



2025 Ultragenyx Impact Report

Going beyond every day.®

ultragenyx

Bruce's Story

Bruce lives with Angelman Syndrome, a rare genetic disorder. His mother, Danielle, has helped build a strong network across the Angelman community. Even as they navigate through complexity as a family, Bruce brings immense light and joy into their lives.

Learn more about [Bruce and Danielle](#).



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 that predict or indicate future events or trends or that are not statements of historical fact. 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 18, 2026, 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, 2025; 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 is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases.

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 2025 report contains disclosures for the period January 1 through December 31, 2025, 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. Percentages and numbers used throughout are estimates or approximates and values may not total to 100%.

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” refers to the wider groups of people working for and with us, including full- and part-time employees and contingent staff.

© May 2026

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



Table of Contents

A Letter From Our CEO	4
About Us	6
Impact Report Approach	8
Innovation	12
Patients	24
People	38
Communities	60
Planet	70
Governance	76
SASB Index	90
GRI Index	93
TCFD Index	98



A Letter From Our CEO

Emil Kakkis, M.D., Ph.D.
Founder, President and CEO

Dear Stakeholders,

The rare disease community is standing at an important crossroads. Across the field, the pace of scientific and technological advancement continues to accelerate, and public awareness and support for rare disease communities is higher than ever before. At the same time, 2025 reminded us that considerable challenges still stand between transformational medicines and the patients and families waiting for them—particularly in rare and ultra-rare diseases where the urgency is highest and the path to access is often the most complex.

It is increasingly clear that the rate-limiting factor to transform the future of rare disease is not our scientific ability to develop meaningful treatments and cures, but the system’s ability to deliver them.

When the stakes are this high, those of us with the expertise and opportunity to bring meaningful change to families with no options have a moral imperative to adapt and find solutions—yet no single organization can close this gap alone. Companies, patient advocates, policymakers, regulators, health systems, and payers must all work together with shared purpose to evolve—and sometimes radically reimagine—outdated systems that were built for a different scientific paradigm. That is why in 2025 we worked alongside rare disease advocates to advance a legislative framework appropriate for the realities of rare disease, building on the foundation established by the Orphan Drug Act.

As we worked in 2025 to build a more promising future for rare disease development, we also continued to relentlessly deliver for patients today—advancing our late-stage programs closer to regulatory approvals and continuing to reach more and more patients around the world with our approved medicines.

We laid important groundwork for two potential gene therapy approvals—DTX401 for people living with glycogen storage disease type Ia, a condition requiring burdensome lifelong metabolic management, and UX111 for Sanfilippo syndrome type A, a progressive and devastating pediatric neurodegenerative disease. As we progress towards our first potential gene therapy launches as a company, we will hold ourselves to the same high standard for broad and timely access that has guided us since day one.

None of this progress would be possible without the dedication and perseverance of our team - who remain “ultra-committed” to our mission to transform the lives of as many patients and families impacted by rare diseases as possible - as well as the continued partnership from rare disease communities, whose input remains our north star. Success in rare diseases requires both generosity and humility, and I welcome continued engagement from our stakeholders so that we can help one another succeed in making the world a better place for all those who are impacted by rare diseases.

Sincerely,

Emil Kakkis, M.D., Ph.D.
Founder, President and CEO



Dr. Emil Kakkis (left) was honored with the first ever Legacy Award by Ron Leuty at the Bay Area Biotech Forum. The award was presented by Ryan Dant (center), a patient advocate living with MPS I and Public Communications Specialist at the Muenzer MPS Research & Treatment Center. Ryan participated in early, groundbreaking MPS I clinical research led by Dr. Kakkis. Mark Dant (right), Ryan’s father, is Executive Director of the Ryan Foundation for Rare Disease Research, which supported early research toward a treatment for MPS I led by Dr. Kakkis.

2025 Awards



Somerville Chamber of Commerce
Innovation Award

Top Workplaces Culture Excellence Awards



About Us

Vision: Lead the future of rare disease medicine

Mission: Transform the lives of people with rare disease

Ultragenyx is a biopharmaceutical company headquartered in Novato, California that is committed to bringing novel products to patients for the treatment of rare and ultra-rare diseases. Our purpose is to lead the future of rare disease medicine as we seek to treat individuals afflicted by serious, debilitating genetic diseases with limited or no treatment options. We recognize the extraordinary stakes for patients and families who face a rare diagnosis, and our desire to make a difference drives us to develop new therapies with the utmost urgency and care.

Our Impact Today: Delivering Four Approved Treatments for Five Rare Diseases Around the Globe



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

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

Mepsevii®
Mucopolysaccharidosis Type VII (MPS VII)



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



Evkeeza®
Homozygous Familial Hypercholesterolemia (HoFH)

Impact Report Approach

Ultragenyx's Impact Report details our corporate responsibility efforts and is guided by a materiality assessment focused on six key pillars that prioritize efforts and resources toward 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 our overall approach to corporate responsibility.

Strategic Framework

Ultragenyx is committed to integrating the principles of ethics, integrity and corporate responsibility throughout our business. We seek to set a powerful example within our industry by enhancing the lives of individuals with rare diseases, enabling broad access to critical medicines, empowering our employees and contributing positively to the communities we serve. We are dedicated to sharing our insights and learnings to benefit patients and advance the broader industry.

In 2025, we surveyed key stakeholders to evaluate our corporate responsibility materiality assessment conducted in 2021 and remain committed to the established pillars: **Innovation, Patients, People, Communities, Planet and Governance**. This framework helps guide the 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.

This report covers a full spectrum of business practices within our company and is meant to provide insight into the values that underpin our work and the ways we engage our stakeholders on various aspects of our corporate responsibility program – from enhancing disclosure strategies to improving transparency.

Innovation



Patients



People



Communities



Planet



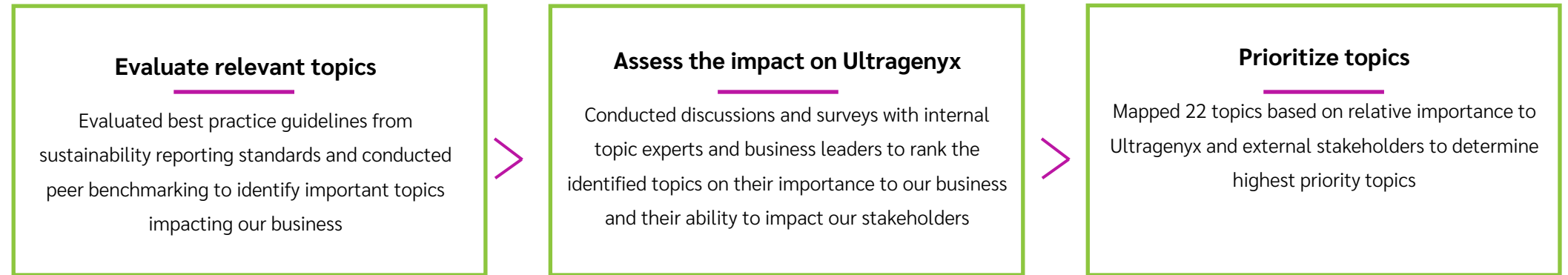
Governance



Materiality Assessment

In 2025, we surveyed key stakeholders to evaluate our corporate responsibility materiality assessment conducted in 2021 and to identify potential shifts in our corporate responsibility priorities. This process considered both internal and external perspectives on the topics that could have the greatest impact on our business and that matter most to our stakeholders.

As part of this effort we:



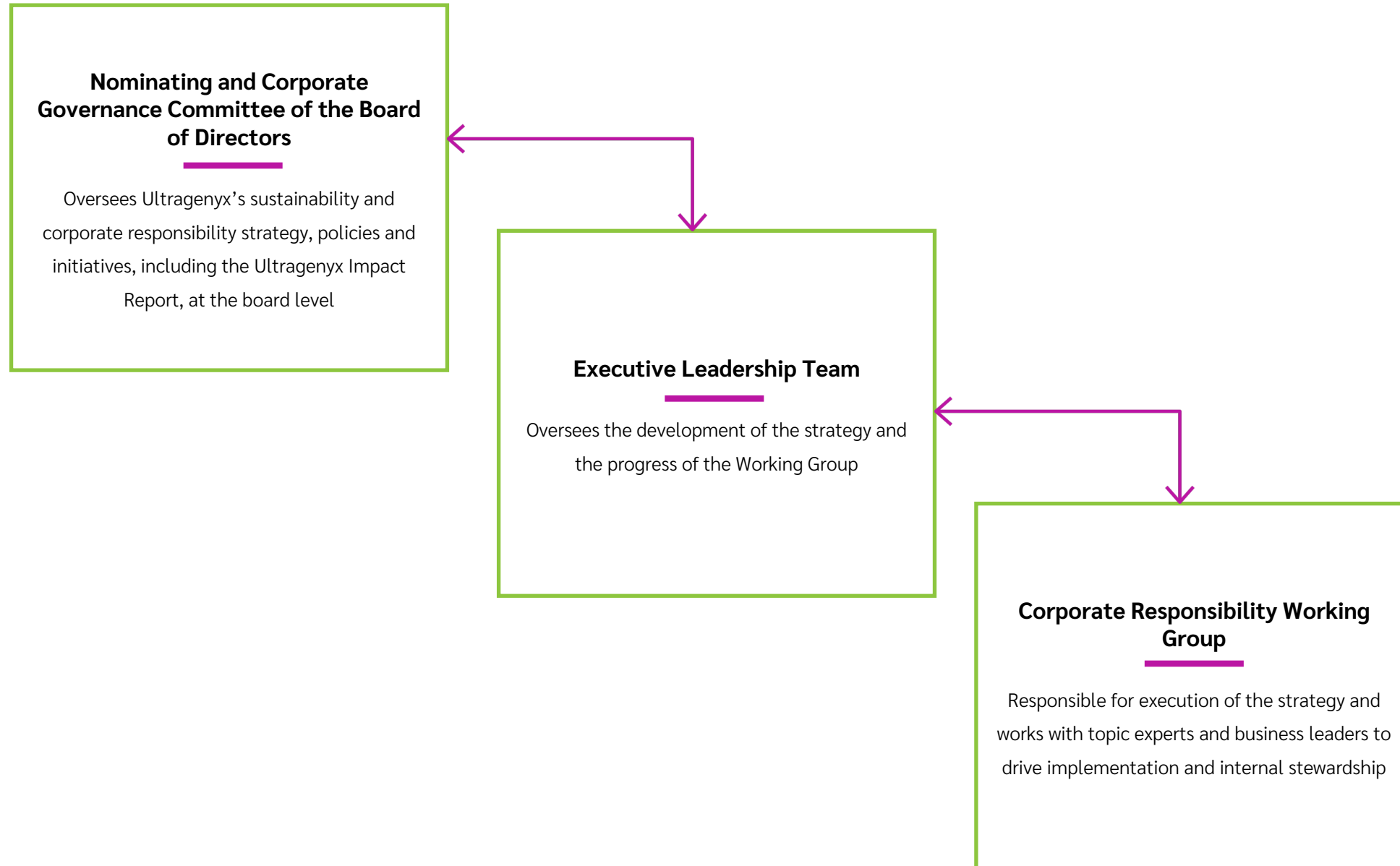
Assessed material topics are listed below. **Bolded** topics have been identified as higher priority in 2025; therefore, updates in this report were prioritized in these areas.

