proprietary Rights).
	Percentage change in: (1) average list price and (2) average net price across U.S. product portfolio compared to previous year	HC-BP-240b.2	See our 2023 Corporate Responsibility Report, Access and Affordability .
	Percentage change in: (1) list price and (2) net price of product with largest increase compared to previous year	HC-BP-240b.3	See our 2023 Corporate Responsibility Report, Access and Affordability .
Drug Safety	List of products listed in the Food and Drug Administration's (FDA) MedWatch Safety Alerts for Human Medical Products database	HC-BP-250a.1	Our products are not listed in the FDA MedWatch. See the FDA FAERS MedWatch website for more information.
	Number of fatalities associated with products as reported in the FDA Adverse Event Reporting System	HC-BP-250a.2	There no fatalities associated with Ultragenyx products in 2023. See the FDA FAERS MedWatch website for more information.
	Number of recalls issued, total units recalled	HC-BP-250a.3	Ultragenyx did not issue any recalls. See the FDA Data Dashboard for more information.
	Total amount of product accepted for takeback, reuse, or disposal	HC-BP-250a.4	Ultragenyx manufactures medicines on a schedule that is designed to avoid their expiration before patient use. In the event that medicines expire before use and are returned, are found to be unsuitable for release or are subject to a recall or withdrawal notice, Ultragenyx does not reintroduce them again for reuse. They get disposed of using regulated and monitored incineration processes.
	Number of FDA enforcement actions taken in response to violations of current Good Manufacturing Practices (cGMP), by type	HC-BP-250a.5	Ultragenyx was not involved in any FDA enforcement actions in response to violations of cGMP. See the FDA Data Dashboard for more information.

SASB Index (cont.)

Topic	Accounting Metric	Code	Response / Reference / Report Location
Counterfeit Drugs	Description of methods and technologies used to maintain traceability of products throughout the supply chain and prevent counterfeiting	HC-BP-260a.1	Ultragenyx has implemented a comprehensive process designed to address counterfeit product risks, including a Field Action procedure and security features like tamper-evident seals and serialization of product labeling. See our 2023 Corporate Responsibility Report, Quality in Supply Chain , section on Counterfeit Drugs.
	Discussion of process for alerting customers and business partners of potential or known risks associated with counterfeit products	HC-BP-260a.2	Ultragenyx has implemented a comprehensive process designed to address counterfeit product risks, including a Field Action procedure and security features like tamper-evident seals and serialization of product labeling. See our 2023 Corporate Responsibility Report, Quality in Supply Chain , section on Counterfeit Drugs.
	Number of actions that led to raids, seizure, arrests, and/or filing of criminal charges related to counterfeit products	HC-BP-260a.3	Ultragenyx had no instances of actions related to counterfeit products in 2023. See our 2023 Corporate Responsibility Report, Quality in Supply Chain , section on Counterfeit Drugs.
Ethical Marketing	Total amount of monetary losses as a result of legal proceedings associated with false marketing claims	HC-BP-270a.1	There have been no reported monetary losses as a result of legal proceedings associated with false marketing claims for the year 2023. See our 2023 Annual Report , page 67 (Item 3. Legal Proceedings).
	Description of code of ethics governing promotion of off-label use of products	HC-BP-270a.2	See our Global Code of Conduct .
Employee Recruitment, Development & Retention	Discussion of talent recruitment and retention efforts for scientists and research and development personnel	HC-BP-330a.1	See our 2023 Corporate Responsibility Report, People chapter.
	(1) Voluntary and (2) involuntary turnover rate for: (a) executives/senior managers, (b) midlevel managers, (c) professionals, and (d) all others	HC-BP-330a.2	See our 2023 Corporate Responsibility Report, Human Capital Development .
Supply Chain Management	Percentage of (1) entity's facilities and (2) Tier I suppliers' facilities participating in the Rx-360 International Pharmaceutical Supply Chain Consortium audit program or equivalent third-party audit programs for integrity of supply chain and ingredients	HC-BP-430a.1	See our 2023 Corporate Responsibility Report, Quality in Supply Chain .
Business Ethics	Total amount of monetary losses as a result of legal proceedings associated with corruption and bribery	HC-BP-510a.1	There have been no reported monetary losses as a result of legal proceedings associated with corruption and bribery for the year 2023. See our 2023 Annual Report , page 67 (Item 3. Legal Proceedings).
	Description of code of ethics governing interactions with health care professionals	HC-BP-510a.2	See our 2023 Corporate Responsibility Report, Interactions with Patients, Caregivers and Healthcare Professionals .

*The SASB metrics are referenced above for informational purposes only with no claim of fulfillment to any given metric.

Global Reporting Initiative (GRI) Index

This index is aligned with the Global Reporting Initiative's Sustainability Reporting Standards. It provides easy access to Core reporting elements and, where available, additional Comprehensive reporting level elements for the period January 1 through December 31, 2023, unless otherwise noted. GRI 1 used: GRI: Foundation 2021.

