2024 Ultragenyx Impact Report

Going beyond every day.®

ultragenyx

The Weinberg Family's Story

Conner lives with Angelman syndrome, a rare genetic disorder. His mother, Ashley, along with their family has helped build an incredible support network across the Angelman syndrome community and use their learnings to help others facing similar journeys.

Learn more about Ashley and Conner

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 and are not intended to create legal rights or obligations. Forward-looking statements inherently involve risks and uncertainties that are often beyond our control, difficult to predict, and could cause actual results to differ materially from those predicted in such statements including changes in economic conditions, slowed or insufficient technological developments, stakeholder engagement, changes in corporate strategy, and changes in the legal or regulatory environment. 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, assumptions that are subject to change in the future, and certifications, representations or data reviewed or provided by third parties. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 19, 2025, and its subsequent periodic reports filed with the SEC. Forward-looking statements are aspirational and are not guarantees or promises that goals or targets will be met. Ultragenyx undertakes no obligation to update any forward-looking or other statements, whether as a result of new information, future events, or otherwise, and notwithstanding any historical practice of doing so. Ultragenyx may determine to adjust any goals and targets or establish new ones to reflect changes in our business. The information included in, and any issues identified as material for purposes of, this document is not an indication that they are considered material to Ultragenyx, our investors, or other stakeholders, or required to be disclosed in our filings, in each case under SEC reporting or any other laws or requirements that may apply to Ultragenyx. In the context of this report, the term "material" is distinct from, and should not be confused with, such term as defined for SEC or other mandatory reporting purposes. Historical clinical trial success rates are not necessarily predictive, and should not be considered a guarantee, of future success rates. Website references and hyperlinks throughout this document are provided for convenience only, and the content on the referenced third-party websites is not incorporated by reference into this report nor does it constitute a part of this report. Ultragenyx assumes no liability for the content contained on the referenced third-party references.

This report is intended to highlight some of Ultragenyx's corporate responsibility efforts during the fiscal year ended December 31, 2024; it is not a comprehensive description or representation of all of our corporate responsibility activities during that time.

This document and the materials or websites cross-referenced include certain statistical information and estimates relating to rare diseases that are based on publications of independent sources. Ultragenyx believes these third-party sources to be reputable but has not independently verified the underlying data sources, methodologies or assumptions. The third-party sources referenced are generally available to the public and were not commissioned by Ultragenyx. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties, and actual events or circumstances may differ materially from events and circumstances reflected in this information.

About This Report

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

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 impact report. This 2024 report contains disclosures for the period January 1 through December 31, 2024, 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 and operations by subsidiaries which are not wholly owned by Ultragenyx are not covered.

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.

Contact

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

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A Letter From Our CEO

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

Founder, President and CEO

Dear Stakeholders,

Since founding Ultragenyx 15 years ago, I've sought to build a next-generation rare disease company that has a meaningful and sustained impact on the entire rare and ultrarare disease community. That has entailed not only engineering our own dynamic approach to drug development but also working on broader education and policy initiatives that meaningfully support diagnosis, drug development, regulatory pathways and clinical care. These efforts have been a part of our DNA from the start. For that reason, this year we are changing the name of our corporate responsibility and sustainability report to the Ultragenyx Impact Report.

2024 was our "get it done" year—a transformative year for our company as we've met intensive timelines to advance multiple late-stage programs with no approved therapies. There is so much entropy and many unknowns in the world of drug development, particularly in disease areas with no precedent approvals. We have been relentless in our efforts to move each of our late-stage programs forward while expanding access to our commercial therapies worldwide. Our Biologics License Application (BLA) seeking accelerated approval for UX111 AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A was recently accepted by the U.S. Food and Drug Administration (FDA), and we are poised to potentially have two more active BLA filings later this year.

Our international growth and collaboration in 2024 has also been impressive. We successfully launched our in-licensed therapy for homozygous familial hypercholesterolemia (HoFH) in Europe and Japan, while increasing access to our other commercial therapies in Latin America. We've also been working within these regions to establish clinical trial sites, line up new drug submissions with regulatory authorities, and reach into more geographies than ever before through expanded access programs.

We've remained highly engaged in advocating for regulatory policy improvements and have seen substantial change in the FDA's relationship with the rare disease community over the past year with the advancement of first-ever treatments, some of which were at risk of being shelved entirely. We will continue to be courageous in our advocacy and engagement efforts to advance rare disease regulatory policy. For example, we believe that an appropriate framework must be put in place to encourage development of therapies for ultrarare diseases that impact extremely small patient populations, and to build upon the success of the existing Orphan Drug Act. Therefore, Ultragenyx plans to advocate this year for new legislation to support an ultrarare regulatory framework.

We see this work as core to our responsibility as both a next-generation rare disease company and a company that wants to do good in the world. We believe all biopharmaceutical companies need to hold themselves to a higher standard because patient lives are in our hands. We are willing to model what that should look like because we believe we can do what's right and have a successful business by being generous with our time and expertise.

I continue to be inspired by the talented team at Ultragenyx that is executing on one of the largest late-stage pipelines in the industry by staying attuned to the possibility of what we can accomplish together. It's incredible to think that we could potentially launch three new therapies over the next couple of years, including our first two gene therapies, bringing first-ever approved treatments to patients without any other real options. As we move forward, our mission remains to transform the lives of people with rare disease and I welcome engagement from our communities on how we can continue to amplify our impact.

Sincerely,

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

Founder, President and CEO

2024 Awards

Top Workplaces Culture Excellence Awards

About Us

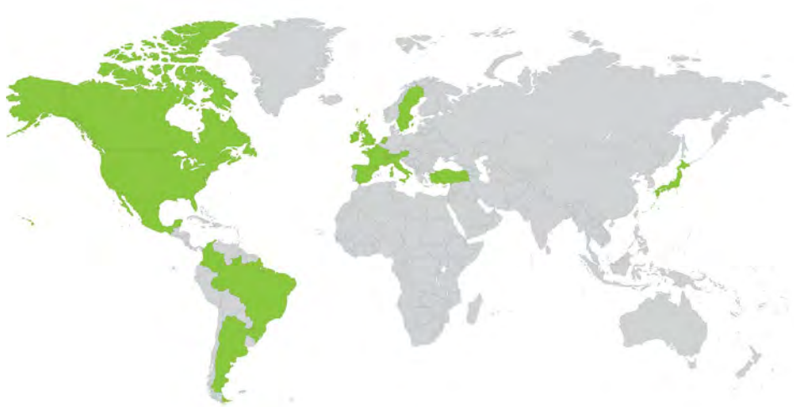
Vision: Lead the future of rare disease medicine

Mission: Transform the lives of people with rare disease

One Ultra Team

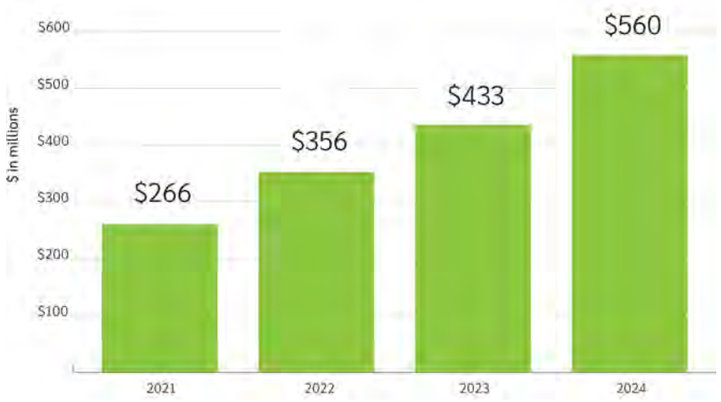
Ultragenyx is a biopharmaceutical company headquartered in Novato, California that is committed to bringing novel products to patients for the treatment of rare and ultrarare diseases, with a focus on serious, debilitating genetic diseases. Our purpose is to lead the future of rare disease medicine as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

1,290+ employees worldwide in 18 countries*



*As of December 31, 2024

Revenue*



*Excluding Bayer and Daiichi collaboration revenue

$698*
million in R&D investment

*From Jan. 1, 2024 - Dec. 31, 2024

>150
locations with clinical studies in 21 countries

>650 patients in 50 countries have been approved for access to Ultragenyx treatments through various global expanded access and patient assistance programs since 2013

Four Approved Treatments for Five Rare Diseases

Crysvita

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

Mepsevii

Mucopolysaccharidosis Type VII (MPS VII)

Dojolvi

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

Evkeeza

Homozygous Familial Hypercholesterolemia (HoFH)

2024 UPDATES

Evkeeza:

Successfully achieved reimbursement access in eight markets. Received marketing approval in Canada and Japan, and an expanded indication in children aged five years and older from the European Commission.

Crysvita:

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

Impact Report Approach

Ultragenyx's Impact Report covers our approach to corporate responsibility and is guided by a materiality assessment, focused on six key pillars that prioritize efforts and resources towards initiatives that significantly impact our business and stakeholders. Ultragenyx's Executive Leadership Team and Board of Directors have oversight responsibility for the Impact Report and corporate responsibility approach.

Strategic Framework

Ultragenyx is committed to ethics, integrity and corporate responsibility, integrating these principles across our business. We seek to set a powerful example, being 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 toward initiatives that we believe 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.

Innovation

Patients

People

Communities

Planet

Governance

Materiality Assessment

In 2021, we conducted a materiality analysis to determine our corporate responsibility 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. Ultragenyx plans to refresh this materiality assessment in the near future.

Evaluate relevant topics

Evaluated best practice guidelines from sustainability reporting standards (SASB, etc.) and conducted peer benchmarking to identify important topics impacting our business

Assess the impact on Ultragenyx

Conducted discussions and surveys with internal topic experts and business leaders to rank the identified topics on their importance to our business and their ability to impact our stakeholders

Topic prioritization taking into account importance to stakeholder

Obtained external stakeholder feedback from analyst reports, investor queries, data from Datamaran (an AI-driven tool) and other resources to determine prioritization of topics

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 & Inclusion**
- **Employee Health & Safety**
- **Workforce Management**

Communities

- Community Relations

Planet

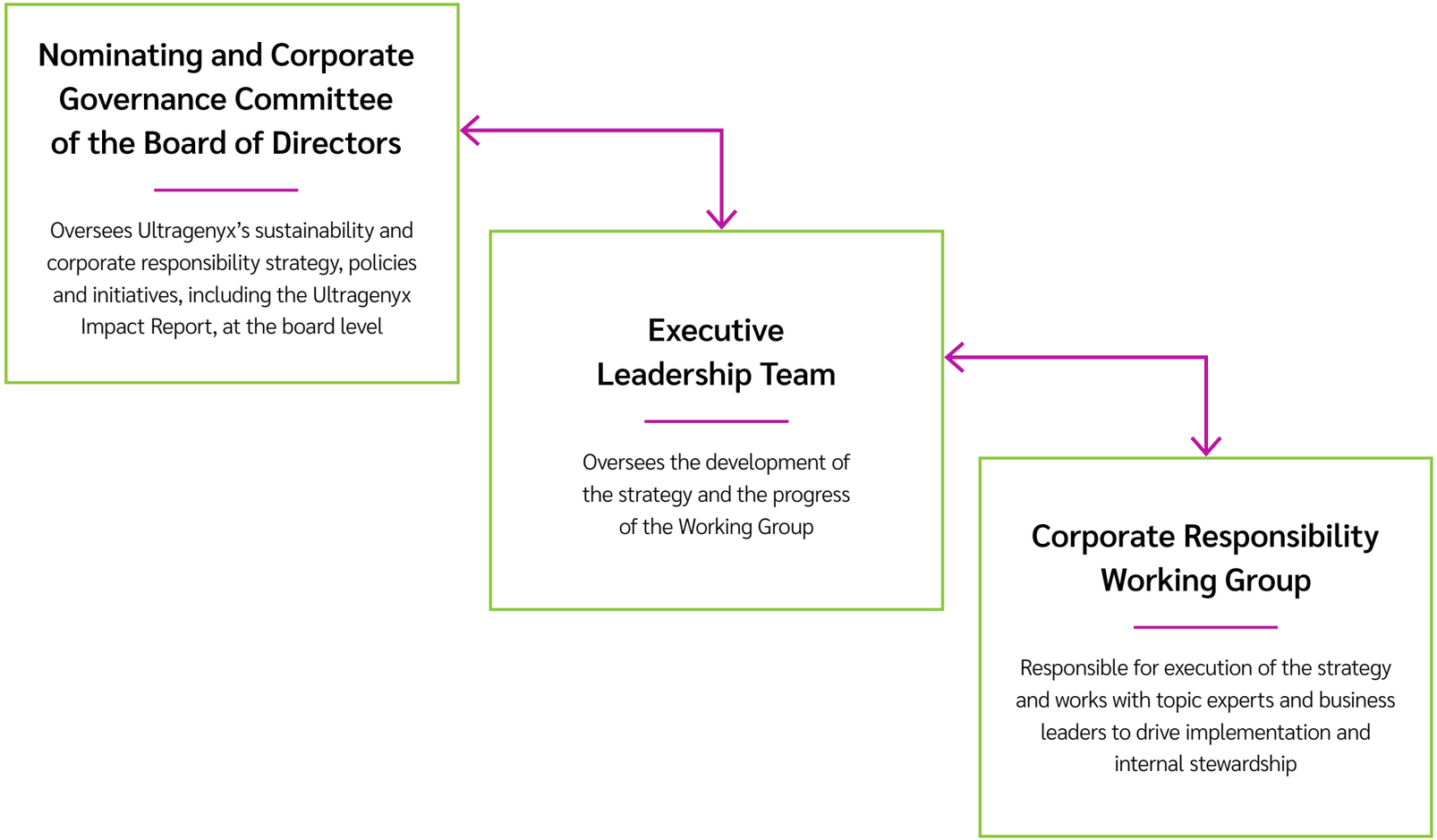
- Climate Change Risks and Management
- Energy Management
- Product Stewardship
- Waste Management
- Water Management

Governance

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

Corporate Responsibility Oversight

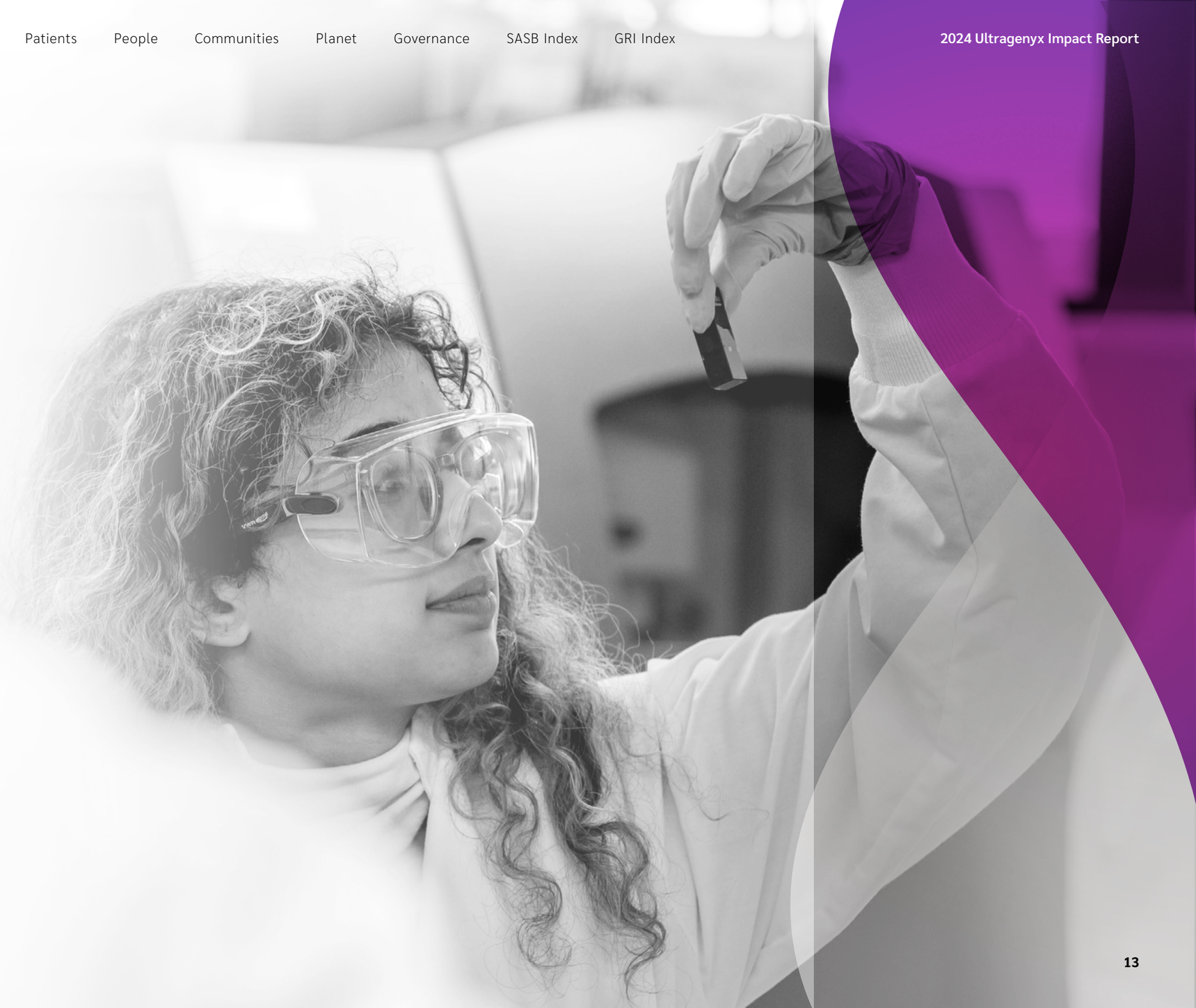
We are committed to integrating corporate responsibility with our overall corporate strategy. This commitment starts at the Board of Directors' level. The Nominating and Corporate Governance Committee of our board regularly reviews and makes recommendations on sustainability and corporate responsibility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



Innovation

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

We are a next-generation rare disease company **committed** to developing and delivering transformative treatments where none exist.