- **Access & Affordability**
- Climate Change Risks & Management
- **Clinical Trial Practices**
- Community Relations
- Employee Equity & Inclusion
- Employee Health & Safety
- Energy Management
- **Ethical Practices & Corporate Behavior**
- Governance Structure & Mechanisms
- Human Rights
- **Impact***
- **Management of the Legal & Regulatory Environment**
- **Patient Safety**
- **Privacy & Data Security**
- **Product Quality**
- Product Stewardship
- R&D
- Risk Management & Business Continuity
- Transparency
- Waste Management
- Water Management
- **Workforce Management**

*Indicates a new topic for 2025

Corporate Responsibility Oversight

Corporate responsibility is integrated within our corporate strategy, and this commitment starts with the Board of Directors. 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 ultra-rare diseases

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

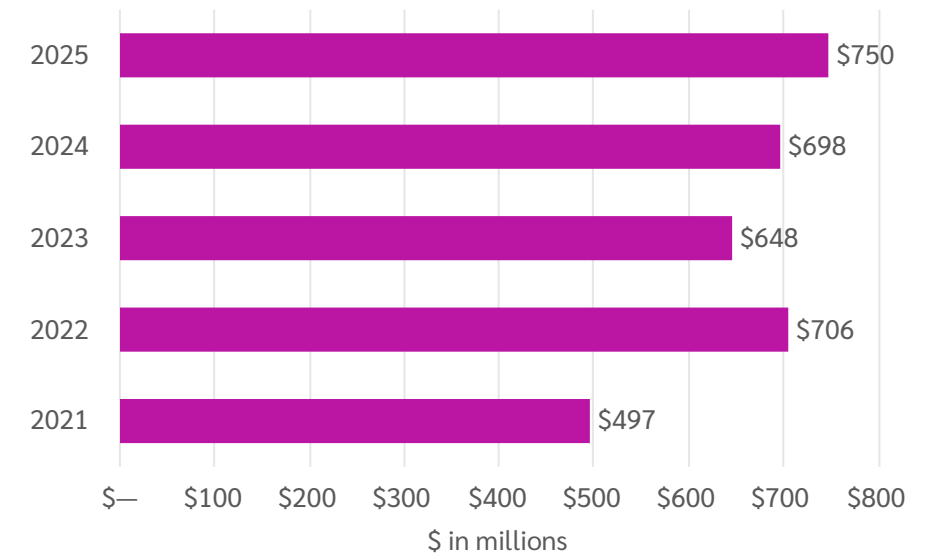
R&D

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. Our patient-focused drug development model and cross-industry collaborations support our mission to transform the lives of people with rare diseases.

We believe innovation in healthcare is critical for the many individuals living with rare diseases who are waiting for an approved treatment. Innovation refers to our processes, initiatives, activities and investments aimed at the efficient and rapid advancement of investigational therapies that maximize patient health outcomes. We make investments in R&D to innovate and develop new therapies. In 2025, we spent approximately 62% 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 prohibits 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.

R&D Spend



For more details, please see our [2025 Annual Report](#).

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 ultra-rare diseases. We are advancing clinical and preclinical development programs across multiple rare disease therapeutic areas. Currently, we are developing five investigational therapies in pivotal clinical programs with the potential to reach thousands of patients. In addition to our clinical efforts, we are actively working on advancing a number of preclinical programs.

Candidate	Description	Therapeutic Area:			Route of Admin	Prevalence ¹
		Phase 1	Phase 2	Phase 3		
GTX-102	ASO activating paternal expression of UBE3A	Angelman Syndrome (AS)			Intrathecal (IT) Infusion	~60,000
UX016²	Small molecule prodrug	GNE Myopathy			Oral substrate replacement	~10,000
UX111	AAV9 SGSH gene therapy	Sanfilippo Syndrome (MPS IIIA)			Intravenous (IV) Infusion	~3,000 - 5,000
DTX401	AAV8-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
UX143 (setrusumab)³	Anti-Sclerostin monoclonal antibody	Osteogenesis Imperfecta (OI)			IV Infusion	~60,000

*As of May 2026

For the latest information on our pipeline, please visit [our website](#).

1. Prevalence in commercially accessible geographies
2. Externally funded by a venture philanthropy agreement through clinical proof-of-concept
3. As of the publication date of this report, based on the Company's analysis of the data across the two global Phase 3 studies, the Company believes there is a basis to engage regulatory agencies to determine if there is a path forward for the program.

Patient-Focused Drug Development

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 individuals living with rare disease.

Our approach includes 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 and Caregiver Advocacy and Engagement](#).

Our differentiated 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.

Our goals with the GTMF are to maintain a stable and secure supply of gene therapies, provide the agility needed to support our gene therapy pipeline, and expand our opportunities for future partnerships.



Ultragenyx receives the Somerville Chamber of Commerce Innovation Award

We celebrated Somerville’s vibrant business community at the Chamber of Commerce Powderhouse Awards Dinner in November 2025. Our team proudly accepted the Innovation Award. Since opening our Somerville, MA facility in 2023, our team’s goal is to continue innovating with groundbreaking ideas. At Ultragenyx, our Somerville site serves as the nucleus of our gene therapy innovation, fueling breakthroughs from the ground up.

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 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 work to protect patient safety and well-being by adhering to appropriate informed consent procedures and good clinical practices. 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.

For more information on the risks related to the discovery and development of our investigational therapies, please see our [2025 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 using a risk-based approach. Through our GCP audit program, we evaluate each site for compliance risks and 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. Learn more about our commitment to quality and safety in our Governance section.

>160 *locations with clinical studies*
across **19** *countries as of Dec. 31, 2025*

Clinical Trials (cont.)

Data Transparency

Data transparency is essential to fostering trust among 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.

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 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 trial participants 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.

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 shared. 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 **100** clinical and non-clinical
ISTs approved and **630** participants
enrolled globally since 2012*

Individuals and families impacted by rare and ultra-rare 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 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 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 ultra-rare 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, we may partner with an academic institution and a patient advocacy group, if applicable, on the ownership and management of data to ensure the greatest reach and benefit for all.

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.

First DMP launched in 2012

More than 900 patients enrolled* at sites in 14 countries

*Enrollment as of December 31, 2025

Collaborations

We participate in multiple industry consortia and partnerships to foster and support collaborative, industry-wide drug development in rare and ultra-rare diseases. Examples of our research collaborations include:

	About the initiative	How Ultragenyx supports the initiative
Angelman Biomarker and Outcome Measure Consortium (ABOM) <i>Member</i>	Launched to drive information sharing, agree on the most important disease domains in Angelman syndrome and develop or modify existing endpoints or biomarkers to better understand how to measure change in clinical studies.	The Ultragenyx Endpoint Development & Strategy, Clinical Development and Patient Advocacy teams participate in an annual Angelman workshop hosted by FAST to talk through previous, current and future biomarkers and endpoints around Angelman.
BeginNGS™ (newborn genomic sequencing) <i>Founding Sponsor</i>	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.	Ultragenyx helps support the ultimate goal of testing 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.
LouLou Foundation CDKL5 Deficiency Disorder Consortium <i>Member</i>	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.	Ultragenyx is committed to sharing information in clear, accessible language and ensuring that families can easily stay informed about our progress. In 2025, we shared an update on nonclinical learnings at a LouLou Foundation meeting, reinforcing our dedication to open communication. We also continue to support natural history studies that advance scientific understanding and help guide future research.
“Living with Osteogenesis Imperfecta: Understanding Experiences Based on Community Insight and Experience” (IMPACT) Survey <i>Supporter</i>	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.

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 diseases. 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 ultra-rare 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 ultra-rare diseases worldwide.

Please see our [2025 Annual Report](#) and our [website](#) for a list of our collaborations.

Rare Bootcamp™

In partnership with EveryLife Foundation for Rare Diseases, 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 biannual, multi-day event. In 2025, we hosted a Rare Bootcamp on the East Coast of the United States for the first time. 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 **12** Rare Bootcamps with more than **230** participants from over **140** organizations in attendance.*

Finding Hope and Spurring Action Through Rare Bootcamp™ and Employee Advocacy

For Ada Lio and Nathan Guo, becoming parents to their son Lucas meant taking on roles they never anticipated: full-time caregivers, medical researchers and fierce advocates. Soon after birth, Lucas began showing symptoms that would lead to a diagnosis of Zhu-Tokita-Takenouchi-Kim syndrome (ZTTK) when Lucas was 8-months old, an ultra-rare neurodevelopmental disease that is often characterized by intellectual disabilities, motor delay and seizures, among other symptoms.

While the family felt fortunate that they didn't experience a long diagnostic odyssey like many families do in rare disease, they eventually hit a wall. "We were told by the doctors, there are no cures, there are no treatments. There is very little known about your son's disorder," Ada shares.

Ada and Nathan soon found hope in the rare disease community. "A lot started with Rare Bootcamp, a multi-day forum hosted by Ultragenyx and EveryLife Foundation for Rare Diseases. The reason we have hope is because we've seen other successes, and we believe there will be more successes as well," explains Nathan.

Rare Bootcamp is designed to provide parents and patient advocates working to develop rare disease therapies with the opportunity to learn from experts in rare disease drug development and connect with peers on a similar path. The program provides practical, actionable guidance on everything from identifying and prioritizing early research priorities to translating research results into a path forward for potential therapies. For Ada and Nathan, it was also a way to uncover new directions in their journey forward.

"Rare Bootcamp provided us a roadmap – here's how to develop clinical models, here's how to think about endpoints and biomarkers, here's how you should think, ultimately, about clinical trials and manufacturing," says Nathan. "For our community, there are still a lot of unknowns. But we are not the first parents to go through this. We must talk to the trailblazers to understand – this is where you should focus your time and energy."

In support of families like Ada and Nathan's, Ultragenyx employees demonstrate their commitment to the rare disease community through direct advocacy and volunteerism opportunities. In 2025, our employees volunteered at the 2025 ZTTK Conference, which brought together more than 180 family members, researchers and clinicians from around the world to accelerate progress toward treatments for ZTTK. We recognize the value of standing alongside rare disease families in their fight for progress.

Now, Ada and Nathan similarly use their experience to support other families, with a tireless commitment to Lucas and other children living with rare diseases. "We're committed to work so hard for him," says Ada. "I believe that working with patients, parents and patient advocacy groups is so important because we are the patients' voice, and we'll do everything we can to help our children."



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.

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.

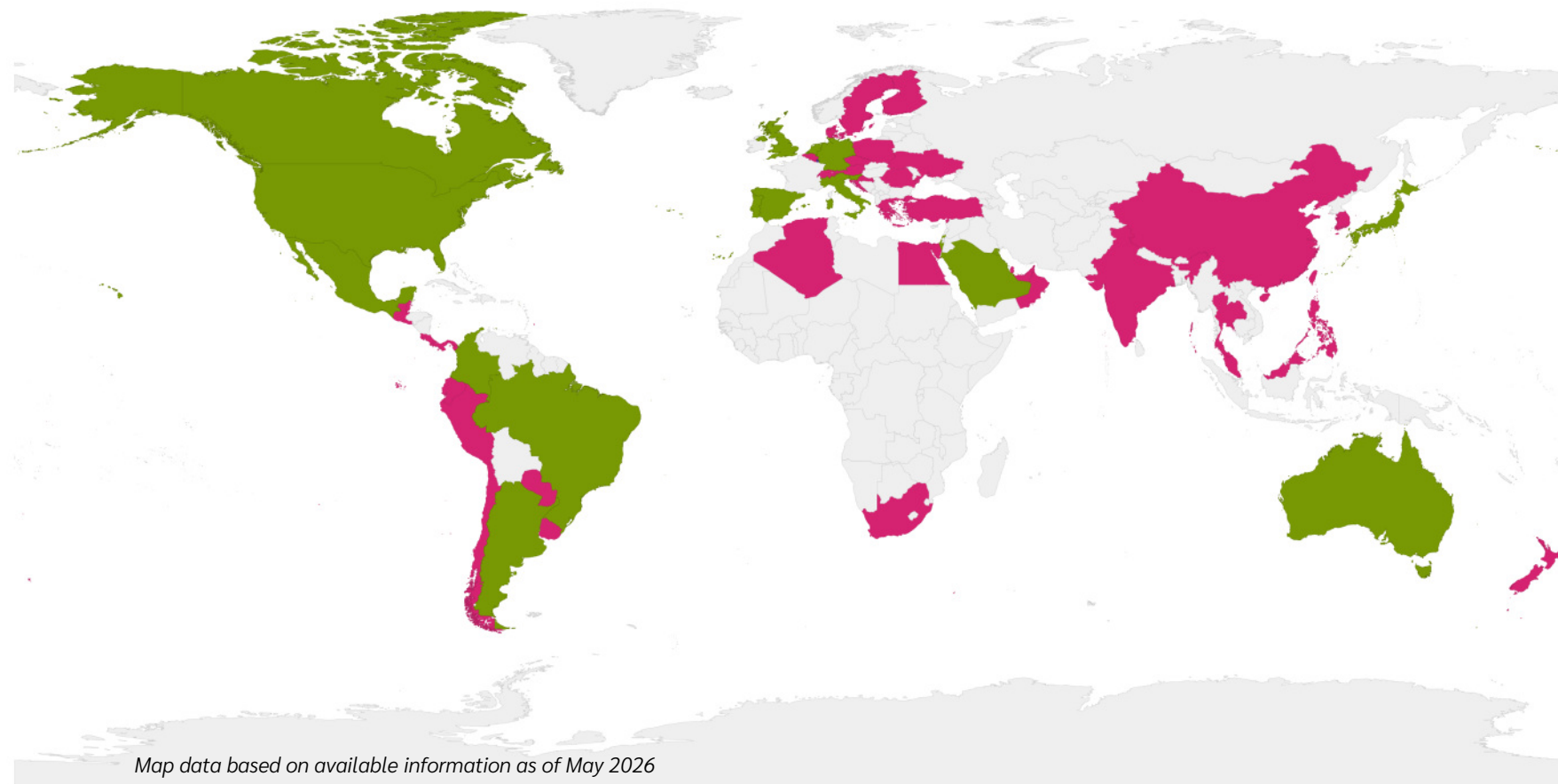
Additionally, we consider the costs of producing high-quality medicines and sustaining a robust global supply chain. We set our medicine prices with global access in mind. In the U.S., we leverage effective reimbursement strategies and use our best efforts to prevent patients from forgoing Ultragenyx therapy for financial reasons. Our patient assistance programs include co-pay support and free access where appropriate. In 2025, U.S. price increases for the commercial products for which we lead promotion were consistent with the Consumer Price Index.