GRI Standard	Disclosure	2023 Location
GRI 2: General Disclosures 2021	2-1 Organizational details	2023 Corporate Responsibility Report, About Us and 2023 Annual Report , Item 1. Business.
	2-2 Entities included in the organization's sustainability reporting	The 2023 Corporate Responsibility Report mainly covers information from the fiscal year ending December 31, 2023, unless otherwise indicated. Questions and inquiries on the reported information can be submitted to cr@ultragenyx.com . 2023 Corporate Responsibility Report, About This Report .
	2-3 Reporting period, frequency and contact point	Ultragenyx publishes a corporate responsibility report annually. 2023 Corporate Responsibility Report, About This Report .
	2-4 Restatements of information	No restatements of information were made.
	2-5 External assurance	No external assurance was performed.
	2-6 Activities, value chain and other business relationships	2023 Annual Report , Item 1. Business.
	2-7 Employees	2023 Corporate Responsibility Report, Workforce Data .
	2-8 Workers who are not employees	Information unavailable.
	2-9 Governance structure and composition	2023 Corporate Responsibility Report, Corporate Governance . 2023 Corporate Responsibility Report, Corporate Responsibility Oversight .
	2-10 Nomination and selection of the highest governance body	2024 Proxy , Nomination of Directors.

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 2: General Disclosures 2021 (cont.)	2-11 Chair of the highest governance body	Daniel G. Welch, Chairperson of the Board. Ultragenyx's Leadership .
	2-12 Role of the highest governance body in overseeing the management of impacts	2023 Corporate Responsibility Report, Corporate Responsibility Oversight . Board Committees and Charters .
	2-13 Delegation of responsibility for managing impacts	2023 Corporate Responsibility Report, Corporate Responsibility Oversight .
	2-14 Role of the highest governance body in sustainability reporting	2023 Corporate Responsibility Report, Corporate Responsibility Oversight .
	2-15 Conflicts of interest	2023 Corporate Responsibility Report, Corporate Governance . Corporate Governance Guidelines .
	2-16 Communication of critical concerns	2023 Corporate Responsibility Report, Risk Management . 2023 Annual Report , Item 1A. Risk Factors.
	2-17 Collective knowledge of the highest governance body	2024 Proxy , Board Diversity, Skills and Experience.
	2-18 Evaluation of the performance of the highest governance body	Corporate Governance Guidelines .
	2-19 Remuneration policies	2024 Proxy , Executive Compensation. 2024 Proxy , Director Compensation.
	2-20 Process to determine remuneration	2024 Proxy , Executive Compensation. 2024 Proxy , Director Compensation.
	2-21 Annual total compensation ratio	2024 Proxy , CEO Pay Ratio.

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 2: General Disclosures 2021 (cont.)	2-22 Statement on sustainable development strategy	2023 Corporate Responsibility Report, Letter From Our CEO . 2023 Corporate Responsibility Report, Corporate Responsibility Approach .
	2-23 Policy commitments	2023 Corporate Responsibility Report, Governance chapter Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-24 Embedding policy commitments	2023 Corporate Responsibility Report, Governance chapter Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-25 Processes to remediate negative impacts	2023 Corporate Responsibility Report, Governance chapter Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-26 Mechanisms for seeking advice and raising concerns	2023 Corporate Responsibility Report, Raising A Concern Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-27 Compliance with laws and regulations	2023 Corporate Responsibility Report, Ethics and Integrity 2023 Annual Report , Item 3 Legal Proceedings.
	2-28 Membership associations	2023 Corporate Responsibility Report, Strategic Collaborations 2023 Corporate Responsibility Report, Public Policy Participation . 2023 Corporate Responsibility Report, Patient Advocacy and Engagement .
	2-29 Approach to stakeholder engagement	2023 Corporate Responsibility Report, Corporate Responsibility Approach .
	2-30 Collective bargaining agreements	2023 Annual Report , Item 1. Business – Human Capital.
GRI 3: Material Topics 2021	3-1 Process to determine material topics	2023 Corporate Responsibility Report, Materiality Assessment .
	3-2 List of material topics	2023 Corporate Responsibility Report, Materiality Assessment .
	3-3 Management of material topics	The 2023 Corporate Responsibility Report describes the management of material topics by section. 2023 Annual Report , Item 1A. Risk Factors.