Aspiration

To optimize and accelerate the field of rare disease drug research and development (R&D).

Our Objectives	2024 Progress Highlights
Develop industry-leading clinical pipeline in rare and ultrarare diseases that have limited or no treatment options	• Setrusumab (UX143) received Breakthrough Therapy Designation from the FDA for the treatment of osteogenesis imperfecta (OI) • Initiated Phase 3 *Aspire* study evaluating the efficacy and safety of GTX-102 for Angelman syndrome • Filed a Biologics License Application (BLA) with the FDA seeking accelerated approval for UX111 AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A (MPS IIIA); the FDA subsequently accepted the BLA and granted the BLA Priority Review with a Prescription Drug User Fee Act (PDUFA) action date of August 18, 2025 • Announced positive topline results from the Phase 3 *GlucoGene* study evaluating DTX401 AAV gene therapy for the treatment of patients with glycogen storage disease type Ia (GSDIa) aged eight years and older • Successfully transferred the manufacturing process of our DTX401 gene therapy for the potential treatment of GSDIa to our gene therapy manufacturing facility
Foster industry-wide and community funded development efforts in rare and ultrarare diseases	• Hosted two Rare Bootcamps with nearly 40 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 • Jointly sponsored workshop with the Reagan-Udall Foundation for the FDA on qualifying biomarkers to support accelerated approval in rare disease drug development • Supported more than 80 clinical and non-clinical investigator-sponsored trials (ISTs) globally, fostering community and investigator-led research initiatives

R&D

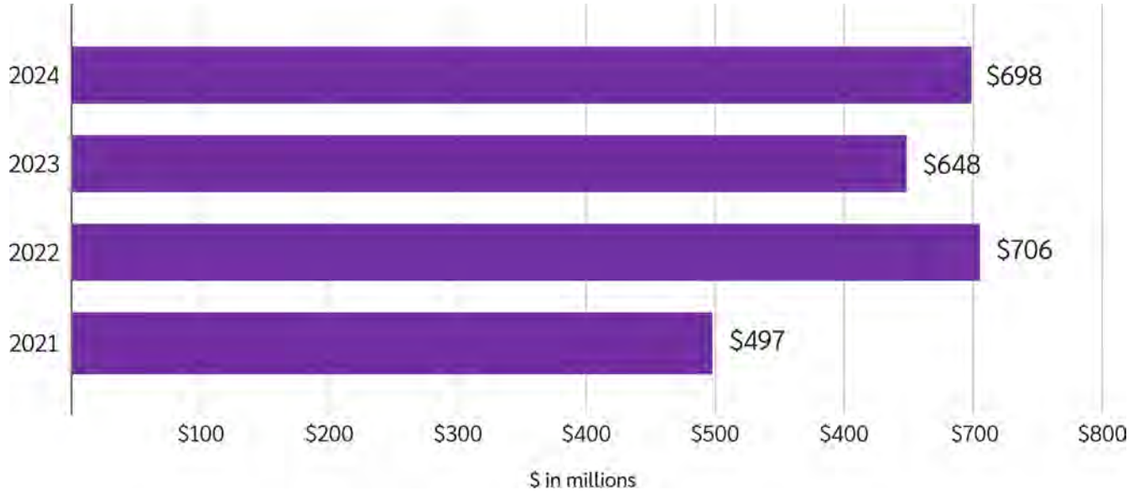
Innovation refers to our processes, initiatives, activities and investments aimed at the efficient and rapid advancement of investigational therapies that maximize patient health outcomes. Our patient-focused drug development model and cross-industry collaborations support our mission to transform the 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 investments in R&D to innovate and develop new therapies. In 2024, we spent approximately 64% 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. We patent or in-license the technologies, inventions and improvements in the U.S. and internationally that we consider important to the development of our products, investigational therapies and processes. We are committed to actively enforcing and defending our intellectual property rights against any infringement or unauthorized use to protect our innovations and competitive advantage. Furthermore, 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.

Our research aims to advance a new program into clinical development every one to two years. At the end of 2024, we had **four** rare disease treatments approved by the FDA in **five** indications and **six** programs in clinical trials.

R&D Spend



For more details, please see our **2024 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

Find right opportunities at reasonable cost, develop rapidly with adaptive designs, and commercialize efficiently and effectively

Research

Pursue high potential programs

- Potent biology in severe diseases
- Treating underlying cause
- Best modality for each disease

Clinic

Accelerate to drive value

- Adaptive trial designs
- Novel endpoints
- High unmet medical need supporting expedited enrollment

Commercial

Patient-centric approach

- Lean commercial team
- Emphasize patient find/support
- Reduced post-approval R&D costs

Our Gene Therapy Manufacturing Capabilities

Our Gene Therapy Manufacturing Facility (GTMF) in Bedford, Massachusetts provides 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.

The GTMF 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, including fill finish, as well as warehouse and office spaces. 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 integrated end-to-end gene therapy R&D capabilities and position us at the forefront of industry developments in gene therapy.

Our goals with the GTMF are to support the optimization of our proprietary Pinnacle PCL™ (Producer Cell Line) 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.

In 2024, we successfully transferred the manufacturing process of our DTX401 (pariglasgene brecaparvovec) gene therapy for the potential treatment of glycogen storage disease type Ia (GSDIa) to our GTMF and ensured the necessary documentation, equipment and controls were in place to enable process performance qualification (PPQ) manufacturing.

Our Pipeline

Our approved therapies and clinical-stage pipeline consist of four modalities: biologics, small molecules, gene therapies and antisense oligonucleotides (ASOs). 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.

Candidate	Description	Phase 1	Phase 2	Phase 3	Route of Admin	Prevalence[1]
UX143	Anti-Sclerostin monoclonal antibody	Osteogenesis Imperfecta (OI)			Intravenous (IV) Infusion	~60,000
UX111	AAV9 SGSH gene therapy	Sanfilippo Syndrome (MPS IIIA)			IV Infusion	~3,000–5,000
DTX401	AAAV8-G6Pase gene therapy	Glycogen Storage Disease Type Ia (GSDIa)			IV Infusion	~6,000
DTX301	AAV8-OTC gene therapy	Ornithine Transcarbamylase (OTC)			IV Infusion	~10,000
UX701	AAV9-ATP7B gene therapy	Wilson Disease (WD)			IV Infusion	~50,000
GTX-102	ASO activating paternal expression of UBE3A	Angelman Syndrome (AS)			Intrathecal Infusion	~60,000

[1]Prevalence in commercially accessible geographies

Therapeutic Area: Bone/Endo | Inborn Error of Metabolism | Neurogenetic

Update to Our Pipeline

UX143 for osteogenesis imperfecta (OI)

In October 2024, UX143 (setrusumab) received Breakthrough Therapy Designation (BTD) from the FDA. This designation aims to expedite the development and review of drugs that are intended to treat serious or life-threatening diseases and whose preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. Setrusumab is currently being evaluated in the Phase 3 *Orbit* study, which is assessing setrusumab's impact on annualized clinical fracture rates versus placebo in patients aged five to 25 years, and the Phase 3 *Cosmic* study, an active-controlled trial comparing setrusumab to IV bisphosphonates in patients aged two to six years, focusing on annualized total fracture rates. Setrusumab 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

In December 2024, Ultragenyx initiated the pivotal Phase 3 *Aspire* study evaluating the efficacy and safety of GTX-102 for Angelman syndrome. In 2024, the company also presented data from the Phase 1/2 study at the 76th Annual American Academy of Neurology Meeting (AAN) in April and the Foundation for Angelman Syndrome Therapeutics (FAST) Global Science Summit in November that demonstrated clinically meaningful improvements across multiple clinical domains, compared to natural history data, where available and confirmed that the Phase 3 *Aspire* study is amply powered to establish the efficacy of GTX-102 on the primary endpoint of change in cognition, as measured by Bayley-4, or the key secondary endpoint of MDRI at Week 48. 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

In October 2024, Ultragenyx shared that the Phase 1/2/3 *Cyprus2+* study demonstrated clinical activity as well as improvements in copper metabolism for patients treated in Cohorts 1, 2 and 3 in Stage 1, which is the Phase 1/2 portion of the study. Multiple responders completely tapered off their standard-of-care treatment with responses seen in all three dose cohorts. UX701 AAV gene therapy has been well tolerated, with no unexpected, related treatment-emergent adverse events and no significant immunologic safety events as of the data cut-off. The company expects to enroll an additional cohort in Stage 1 at a moderately increased dose and with an optimized immunomodulation regimen to enhance the efficiency and efficacy of the gene therapy, with the objective of having the majority of patients come off of standard-of-care treatment before selecting a dose for the randomized placebo-controlled stage of the study. UX701 has received Orphan Drug and Fast Track designations in the U.S., and Orphan medicinal product designation in the EU.

Therapeutic Area: Bone/Endo | Inborn Error of Metabolism | Neurogenetic

Update to Our Pipeline (cont.)

UX111 for Sanfilippo syndrome type A (MPS IIIA)

In December 2024, Ultragenyx filed a BLA with the FDA seeking accelerated approval for UX111 AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A. The FDA subsequently accepted the BLA and granted the BLA Priority Review with a PDUFA action date of August 18, 2025. 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™ 20th annual research meeting in February 2024. These findings showed rapid and sustained decreased levels of heparan sulfate (HS) levels in cerebrospinal fluid (CSF), correlating with improved long-term cognitive development as of the data cut-off. Additional data were presented in February 2025 that demonstrated treatment with UX111 led to significant improvements across multiple clinical domains compared to natural history data from untreated patients. These clinical endpoints were correlated with substantial and sustained reduction in levels of CSF-HS as of the data cut-off. 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)

In May 2024, Ultragenyx announced positive topline results from the Phase 3 *GlucoGene* study for the treatment of patients aged eight years and older. The study achieved its primary endpoint, demonstrating that treatment with DTX401AAV gene therapy resulted in a statistically significant and clinically meaningful reduction in daily cornstarch intake compared with placebo at Week 48. Additional data in crossover patients (previously treated with placebo) were released by the company in November 2024 and demonstrated a higher and faster mean reduction in daily cornstarch intake at Week 30 post-treatment with DTX401. These patients were able to titrate cornstarch much more rapidly once they were confirmed to have been treated and had timely direct access to their glucose levels. Patients from the original DTX401 treatment arm who have reached 78 weeks also continued to reduce their daily cornstarch intake, while maintaining glycemic control. DTX401 has received Orphan Drug designation, RMAT designation and Fast Track designation in the U.S., and PRIME and Orphan medicinal product designations in the EU.

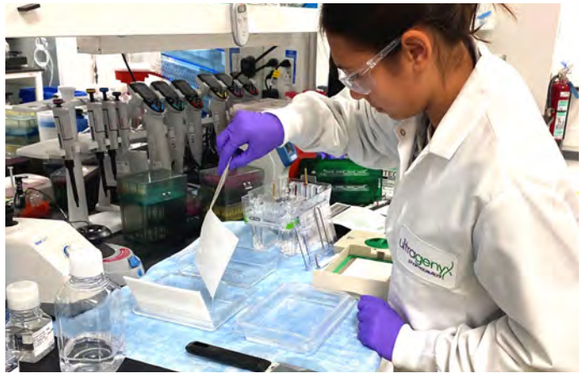
DTX301 for ornithine transcarbamylase (OTC) deficiency

The Phase 3 *Enh3ance* study has completed enrollment with 37 patients, randomized 1:1 to DTX301 AAV gene therapy or placebo. The pivotal, 64-week study will evaluate the primary endpoints of response as measured by change in 24-hour ammonia levels and removal of ammonia-scavenger medications and protein-restricted diet. DTX301 has been granted Orphan Drug designation and Fast Track designation in the U.S., and Orphan medicinal product designation in the EU.

Therapeutic Area: Bone/Endo | Inborn Error of Metabolism | Neurogenetic

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, as well as 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 **2024 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, our procedures require that we carefully assess available resources, clinical trial experience and the suitability of its facilities, staff and equipment.

Every clinical trial site is expected to be 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 to be 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 regularly monitor for any deviations from GCP standards, misconduct or violations of patient rights or safety. In cases where such issues are identified, our procedures require corrective measures to be implemented, severity impact assessments to be conducted and, if necessary, for these findings to be reported to the FDA and comparable regulatory authorities in other countries.

As of 2024

>150 *locations with clinical trial sites across* **21** *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 have designed processes to be informed by 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 consistent 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, as appropriate. 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 under our procedures, meaning no names or other personally identifiable information (PII) is to be 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.

Representation 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 **80** clinical and non-clinical ISTs approved and **600+** 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 representation 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.

A lack of adequate representation in clinical research 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 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 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 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, inclusion and belonging to enhance our employees' ability to engage effectively with different communities
- 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.

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, 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.

We currently have DMPs for MPS VII, LC FAOD, XLH, TIO and GSDIa.

DMP Highlights

*First **DMP** launched in 2012*

*Nearly **1,000** patients enrolled* at sites in **13** countries*

*Enrollment as of December 31, 2024

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 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 our operations and across the stages of development, manufacturing and distribution of our medicines.

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 employing 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 all applicable regulations and international standards, collectively referred to as “good practice” or 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, continued 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

We have a robust supplier oversight program with the aim of requiring high standards for ourselves and our partners. 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. Additionally, we periodically 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 2024

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.

At the heart of our mission to be the leader in rare disease medicine is our commitment to patient safety and the trust that patients, healthcare professionals, regulatory authorities and the general public place in us. Our Global Drug Safety & Pharmacovigilance department maintains a global pharmacovigilance (PV) system with robust medical and safety surveillance and designed with comprehensive, inspection-ready systems and procedures designed to comply with laws, Good Pharmacovigilance Practices and other GxP regulations, standards and industry best practices.