Standing at the entrance of Ultragenyx headquarters, the bronze bull and child statue serves as a powerful symbol of the rare and ultra-rare diseases that patients courageously confront. The child represents patients, while the bull embodies the challenges posed by these conditions. At Ultragenyx, our mission is to help protect patients from these burdens through the development of innovative therapies. Each time we reach a significant milestone, we commemorate the achievement with a "Bull Ceremony," celebrating the dedication, perseverance, and impact of our teams in advancing hope for patients and families.

Access to Our Therapies (cont.)

Reaching patients around the world with Ultragenyx medicines



Timely Care for Rare Disease Patients Around the World

Our established 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, the Middle East and Japan. Specifically, we’ve received recommendations for reimbursement for Evkeeza® in seven countries outside of the U.S. – Italy, Canada, Japan, Netherlands, Spain, United Kingdom and Luxembourg. It is also available via early access programs in 13 additional countries including Austria and France.

As we look ahead to potential gene therapy approvals, we will be guided by the same principle of timely global access as we build our commercial infrastructure.

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 diseases and their families in receiving proper diagnoses and optimal care. Our commitment to developing new medicines for both children and adults with rare and ultra-rare 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.

*More than **700** patients in **50** countries have been approved for access to Ultragenyx medicines through various global expanded access programs and patient assistance programs since 2013*

Patient and Caregiver Advocacy and Engagement

Ultragenyx was built hand-in-hand with the rare disease communities we serve. Our priority is to meet patients and families where they are, from diagnosis to participating in clinical research and accessing care. They are our partners at every stage, from designing research and clinical trials through approval and commercialization. There are a variety of ways that we endeavor to ensure every decision is informed by those who know the disease experience best.

Our patient advocacy work includes the following:

- **Establishing** partnerships with global patient organizations to best support patients, caregivers and disease communities as well as incorporate their perspectives and priorities in the development of therapies to address unmet needs.
- **Engaging & Empowering** patients, caregivers 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 on our clinical programs.



Members of the Ultragenyx Patient and Caregiver Advocacy team.

Partnering with Patient Advocacy Organizations

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

In 2025, we attended **over 45** patient advocacy conferences, meetings and town halls across the globe. We engaged with advocacy leaders and rare disease community members, sponsored educational events and appropriately shared new information on our pipeline research programs.

We actively participate in disease awareness initiatives to shine a light on the challenges patients and caregivers face every day. By collaborating with international advocacy organizations and hosting events, we help increase education, empower communities and amplify patient and caregiver voices. These efforts strengthen the connections to the communities we serve and ensure their needs remain at the heart of our work.

Patient and Caregiver Leadership Councils

Patient and Caregiver Leadership Councils (LCs) are initiatives led by our patient advocacy team to include patient community perspectives and lived experiences across functional areas of product development.

Through regular dialogue, LC members provide critical insights that are applicable to multiple functions across Ultragenyx. Our work is strengthened by understanding disease impact, unmet needs, treatment challenges and quality of life considerations, and using this information to shape the design of clinical trials and the development of new treatments. Patient and Caregiver involvement is critical to making informed decisions in drug development and LCs provide a framework for cross-functional leaders to turn patient and caregiver insights into actionable impact.

We currently host the following community leadership councils:

- Angelman Caregiver Leadership Council
- Global Gene Therapy Advisory Council
- GSDIa Leadership Council
- Latin American Patient Leadership Council
- LC-FAOD Patient Leadership Council
- MPS IIIA Caregiver Council
- OI Leadership Council
- XLH Patient Education Advisory Council

In addition to reoccurring Leadership Councils, we also facilitate roundtables, such as “Partners in Progress” held with the Wilson Disease community. This discussion with a dozen patients and caregivers, some newly diagnosed and others living with Wilson disease for over 50 years, explored diagnostic journeys, the burdens of current treatment regimens, questions around gene therapies and the kinds of lasting support caregivers need most.

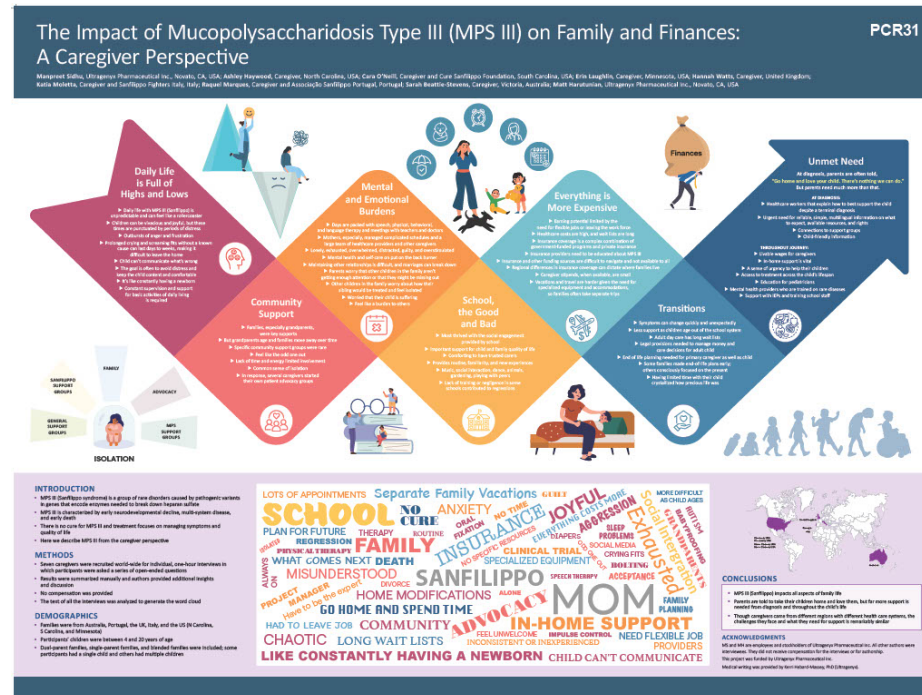
Scientific Posters and Presentations

Scientific posters and presentations are vital tools for sharing patient insights, research findings and raising disease awareness with the broader community.

By visually highlighting patient and caregiver experiences and real-world challenges, these materials inform clinical research and guide the development of therapies that address what matters most to patients and caregivers. These efforts help amplify the patient and caregiver voice across the rare disease ecosystem.

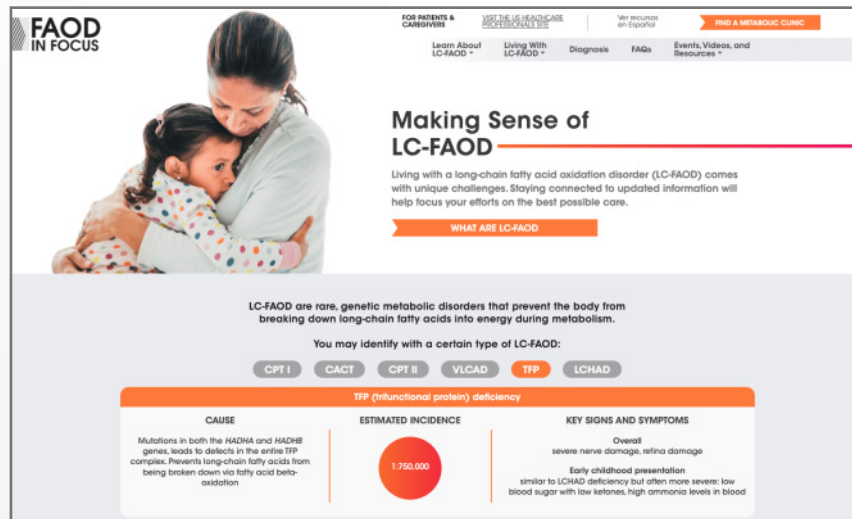
One example is mucopolysaccharidosis Type III (MPS III), also known as Sanfilippo syndrome, where we learned through interviews and shared stories about the wide range of complex needs families and caregivers face. The impact of this poster highlighted that the unseen challenges of Sanfilippo syndrome can be just as significant as the visible ones, and that education, resources and strong professional and community networks are important to improving quality of life. Additionally, we co-created two posters with the GSDIa community to highlight the real-world challenges of both living with GSDIa and navigating the health care system. This work helps to articulate often unseen demands and underrecognized issues that impact patients and families every day.

Additionally, Ultragenyx, alongside Mereo BioPharma, was recognized in 2025 for excellence in patient engagement, earning the Black Pearl Award by EURODIS. This award celebrates dedication, innovative thinking and commitment to the rare disease community. This recognition reinforces our mission to address unmet needs, drive impactful therapies and create lasting value for the communities we serve.



Providing Educational Resources

Our patient-focused websites provide customized education and rare disease awareness to patients and their families.



www.faodinfofocus.com

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

www.mpsviiinfofocus.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.understandingsdia.com

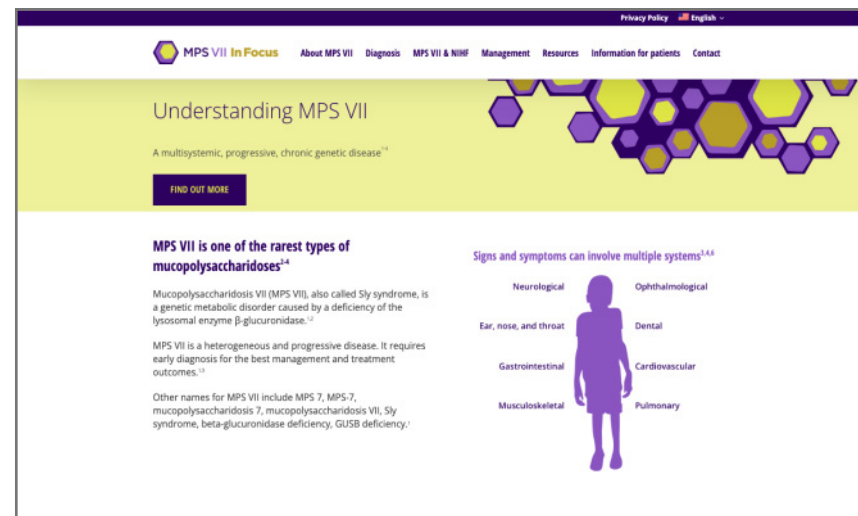
A disease education website about glycogen storage disease type Ia (GSDIa) available in English

www.thinkmpsiii.com

A disease education website about mucopolysaccharidosis type IIIA (MPS IIIA) also known as Sanfilippo syndrome type A available in English

www.ultraclinicaltrials.com

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





“What guides all of our decisions is ensuring that Bruce is living his best life.”