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 201: Economic Performance 2016	201-1 Direct economic value generated and distributed	2023 Annual Report , Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations.
	201-2 Financial implications and other risks and opportunities due to climate change	2023 Corporate Responsibility Report, Reducing Our Environmental Impacts .
	201-3 Defined benefit plan obligations and other retirement plans	2023 Annual Report , Item 15. Exhibits and Financial Statement Schedules.
GRI 203: Indirect Economic Impacts 2016	203-1 Infrastructure investments and services supported	2023 Corporate Responsibility Report, Access and Affordability . 2023 Corporate Responsibility Report, Communities chapter .
	203-2 Significant indirect economic impacts	2023 Corporate Responsibility Report, Access and Affordability . 2023 Corporate Responsibility Report, Communities chapter .
GRI 205: Anti-corruption 2016	205-1 Operations assessed for risks related to corruption	2023 Annual Report , Item 1A. Risk Factors. 2023 Corporate Responsibility Report, Responsible Supply Chain Management . Global Code of Conduct . Global Standard for Suppliers .
	205-2 Communication and training about anti-corruption policies and procedures	2023 Corporate Responsibility Report, Ethics and Integrity . Global Code of Conduct . Global Standard for Suppliers .
	205-3 Confirmed incidents of corruption and actions taken	2023 Corporate Responsibility Report, Ethics and Integrity .
GRI 206: Anti-competitive Behavior 2016	206-1 Legal actions for anti-competitive behavior, anti-trust, and monopoly practices	2023 Annual Report , Item 3 Legal Proceedings.
GRI 303: Water and Effluents 2018	303-1 Interactions with water as a shared resource	2023 Corporate Responsibility Report, Managing Waste and Water .
GRI 305: Emissions 2016	305-5 Reduction of GHG emissions	2023 Corporate Responsibility Report, Reducing Environmental Impacts .
GRI 306: Waste 2020	306-2 Management of significant waste-related impacts	2023 Corporate Responsibility Report, Managing Waste and Water .

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 401: Employment 2016	401-1 New employee hires and employee turnover	2023 Corporate Responsibility Report, Human Capital Development . 2023 Corporate Responsibility Report, DEIB in Recruitment .
	401-2 Benefits provided to full-time employees that are not provided to temporary or part-time employees	2023 Corporate Responsibility Report, Employee Compensation and Benefits . Benefits .
	401-3 Parental leave	2023 Corporate Responsibility Report, Employee Compensation and Benefits . Benefits .
GRI 403: Occupational Health and Safety 2018	403-1 Occupational health and safety management system	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-2 Hazard identification, risk assessment, and incident investigation	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-4 Worker participation, consultation, and communication on occupational health and safety	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-5 Worker training on occupational health and safety	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-6 Promotion of worker health	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-8 Workers covered by an occupational health and safety management system	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-9 Work-related injuries	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .
	403-10 Work-related ill health	2023 Corporate Responsibility Report, Occupational Health, Safety and Wellness .

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 404: Training and Education 2016	404-1 Average hours of training per year per employee	2023 Corporate Responsibility Report, Employee Learning and Development .
	404-2 Programs for upgrading employee skills and transition assistance programs	2023 Corporate Responsibility Report, Employee Learning and Development . 2023 Corporate Responsibility Report, Career Development .
	404-3 Percentage of employees receiving regular performance and career development reviews	2023 Corporate Responsibility Report, UltraPerformance Management .
GRI 405: Diversity and Equal Opportunity 2016	405-1 Diversity of governance bodies and employees	2023 Corporate Responsibility Report, Workforce data (overall diversity data). 2023 Corporate Responsibility Report, Board Diversity (board diversity).
GRI 406: Non-discrimination 2016	406-1 Incidents of discrimination and corrective actions taken	2023 Corporate Responsibility Report, Raising A Concern .
GRI 408: Child Labor 2016	408-1 Child Labor 2016 408-1 Operations and suppliers at significant risk of incidents of child labor	2023 Corporate Responsibility Report, Responsible Supply Chain Management Global Code of Conduct . Global Standard for Suppliers .
GRI 409: Forced or Compulsory Labor 2016	409-1 Operations and suppliers at significant risk for incidents of forced or compulsory labor	2023 Corporate Responsibility Report, Responsible Supply Chain Management Global Code of Conduct . Global Standard for Suppliers .
GRI 413: Local Communities 2016	413-1 Operations with local community engagement, impact assessments, and development programs	2023 Corporate Responsibility Report, Communities chapter . Includes information on local community engagement, including corporate philanthropy and volunteering.
GRI 415: Public Policy 2016	415-1 Political Contributions	2023 Corporate Responsibility Report, Public Policy Participation .

GRI Index (cont.)

GRI Standard	Disclosure	2023 Location
GRI 416: Customer Health and Safety 2016	416-1 Assessment of the health and safety impacts of product and service categories	2023 Corporate Responsibility Report, Safety .
	416-2 Incidents of non-compliance concerning the health and safety impacts of products and services	2023 Corporate Responsibility Report, Quality . 2023 Corporate Responsibility Report, Safety . FDA Data Dashboard .
GRI 417: Marketing and Labeling 2016	417-1 Requirements for product and service information and labeling	2023 Annual Report , Item 1. Business. Crysvita . Mepsevii . Dojolvi . Evkeeza .
	417-2 Incidents of non-compliance concerning product and service information and labeling	2023 Annual Report , Item 1A. Risk Factors.
	417-3 Incidents of non-compliance concerning marketing communications	2023 Corporate Responsibility Report, Interactions with Patients, Caregivers and Healthcare Professionals . 2023 Annual Report , Item 1A. Risk Factors.
GRI 418: Customer Privacy 2016	418-1 Substantiated complaints concerning breaches of customer privacy and losses of customer data	2023 Corporate Responsibility Report, Data Privacy .



2023 Corporate Responsibility Report

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