Safety information from all sources globally is continually evaluated throughout a product's lifecycle, from early development phase and for as long as the product is commercialized to provide for a positive benefit-risk profile.

This information comes from many potential sources and may include but is not limited to:

- Employees, contractors, consultants
- Clinical trials
- Disease monitoring programs
- Patient access programs such as compassionate and named patient use
- Patients, 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

All Ultragenyx workforce, representatives or agents working on behalf of Ultragenyx are required to complete training to understand the safety reporting process and their role to report safety information including AEs and product quality complaints (PQCs) for all Ultragenyx medicines.

Our 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.

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 Crysvita 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 **2024 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 are 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 an effort to better understand how to measure change in clinical studies.

We are 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.

We are 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 are 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 are expected to be shared with the entire community to aid CDD clinical trial design and inform therapeutic development for related neurodevelopmental disorders.

We are 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 caregivers. 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.

ULTRAGENYX IN ACTION STORY

Ultragenyx Joins Reagan-Udall Foundation for the FDA to Support Workshop on Accelerated Approval in Rare Diseases

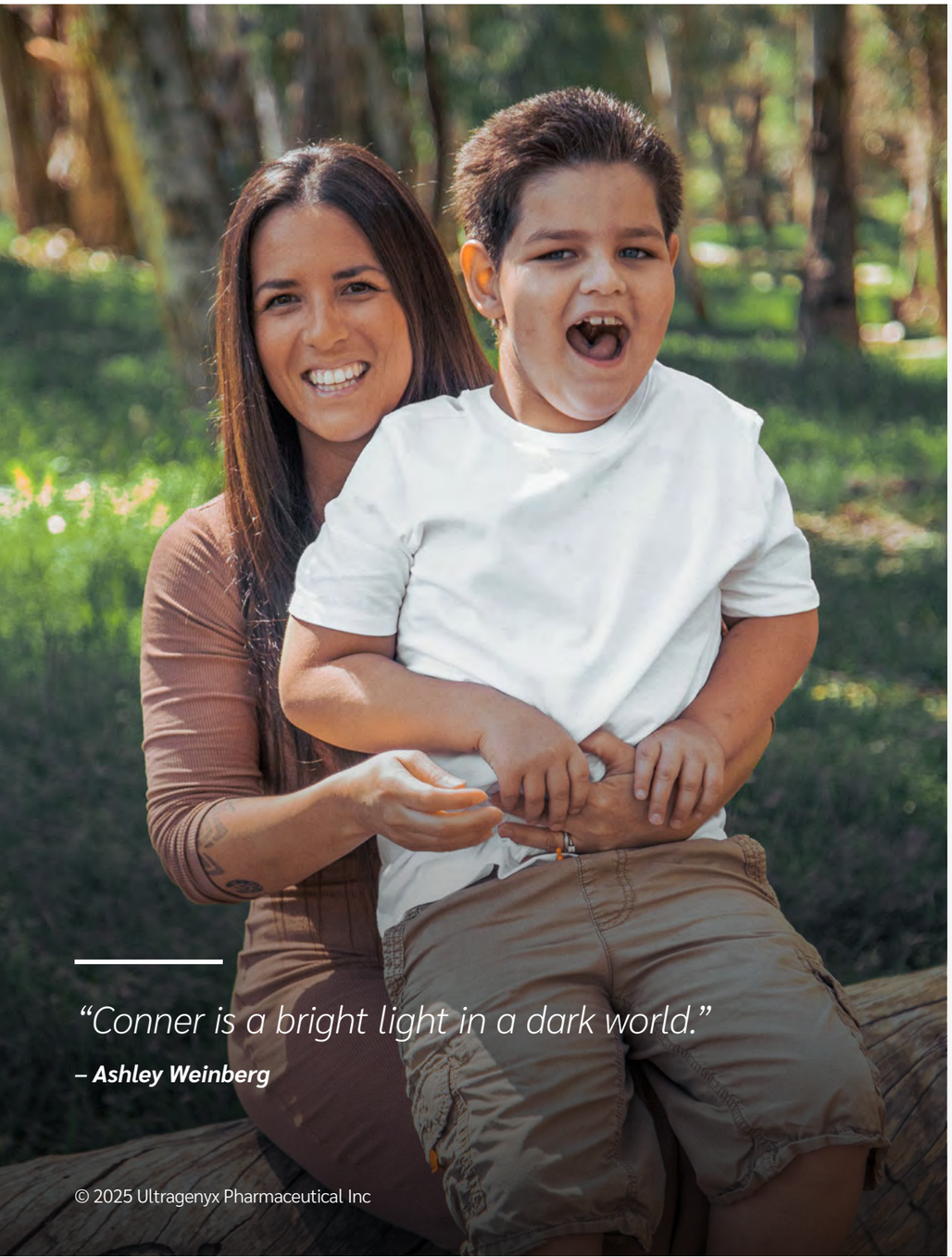
In February 2024, our team collaborated with the Reagan-Udall Foundation for the FDA, to bring together clinicians, scientific researchers, drug developers and patient advocates to discuss the qualification of cerebrospinal fluid-heparan sulfate (CSF-HS) as a primary biomarker in neuropathic mucopolysaccharidoses (MPS) disorders, including Sanfilippo syndrome (MPS IIIA). Advocacy participants included leaders from the Cure Sanfilippo Foundation, the National MPS Society and the Ryan Foundation.

At the heart of this endeavor was the urgent need to provide equitable access to the Accelerated Approval Pathway to children suffering from these devastating ultrarare disorders. The pathway allows the FDA to approve certain drugs based on endpoints that can be measured earlier than traditional clinical endpoints. The workshop served as a platform for leaders in the field to present comprehensive data on disease pathology, fostering a unified approach to validate CSF-HS as a primary disease activity biomarker. This aligns with the FDA's 2020 guidance on rare diseases involving single enzyme defects with substrate deposition, paving the way for new treatments via the accelerated approval pathway.

Over the years, multiple clinical studies have faltered due to stringent FDA criteria, despite the clear correlation between the reduction of CSF-HS and positive clinical outcomes. With this, there is an urgent need for regulatory frameworks to keep pace with scientific advancements to ensure that promising therapies are not left behind.

Since the workshop, Ultragenyx and other drug sponsors have reached agreement with the FDA that CSF-HS is a reasonable surrogate endpoint that could support submission of a biologics license application (BLA) through the accelerated approval pathway. In December 2024, Ultragenyx submitted its BLA seeking accelerated approval for UX111 AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A.

As we continue to advance our UX111 program, it is our hope that strategic collaborations such as this will enable us to progress our work and significantly improve the lives of those affected by Sanfilippo syndrome and other MPS disorders.



"Conner is a bright light in a dark world."

– Ashley Weinberg

CONNER'S STORY

Living with Angelman Syndrome

For the first year of Conner Weinberg's life, his family had every reason to believe that he was healthy. He was quiet, content and brought his family tremendous joy. But when he began to miss major milestones – unable to sit up, crawl, or speak – his mother Ashley sought the help of their pediatrician, who recommended early intervention therapy.

Despite seeking answers from specialists for years, it wasn't until Conner turned five that a pediatric resident suggested the family seek genetic testing. The results revealed that Conner was living with Angelman syndrome (AS), a rare genetic disorder that can lead to developmental delay, communication impairments, motor impairment and debilitating seizures.

"I know many people go through a grieving process when receiving a diagnosis, but for us, there was also a feeling of relief. There was the grief of knowing that this was forever, but it was a relief to find a community of people who had lived this life," Ashley shares.

Through the AS community, the family not only found resources to support Conner's care but also an incredible support network. Ashley became actively involved with the Angelman Syndrome Foundation (ASF), driven by her desire to help other families facing similar journeys. As part of her work, Ashley coordinates local events, including ASF's annual walk in the Weinbergs' hometown of San Diego, to raise greater awareness for the community.

As the family connects with new people along the way, Conner's bravery and resilience have a profound impact, creating unbreakable bonds with everyone he meets.

"Conner has this way of bringing people in—you can't help but fall in love with him! He has teachers that haven't taught him since preschool but still reach out to check-in. His first therapist still sends messages to see how he's doing," she shares. "The best part about being part of the Angelman community is the fact that everyone you talk to is always willing to support. It's a family."

Evaluating GTX-102 to reactivate expression of the UBE3A gene in patients with AS

Ultragenyx's GTX-102 is an investigational antisense oligonucleotide (ASO) therapy designed to inhibit expression of UBE3A-ATS in order to prevent silencing of the paternally inherited allele of the UBE3A gene and reactivate expression of the deficient protein. A Phase 1/2, open-label, multiple-dose-escalating study evaluating the safety and tolerability of GTX-102 and its effect on all major domains of AS in pediatric patients completed enrollment in January 2024.

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.

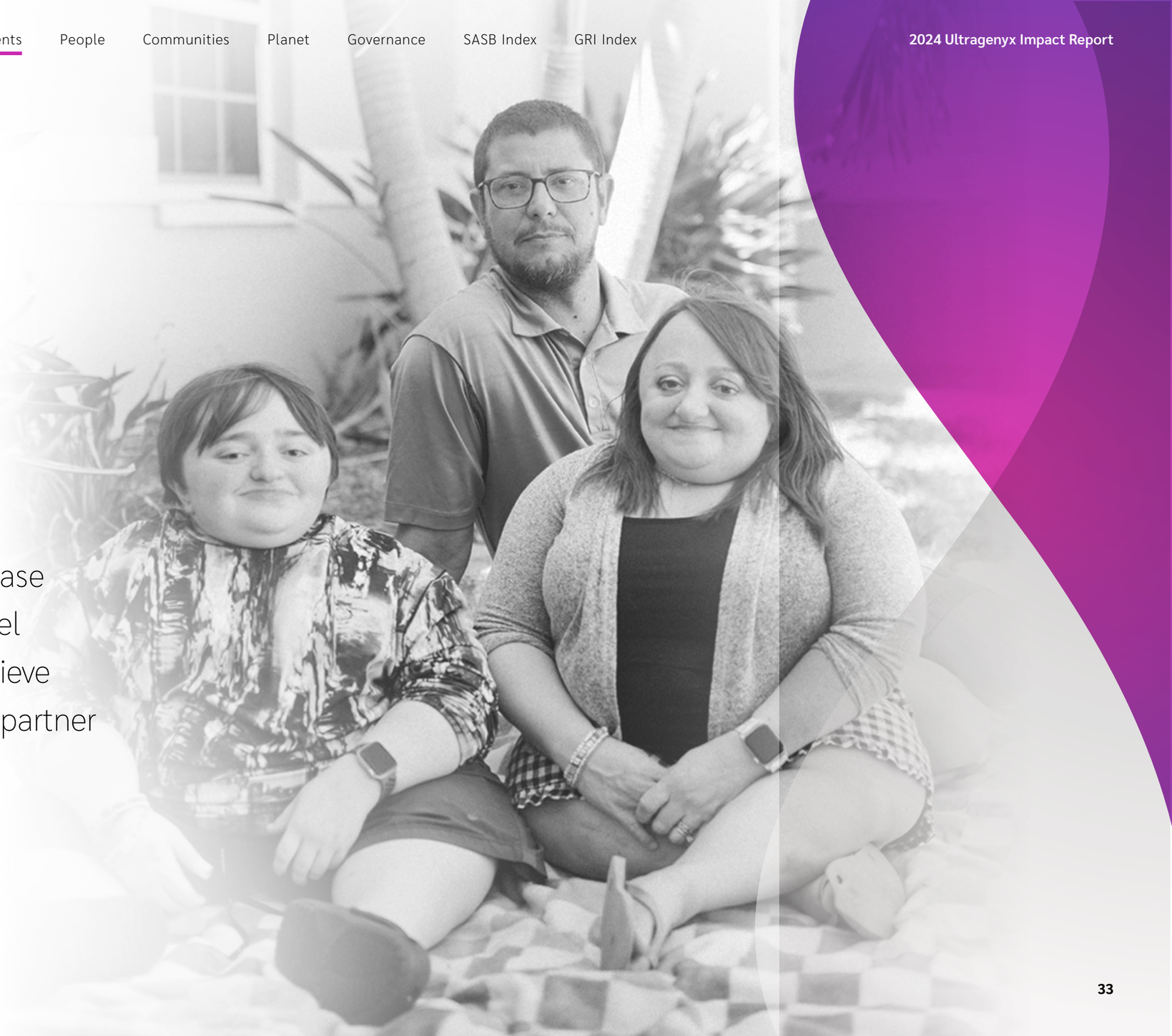
The event has evolved based on participant feedback. For example, on the research side, we now conduct a working group to assess scientific gaps in knowledge associated with each disease and the appropriate therapeutic modality to prioritize. In addition, we have added a panel discussion and lecture 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.

*Since 2017, we have held **10** Rare Bootcamps with more than **180** participants from over **120** 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.



Aspiration

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

Our Objectives	2024 Progress Highlights
Achieve majority access through responsible pricing and support services	• We continue to align U.S. price increases consistent with the **Consumer Price Index for commercial products for which we are responsible for promotion**
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	• Initiated **policy** discussions to advocate for **legislation** that supports the development of treatments for ultrarare diseases, to help ensure that these patient communities are not left behind • Successfully advocated for the passage of newborn screening legislation in Alabama and Tennessee so that when new conditions are added to the Recommended Uniform Screening Panel (RUSP) those states will add those conditions to their state panels within a specific period of time
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	• Attended **50** 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 to Our Therapies

We believe the greatest impact we can have on the lives of individuals with rare disease is to invest in developing transformative treatments and do our best to make them accessible to anyone who can benefit while engaging and supporting rare disease families along every step of their journey.

We believe 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 aim to evaluate each medicine's value based on a number of factors, such as healthcare economics, clinical data and comparisons to similar therapies.

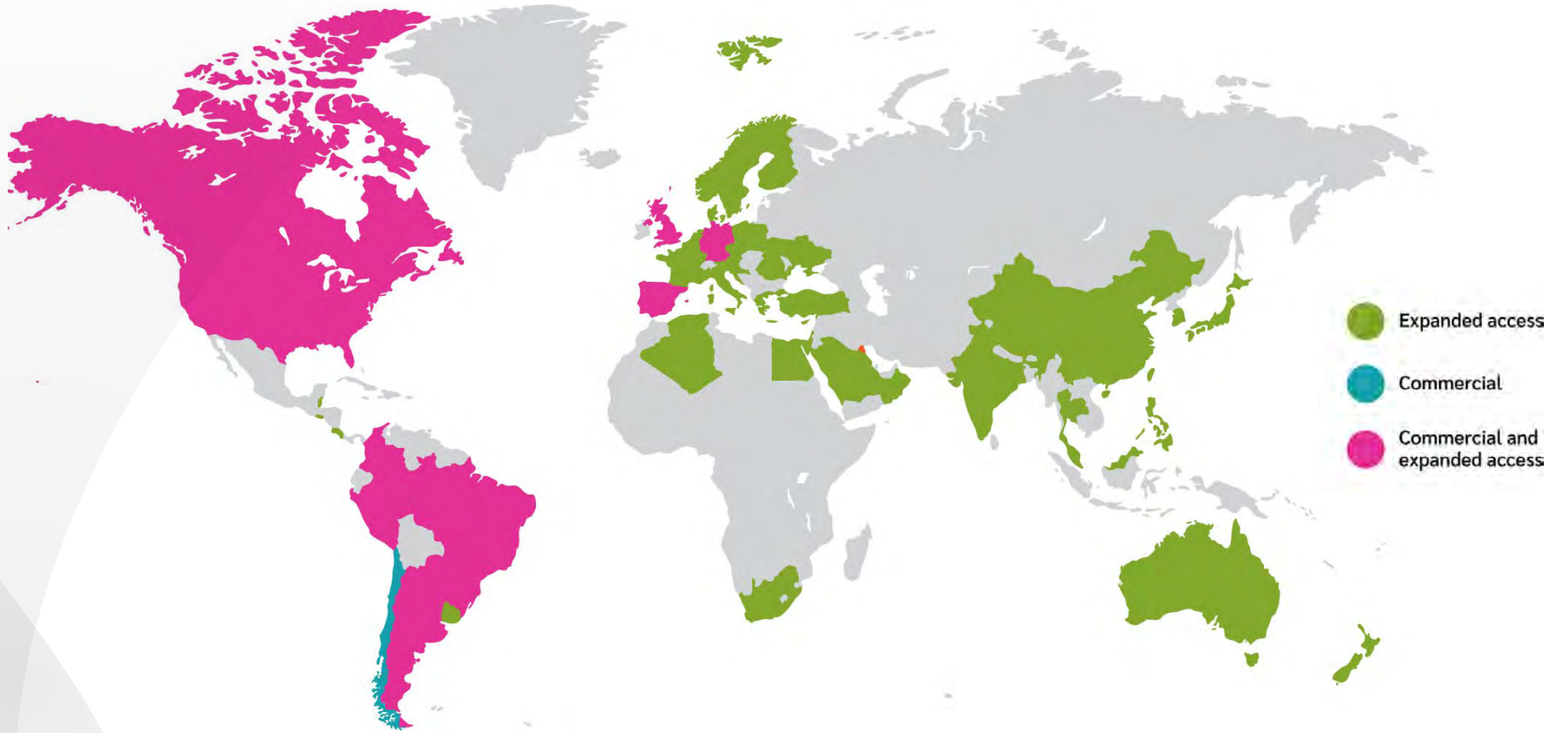
Additionally, we consider the costs of producing high-quality medicines and sustaining a robust global supply chain. We set our medicine prices with global pricing in mind. In the U.S., we leverage effective reimbursement strategies and strive to use our best efforts to prevent patients from foregoing Ultragenyx therapy for financial reasons. Our patient assistance programs include co-pay support and free access where necessary. In 2024, U.S. price increases for the commercial products for which we lead promotion were consistent with 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.