– Danielle Kowalski

BRUCE’S STORY

Living with Angelman Syndrome

Danielle Kowalski remembers how quickly one ordinary moment shifted her family’s life. “It was Easter Sunday, and we were taking a video of our son Bruce having his first glimpse of the basket that the Easter Bunny brought for him,” she shares. “Suddenly, his head dropped and bobbed a bit. I thought maybe his neck was just tired or not strong yet.” After sharing the video with Bruce’s grandma Mémé, an experienced Registered Behavior Technician supporting people with various intellectual and developmental disabilities, she immediately identified Bruce’s head drop as a seizure and told Danielle to contact a doctor.

Bruce was diagnosed with Angelman syndrome, a rare, neurogenetic disorder that causes cognitive and motor impairment, difficulty with communication, balance issues, sleep disruption and seizures. With an answer in hand, the family was determined to give Bruce the best chance possible to reach his full potential, even as they faced hurdles. “We received a packet in the doctor’s office that had the list of symptoms and risks outlined, detailing how this would impact his ability to function and live the life we dreamed of for him,” explains Danielle. “I asked the doctor how do we treat this? And he said, ‘You don’t. This will require 24/7 lifelong care.’”

From there, the family focused on what they could control: building the right team of healthcare professionals, researching therapeutic options and potential clinical studies and connecting with other families living with Angelman syndrome to better understand what the future might hold. In becoming active members of the community, the family was able to ease the isolation they were feeling.

“Some days I feel like I can take this on and I’m a strong advocate. Other days I honestly don’t know how I’m going to get through the next hour, besides taking it moment-by-moment,” says Danielle. “But being around the Angelman community is incredibly inspirational and supportive, just knowing others are managing through too.”

Even as they navigate through complexity as a family, Bruce brings immense light and joy into their lives. He loves swimming, playing with his dog, listening to music and learning about science. He adores his brother Grady and being with his family, who all contribute to ensuring that Bruce always feels heard and loved.

With agility and flexibility, the family continues to fight for Bruce’s future. It is their hope that one day, another family will be handed a different packet – one that includes treatments to help their child living with Angelman syndrome.

Angelman syndrome is a lifelong condition caused by changes that disrupt the expression or function of the ubiquitin-protein ligase E3A (UBE3A) gene, resulting in profound developmental delays and cognitive impairment. In July 2025, the Phase 3 Aspire study of GTX-102 completed enrollment ahead of plan with participants ages 4 to 17 with full maternal UBE3A gene deletion, randomized across 28 global sites. In October 2025, Ultragenyx dosed the first patient in the Aurora study, which will expand the population of Angelman patients treated to include both younger and older patients and those with other non-deletion AS genotypes not included in the Phase 3 Aspire 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 leading efforts to put patients at the forefront from drug development through commercialization, providing access to innovative therapies through responsible pricing, and accelerating

the development timelines for new medicines. Efforts also include shortening the time for those with a rare disease to receive an accurate diagnosis, contributing to ensuring an environment that allows development of innovative technologies, including gene therapy, to flourish (including the regulatory environment as well as reimbursement landscape), and ensuring that rare diseases are treated as a priority in markets worldwide. We establish these priorities and positions in consultation with our executive leadership 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 more than 30 companies that are collectively investing more than \$17 billion annually in R&D and that have over 250 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

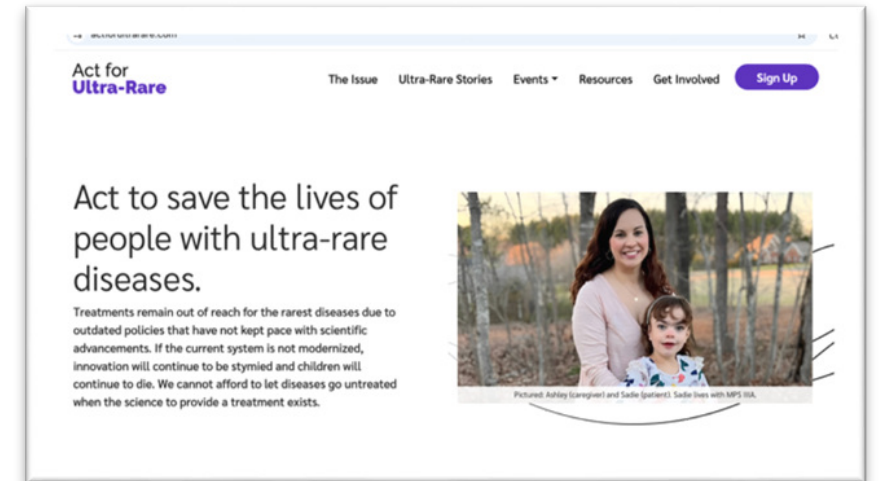
Public Policy Participation (cont.)

Eric Crombez, M.D. appointed to the Massachusetts Rare Disease Advisory Council

In 2025, Dr. Eric Crombez, Ultragenyx’s chief medical officer and executive vice president, was sworn in as a member of the Massachusetts Rare Disease Advisory Council (RDAC). Surrounded by colleagues and his husband Ryan, Dr. Crombez officially joined the RDAC, a group established in 2020 with a mission to “provide guidance and recommendations on rare disease incidence and the status and needs of the rare disease community to the governor, the legislature, and the department of public health with the goal of improving the lives of people impacted by a rare disease in the Commonwealth.” Dr. Crombez was appointed by Governor Maura Healy and is expected to serve on the council for 3 years.



Launched in February 2025, Act for Ultra-Rare brings together the rare disease community, innovators and policymakers to accelerate therapies for ultra-rare diseases.



The website was launched and is sponsored by Ultragenyx.

Public Policy Participation (cont.)

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

Patient Experience

Patients should be at the forefront from development through commercialization

Market Access

At a minimum, a majority of patients who can benefit from our products should have access to them as soon as possible following regional regulatory approval

Pricing and Reimbursement

Achieving majority access to treatments while advocating for responsible pricing and flexible payment models for innovative therapies; reimbursement should be provided regardless of regulatory pathway to approval

Diagnostic Odyssey

Shortening the timeline from diagnosis to treatment access through initiatives such as NBS and genetic testing

Innovative Technologies

Global healthcare systems need to adapt to fully realize the promise of innovative technologies, such as gene therapies, that will not require chronic administration

Accelerated Development

Rare disease drug development globally should be streamlined to shorten translational research, clinical and manufacturing timelines

Rare Disease Policy Advancement and Protection

Rare diseases should be treated as a priority in all global markets; we proactively pursuing rare disease policies globally regarding definitions and incentives and protecting existing rare disease policies



In April 2025, a group of ultra-rare disease advocates, including Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx as well as Ultragenyx senior leadership, gathered in Washington, D.C. to educate Congressional Members and staffers about the current challenges preventing treatments from getting developed.

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. The federal government has a limited role in state NBS programs as it historically provided some funding for state programs and produced the Recommended Uniform Screening Panel (RUSP) – a list of the conditions that should be included on state screening panels – via the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children (ACHDNC). The ACHDNC was dissolved in April 2025, leaving the future of NBS uncertain. We advocate for:

- Reinstatement of a restructured ACHDNC comprised of a broader swath of rare disease stakeholders, including patients and caregivers, that can review conditions for addition to the RUSP
- Ensuring that the reinstated, restructured ACHDNC receives sufficient funding to review as many conditions as possible each year
- Reauthorizing the elements of the Newborn Screening Saves Lives Reauthorization Act that allow states to improve and expand their NBS programs, support parent and provider education, ensure laboratory quality and facilitate adding conditions to the RUSP
- Ensuring adequate funds are provided to states to implement screening of all RUSP conditions
- Increasing understanding of the benefits of NBS and current challenges the system faces

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 no state currently screens for all 40 core conditions and all 26 secondary conditions on the 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 2025, Ultragenyx successfully advocated for the formation of Sunshine Genetics, a state-funded newborn sequencing pilot in Florida that partners with the Florida Newborn Screening public health program. Sunshine Genetics will screen for 250-600 rare conditions in partnership with hospitals throughout Florida.

Advocating for the Successful Development of Treatments for Ultra-rare Diseases

Challenges in Ultra-rare Disease Drug Development: Despite the success of the Orphan Drug Act (ODA), there are substantial regulatory and commercial hurdles in developing treatments for ultra-rare 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 ultra-rare diseases, to take charge of therapy development.

Ultragenyx’s Advocacy for Legislation to Support Development of Therapies for Ultra-rare 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:

- Define “ultra-rare” disease
- Require rational study design for ultra-rare disease programs:
- Expand the definition of “substantial evidence” in Section 505 of the Federal Food, Drug, and Cosmetic Act to account for additional data that can demonstrate efficacy beyond randomized controlled trials (e.g., natural history data, real-world evidence, etc.)
- Require FDA acceptance of biomarkers that represent the underlying disease (“primary disease biomarkers”) and/or intermediate clinical endpoints as primary endpoints in pivotal trials for ultra-rare disease treatments
- Ensure ultra-rare disease therapies approved through the use of primary disease biomarkers and/or intermediate clinical endpoints receive traditional approval instead of accelerated approval
- Stimulate investment into ultra-rare diseases:
- A 50% tax credit for clinical trial costs (capped at \$100 million of clinical expense per program)
- 10 years of marketing exclusivity
- Waiver of application user fees



“I see my work as an extension of my relationship with Ben – a way to continue my bond with him.”

- Jennifer Siedman

BEN'S STORY

Understanding Sanfilippo Syndrome

For Jennifer Siedman, memories of her son Ben bring tremendous joy – his love of crazy socks that captured his spirited personality, the way he dipped his steak in mashed potatoes with gusto, and his smile, which started in his eyes before spreading across his whole face.

When Ben was diagnosed with Sanfilippo syndrome type B at just 15 months old, the family's world changed forever. They learned that for children like Ben, Sanfilippo syndrome can cause 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. They also learned that the median life expectancy for those living with this condition is 15 years old. “When Ben was diagnosed, there really was no roadmap for what might lie ahead, especially emotionally, and it was all very isolating,” Jennifer shares.

Despite their initial shock and sadness, the family viewed Ben's early diagnosis as an opportunity to take action, pursuing every avenue possible to help him build and maintain skills. When Ben was five, Jennifer and her husband Stuart formalized their efforts by establishing Ben's Dream: The Sanfilippo Research Foundation. Over the years, their fundraising efforts supported the critical research of Dr. Haiyan Fu, leading to the development of the investigational gene therapy, UX111, for people living with Sanfilippo syndrome type A.

“When I speak about the impact of rare disease, I often use the analogy of parents and patients being both drivers and passengers in the experience,”

Jennifer explains. “Not only are we trying to drive the medical care of our child in an environment that often feels like an uphill climb, but we are also big drivers of the drug development process. We drive registries, natural history studies, provide funding, but still, so much of the process is out of our control.” After many years of effort, the day came when the Siedman family had to face a heartbreaking reality: science wasn't going to advance quickly enough for Ben. Yet, they continue their work, with the hope that their journey can create pathways for other families navigating the complexities of rare disease.

“Right from the beginning, I knew that alongside my hope to find a treatment for my child, I also hoped to change the path for other Sanfilippo families,” she says. “When a family with a new diagnosis calls me, I encourage them to find a community of other parents to lean on. I remind them that the child in front of them is full of love and potential and their goal today should be the same goal they had on the day their child was born – to help them be the best person they can be.”

UX111 (rebisufligene etisparvovec and formerly ABO-102) is an investigational 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 in the brain that results in progressive cell damage and neurodegeneration that is associated with the disorder. UX111 for Sanfilippo syndrome type A (MPS IIIA). In April 2026, the Company announced that the FDA had accepted its resubmitted BLA seeking accelerated approval of UX111 and assigned a PDUFA action date of Sept 19, 2026. 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.

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.

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.

Our people are:

Ultra-focused

Our team works together fearlessly to uncover new possibilities.

Ultra-curious

Our team applies their biggest ideas in courageous ways. Instead of asking “why?” they ask “why not?”

Ultra- impactful

Our team works hard to make a difference for those who need it most.

Ultra-dedicated

Our team recognizes their biggest challenges yield rare possibilities.

Ultra-innovative

Our team takes rare and dynamic challenges head on.