Our Gene Therapy Manufacturing Facility (GTMF) opened in June 2023

Access to Our Therapies (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. In an effort 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.

Since we have entered into our collaboration agreement with Regeneron, Ultragenyx has expanded access to Evkeeza in Canada, Europe and Japan. Specifically, we've received recommendations for reimbursement in seven countries outside of the U.S. – Italy, Canada, Japan, Netherlands, Spain, United Kingdom and Luxembourg.

Access to Our Therapies (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** 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. 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 feasible, 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.

*More than **650** patients in **50** countries have been approved for access to Ultragenyx medicines through various global expanded access and patient assistance programs since 2013*

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 into clinical research and through approval and commercialization.

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 burden of the diseases on daily life and understand the impact of our medicines on patients and families.

Our patient advocacy 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, policy implications and their impact on the rare disease ecosystem.
- **Engaging, Empowering, and Activating** patients and community members to advocate for equitable access to rare disease diagnostics and treatments.
- **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 patient and patient community perspectives and lived experiences across 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.

Partnering with Patient Organizations

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

To further this purpose, in 2024, we attended **50** 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:

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

In 2024, the council 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 disease monitoring program (DMP) results, as appropriate.

Our Educational Resources:

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

www.mpsviiinfocus.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.ultraclinicaltrials.com
A global clinical trial recruitment website for caregivers and people living with rare diseases

Our Rare Journey Resources, at **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.

"I've always been a fighter, and so has Emma."

– Amber Gray

AMBER AND EMMA'S STORY

Living with Osteogenesis Imperfecta

Every Saturday morning, Amber and Emma Gray embark on a cherished "mother-daughter date day" where the duo grab treats from their favorite coffee shop and play games together, content in each other's company. These simple moments of connection have carried Amber and Emma through seasons of immense challenges and change, as they each navigate living with osteogenesis imperfecta (OI).

For people living with OI like Amber and Emma, inadequate production of new bone and excess bone resorption can result in decreased bone mineral density, bone fragility and weakness. OI can also lead to bone deformities, abnormal spine curvature, pain, decreased mobility and short stature. "Sometimes, we don't need a lot of trauma to break," Amber shares. "Emma recently broke her clavicle simply by putting her arm back in the passenger seat of the car."

When Amber was first born in 1984, doctors were unsure if she would survive. Yet, from a young age, Amber demonstrated an unwavering spirit, fighting the odds stacked against her. She participated in groundbreaking medical research at the National Institutes of Health, becoming one of the first children to participate in their OI program. Through countless tests and procedures, Amber contributed to critical research across the field. She was determined to not simply survive, but thrive.

Years later, when Amber's daughter Emma was born with OI, doctors provided the same bleak prognosis as her own mother was given. With this, her fierce maternal instinct kicked in. Today, she continues to fight tirelessly to ensure Emma receives the best possible care, even when it at times meant prioritizing Emma's needs over her own.

As they navigate living with OI, Amber and Emma's bond is unbreakable. "We're attached at the hip," Amber shares. Together, they find joy in life's simple pleasures – warm cups of coffee, trips to the library and neighborhood walks. Their camaraderie also extends to the larger OI community, who over the years, the family has both leaned on and offered their support to, participating in advocacy events across the country. Ultimately, it is the strength they draw from one another and the OI community that keeps them fighting each day.

Evaluating UX143 to improve bone density and reduce fracture rate

UX143 (setrusumab) is an investigational, fully human monoclonal antibody that inhibits sclerostin, a negative regulator of bone formation being developed under a collaboration and license agreement between Mereo BioPharma and Ultragenyx. Inhibiting sclerostin is expected to increase new bone formation, bone mineral density and bone strength in OI. Ultragenyx is developing UX143 in pediatric and young adult patients across OI sub-types I, III and IV with two late-stage trials: the pivotal Phase 2/3 *Orbit* study and Phase 3 *Cosmic* study.

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 , 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 Policy, Government and Public Affairs 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 25 companies that are collectively investing more than **$17 billion** annually in R&D and that have over 200 treatments either approved or in development. 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 establish rare diseases as a priority in global markets
Pricing and Reimbursement	Proactively engaging with payers to with the goal 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

Members of our senior management team and 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 33 to 68, and only one state screens for all 38 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 2024, Ultragenyx successfully advocated for the passage of legislation in Alabama and Tennessee so that when new conditions are added to the RUSP, those states will add the conditions to their state panels within a specific period of time and that they have the requisite funding to do so (RUSP-alignment legislation).

In 2025, Ultragenyx plans to work to pass RUSP-alignment legislation in Washington and Wisconsin.

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.

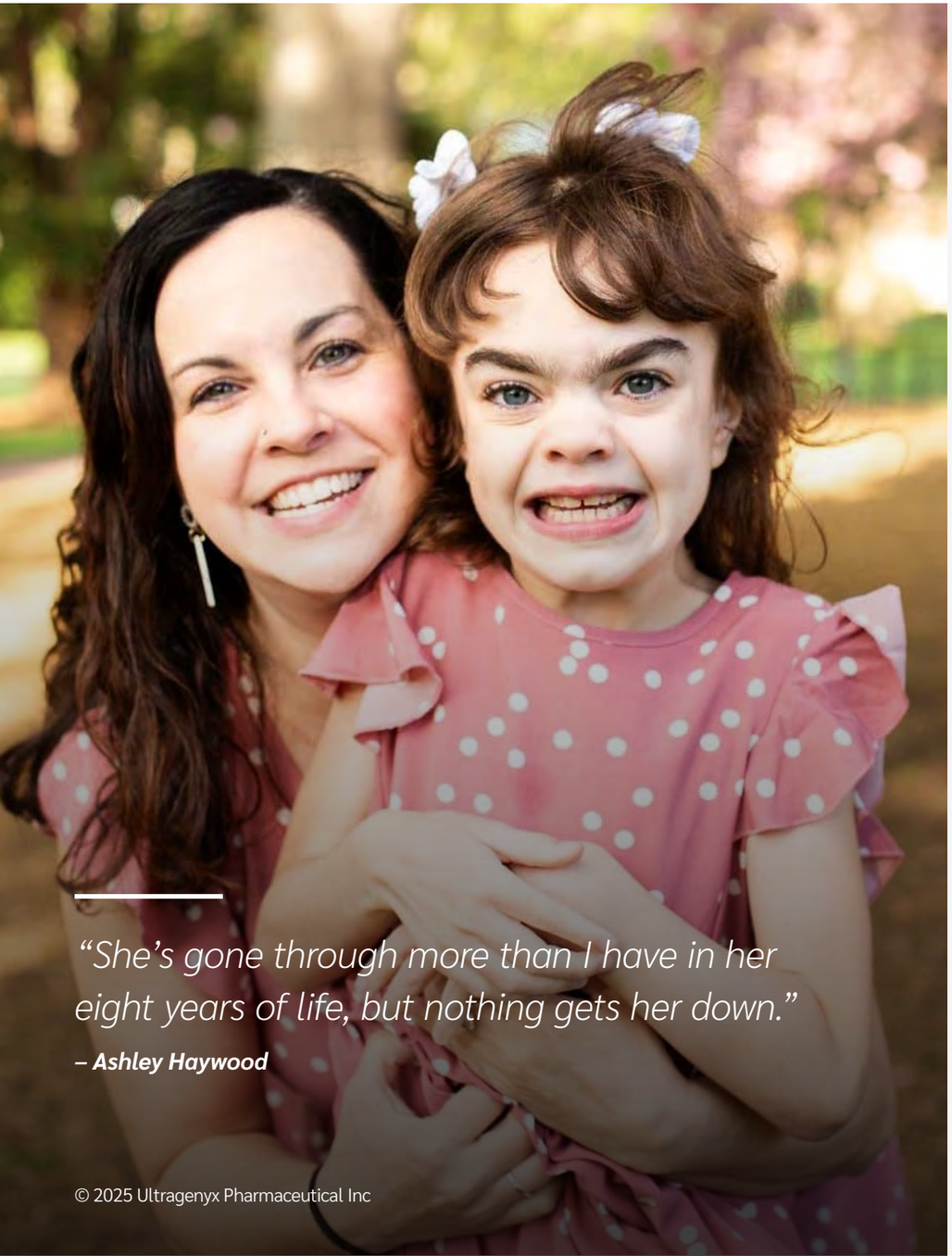
Advocating for the Successful Development of Specific Treatments for Ultrarare Diseases

Challenges in Ultrarare Disease Drug Development: Despite the success of the Orphan Drug Act (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 Legislation to Support Development of Therapies for Ultrarare Diseases: In response to these challenges, Ultragenyx is proposing that new legislation be implemented to provide an appropriate framework that encourages development of therapies for diseases with very small patient populations that will be a supplement to the existing ODA.

Key elements of the proposed framework include:

- Clarifying that, for ultrarare disease therapies, the "substantial evidence of efficacy" standard is met by utilizing a "totality of the evidence" standard — that, based on the totality of the scientific evidence available, the known and potential benefits of the therapy outweigh the known and potential risks
- Acceptance of alternative study designs and analyses for ultrarare diseases
- Recognition of qualifying biomarkers that represent underlying disease ("primary disease biomarkers") as primary endpoints in pivotal clinical trials
- Assurance that therapies approved through the use of primary disease biomarkers are traditional approvals, not accelerated approvals
- 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



“She’s gone through more than I have in her eight years of life, but nothing gets her down.”

– Ashley Haywood

SADIE’S STORY

Living with Sanfilippo Syndrome

For 8-year-old Sadie Rae Haywood and her family, every day is treasured. In 2016, at just three months old, Sadie was diagnosed with Sanfilippo syndrome. Her mom, Ashley, had a feeling something wasn’t right and asked for testing – a distant relative had lived with the rare condition. Though her doctors were initially skeptical, the results confirmed the family’s suspicions.

Sanfilippo syndrome type A (MPS IIIA) is a rare disease that primarily affects the central nervous system and is characterized by rapid neurodegeneration, with onset in early childhood. Children with Sanfilippo syndrome experience global developmental delay leading to the decline of cognitive function, which plays a key role in acquiring new skills including walking, communicating and performing many everyday tasks. The median life expectancy is 15 years old.

Ashley and her family refused to give up hope in the face of Sadie’s diagnosis, determined to give their daughter every possible opportunity. From the time she was born, Sadie was enrolled in physical, occupational and speech therapy. She eventually participated in a clinical trial, laying a foundation that helped her maintain skills as the disease progressed.

Over the years, Ashley became a tireless advocate, not only for Sadie, but the entire Sanfilippo community. She and her family began sharing Sadie’s story on social media, attracting a tremendous following and acting as a vital resource for other families, sharing their learnings and raising awareness of the condition.

The family partnered with the Cure Sanfilippo Foundation, where today, Ashley spends time connecting with families new to the community to provide the support and resources they need.

Today, Sadie is a fun and joyful child who loves to sing and be snuggled. “She makes me proud all the time,” Ashley says. By sharing Sadie’s story, Ashley hopes to advocate for even greater resources across the Sanfilippo community in the years ahead, to ensure that other children and their families know that they are not alone.

UX111 (rebisufligene etisparvovec and formerly ABO-102) is Ultragenyx’s investigational in vivo AAV gene therapy for people living with Sanfilippo syndrome type A. The therapy is designed to address the underlying sulfamidase enzyme deficiency responsible for abnormal accumulation of heparan sulfate, a glycosaminoglycan, in the brain that results in progressive cell damage and neurodegeneration that is associated with the disorder. In December 2024, Ultragenyx submitted a BLA to the FDA for accelerated approval of UX111 for the treatment of MPS IIIA following successful meetings with the FDA, aligning that cerebral spinal fluid heparan sulfate (CSF-HS) can be used as a reasonable surrogate endpoint.

People

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

We are **committed** to working to maintain an 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	2024 Progress
Maintain a positive workforce culture by achieving a total turnover rate below the industry average and continuing to have high employee engagement	• Total turnover was **10.9%**, 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 **88%** in our employee engagement survey
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	• Launched Executive Edge program to support development of leadership skills at VP and SVP level • Nearly **7%** of our global workforce participated in the on-demand career coaching services we offer • Hosted **35** students across our emerging talent programs
Continue to strengthen and expand inclusion and belonging through talent acquisition and management efforts, including candidate pipelining, interview processes, ongoing education, awards, promotions and succession planning	• Of employees promoted globally, **58.9%** self-reported as female and **41.1%** self-reported as male, while employees who self-identified as racially or ethnically diverse represented **45.5%** of U.S. promotions and U.S. employees who self-reported as White represented **54.0%** of U.S. promotions • Launched resource library for inclusion excellence to further enhance team connection, awareness and education on various topics related to inclusion and belonging
Implement a robust and comprehensive health and safety management system framework and audit process	• Launched the Global Environmental, Health and Safety (EHS) Internal Audit program • Developed and deployed new standards including Office Safety and Asphyxiant Gas Monitoring

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. 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.

COURAGEOUS

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

RELENTLESS

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

DYNAMIC

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

POSSIBILITY

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

Human Capital Development

Ultragenyx has more than **1,290** employees globally. We are dedicated to building a global 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 6% in 2024, which is below the U.S. and global averages for our industry (according to Aon Radford's Salary Increase and Turnover Studies).

We believe we have created a culture that supports us in performing at our very best to support our mission to transform the lives of people living with rare diseases. 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 seek to 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.

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 inclusion and belonging 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, happy hours, milestone celebrations, summer and holiday events, and more. These events are held both virtually and in-person.

Turnover in 2024

Total Turnover Rate	**10.9%**
Voluntary Turnover Rate	**6.0%**

UltraPerformance Management

We refer to our performance management system as UltraPerformance, which represents a holistic approach to management by evaluating performance against a set of 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, receive further development and advance within the company.

The core of UltraPerformance involves establishing and periodically reviewing annual employee objectives 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, 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.**

Enhancing Performance Management

Our UltraPerformance management 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

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, which 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 an 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.

In 2024, we developed and piloted a new executive education program, Executive Edge, to support senior leaders to excel in leading through the complexity of our constantly changing business landscape. They received a 360 evaluation with customized coaching and multiple personalized assessments in change readiness and people management to identify high-value areas for growth. They attended hands-on workshops to immerse in learning and apply the tools to real, current organizational challenges, then received blueprints for sharing and amplifying what they learned with their teams. To sustain and deepen their impact, they also benefited from in-depth group coaching and access to an online library of resources.

Impact

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

- Raised our annual engagement index and inclusion and belonging 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

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 to Senior Leaders
Teaches skills for developing clear messaging, memorable content and effective delivery style to business presenters delivering senior-level presentations

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. BJ Miller, Bonnie Wan, Dr. Shauna Shapiro and Justin Michael Williams.

In 2024

*We offered **62** employee development workshops*

*When surveyed about their experience, **99%** of employees who attended at least one development workshop found it to be useful for their job*

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.

To date, we have trained hundreds of employees on the tools and methodologies of Lean Six Sigma. Additionally, we curated and customized a set of Lean Six Sigma tools and resources for projects so that our teams can be 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. And finally, we launched a Continuous Improvement Community of Practice to bring interested practitioners together to share tools, learnings, and best practices.

Green Belt Project Spotlight: Improving the Talent Acquisition Interview Process

Problem:

The time between a candidate's first interview with a hiring manager to completion of a panel interview takes an average of 23 days and many candidates are withdrawing their applications while in the interview process for other opportunities.

Solution:

Streamlined the interview process by implementing the following changes:

- Reduced the number of interview panel members from approximately 10 to 6
- Decreased the number of onsite interviews to the 1-2 finalists that we are willing to make an offer
- Requested panel members to complete scorecard feedback within 24 hours

Results:

- Reduced the cycle time from hiring manager interview to completion of the panel interview to 8 calendar days
- Reduced our travel expense by approximately $1,400 per candidate for each candidate not brought onsite for a final interview
- Saved our interviewers an average of 3 hours of time per candidate that could be reallocated to business needs by reducing the number of interviewers on each panel

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.

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 2024, more than 300 sessions of coaching were provided to our global workforce through this program. Participants rated the quality of sessions an average 5 out of 5 stars.

Executive Edge

At a time when our industry faces increased complexity, it's more important than ever to fully equip our leaders with the skills required to effectively navigate this environment. Executive Edge, a new program launched in 2024, aims to identify exceptional leaders across our business and help them elevate their impact. The program includes modules focused on strategic decision-making, navigating and leading change, mobilization of teams, and consistent demonstration of our company values and culture.

Ultimately, we want leaders to walk away from this program with new tools to unlock and maximize potential from their teams, fostering empowerment and excellence in what they do. This year across the company, 26 VPs and SVPs participated in the pilot program, which we expect to expand to new cohorts in the years ahead.

Emerging Talent Programs

Ultragenyx is committed to fostering the next generation of STEM talent by providing a range of opportunities to learn from a world-class team at the forefront of biotechnology.

Through mentorship, hands-on experience, and exposure to advanced scientific techniques, we aim to equip students with the skills they need to succeed while building a pipeline of talent ready to make meaningful contributions to the biopharmaceutical industry.

In 2024, we strengthened our partnership with Biotech Partners, a program designed to equip underrepresented youth in the Bay Area with the skills and confidence to pursue STEM careers. As part of this initiative, we hosted **11** rising seniors from Oakland Technical High School for an eight-week summer internship across various departments at our Bay Area locations.

As part of our Emerging Talent program, we also hosted undergraduate, graduate and MBA students in our internship, co-op and APPE programs across our North American locations, and sponsored our first Purdue University Fellow within the Regulatory Affairs team in 2024. Emerging Talent participants engaged in our 'Business of Biotech' learning series, executive leadership fireside chats, and a final poster presentation highlighting their achievements. We hosted a total of **35** Emerging Talent program participants in 2024.

To further inspire early talent, we welcomed over 100 local high school students to our Novato lab, where they gained insights into the rare disease landscape, cutting-edge biotech technology and the scientists behind our therapies.

Through our programs, we aimed to expand our global presence by engaging with students in the Latin America and Europe, Middle East and Africa regions in our internship program, reinforcing our ongoing commitment to empower the next generation of STEM leaders worldwide.

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 "YourVoice" employee engagement survey as a listening platform to pinpoint key areas of focus and take action on opportunities that are expected to improve employee experience and engagement. We solicit feedback from our employees at least once per year so that we can hear about their experience and identify ways to improve in areas such as connection, collaboration, meeting effectiveness, continuous improvement and more.

In addition, we use the survey to measure three people-related corporate goals. The areas we measure are focused on:

- Enhancing employee engagement at Ultragenyx
- Strengthening how our managers and leaders support Inclusion Excellence in the workplace
- Improving health & well-being in the workplace to reduce stress and balance work and personal life

We conducted our most recent survey in October 2024 and **78%** of eligible employees[1] participated in the survey. In this survey, we included a smaller set of targeted questions on topics including people-related corporate goals and key strategic initiatives and efforts. Based on the results from the recent survey, we achieved or outperformed on all three of our "Thriving Culture" 2024 corporate goals, including employee engagement, inclusion and health and 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 continues to be a key strength for us as a company, as demonstrated by employees having a positive perception of their direct manager's support of an inclusive environment, and managers creating an environment where people can grow and develop.
- Our employees have confidence and trust in our leadership team and their commitment to people and purpose.

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

[1]Employees who have worked for Ultragenyx for at least 90 days were eligible to participate in the survey.

Employee Engagement (cont.)

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 88% overall in 2024, with 95% of our employees responding that they are proud to work at Ultragenyx, 88% feel a personal sense of accomplishment from their work, and 85% intend to stay at Ultragenyx for at least the next 12 months and recommend Ultragenyx as a good place to work.

External Recognition

In addition to strong employee engagement results, our achievements over the past year have been consistently recognized. Notably, we were named one of the Top Places to Work in the USA for two consecutive years and recognized by the Boston Globe for the third consecutive year, alongside accolades from the San Francisco Chronicle. We also received the Cultural Excellence Awards from Top Workplaces for Employee Appreciation, Work-Life Flexibility, Professional Development, Leadership, Compensation & Benefits, and Purpose & Values. Mogul acknowledged us as one of the top 100 workplaces for diverse professionals. Additionally, we were recognized as one of the Best Places for Working Parents in Massachusetts.

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 & Ultra Sharp Recognition Program and the Rare Pearl Spotlight Award – presented by the EmpowerX Women in Biotech employee community group – 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.

Ultra Sharp

U EARNED IT

rare pearl

UltraRare Award

Inclusion Excellence

We are committed to fostering an 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 multi-year vision for developing an inclusive environment aims at going beyond words, with meaningful, tangible actions and results that reflect our values. To realize this vision, we have identified four strategic 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, integrating a culture of inclusion and belonging into our activities.
- **Organizational Talent**
  We focus on attracting, developing and promoting the best talent.
- **Accountability and Transparency**
  We commit to working to drive an inclusive culture, integrate it into our operations, and transparently share our progress.

Employee Community Groups

Employee community groups are voluntary, employee-led groups that contribute to the culture at Ultragenyx and are equipped with a charter, mission and support team.

Community groups are created with the purpose of building inclusive spaces for individuals who share common aspects of identity, are open to all employees and are structured with the objective of advancing our business's goals.

Members of an Ultragenyx community group hosted a Black Professionals in Biopharma Networking event in December 2024

A highlight from our recent accomplishments:

Launched Inclusion Excellence Resource Library

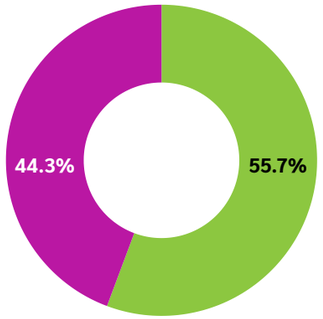
With input from our community, leaders of employee groups and executives, we built a one-stop-shop library offering teams and individuals tools to deepen connection and belonging. This library is a key initiative to further the people and leadership strategic pillars outlined above. Teams can learn how to add conversations on relevant topics to their existing meetings in accessible-yet-powerful ways. Leaders can examine options to enhance team connection and awareness and select workshops on various topics. The library also promotes resources from our community groups, and partners with them in elevating the level of awareness throughout the employee population.

Workforce Data

Total Number of Employees: 1,294*

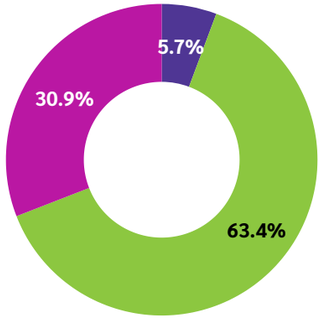
Gender Breakdown (Total Employees)

- Employees who self-reported as female
- Employees who self-reported as male



Age Breakdown (Total Employees)

- <30 years of age
- 30-50 years of age
- >50 years of age



Number of Employees in Management Positions: **457**

50.3%
% of employees in all management positions who self-reported as female**

49.7%
% of employees in all management positions who self-reported as male**

41.2%
% of employees in VP-level positions and above who self-reported as female

58.8%
% of employees in VP-level positions and above who self-reported as male

54.7%
% of employees in STEM-related positions who self-reported as female***

45.3%
% of employees in STEM-related positions who self-reported as male***

Number of Employees Promoted: **219**

58.9%
% of employees promoted who self-reported as female

41.1%
% of employees promoted who self-reported as male

45.5%
% of U.S. employees promoted who self-reported and identified as racially or ethnically diverse

54.0%
% of U.S. employees promoted who self-reported and identified as White

Number of Employees on the Executive Leadership Team (XLT): **9**

Number of XLT members who self-reported as female

Number of XLT member who self-reported as male

Number of XLT members who self-reported and identified as racially or ethnically diverse

Number of XLT members who self-reported and identified as White

*All data as of December 31, 2024.

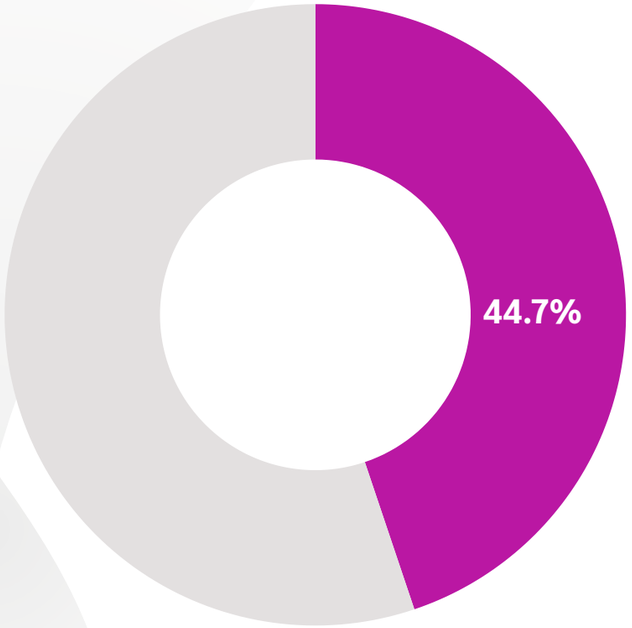
**Percentage of total management positions. Total management positions include all management positions at Ultragenyx, including employees in manager roles.

***Percentage of total STEM positions.

Workforce Data (cont.)

Total Number of U.S. Employees: 1,081*

% of U.S. employees who self-reported and identified as racially or ethnically diverse



Breakdown of U.S. employees by self-reported ethnicity

27.6%	Asian
5.6%	Black / African American
8.1%	Hispanic or Latino
54.3%	White
3.4%	American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races
1.0%	Not Specified

Number of U.S. employees in management positions: **395**

25.1%	Asian
3.0%	Black / African American
5.8%	Hispanic or Latino
63.8%	White
1.8%	American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races
0.5%	Not Specified

According to our 2024 internal employee* engagement survey:

93% of our employees* feel that their managers support inclusion in the workplace

90% feel that their managers model inclusive behaviors

87% feel that their managers create an environment where people feel they belong

*Refers to employees that participated in the survey.

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

Recruiting Top Talent

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 goal is to build a broad pipeline of candidates who feel a sense of belonging from the very start of their relationship with Ultragenyx. We do so by implementing thoughtful recruitment strategies that include assembling well-rounded interview teams to focus on “screening in” talent rather than “screening out.” We also host virtual open houses and career fairs to expand our outreach and connect with a broader range of potential candidates.

We provide interview skills training for our employees, equipping them with tools to conduct inclusive interviews. This training supports our company goals of being an employer of choice for the best talent and creating a sense of belonging for all individuals.

These intentional efforts have resulted in an increase in the depth and range of candidates interviewing for roles.

In 2024

*Nearly **30%** of new hires came through our employee referral program*

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.

2024 Highlights:

Launched Global EHS Internal Audit program to assess site EHS management system effectiveness and conformance with regulatory and internal EHS requirements at our key facilities.

Implemented Workplace Violence Prevention Plan in partnership with Global Security to support proper response planning for violent incidents, track and investigate workplace violence incidents, provide workplace violence training to all employees, and regularly conduct security hazard assessments.

Deployed Driver Safety training for personnel regularly operating motor vehicles on behalf of Ultragenyx to familiarize individuals with defensive driving techniques, safe driving practices and company policies.

Partnered with Human Resources to update employee job descriptions with the physical requirements considered essential to the performance of each job at our primary U.S. sites.

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

Obtained a Radioactive Materials License at our Somerville, Massachusetts location to allow us to use radio-labeled synthetic organic molecules to perform enzymatic assays, significantly enhancing our capability to develop advance innovative medicines for rare and ultrarare diseases. A comprehensive radiation protection program has been implemented to support the safe conduct of our activities and operations involving ionizing radiation.

Enhanced our safety culture by establishing and communicating targets to increase safety observation reporting, improving Corrective and Preventive Action (CAPA) closure rates, increasing proactive ergonomic assessments, and mitigating high risk issues including asphyxiant gases, isoflurane, roof safety, fall protection, laser safety and potent pharmaceutical compound handling.

Occupational Health, Safety and Wellness (cont.)

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 opportunitiesfor ERPP improvement.

We also have a mass notification system designed to rapidly alert our workforce of emergency situations or potential threats and direct them on how to respond.

2024 Safety Data*	
Lost Time Incident Frequency Rate (LTIFR)**	**1.20**
Total Recordable Incident Rate (TRIR)***	**0.72**
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

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 held at our primary sites at no cost for Ultragenyx employees and contractors. Personnel may also request flu shots from our Occupational Health provider if a flu clinic is not available at their location. Flu shots are covered for free in the U.S. for employees plus family members or 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.

Blood Drives involving more than 70 Ultragenyx volunteers collected enough blood to help potentially save the lives of over 200 people injured in accidents or undergoing medical treatment.

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.

Expanded preventative ergonomic assessments for remote and international employees by conducting ergonomic assessments for computer workstations as well as laboratory ergonomic seminars at our research facilities.

Employee Compensation and Benefits

We believe 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 our employees worldwide, an integral component of our broader focus on inclusion excellence. 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 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 designed to enable a comprehensive examination of compensation across roles, departments and demographic groups. With real-time monitoring capabilities, we strive to 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 sharing 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 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.

More information can be found on our **Career webpage**.

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

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	2024 Progress Highlights
Support charitable organizations that contribute meaningfully to the health and well-being of the communities where we operate and beyond	• Approved approximately **$3 million** in charitable donations, independent medical education and health-related grants across more than **20** countries • Provided support to organizations focused on **emergency and humanitarian relief**, including contributions to the Peace Boat Disaster Relief Emergency Fund, Governo Do Estado Rio Grande do Sul, Community Foundation of Western North Carolina, and Banco de Alimentos de Valencia
Expand opportunities for employee volunteerism	• Partnered with Life Science Cares and other organizations to host the 2nd annual **Ultragenyx Global Days of Service** with employees volunteering in almost 60 service projects • More than **60%** of employees signed up to participate in Days of Service projects • Employees completed more than **3,700** paid volunteer hours throughout the year

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 believe 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 contributing to local initiatives. In 2024, approximately 600 full-time and part-time employees recorded donating more than 3,700 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 community group 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, Canada, Japan, the Latin America Region and the Europe/Middle East/Africa Region.