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

GENEROUS

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

DYNAMIC

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

COURAGEOUS

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

POSSIBILITY

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

RELENTLESS

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

Human Capital Development

As of December 31, 2025, Ultragenyx had approximately **1,370** 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 an environment where they can do their best work and where they want to stay long-term. Our total turnover rate in 2025 was 10.4% and voluntary turnover was 5.8%, 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.

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.

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 weighed 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.

The goals of our employee learning and 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.

With AI increasingly becoming part of the way we work, we developed a set of AI trainings and tools to educate employees on the ethical and compliant use of AI and understand the use-cases where it can unlock their capacity. For more information on our AI strategy, see [Improving How We Work](#).

Learning & Development Impact

Our learning and 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

**The Perceptyx Benchmark Database and benchmark library continue to expand, providing an ever-growing foundation for actionable insights. Our database now includes 531 organizations and 20 million respondents who have participated in surveys over the past three years (2022–2024). We are currently tracking data on 710 commonly utilized scaled survey items, reflecting a 5% year-over-year growth in total respondents*

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

In addition to required learning, we offer a broad set of optional development workshops across topics such as leadership, team effectiveness, wellbeing and resilience, communication and presentation skills, and practical use of AI tools. These offerings are refreshed throughout the year to reflect evolving business needs and employee demand.

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. In 2025, speakers included Soren Gordhamer, Dr. Luana Marques and Dr. Clarence B. Jones.



In 2025

We offered >80 employee learning & development workshops.

When surveyed about their experience, 97% 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.

We have continued to create value for the company through delivery of Continuous Improvement (CI) & Lean Six Sigma (LSS) Projects. To date, we have trained and certified hundreds of employees on the tools and methodologies of LSS including LSS Champions Green Belt, White Belt, Lean Foundations and Kaizen Leader, and have trained employees in each of our functions. As a requirement for the Green Belt and Kaizen leader certification, participants are required to deliver a workplace project. Since inception, the projects have delivered over \$1M in cost savings and over \$200K in cost avoidance, reduction or elimination of risks, cycle time reduction and process clarity and maturity.



In addition to our training and certification program, we curated and customized a set of LSS 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 the Ultragenyx Continuous Improvement Community of Practice to bring interested practitioners together to share tools, learnings and best practices.

Improving How We Work (cont.)

Spotlight on our Continuous Improvement Community of Practice (UGX CI CoP)

Each quarter we host short, 30-minute learning sessions with Continuous Improvement (CI) practitioners across the company to share Continuous Improvement tools and best practices that leaders, employees and teams can apply in their day-to-day work to increase efficiency and effectiveness, so they spend more time focusing on more meaningful work. As part of our community of practice, we are building and sharing quick reference guides (QRGs) for each of our topics. A sample of the topics we have covered include process mapping and SIPOC, lessons learned, root cause analysis, and mistake proofing.

Sample of our Ultragenyx Continuous Improvement Quick Reference Guide

Ultragenyx Continuous Improvement Quick Reference Guide

DMAIC Define Phase – SIPOC

Why do we use a SIPOC?
To develop a high-level view of the process and its elements to give decision makers a visual of the process and to agree on common language.

What is a SIPOC?
SIPOC is the Suppliers, Inputs, Process, Outputs, and Customer for a given process.

When do we use a SIPOC?
In the early stages of a DMAIC process to introduce team members and stakeholders to the process and agree on common language.

How do we use a SIPOC?
Identify the process owner and all of the process. Next list the process steps and outcomes of the overall process. Then list the suppliers and the outputs (customers).

What's next?
Develop your process map for the 5

Suppliers	Inputs	Process	Outputs	Customers
Definition: Whoever provides the inputs to your process.	Materials, resources, data, information, guidelines required to execute your process.	A process is a collection of activities that takes one or more inputs and creates an output that is of value to the customer.	The products or services resulting from the a process, which address a customer's need.	Whoever receives the outputs of your process – may be internal or external.
Example: Customer, Collection Agency	Customer Complaint, Billing, Question	Start boundary, Receive call	Credit card resolution, Accurate statement	Credit card customer, Collection agency

Ultragenyx Continuous Improvement Quick Reference Guide

Root Cause Analysis – The Five Whys

Why do we use the Five Whys?
To get the root cause of a problem.

What is the Five Whys?
A technique that can be used to identify suspected root causes of a problem by guiding the team into a deeper understanding of the problem and potential links from effect to cause.

SAMPLE

Ask: WHY? → Answer: BECAUSE...
 Ask: WHY? → Answer: BECAUSE...
 Ask: WHY? → Answer: BECAUSE...
 Ask: WHY? → Answer: BECAUSE...
 Ask: WHY? → Answer: BECAUSE...
 Suspected Root Cause

Ultragenyx Continuous Improvement Quick Reference Guide

DMAIC Define Phase – Process Mapping

Why do we process map?
To understand, evaluate, and mature business process, and identify opportunities to improve the efficiency of a process by identifying bottlenecks, gaps, and other breakdowns.

What is a process map?
A graphical representation of the flow or work including the steps, actions, and hand-offs in a business process. Types of process mapping include basic flowchart, alternative path flowchart, and deployment flowchart ("swim lanes").

When do we use process maps?

- To better understand the current state or as-is process to investigate where problems might occur and identify "quick wins" (easy, fast and cheap to implement, reversible, and within the team's control).
- To document the future state or to-be state process after improvement opportunities have been implemented.
- To document the procedure for training or SOPs.

Key questions to ask

- What processes are well understood? What processes are opaque for us?
- Where do we have resource gaps, capacity constraints, or potential fail points?

Best Practice Tip: Use the Deployment Flowchart "swim lanes" process map when possible.

SAMPLES

PREFERRED Deployment Flowchart "swim lanes"
Can be vertical or horizontal

Basic Flowchart **Alternative Path Flowchart**

© 2025 Ultragenyx Pharmaceutical Inc. All Rights Reserved. ultragenyx

Taken together, these efforts continue to help us embed Continuous Improvement in everything that we do at Ultragenyx. In fact, our October 2025 YourVoice Employee Engagement Survey results demonstrated the impact of the CI work that we have been driving across the company with achievement of a 9-point increase in our Continuous Improvement (CI) benchmarks from 2024 showing our improvement to getting to the root cause of problems and sharing learnings and best practices across Ultragenyx.

Improving How We Work (cont.)

AI Philosophy, Strategy & Taskforce

We developed and implemented an AI strategy to identify opportunities to embed AI solutions across all parts of our business. To guide this approach, we crafted an AI Philosophy Statement:

Our AI Philosophy

As a company, we believe in and value human intelligence – knowing that our best thinking comes from the vast and diverse experiences and expertise we collectively bring to our company mission and vision. This belief is foundational as we begin to thoughtfully explore AI to see how it can augment and support the important work we do – but in a way that doesn't replace the essential human intelligence that is core to our work and impact. As with any other technology we use, it's important that we all have awareness and understanding of the benefits, risks and acceptable uses and policies for AI, so we can navigate this new territory in a secure and responsible way.

AI Steering Committee & Taskforce

To launch our AI efforts, we created an AI Steering Committee to oversee our AI strategy and engaged a cross-functional AI Taskforce to help identify high-impact use cases and opportunities for the company.

Key pillars of our AI Strategy are to:

- Increase Employee Capacity & Satisfaction
- Accelerate Drug Development, Production & Discovery
- Enhance Market Leading Rare Disease Commercialization

Our pillars are supported by a set of essential foundational elements, including:

- Governance & Oversight
- Change Management & Communication
- Infrastructure & Security
- AI Training
- AI Communication and Awareness
- External AI Monitoring and Learning
- AI Project Identification, Assessment and Prioritization

Further, we delivered a set of foundational AI trainings to the organization, such as:

- AI & U: Safe & Compliant Use and Our Philosophy at Ultragenyx
- AI & U: Effective Prompting
- Gaining Efficiency with AI Tools

Career Development

We strive to be a place where employees can do the best work of their career and where they can experience profound professional and personal growth. In addition to our set of employee learning and development offerings, we have career development tools and programs designed to support employees growing their careers.

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 2025, more than 70 sessions of coaching were provided to our global workforce through this program.

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

We invest in various training and development programs that are designed to build and strengthen our employees' leadership and professional skills. The goal of our LEAD program, which started in 2018, is to build the next level of leaders that will help us evolve our company and support our culture as we grow. Each of the six LEAD cohorts since the program began have been cross-functional and balanced geographically and by background and experience, with the aim of being inclusive of remote employees. The program provides workshops, business simulations, mentorship and executive leadership talks to help equip participants with the skills to activate change throughout the company. In 2025, 30 directors and senior directors participated in the LEAD program.

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.

Ultragenyx Role Expectations Framework

We developed and launched a more detailed framework that outlines the set of expectations for Associate Director and above roles in a consistent way. This document is designed to be used to guide performance management, future promotions, and talent management decisions within respective functions. The framework includes what we expect individuals at each level to exemplify so they understand the what and the how for their role across three key areas: the scope of the work for their role and the behaviors and competencies that reflect our company values and exemplify our culture.



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 2025, we strengthened our partnership with Biotech Partners, a program designed to equip youth in the Bay Area with the skills and confidence to pursue STEM careers. As part of this initiative, we hosted 10 high school students 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 welcomed undergraduate, graduate and MBA students through internships, co-op placements and APPE programs across our North American locations. Participants engaged in our signature 'Business of Biotech' learning series, executive leadership fireside chats and concluded with poster presentations showcasing their contributions. In 2025, we hosted **40** Emerging Talent participants, reinforcing our commitment to developing the next generation of industry leaders. We transitioned four students into full-time roles and provided contract opportunities to three others, demonstrating the strength and impact of our talent pipeline.

To inspire the next generation of innovators, we connected local high school students through lab tours, classroom presentations and community outreach events. These experiences offered students a firsthand look at the rare disease landscape, cutting-edge biotech technologies and the scientists driving our therapies.

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



Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx pictured with Ultragenyx Emerging Talent program participants.

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, manager effectiveness, benefits, growth and development, and more. We conducted our most recent survey in October 2025 and **~92%** of eligible employees* participated in the survey.

In addition, we use the survey to measure three people-related, “Thriving Culture” 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

Based on the results from the recent survey, we achieved or outperformed all three of our “Thriving Culture” 2025 corporate goals, including employee engagement, inclusion and health and well-being.

The following highlights some of our company-wide strengths:

- Our employees generally have a strong understanding of our purpose and vision, they understand our goals and priorities for the next 12 months, and they see a clear link between their work and Ultragenyx’s goals and priorities
- Employees generally continue to have a strong sense of 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 generally having a positive perception of their direct manager’s support of an inclusive environment, and employees feeling supported and encouraged by their managers to balance work and personal life

- And our employees feel resourceful and dynamic, and when something comes up in their work, they usually know who to ask for help

Finally, in the areas where we have increased our focus and attention over the past two years – Continuous Improvement (CI), Meeting Effectiveness, and Connection & Collaboration – all three categories have shown improvement from 2023 and 2024 internal benchmarks.

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.



* 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 86% overall in 2025, with 92% of our employees responding that they are proud to work at Ultragenyx, 86% feel a personal sense of accomplishment from their work, and 84% intend to stay at Ultragenyx for at least the next 12 months, and 81% 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 fourth consecutive year and by the San Francisco Chronicle for the second consecutive year. We also received the Cultural Excellence Awards from Top Workplaces for Employee Appreciation, Compensation & Benefits, Employee Well-Being, Innovation, Leadership, Professional Development, Purpose & Values, Work-Life Flexibility, and DE&I Practices.

Employee Recognition

Our recognition programs are designed to foster 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 to 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

Inclusive 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.

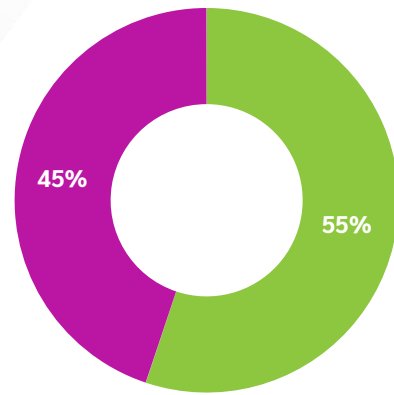
Community groups are created with the purpose of building inclusive spaces for individuals who share common aspects of identity and are open to all employees.