In 2024

*Approved approximately **$3 million** in charitable donations, independent medical education and health-related grants across more than **20** countries.*

*Employees completed almost **60** service projects around the world during our Days of Service campaign.*

Ultragenyx Global Days of Service

During our 2024 Global Days of Service, more than 60% 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 nearly 60 service projects around the world.

Key highlights from this initiative include:

Significant Impact and Outreach

- Completing numerous environmental projects to enhance local parks and conservation areas
- Packing domestic aid and hygiene kits to be delivered to various shelters, food banks and community support agencies
- Preparing meals and packing grocery boxes for organizations addressing food insecurity and supporting vulnerable populations in local communities
- Building STEM/STEAM kits to be delivered to youth within underrepresented communities to help foster interest and excitement in STEM pathways

Enjoyable and Rewarding Experiences

Employees across various locations actively participated in varied 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.

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 2024, Ultragenyx proudly supported:

Raising Money to Directly Support Rare Disease Advocacy
(New York)

Ultragenyx hosted the 2024 Rare Affair fundraising event, now in its 12th year. Each year, the Rare Affair has focused on raising money and visibility for a different rare disease advocacy organization. The 2024 event gathered hundreds of biotech executives, venture capitalists and rare disease advocates to raise money for the Wilson Disease Association to support its mission to provide support and hope to people impacted by Wilson Disease.

A Safe Camp Experience for Children with Serious Illness
(Washington)

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.

Enhancing Lives of People Living with Disabilities
(U.S.)

Provided funding to Canine Companions, a national organization supporting people with disabilities by providing highly trained service dogs.

Home Away from Home for Families of Seriously Ill Children
(California)

Supported Family House, which provides free, temporary housing to families of seriously ill children receiving treatment at the University of California San Francisco Benioff Children's Hospital.

Improving Quality of Life for Families
(Mexico)

Supported Fundacion IMSS, which supports health research and social development for the Mexican Social Security Institute and their families. Ultragenyx's donation was directed to buying FIMSS Executive Accompaniment Chair-Beds for caregivers to have a place to sit/sleep. (Mexico)

Peer Education and Support for Parents of Children with Special Needs
(Pennsylvania)

Parent to Parent USA is dedicated to offering emotional and informational support to families with children who have special needs. Ultragenyx's donation was directed to the Support Parent Summit, where volunteers undergo a comprehensive, evidence-based training so they can go beyond empathizing with, and serving, their peers.

Raregivers™ .global

Raregivers is a global network that delivers mental health and wellness services to caregivers, patients and professionals in rare, chronic and complex disease communities – from sustainable psychosocial training and transformative retreats to a connective peer-to-peer network. As part of the Ultragenyx Global Days of Service, the company committed to donating $25,000 to a charitable organization nominated by employees if more than 40% of employees recorded volunteer hours in UltiPro during Days of Service. This goal was met, and Raregivers, was selected as the employee-nominated non-profit to receive the Ultragenyx Global Days of Service Award. The award will support Raregivers' wide range of family support programs, including the global expansion of its Emotional Journey Map & Guidebook, a multilingual, interactive guide for caregivers, patients, professionals and other individuals and organizations involved in supporting those living with rare disease.

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.

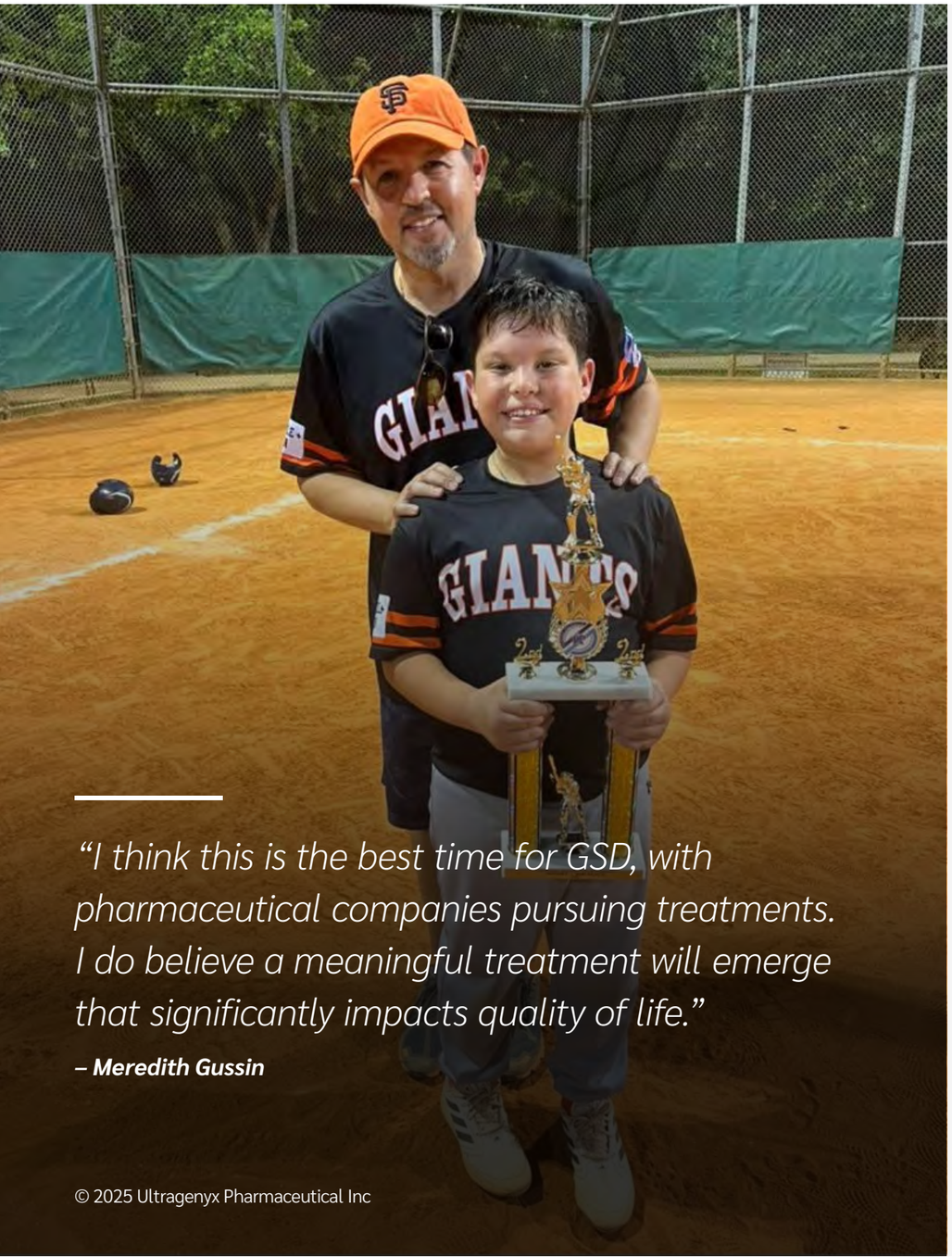
To promote compliance with applicable standards and guidelines, our funding undergoes rigorous evaluation, that is based on:

- 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 2024

*Grants were provided to more than **180** organizations, across approximately **250** programs, focused on medical education, rare disease awareness, and advocacy efforts in over **20** countries.*



"I think this is the best time for GSD, with pharmaceutical companies pursuing treatments. I do believe a meaningful treatment will emerge that significantly impacts quality of life."

– Meredith Gussin

JAMIE'S STORY

Living with GSDIa

For those living with glycogen storage disease type Ia (GSDIa) – a serious, ultrarare genetic disease resulting in the inability to regulate blood sugar – a single moment's delay in ingesting oral glucose replacement therapy (raw cornstarch) can be life-threatening.

This is the reality Jamie Gussin and his family have faced since he was born in 2012. Shortly after birth, Jaime experienced dangerously low blood sugar levels. Despite initial reassurances from doctors that everything was normal, his mother Meredith instinctually knew something was wrong when Jamie required around-the-clock feeding to remain awake and engaged. She pushed for answers, and six weeks later Jamie was diagnosed with GSDIa.

The first few years of Jamie's life were incredibly challenging as the family grappled with managing the daily burden of the disease. After struggling to constantly monitor Jamie's blood sugar levels – testing his blood up to 20 times a day – eventually, his parents made the difficult decision to get him a gastrostomy tube to provide the nutrients he needed. "There was a lot of grief overcome, having a child with a device in his body. But it has been an absolute lifesaver," Meredith shares. Yet, even with the support of the device, Jamie continued to face a number of risks and challenges managing his disease.

As Jamie has grown older, the family has always made an effort to help him experience life as a typical preteen, even while he faces significant limitations like not being able to sleep over at friends' houses or go to camp.

Jamie and his dad bond over their love for Chicago sports and enjoy playing baseball, basketball and tennis together. Their family also loves to travel, though the logistics of managing Jamie's care while on the road require extensive preparation to ensure they have enough cornstarch and emergency supplies on hand.

The Gussins have also become deeply involved in the GSD community, with Meredith serving on the board of The Children's Fund, an organization that advocates for continued research for children like Jamie. She remains hopeful that future medical advances like gene therapy will allow him and others with GSDIa to live fuller, freer lives. "We appreciate every moment – the time we get to be together, our joy, health and happiness," she explains. "While there are a lot of fears, we live our lives and try to give him the most wonderful experiences we can."

Most recently, the family embarked on a trip to Egypt. With careful planning and support, they made the most of their time together, knowing that every moment counts. Nevertheless, Jamie became sick with a stomach bug and they ended up with an emergency hospital stay during their layover home in Germany for five nights. Emergencies are a part of life with GSDIa and the fear never leaves the Gussin family. The hope for a cure is more intense now since this incident.

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 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.

2024 HIGHLIGHTS

Revolutionizing Aid Delivery
Supported Global Medic Canada, which provides people affected by poverty, disaster or conflict with humanitarian aid. (Canada)

Supporting Children Across Brazil
Donated to Gotas de Flor com Amor, a social program serving the children of Brazil and their families with educational, cultural and artistic opportunities, counseling, and healthcare. (Brazil)

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. (California, Massachusetts)

Fostering Belonging for Children in Need
Sponsored SOS Kinderdorf, an organization focused on ensuring that children and young people without parental care or at risk of losing it grow up with the relationships and support they need to become their strongest selves. (Austria)

Ultragenyx Recognized by Ritter Center for Commitment to Unseen Communities

In May, the Ritter Center, which provides medical, housing and food services to Marin County, California residents experiencing economic insecurity and homelessness, recognized Ultragenyx as its 2024 Corporate Impact Honoree for our dedication to improving the lives of communities that can often feel invisible, both patients with rare diseases and those who are unhoused.

At the 4th Annual Under the Stars: Dare to Dream event, our organizations came together with 200 members of the local community as well as San Rafael Mayor Kate Colin, raising $150,000 for center. The event followed our Ultragenyx Global Days of Service, where our team volunteered their time to support the non-profit's efforts.

"Generosity is a core value at Ultragenyx, and this is reflected by our team members day in and day out. The Ultragiving employee community group at our company has driven a rich and substantive collaboration with the Ritter Center for years and one that many of us have been personally involved in, which makes this recognition meaningful for all of us," shares Ernie Meyer, chief human resources officer and executive vice president, about the honor.

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 a variety of young minds.

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

2024 HIGHLIGHTS

California-based support:

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

Expanding horizons in STEM
Sponsored Expanding Your Horizons Sonoma County, a one-day conference for 7th and 8th grade girls consisting of hands-on STEM workshops led by local women working in the sciences.

Ensuring Access to the Arts
Donated to Marin School of the Arts, an inclusive and supportive specialized arts program within Novato High School in California.

Sparking Curiosity in Science
Participated in the Buck Institute for Research on Aging's North Bay Science Discovery Day, a free science festival designed to spark children's interest in science, technology, engineering and mathematics.

Changing the Face of Science
Supported Scientific Adventures for Girls' afterschool STEM programs for TK-6th grade students, with a special focus on girls of color from low-income families and communities.

Massachusetts-based support:

Promoting Creativity in STEAM
In Massachusetts, we supported students chosen by Bedford Creativity to compete in the Destination Imagination Global Finals. This competition encourages students to apply their STEAM knowledge creatively to solve complex, real-world problems.

Supporting the Next Generation of Scientists
Sponsored the Biomedical Science Careers Program, which provides students with the information and support needed to achieve their goals through conferences and workshops.

Ensuring Future Success in Life Sciences
Supported the Massachusetts Biotechnology Education Foundation's mission is to build a sustainable life sciences workforce in the region through educational programs that engage and excite teachers and students.

Enabling Equitable Access to Education
Provided funding to Project LEARN, Inc., to support innovative programs to students, families and educators in Lowell, Massachusetts.

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 employee community group 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.

Supporting Safe Communities
Donated to the holiday fundraiser hosted by the California Highway Patrol, which raised funds for CAHP Widows and Orphans Trust Fund, the CHP 1199 Foundation, Mothers Against Drunk Driving and Sober Graduation programs throughout Marin County.

Fighting Poverty to Uplift Lives
Supported Samaritan House of San Mateo County to deliver essential services and personalized support to those living in poverty to help families not only to be fed, clothed, healthy and housed but gain the stability to become a successful part of the local community.

Helping Mothers and Children Sparkle
Donated funding to the Sparkle Foundation, which aims to support single mothers and their children with scholarship funds supporting them across the fields of sports, art, education and technology.

Inspiring Confidence and Joy in Children
Provided support to Wonderfund, which serves Massachusetts children and families who have been impacted by abuse and neglect with emergency aid services, enrichment opportunities and everyday essentials.

Team members in Massachusetts volunteered at Cradles to Crayons during Ultragenyx's 2024 Days of Service

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 2024, we directed our support to organizations dedicated to offering emergency and humanitarian relief in the aftermath of several natural disasters:

- Donated to the **Peace Boat Disaster Relief Emergency Fund**, which supported communities following the Ishikawa Noto Peninsula Earthquake in Japan.
- Contributed to **Governo Do Estado Rio Grande do Sul**, providing aid following catastrophic flooding of Rio Grande do Sul in Brazil.
- Supported the **Community Foundation of Western North Carolina** with emergency and disaster relief following Hurricane Helene.
- Assisted the **Banco de Alimentos de Valencia (Food Bank of Valencia)** with relief following flooding in Spain.

Employees in Canada packed aid kits for Global Medic during Ultragenyx's 2024 Days of Service

Planet

Managing our environmental impact and promoting sustainability

We are **committed** to implementing an environmental strategy that helps to minimize our environmental footprint across our business.