Workforce Data

Total Number of Employees: 1,371*

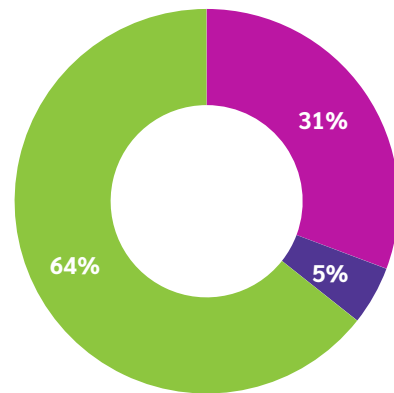
Gender Breakdown (Total Employees)

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



Age Breakdown (Total Employees)

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



Total Number of Employees in Management Positions: 518



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



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



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



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



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



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

Number of Employees Promoted: 162



% of employees promoted who self-reported as female



% of employees promoted who self-reported as male



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



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

* As of December 31, 2025

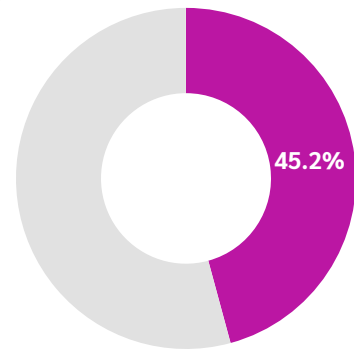
** Percentage of total management positions. Total management positions include all management positions at Ultragenyx, including employees in manager roles.

*** Percentage of total STEM positions.

Workforce Data (cont.)

Total Number of U.S. Employees: 1,144*

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



According to our 2025 internal employee* engagement survey:

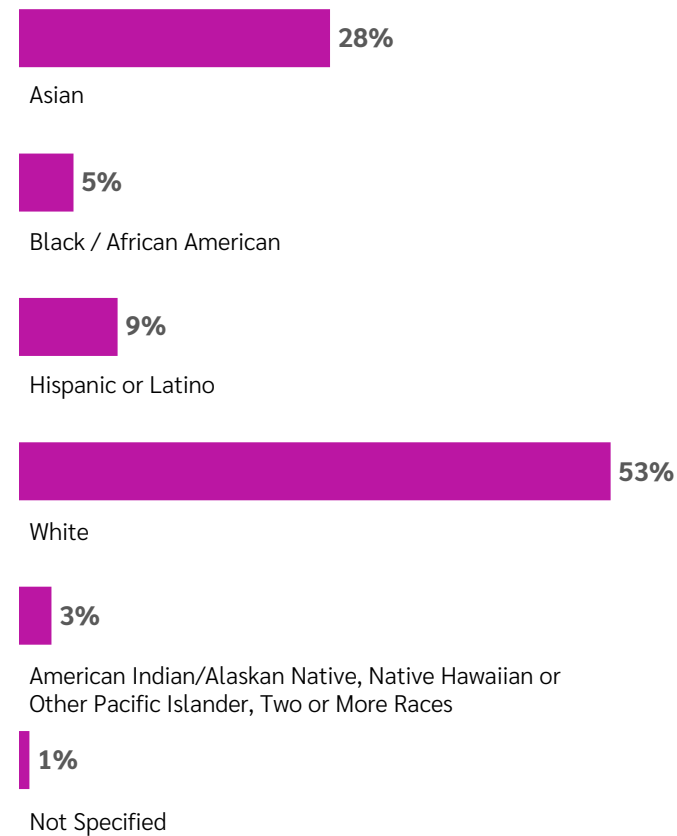
91% of our employees* feel that their managers support inclusion in the workplace

90% feel that their managers model inclusive behaviors

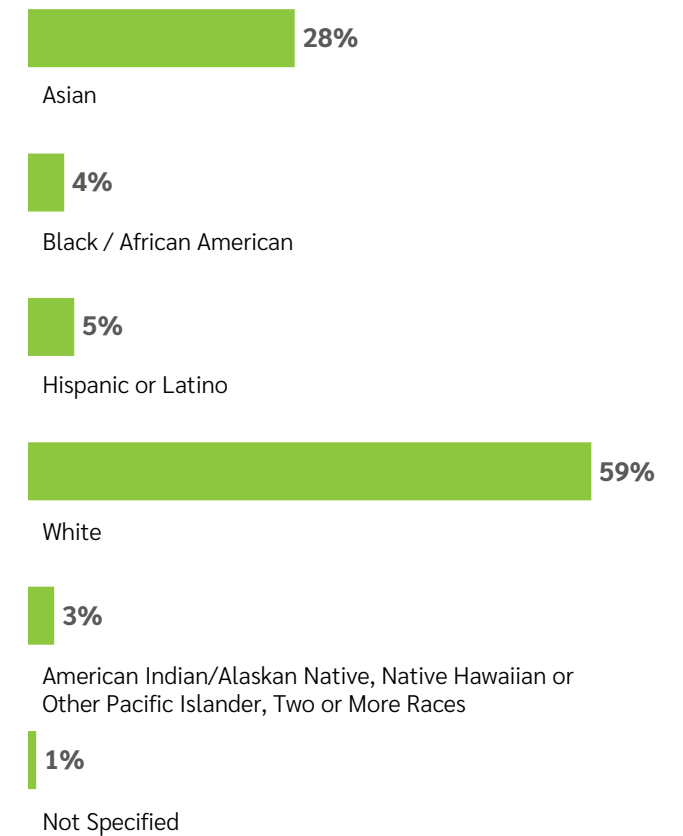
88% feel that their managers create an environment where people feel they belong

*Refers to employees that participated in the survey.

Breakdown of U.S. Employees by Self-Reported Race/Ethnicity



Number of U.S. employees in management positions: 445



*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 for 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 effective 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 2025

Nearly **28%** of new hires came through our employee referral program

Occupational Health and Safety

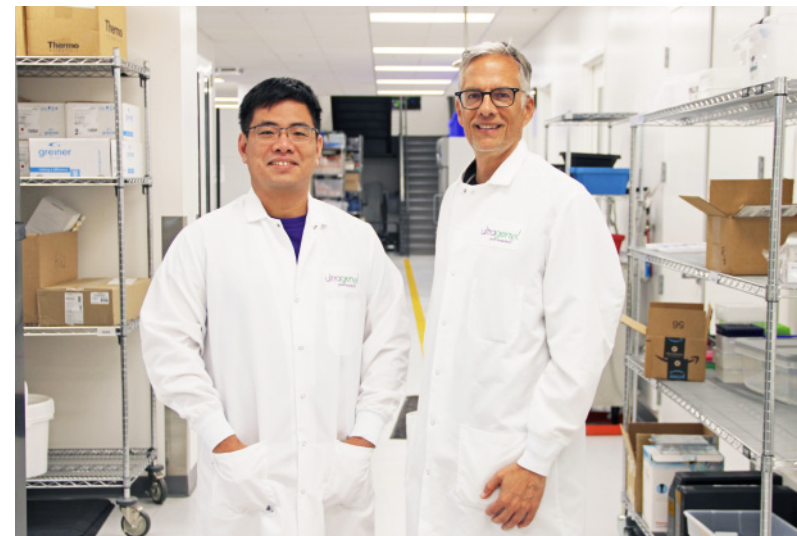
The health and safety of our workforce is a key priority.

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. It 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.

2025 Highlights:

- **Implemented an enterprise EHS Compliance Calendar** designed to proactively ensure regulatory compliance, improve operational consistency across sites, improve record keeping and audit readiness, and designated ownership with escalation paths.
- **Launched a Contractor Management program** including vendor pre-qualification contractor orientation, pre-job risk assessment, monitoring and post-job evaluation.
- **Designed and implemented a Tiered Management System** with key performance indicators to monitor EHS performance and improve outcomes.
- **Published monthly EHSS Performance Reports** to provide an enterprise-wide view on incidents, compliance, targets and training to stakeholders and the executive sponsor.
- **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, contractor management, and potent pharmaceutical compound handling.



Occupational Health and Safety (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, which are designed to familiarize our workforce with site emergency procedures, establish proficiency in executing an orderly evacuation and determine accurate occupant accountability. Reviews are carried out after each drill to identify the effectiveness and opportunities for ERPP improvement.

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.

2025 Safety Data*

Lost Time Incident Frequency Rate (LTIFR)**	0.77
Total Recordable Incident Rate (TRIR)***	0.46
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

Occupational Health Services

Ultragenyx has global processes in place designed to recognize, evaluate, and control occupational health risks. These processes provide for access to designated clinicians or occupational health partners for medical case management, including early intervention, diagnosis, conservative treatment and safe return-to-work procedures, where required. We address the quality of these services through standardized protocols, compliance reviews, and ongoing improvement measures.

It is our policy to safeguard the privacy and confidentiality of employee medical records in compliance with applicable laws and regulations. Participation in occupational health programs and any health-related information cannot be used for favorable or unfavorable treatment of workers.

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 then go to senior leadership for thorough review 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:

- **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
- **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.

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

Employee Compensation and Benefits (cont.)

Employee Wellness

We continue to provide our employees with wellness offerings to support their physical and mental health, such as:

- **Lyra** offers mental wellness screening and stress management, and up to eight free coaching sessions and eight free therapy sessions each year to employees globally and covered dependents.
- **Mindfulness and Meditation programs and informal support groups** are available to employees struggling with caregiving, isolation and stress.
- **Annual flu shot clinics** are held at select 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.
- **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.
- **Semi-annual blood drives** for Ultragenyx volunteers to help potentially save the lives of people injured in accidents or undergoing medical treatment.



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.

Charitable Giving

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

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

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 2025, approximately **450** full-time and part-time employees recorded donating more than **2,800** 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.



Employees in Novato, CA packed STEM Kits during Ultragenyx's 2025 Global Days of Service

In 2025

Approved approximately **\$3.3 million** in charitable donations, independent medical education and health-related grants across more than **20** countries.



Ultragenyx volunteers in California (top) and Italy (bottom) participated in environmental cleanup activities during the annual Global Days of Service

Ultragenyx Global Days of Service

In 2025, we hosted our 3rd annual Global Days of Service. With the support of our nonprofit partners, 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 **40** service projects around the world.

Key highlights from this initiative include:

Significant Impact and Outreach

- Completing numerous environmental projects to enhance local parks, schools and conservation areas
- Packing domestic aid and hygiene kits and making reusable totes, no-sew blankets and dolls 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

Global Days of Service provided employees across regions and roles with meaningful opportunities to step away from day-to-day responsibilities and contribute to causes they care about

strengthening engagement while fostering connection. According to the 2025 Global Days of Service feedback survey, **99%** of respondents reported enjoying their volunteer experience, underscoring the program’s effectiveness in creating positive, energizing moments for employees.

In addition, **87%** of participants met new colleagues through their volunteer activities, highlighting the program’s role in building relationships across teams, locations, and functions.

Strengthening Community Ties

Through service at food banks, schools, and environmental sites, employees deepened their understanding of local needs while building meaningful relationships with community partners. In 2025, **97%** of Global Days of Service participants who completed the feedback survey reported learning something new about a nonprofit organization or the community they served—from increased awareness of food insecurity to insights into local environmental conservation efforts. These experiences strengthened employees’ connection to the communities where they live and work, reinforcing our commitment to being a trusted, engaged community partner.

Rare Disease Community Support

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

In 2025, Ultragenyx proudly supported:

Raising Money to Directly Support Rare Disease Advocacy (California)

Ultragenyx hosted the annual Rare Affair fundraising event, now in its 11th year. Each year, the Rare Affair has focused on raising money and visibility for a different rare disease advocacy organization. The 2025 event gathered hundreds of biotech executives, venture capitalists and rare disease advocates to raise money for the Association for Glycogen Storage Disease (AGSD) to support its mission to promote the best interests of all persons and families affected by Glycogen Storage 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 Nutrition, Health, Interconnectedness and Economic Opportunities (Colombia)

Donated to the D.K. Kim Foundation to support residents in Algarrobo, Colombia impacted by Huntington's Disease and other neurodegenerative diseases. Initiatives aim to provide access to nutritious food through sustainable agriculture, empower individuals and families to generate sustainable income, foster social support networks, and enhance education and awareness about Huntington's Disease within the community.