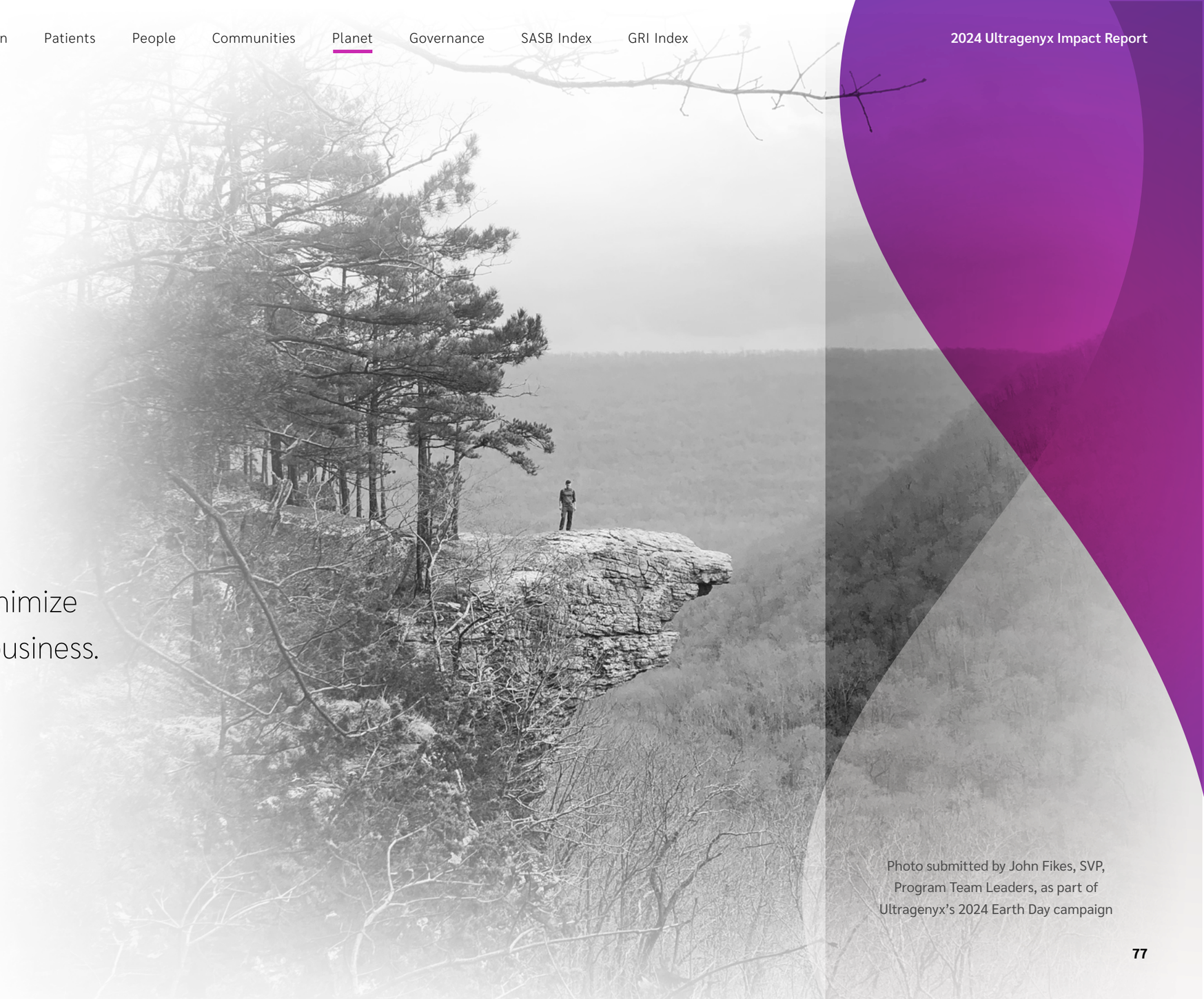


Photo submitted by John Fikes, SVP, Program Team Leaders, as part of Ultragenyx's 2024 Earth Day campaign

Aspiration

To conduct business in an environmentally responsible manner and strive to continuously improve our performance to benefit our employees, patient and physician communities, the localities where we work, and the environment.

Our Objectives	2024 Progress Highlights
Continue to implement improvements to reduce our environmental footprint	• Purchased **100% renewable electricity** at our headquarter campus in Novato, California • Strengthened our waste vendor partnerships to expand diversion and circularization programs • Achieved **My Green Lab Green certification** at our Translational Sciences lab in Novato, California
Develop an environmental strategy	• Continued and expanded the collection of environmental data across our facilities • **Engaged with employees** to increase awareness of sustainability practices both within Ultragenyx and outside the workplace

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 seek to design our facilities with sustainability in mind.

We utilize policies and procedures for environmental data collection in alignment with the Greenhouse Gas (GHG) Protocol, International Organization for Standardization (ISO) and other best practice standards and frameworks. The policies set standards for tracking environmental information and 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. This information enables our team to monitor our performance.

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.

My Green Lab 'Green' Certification in Novato, California

My Green Lab is a nonprofit organization made "for scientists, by scientists" that develops, implements and inspires sustainable lab standards. In 2024, our Translational Sciences team successfully completed the company's first My Green Lab certification program in the Novato, California lab space. The effort was driven by a cross-functional team of scientists as well as EHS&S, Lab Operations and Facilities professionals.

Based on results from the baseline assessment, the team identified and implemented numerous improvements focused on energy use, waste management and lab user behavior. Some examples include:

- A waste assessment and updated waste management processes to divert nearly all non-hazardous single use plastics from landfill.
- Implemented a polystyrene recycling program.
- Participated in the My Green Lab Freezer Challenge to reduce freezer energy consumption and increase freezer longevity.
- Performed an HVAC air balancing analysis to increase ventilation efficiency and reduce building energy consumption while still meeting minimum requirements for air circulation within the lab space.
- Conducted an energy audit to obtain energy consumption metrics for lab-specific equipment.
- Initiated an equipment shutdown program to decrease the lab's plug load and increase equipment longevity.

Photo submitted by Eric Knight, Executive Director of Global EHS

Following a rigorous six-month project schedule, the team completed the final certification assessment in July 2024 and achieved a 'Green' level – the highest certification achievement level recognized by My Green Lab.

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 2024, Ultragenyx purchased **1,945 MWh** of renewable electricity through this initiative. Additionally, we purchase renewable electricity for a portion of our energy use at our sites in Brisbane, California and Bedford, Massachusetts.

Sustainable Transportation

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

Eco-Efficient Facility Operations

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.

Photo submitted by Lisa Moore, Sr. Director, Program Management

Engaging Our Workforce in Sustainability

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

In recognition of Earth Day, in April 2024, employees at our Gene Therapy Manufacturing Facility were invited to an unveiling celebration of the site's new outdoor seating area. The tables and benches purchased for this area were made from plastic lumber resulting from the Red2Green program. All Ultragenyx sites in Massachusetts utilize the Red 2Green program to recycle regulated medical waste (RMW), which would otherwise be treated and disposed of via incineration. In 2024, we diverted more than 60,000lbs of RMW from Ultragenyx operations through the Red2Green program.

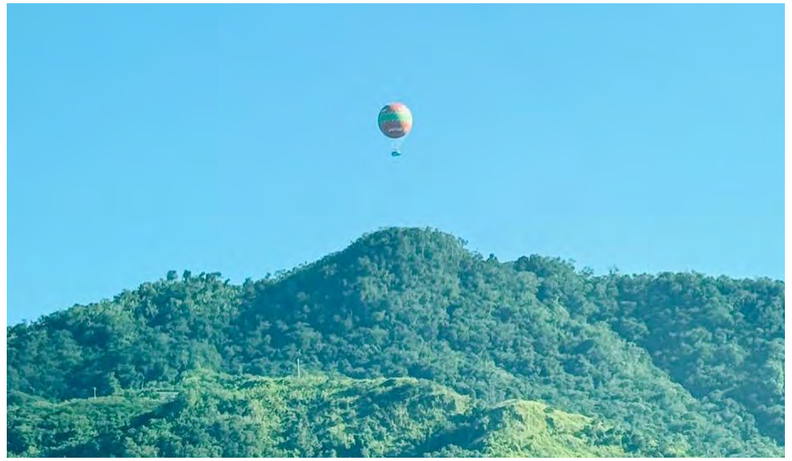


Photo submitted by Mayte Vega-Torres, Lab Operations Technician

Additionally, employees were invited to submit original photos of nature and the environment in recognition of Earth Month in April. For each employee that submitted a photo, the Ultragenyx EHS&S team donated one tree to be planted via The Canopy Project. More than 125 employees participated in this initiative, and we are proud to include several employee-submitted photos within the Planet chapter of this report.

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 to recycle pipette tip boxes, rigid lab plastics and polystyrene. Through this partnership, Ultragenyx diverted more than 2,500 lbs of waste in 2024.

We also partner with vendors to recycle Regulated Medical Waste into plastic lumber and in 2024 diverted more than 60,000 lbs of RMW from incineration. Furthermore, our lab and manufacturing facilities have introduced reusable gowns, garments and lab coats for use within lab spaces and clean rooms. Any single use gowns are to be sent for recycling along with single use gloves.

In 2024

*More than **60,000 lbs** of laboratory waste diverted from incineration through our recycling partnerships.*

Photo submitted by Juan Ignacio Pérez, Associate Director, Market Access & Government Affairs

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.

Supply Chain Sustainability

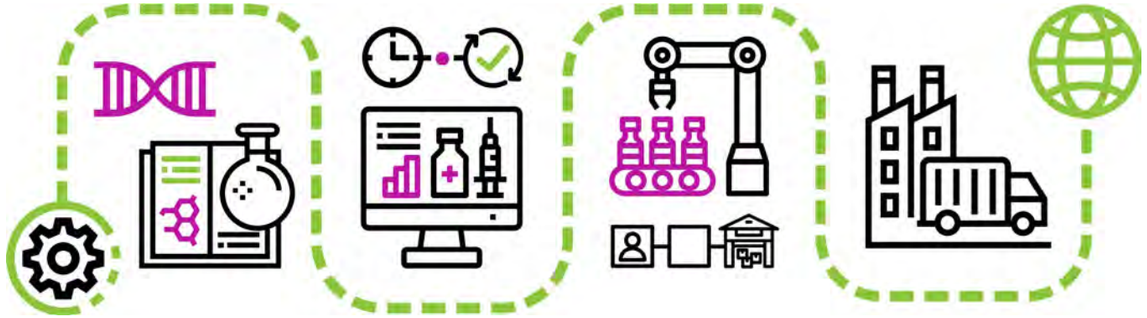
Our commitment to operating in an environmentally responsible manner extends to our supply chain.

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 periodically 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:

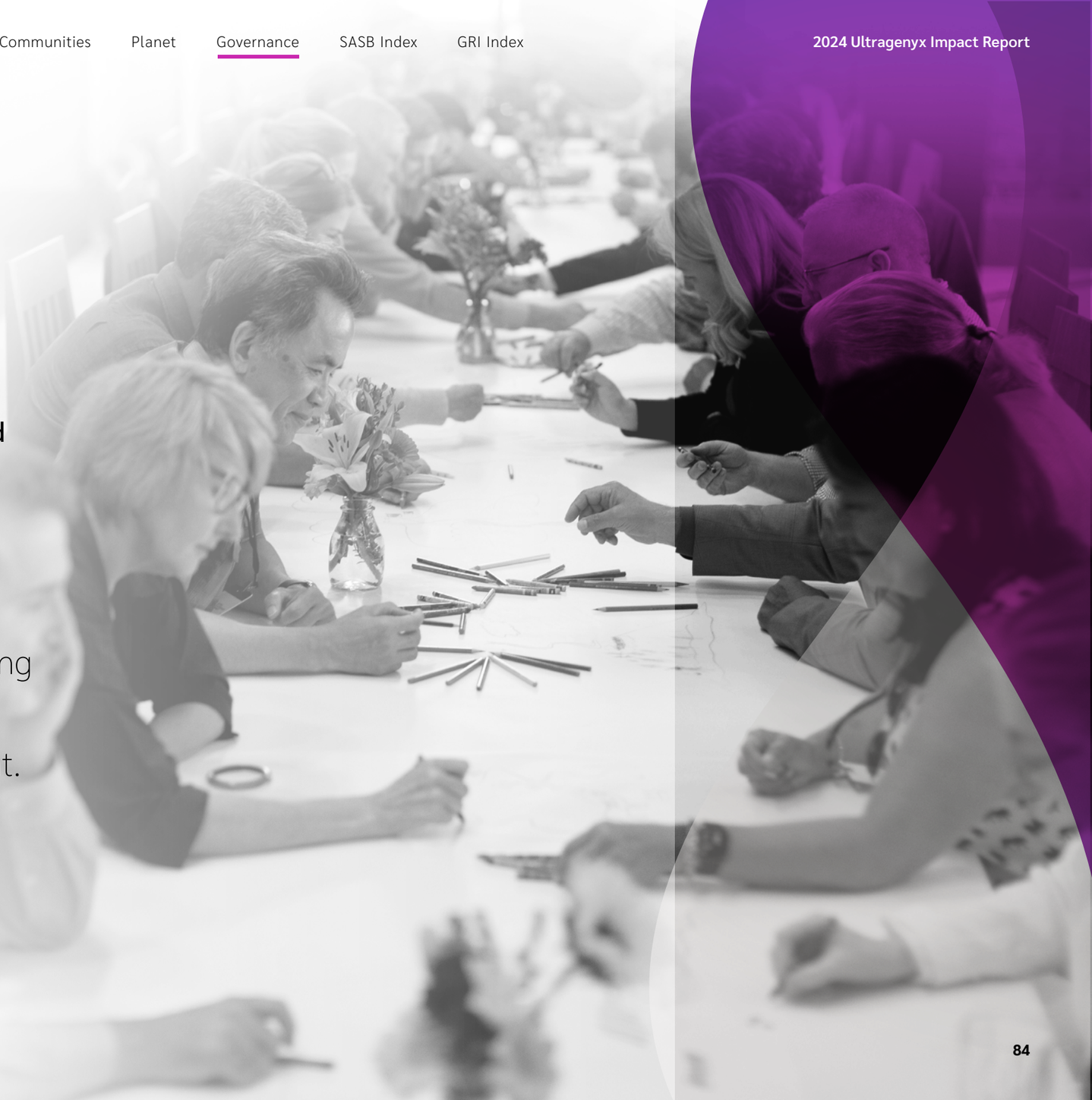
- Consolidating small volume orders to help reduce the number of shipments and prioritizing direct-to-patient shipments whenever feasible to eliminate the need for extra transportation and handling.
- Conducting long-term stability studies for our products with the goal to maximize shelf life for our 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.

In 2024, our Product Supply team made efficiency improvements within our Dojolvi supply chain that resulted in significant carbon emissions savings. These improvements include shipping bulk containers from the Drug Substance manufacturing facility to the Drug Product manufacturing facility using ocean freight as opposed to air freight. Additionally, we relocated the fill site to Canada. We estimate that these improvements will yield an annual carbon emissions savings of over 36 MTCO2eq compared to the previous process.



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	2024 Progress Highlights
Act responsibly and with integrity and provide annual, targeted training to our workforce on Ultragenyx's ethical standards	• **Over 90%** of 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	• 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 supplierss	• Maintained a **Global Human Rights policy**, aimed at holding ourselves and our vendors to its standards • Continued compliance with the **U.S. Department of Treasury's OFAC regulations** and other applicable international equivalent 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.

Board Infromation

- *Average age of 61 years*
- *Average tenure of 8 years*

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 is comprised of 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 May 1, 2025, 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**.

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

Best Practices

- Ongoing shareholder engagement program
- Board that represents varied talents, skills, backgrounds and expertise
- Minimum stock ownership requirements for directors and named executive officers
- 100% attendance of board and committee meetings in 2024 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 SEC requirements
- Annual board and committee self-evaluations
- Prohibition against hedging transactions

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 2024, the board and the committees reviewed with management various risks and mitigation strategies, including those related to:

- 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 governance 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 **2024 Annual Report**.