Improving the Lives of Individuals Affected by Muscular Dystrophy (North Macedonia)

As part of our end of year Proactive Giving program, donated to the Muscular Dystrophy Association Macedonia, which helps provide vital support through resources, advocacy and raising awareness about this rare and debilitating 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 ultra-rare diseases, and empower healthcare professionals to bridge clinical, research and practice gaps.

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 2025

Grants were provided to **180** organizations, across approximately **230** programs, focused on medical education, rare disease awareness, and advocacy efforts in over **20** countries.



“We are excited about what the future holds and remain hopeful for new treatments in the near future.”

– Lisa Hodes

LISA'S STORY

Raising children living with Glycogen Storage Disease Type Ia

For Lisa Hodes and her family, life has often been measured in 30-minute intervals. When both of her daughters, Samantha and Katie, were diagnosed with Glycogen Storage Disease Type Ia (GSDIa), every half hour meant another feeding, another heel prick to check blood sugar levels, another moment of vigilance to keep them alive.

For those living with GSDIa, a rare genetic disorder that results in the buildup of glycogen in the body's cells and an inability to regulate blood sugar levels, a prescribed restricted diet and strict around-the-clock doses of raw cornstarch is critical to sustaining bodily functions.

“In the very early years, we struggled to keep our daughters alive,” Lisa shares. “The fear of missing a feeding was so intense that I basically attended preschool with them.”

As time went on, the family continued to adapt. But with every new stage of life, new challenges emerged. Extremely strict diets made it difficult for the girls to attend school, playdates, class trips and birthday parties. To simply leave the house, a bag was always packed with premeasured cornstarch, snacks, water, emergency rescue supplements and emergency protocol letters.

“There is no such thing as spontaneity. Anything that might require a change in schedule or have an effect on blood sugar levels – traffic, flight delays, meetings, activities, extreme weather, the inability to get food or water – can

cause a life-threatening episode for someone with GSDIa,” Lisa explains. “The mental burden is immense.”

Yet even through these challenges, the Hodes found community. In 2004, the family attended a fundraiser where they discovered The Children's Fund for GSD Research. Lisa immediately became a dedicated board member. To date, the foundation has funded over 60 studies and awarded almost \$9 million to the most promising scientists and institutions around the world to support cutting-edge research.

“Those first few years were frightening and lonely. There wasn't anyone to talk to, ask for help or guidance, or just commiserate with,” Lisa reflects. “The foundation provided a community for us and gave me a new purpose.”

Lisa, her family, and the GSDIa community continue to look forward to the promise of emerging treatments. “We have hope that new advances will alleviate some of the burden and allow our children to live the carefree lives they deserve,” she shares.

GSDIa is an ultra-rare, inherited metabolic disorder that is estimated to affect ~2,500 individuals in the United States and 6,000 individuals globally. Ultragenyx is developing DTX401 (pariglasgene breccaparvovec), an investigational AAV8 gene therapy as a treatment for GSDIa. . The FDA has granted a PDUFA action date of August 23, 2026 for the Company's BLA seeking approval of DTX401. If approved, DTX401 would become the first therapy to directly address the underlying cause of GSDIa.

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.

2025 Highlights

Amplifying Support for Access to Care

Provided a matching gift to the Ritter Center’s annual fundraiser “Under the Stars” to support public health and access to care for at-risk communities

Promoting Public Health and Wellness

Donated to the Marin Community Clinic to support their mission to promote health and wellness through excellent, compassionate care—ensuring access to high-quality medical, dental, behavioral health, and specialty services for the underserved across Marin County.

Access to Healthcare and Nutrition in Argentina

Supported Asociación Civil Humanos con Aprendizajes que Brinden Libertades y Oportunidades to achieve their goal of improving the lives of vulnerable communities and strengthening their holistic development.



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.

2025 Highlights

California-based support:

Partnership with Biotech Partners

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

Developing Future Leaders

Provided a donation to the California Lifesciences Association to help fund their 2025 engagement plans. Helping to open doors to internships and careers for high school students in San Diego and inspiring, educating and connecting emerging scientists and entrepreneurs to grow and diversify biotech in Los Angeles.

Promoting STEAM Education for Local Youth

Donated to the YMCA of greater San Francisco to provide STEAM Startup Kits and offer high-quality STEAM activities for local youth who participate in their afterschool program.

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 and Scientific Adventures for Girls' STEAM Festival. Both are free science festivals 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. Also supported Community Resources for Science's Joyful Explorations in STEM Learning program, supporting thousands of TK-8th grade students from the East Bay by providing hands-on science experiences, diverse STEM role models, and teacher training.

Massachusetts-based support:

Promoting Creativity in STEAM

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.

Igniting Excitement and Confidence in STEM among Underrepresented Girls

Provided support to Science Club for Girls to help deliver programs such as their Junior Mentors Program, and Mentoring Program for K-12 continuum of high-quality, hands-on, free out-of-school-time STEM education and mentorship programs for girls from underrepresented communities.

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.

2025 Highlights

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.



Ultragenyx volunteer in Massachusetts supported the Billerica Food Pantry as part of Ultragenyx's 2025 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.

2025 Highlights

Supporting Wildfire Recovery

Donated to the California Community Fund and NORD which supported communities in Los Angeles, California, to recover from devastating wildfires.

Providing Flood Relief

Contributed to Community Foundation of the Texas Hill Country, providing aid following catastrophic flooding in Texas.

Delivering Hurricane Emergency Aid

Supported Direct Relief with emergency and disaster relief following Hurricane Melissa in the Caribbean.



During 2025 Global Days of Service, Ultragenyx volunteers in Japan created Kiwanis Dolls to support children in the hospital

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.

Reducing Environmental Impacts

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

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. This information enables our team to monitor our performance. In 2025, we implemented a new tool to more efficiently measure and monitor our environmental data across all sites globally.

Ultragenyx acknowledges the impacts and risks posed by climate change on our business operations and stakeholders. In 2025, we conducted an inaugural Climate Risk Assessment to identify actual and potential impacts of climate-related risks and opportunities in our business, including both physical risks and transitional risks. The scope of this assessment encompassed all Ultragenyx facilities as well as select key supplier locations. Our climate 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. For more information, please refer to our [Task Force on Climate Related Financial Disclosures \(TCFD\) Index](#).

My Green Lab Certification in Somerville, MA

Building on the success of achieving My Green Lab Green Certification at our Translational Sciences lab in Novato, California in 2024, Ultragenyx initiated the certification process for our laboratory site in Somerville, Massachusetts. My Green Lab, a nonprofit organization created “for scientists, by scientists,” sets globally recognized standards for sustainable laboratory practices.

The Somerville certification effort is being driven by a cross-functional team of scientists, EHS&S, Lab Operations, and Facilities professionals. The team is currently conducting a baseline assessment to identify opportunities for improvement in areas such as energy efficiency, waste reduction, and sustainable lab behaviors. Potential initiatives may include optimizing equipment energy use, expanding recycling programs, and implementing best practices for resource conservation.

This certification process reflects our commitment to embedding sustainability into scientific operations and advancing environmental stewardship across all Ultragenyx laboratory sites.

Reducing Environmental Impacts (cont.)

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

Green 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 matching the GHG emissions associated

with our electricity use. Purchasing renewable electricity supports the City of Novato’s Climate Action Plan 2030 (CAP2030), which outlines a strategic framework for the city to help support a 40% reduction in statewide emissions below 1990 levels by 2030. Additionally, we purchase renewable electricity for a portion of our energy use at our sites in Brisbane, California.

At our Gene Therapy Manufacturing Facility in Bedford, Massachusetts, we participate in the Bedford Community Choice Aggregation (CCA) program, an initiative offered by the town of Bedford to provide more renewable energy to Bedford residents and businesses.

Through this program, we are supplied with **~100%** renewable electricity through a mix of Massachusetts requirements and voluntary additional renewable energy. All additional renewable energy in the Bedford CCA program comes from sources designated as MA Class I, meaning they are physically part of the New England electricity grid.

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.

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. Additionally, we have installed building management systems that have improved efficiency in use of lighting, heating and cooling, and replaced main passenger elevators with higher-efficiency models.

Airflow Efficiency Project at our Translational Science Laboratory

In April 2025, Ultragenyx implemented an HVAC optimization project at the Translational Science Laboratory in Novato, CA, reducing laboratory airflow by ~30%.

The modifications have been documented in our Environmental Data Repository (EDR), and ongoing monitoring is tracking energy and cost savings.

Preliminary data* indicates an average ~11% reduction in electricity usage (kWh) and average ~8% decrease in associated costs since April 2025, with further analysis underway. This initiative reinforces Ultragenyx’s commitment to lowering energy consumption, reducing HVAC maintenance costs, and enhancing laboratory safety through improved airflow management.

**Data as of May 2026*

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 impact.

In recognition of Earth Month, in April 2025, employees were invited to submit original photos of nature and the environment. For each employee that submitted a photo, the Ultragenyx EHS&S team donated one tree to be planted via The Canopy Project, which works to preserve biodiversity through reforestation. We are proud to include several employee-submitted photos within the Planet chapter of this report.



Photo submitted by Melissa Wolf, associate director, endpoint development and strategy



Photo submitted by Laurence Messerli, senior manager, HR operations EMEA

Managing Waste and Water

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



Photo submitted by Angela Nordhaus, senior director, commercial training and development

We have procedures in place, along with training and compliance audits, to promote appropriate handling and disposal of our waste streams.

Lab Waste Management

We partner with Polycarbin to recycle pipette tip boxes, rigid lab plastics and polystyrene. Through this partnership, Ultragenyx diverted more than **2,700** lbs of waste in 2025. We also partner with vendors to recycle Regulated Medical Waste (RMW) into plastic lumber and in 2025 diverted more than **63,000 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, where available, along with single use gloves.

Office Waste Management

Ultragenyx has put in place comprehensive waste management practices across its office locations, including recycling and composting programs to divert waste from landfills. We partner with specialized recycling services for the secure shredding and recycling of confidential information.

Ultragenyx acknowledges the rapid growth of global e-waste and is committed to reducing waste through circular economy practices. Our E-Waste Policy requires employees to separate e-waste from other waste streams and provides that all materials are handled by certified recyclers that follow environmentally responsible practices.

In 2025, we launched a strategic sustainability initiative across our West Coast offices to significantly reduce single-use cup consumption and promote reusable alternatives. This effort reflects our commitment to embedding environmental stewardship into daily operations, engaging employees in meaningful sustainability practices, and advancing Ultragenyx's broader objectives for waste reduction and responsible resource management.

Managing Disposal of Active Pharmaceutical Ingredients (APIs)

Ultragenyx manages wastewater from our sites in compliance with applicable regulatory requirements. This includes managing effluents and wastewater using site-specific strategies and technologies to reduce active pharmaceutical ingredient (API) levels in wastewater, support compliance with permit requirements, and prevent environmental risks from discharge. In 2025, Ultragenyx achieved ~100% compliance with discharge requirements and continues to evaluate treatment solutions to further reduce potential environmental impacts across our operations.

Assessing Water Risks

Water scarcity is a growing issue globally. Ultragenyx is committed to responsibly managing water impacts across our operated facilities. In 2025, we conducted a comprehensive Water Risk Assessment across all leased and owned facilities using the World Resources Institute (WRI) Aqueduct Water Risk Atlas Tool. Multiple water-related risk indicators were evaluated, with particular emphasis on water stress. These insights will guide future risk mitigation planning and help us identify opportunities to strengthen water management practices, especially at facilities located in water-scarce areas, advancing our broader sustainability strategy.

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 in 2025 include:

- Transitioning to ocean freight shipping lanes for Dojolvi drug substance transportation between Germany and Canada, reducing the associated carbon footprint compared to air freight alternatives.
- Optimizing domestic shipping methods by switching from next-day air to less-than-truckload delivery for U.S. Dojolvi finished goods shipments, significantly reducing our carbon footprint by replacing air transport with less emissions intensive ground transportation.
- Strategically relocating drug product production for Dojolvi from Germany to Canada, eliminating the need for air shipment of heavy glass bottles and reducing associated transportation emissions.