Business Continuity Management and Disaster Recovery

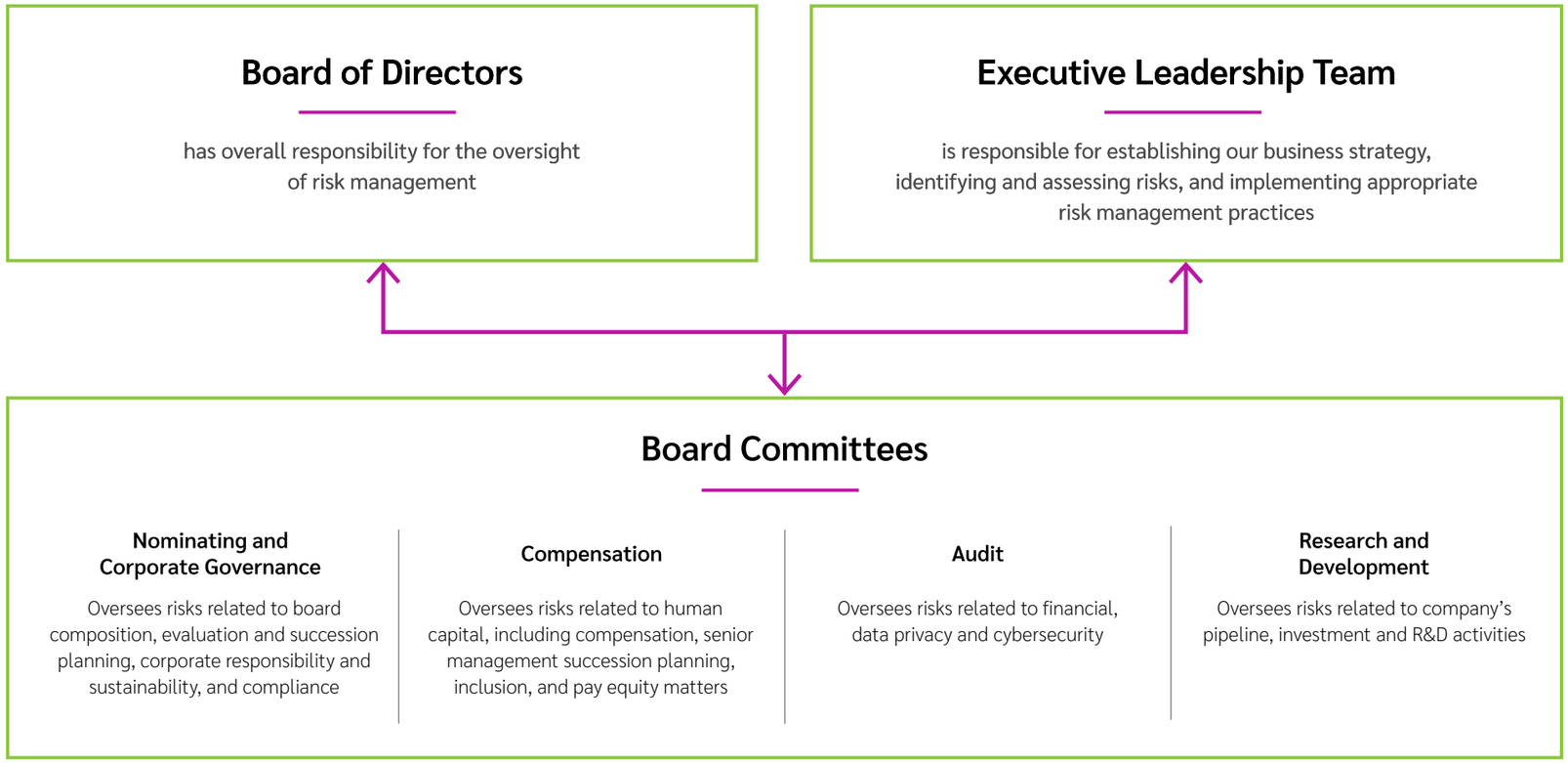
Ultragenyx has a Business Continuity Management (BCM) and Disaster Recovery (DR) program, guided by best practices from organizations such as the International Organization for Standardization (ISO), including ISO 22301. The overarching goal is to continually improve practices and foster the uninterrupted 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
- Crisis management and communications for coordinating company-level responses

Business Continuity Plans are annually reviewed and regularly updated to reflect the evolving business environment. Additionally, we have IT DR 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.

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 strive to enforce our policies and requirements with appropriate disciplinary actions, when necessary, and to take a zero-tolerance approach to violations 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 global, as well as regional, compliance committees 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, leveraging live observations and data analytics.
- 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.

Ethics and Integrity (cont.)

We consistently perform a range of monitoring and auditing activities across business operations, in collaboration with our internal audit, finance, compliance and legal teams. We use technology and automated tools to monitor and report on compliance matters. In addition, we distribute an annual compliance culture survey and conduct annual risk assessments to understand our compliance culture and potential risk areas.

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 their understanding of the code of conduct. Every year, an annual compliance culture survey is sent to all employees to gauge our compliance culture. In 2024, **100%** of full-time employees have received training on the code of conduct and other ethical standards within the last three years.

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 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 helps to ensure 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 are responsible for taking 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.

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 "3 Rs:"

Replace: Use non-animal methods for experiments whenever feasible, 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, including 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 animal care, welfare and scientific programs for research. The IACUC reviews 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 international 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, German, Japanese, Italian and Turkish**. Our compliance program is overseen by a management-level global compliance committee, as well as regional compliance committees, that provide guidance with respect to healthcare law compliance. The global compliance committee meets at least quarterly.

Ultragenyx is committed to meeting all applicable 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 varied sources – including patients, clinical trial participants, customers, healthcare providers and our employees – are integral to our operations.

In 2024

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

We are dedicated to protecting the privacy and integrity of this information and related holders by adhering to applicable 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 protection 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 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.

Cybersecurity

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

Governance	Training and Awareness	Business Resilience and Compliance	Identifying and Mitigating Cybersecurity Risks
• 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 in part by industry standards and best practices, including 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.	• We provide information security training to employees at least 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 regular phishing exercises, 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.	• We have documented the appropriate disaster recovery plans for 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.	• 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 and as needed. • Targeted audits and penetration tests were conducted throughout 2024 by internal and external entities. In 2024, 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 identify any areas of weakness. We documented findings and executed remediation plans. Our security team also performed various vulnerability scanning throughout the year. • We maintained a comprehensive inventory of our systems in a central asset management tool with the goal of supporting accurate records and efficient management. • We enhanced our security posture by implementing robust solutions for cloud security, data loss prevention, and privileged access management. • To strengthen our security governance and risk management, we adopted a comprehensive governance, risk and compliance solution. Additionally, we implemented a risk monitoring service to regularly assess our security performance and that of our vendors.

Responsible Supply Chain Management

We believe responsible supply chain management plays an important role in achieving our mission.

Our **Global Standard for Suppliers** complements and supplements 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. We also have a **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 applicable 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 2024 Impact Report, **Access to our Therapies**.
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 2024 Impact 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 2024. 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 2024. See our **2024 Annual Report,** page 68 (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 2024 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 2024 Impact Report, **Access to our Therapies**, **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	In 2024, Ultragenyx did not have any settlements of ANDA litigation that involved payments and/or provisions to delay bringing an authorized generic product to market. See our **2024 Annual Report**, pages 14-17 (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 2024 Impact Report, **Access to our Therapies**.
	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 2024 Impact Report, **Access to our Therapies**.
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 were no fatalities associated with Ultragenyx products in 2024. 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 in 2024. 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 are 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 2024 Impact 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 2024 Impact 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 2024. See our 2024 Impact 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 2024. See our **2024 Annual Report**, page 68 (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 2024 Impact 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 2024 Impact 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 2024 Impact 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 2024. See our **2024 Annual Report**, page 68 (Item 3. Legal Proceedings).
	Description of code of ethics governing interactions with health care professionals	HC-BP-510a.2	See our 2024 Impact 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, 2024, unless otherwise noted. GRI 1 used: GRI: Foundation 2021. Additionally, we map our GRI disclosures against several of the UN Sustainable Development Goals (SDGs).

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 2: General Disclosures 2021	2-1 Organizational details		2024 Impact Report, **About Us** and **2024 Annual Report**, Item 1. Business.
	2-2 Entities included in the organization's sustainability reporting		The scope of the 2024 Impact Report covers Ultragenyx's wholly-owned operations globally. | 2024 Impact Report, **About This Report**.
	2-3 Reporting period, frequency and contact point		Ultragenyx publishes an impact report annually. 2024 Impact 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		**2024 Annual Report**, Item 1. Business.
	2-7 Employees	8, 10	2024 Impact Report, **Workforce Data.**
	2-8 Workers who are not employees	8	Information unavailable.
	2-9 Governance structure and composition	5, 16	2024 Impact Report, **Corporate Governance.** | 2024 Impact Report, **Corporate Responsibility Oversight.**
	2-10 Nomination and selection of the highest governance body	5, 16	**2025 Proxy**, Nomination of Directors.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 2: General Disclosures 2021 (cont.)	2-11 Chair of the highest governance body	16	Daniel G. Welch, Chairperson of the Board. | **Ultragenyx's Leadership**.
	2-12 Role of the highest governance body in overseeing the management of impacts	16	2024 Impact Report, **Corporate Responsibility Oversight**. | **Board Committees and Charters**.
	2-13 Delegation of responsibility for managing impacts		2024 Impact Report, **Corporate Responsibility Oversight**.
	2-14 Role of the highest governance body in sustainability reporting		2024 Impact Report, **Corporate Responsibility Oversight**.
	2-15 Conflicts of interest	16	2024 Impact Report, **Corporate Governance**. | **Corporate Governance Guidelines**.
	2-16 Communication of critical concerns		2024 Impact Report, **Risk Management**. | **2024 Annual Report**, Item 1A. Risk Factors.
	2-17 Collective knowledge of the highest governance body		**2025 Proxy**, Building the Right Board.
	2-18 Evaluation of the performance of the highest governance body		**Corporate Governance Guidelines**.
	2-19 Remuneration policies		**2025 Proxy**, Executive Compensation. | **2025 Proxy**, Director Compensation.
	2-20 Process to determine remuneration		**2025 Proxy**, Executive Compensation. | **2025 Proxy**, Director Compensation.
	2-21 Annual total compensation ratio		**2025 Proxy**, CEO Pay Ratio.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 2: General Disclosures 2021 (cont.)	2-22 Statement on sustainable development strategy		2024 Impact Report, **A Letter From Our CEO**. | 2024 Impact Report, **Impact Report Approach**.
	2-23 Policy commitments	16	2024 Impact Report, **Governance chapter** | **Global Code of Conduct.** | **Global Standard for Suppliers.** | **Human Rights Policy**.
	2-24 Embedding policy commitments		2024 Impact Report, **Governance chapter** | **Global Code of Conduct.** | **Global Standard for Suppliers.** | **Human Rights Policy**.
	2-25 Processes to remediate negative impacts		2024 Impact Report, **Governance chapter** | **Global Code of Conduct.** | **Global Standard for Suppliers.** | **Human Rights Policy**.
	2-26 Mechanisms for seeking advice and raising concerns	16	2024 Impact Report, **Raising A Concern** | **Global Code of Conduct**. | **Global Standard for Suppliers**. | **Human Rights Policy**.
	2-27 Compliance with laws and regulations		2024 Impact Report, **Ethics and Integrity** | **2024 Annual Report**, page 68 (Item 3. Legal Proceedings).
	2-28 Membership associations		2024 Impact Report, **Strategic Collaborations** | 2024 Impact Report, **Public Policy Participation**. | 2024 Impact Report, **Patient Advocacy and Engagement**.
	2-29 Approach to stakeholder engagement		2024 Impact Report, **Impact Report Approach**.
	2-30 Collective bargaining agreements	8	**2024 Annual Report**, Item 1. Business – Human Capital.
GRI 3: Material Topics 2021	3-1 Process to determine material topics		2024 Impact Report, **Materiality Assessment**.
	3-2 List of material topics		2024 Impact Report, **Materiality Assessment**.
	3-3 Management of material topics		The 2024 Impact Report describes the management of material topics by section. | **2024 Annual Report**, Item 1A. Risk Factors.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 201: Economic Performance 2016	201-1 Direct economic value generated and distributed	8, 9	**2024 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	13	2024 Impact Report, **Reducing Environmental Impacts**.
	201-3 Defined benefit plan obligations and other retirement plans		**2024 Annual Report**, Item 15. Exhibits and Financial Statement Schedules.
GRI 203: Indirect Economic Impacts 2016	203-1 Infrastructure investments and services supported	5, 9, 11	2024 Impact Report, **Access to our Therapies**. | 2024 Impact Report, **Communities chapter**.
	203-2 Significant indirect economic impacts	1, 3, 8	2024 Impact Report, **Access to our Therapies**. | 2024 Impact Report, **Communities chapter**.
GRI 205: Anti-corruption 2016	205-1 Operations assessed for risks related to corruption	16	**2024 Annual Report**, Item 1A. Risk Factors. | 2024 Impact Report, **Responsible Supply Chain Management**. | **Global Code of Conduct.** | **Global Standard for Suppliers**.
	205-2 Communication and training about anti-corruption policies and procedures	16	2024 Impact Report, **Ethics and Integrity**. | **Global Code of Conduct.** | **Global Standard for Suppliers**.
	205-3 Confirmed incidents of corruption and actions taken	16	2024 Impact Report, **Ethics and Integrity**.
GRI 206: Anti-competitive Behavior 2016	206-1 Legal actions for anti-competitive behavior, anti-trust, and monopoly practices	16	**2024 Annual Report**, Item 3 Legal Proceedings.
GRI 303: Water and Effluents 2018	303-1 Interactions with water as a shared resource	6, 12	2024 Impact Report, **Managing Waste and Water**.
GRI 305: Emissions 2016	305-5 Reduction of GHG emissions	13, 14, 15	2024 Impact Report, **Reducing Environmental Impacts**.
GRI 306: Waste 2020	306-2 Management of significant waste-related impacts	3, 6, 8, 11, 12	2024 Impact Report, **Managing Waste and Water**.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 401: Employment 2016	401-1 New employee hires and employee turnover	5, 8, 10	2024 Impact Report, **Human Capital Development**.
	401-2 Benefits provided to full-time employees that are not provided to temporary or part-time employees	3, 5, 8	2024 Impact Report, **Employee Compensation and Benefits**. | **Benefits**.
	401-3 Parental leave	5, 8	2024 Impact Report, **Employee Compensation and Benefits**. | **Benefits**.
GRI 403: Occupational Health and Safety 2018	403-1 Occupational health and safety management system	8	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-2 Hazard identification, risk assessment, and incident investigation	8	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-4 Worker participation, consultation, and communication on occupational health and safety	8, 16	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-5 Worker training on occupational health and safety	8	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-6 Promotion of worker health	3	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-8 Workers covered by an occupational health and safety management system	9	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-9 Work-related injuries	3, 8, 16	2024 Impact Report, **Occupational Health, Safety and Wellness**.
	403-10 Work-related ill health		2024 Impact Report, **Occupational Health, Safety and Wellness**.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 404: Training and Education 2016	404-2 Programs for upgrading employee skills and transition assistance programs	8	2024 Impact Report, **Employee Learning and Development**. | 2024 Impact Report, **Career Development**.
	404-3 Percentage of employees receiving regular performance and career development reviews	5, 8, 10	2024 Impact Report, **UltraPerformance Management**.
GRI 406: Non-discrimination 2016	406-1 Incidents of discrimination and corrective actions taken	5, 8	2024 Impact 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	5, 8, 16	2024 Impact 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	5, 8	2024 Impact 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		2024 Impact Report, **Communities chapter**. | Includes information on local community engagement, including corporate philanthropy and volunteering.
GRI 415: Public Policy 2016	415-1 Political Contributions	16	2024 Impact Report, **Public Policy Participation**.

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2024 Location
GRI 416: Customer Health and Safety 2016	416-1 Assessment of the health and safety impacts of product and service categories		2024 Impact Report, **Safety**.
	416-2 Incidents of non-compliance concerning the health and safety impacts of products and services	16	2024 Impact Report, **Quality**. | 2024 Impact Report, **Safety**. | **FDA Data Dashboard**.
GRI 417: Marketing and Labeling 2016	417-1 Requirements for product and service information and labeling	12	**2024 Annual Report**, Item 1. Business. | **Crysvita**. | **Mepsevii**. | **Dojolvi**. | **Evkeeza**.
	417-2 Incidents of non-compliance concerning product and service information and labeling	16	**2024 Annual Report**, Item 1A. Risk Factors.
	417-3 Incidents of non-compliance concerning marketing communications	16	2024 Impact Report, **Interactions with Patients, Caregivers and Healthcare Professionals**. | **2024 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	16	2024 Impact Report, **Data Privacy**.

2024 Ultragenyx Impact Report

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