For more information on our supply chain management, please see [Responsible Supply Chain Management](#).

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, safety, quality, data protection and security and responsible procurement.

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 regularly reviewing our practices.

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

Board of Directors

Our Board of Directors provides us with strategic guidance as we work to advance our mission to transform the lives of people with rare disease. Our board is comprised of experienced leaders who represent a broad range of talents, skills, backgrounds and expertise and an average tenure of nine years. 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, 2026, 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
- At least 88% attendance of board and committee meetings in 2025 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 2025, 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
- The company's AI policy and related governance framework
- Cybersecurity and security programs related to our information technology systems
- The continued appropriateness of the company's classified board and other corporate defense features of the company
- The company's approach to evaluating our clinical and preclinical programs
- Clinical study results for the company's late-stage 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 [2025 Annual Report](#).

Business Continuity Management and Disaster Recovery

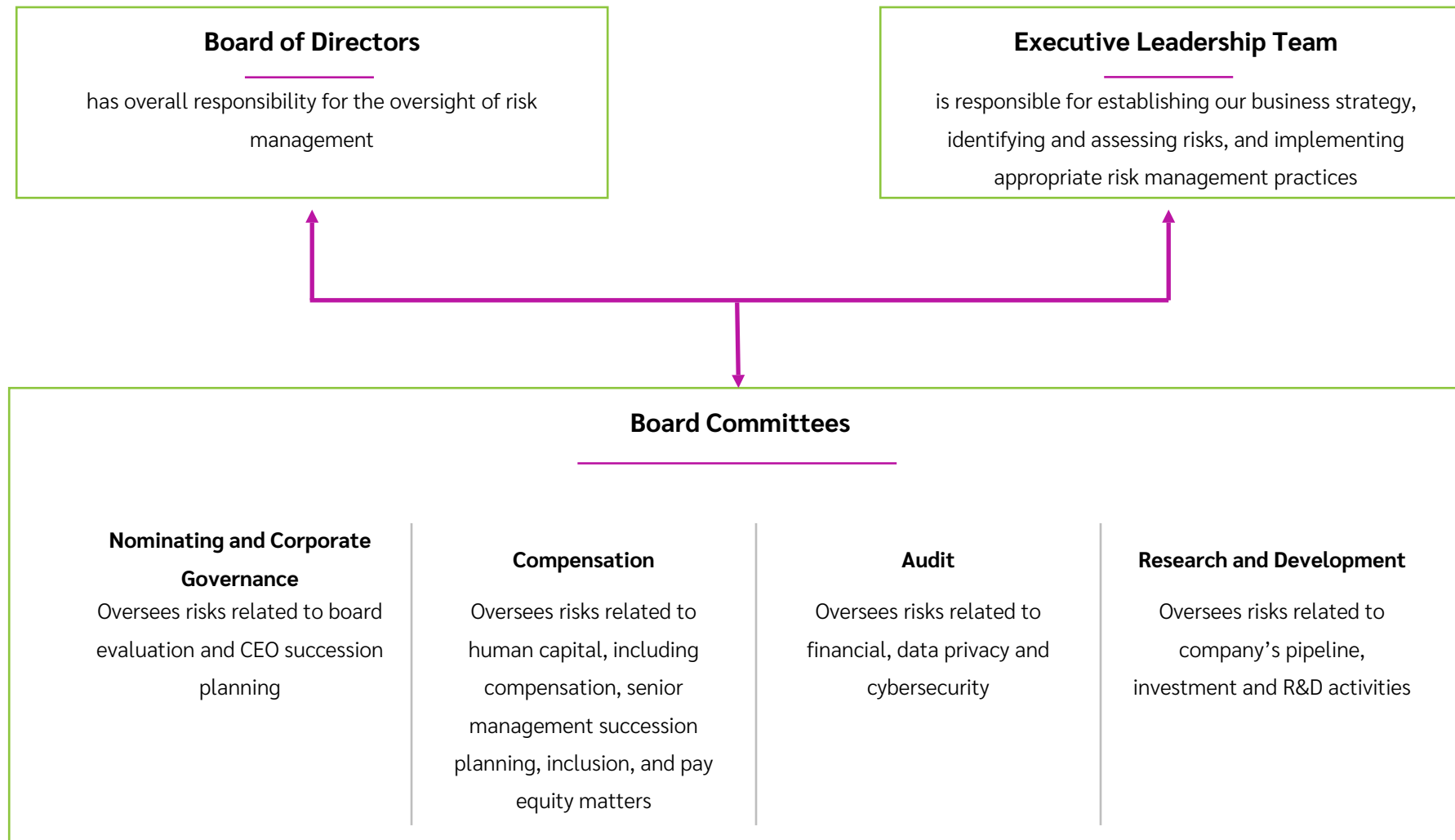
Ultragenyx has a Business Continuity Management (BCM) and Disaster Recovery (DR) program, guided by best practices from organizations such as the International Organization for Standardization (ISO), including ISO 22301. The overarching goal is to continually improve practices and foster the uninterrupted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

For information on our approach to Artificial Intelligence (AI), please refer to [AI Philosophy, Strategy & Taskforce](#).

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.

On an annual basis, employees are expected to receive and acknowledge their understanding of the code of conduct. 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.

~100%

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

~100%

U.S. employees, including part-time and temporary employees, must take a harassment prevention training course every year

~100%

Employees receive training on the code of conduct and other ethical standards

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.

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 ultra-rare 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. Read on to learn more about our [Quality In Supply Chain](#).

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.

For more information on how we consider environmental sustainability in our supply chain, please see [Supply Chain Sustainability](#).

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, data integrity 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.

One voluntary Class III recall was issued in 2025 for a single batch of Crysvida in Colombia. The issue identified does not affect the safety, efficacy, or performance of the product, and all potentially impacted units were accounted for at the conclusion of the voluntary recall.

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.

Patient 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 (GVP) 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
- Investigator Sponsored Trials (ISTs)
- Disease monitoring programs (DMPs) and other Post-Marketing Safety Studies (PASS)
- 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 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 adverse event (AE) and product quality complaints (PQC) reporting processes for all Ultragenyx medicines and the important role they have to ensuring their safety and effective use.

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.

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 2025

*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 commitment to high standards of data security and compliance.

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 data processed by Ultragenyx (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.

In 2025

*Ultragenyx had **no** known security breaches or incidents resulting in material impact to our operations.*

Governance	Training and Awareness	Business Resilience and Compliance	Identifying and Mitigating Cybersecurity Risks
<ul style="list-style-type: none"> Board-level oversight is assigned to our Audit Committee. The CIO reports to the Audit Committee at least on an annual basis to provide an overview of the risks, processes, procedures, recommendations and an overall assessment of our cybersecurity program. Strategic and tactical oversight and direction for cybersecurity-related matters (cyber program strategy, program deficiencies and maturation, program and process review and testing, training and awareness, and reporting) are led by our head of information security, who reports directly to the CIO. Our cybersecurity approach is informed by 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. 	<ul style="list-style-type: none"> 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 also launched a companywide training program on responsible use for Generative AI. We issue regular employee awareness communications, including email newsletters. These communications are intended to consistently inform users about the escalation process to follow if they notice anything suspicious and remind them to report any security incidents to our security email address. 	<ul style="list-style-type: none"> We have documented the appropriate disaster recovery plans for Ultragenyx critical systems. We also have documented our incident response plan’s process and have performed tabletop exercises annually. We updated our critical vendors disaster recovery plans for our information technology systems. We purchase a fixed amount of cybersecurity and crime insurance coverage to help mitigate some of the risk and potential liability from cybersecurity breaches, including claims related to data privacy regulatory matters, third-party lawsuits and the costs associated with data breach events. We require that new and existing systems are built to comply, and remain in compliance with regulatory requirements, including SOX and GxP programs. 	<ul style="list-style-type: none"> We have ongoing managed vulnerability scanning and patching through our vulnerability management program. We monitor internal and external cybersecurity threats and review and revise our cybersecurity defenses on a regular cadence and as needed. Targeted audits and penetration tests were conducted throughout 2025 by internal and external entities. In 2025, 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 maintaining robust solutions for cloud security, data loss prevention and privileged access management.

SASB Index

The following index lists the activity and accounting metrics from the Sustainability Accounting Standards Board (SASB) Biotechnology & Pharmaceuticals Sustainability Accounting Standard (Industry Standard | Version 2023-12) with associated response, reference or report location.

Accounting Metric	Code	Response / Reference / Report / Location
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 region, of management process for ensuring quality and patient safety during clinical trials	HC-BP-210a.1	See our 2025 Impact Report, Clinical Trials , Quality and Patient Safety .
	Number of inspections related to clinical trial management and pharmacovigilance that resulted in: (1) entity voluntary remediation or (2) regulatory or administrative actions taken against the entity	HC-BP-210a.2	There were no inspections related to clinical trial management and pharmacovigilance that resulted in either Voluntary Action Indicated (VAI) or Official Action Indicated (OAI) in 2025. For U.S. information, see FDA Compliance Dashboard .
	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 2025. See our 2025 Annual Report , page 65 (Item 3. Legal Proceedings).

SASB Index (cont.)

Topic	Accounting Metric	Code	Response / Reference / Report / Location
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 ultra-rare diseases, which fall outside the 2025 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 2025 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.
Affordability & Pricing	Percentage change in: (1) weighted average list price and (2) weighted average net price across product portfolio compared to previous reporting period	HC-BP-240b.2	See our 2025 Impact Report, Access to our Therapies .
	Percentage change in: (1) list price and (2) net price of product with largest increase compared to previous reporting period	HC-BP-240b.3	See our 2025 Impact Report, Access to our Therapies .
Drug Safety	Products listed in public medical product safety or adverse event alert databases	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	HC-BP-250a.2	There were no fatalities associated with Ultragenyx products in 2025. See the FDA FAERS MedWatch website for more information.
	(1) Number of recalls issued, (2) total units recalled	HC-BP-250a.3	See our 2025 Impact Report, Quality in Supply Chain .
	Total amount of product accepted for takeback, reuse, or disposal	HC-BP-250a.4	Ultragenyx manufactures medicines on a schedule that is designed to avoid their expiration before patient use. In the event that medicines expire before use and are returned, are found to be unsuitable for release or are subject to a recall or withdrawal notice, Ultragenyx does not reintroduce them again for reuse. They get disposed of using regulated and monitored incineration processes.
	Number of enforcement actions taken in response to violations of good manufacturing practices (GMP) or equivalent standards, 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 2025 Impact Report, Quality in Supply Chain , section on Counterfeit Products.
	Discussion of process for alerting customers and business partners to 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 2025 Impact Report, Quality in Supply Chain , section on Counterfeit Products.
	Number of actions that led to raids, seizure, arrests, or filing of criminal charges related to counterfeit products	HC-BP-260a.3	Ultragenyx had no instances of actions related to counterfeit products in 2025. See our 2025 Impact Report, Quality in Supply Chain , section on Counterfeit Products.
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 2025. See our 2025 Annual Report, page 65 (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 2025 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 2025 Impact Report, Human Capital Development , and our 2025 Annual Report , (Item 1. Business – Human Capital).
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 2025. See our 2025 Annual Report , page 65 (Item 3. Legal Proceedings).
	Description of code of ethics governing interactions with health care professionals	HC-BP-510a.2	See our 2025 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.

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, 2025, unless otherwise noted. Additionally, we map our GRI disclosures against several of the UN Sustainable Development Goals (SDGs). *GRI 1 used: GRI: Foundation 2021.*

GRI Standard	Disclosure	UN SDG	2025 Location
GRI 2: General Disclosures 2021	2-1 Organizational details		2025 Impact Report, About Us and 2025 Annual Report , Item 1. Business.
	2-2 Entities included in the organization’s sustainability reporting		The 2025 Impact Report mainly covers information from the fiscal year ending December 31, 2025, unless otherwise indicated. Questions and inquiries on the reported information can be submitted to impact@ultragenyx.com . 2025 Impact Report, About This Report .
	2-3 Reporting period, frequency and contact point		Ultragenyx publishes an Impact Report annually. 2025 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		2025 Annual Report , Item 1. Business.
	2-7 Employees	8, 10	2025 Impact Report, Workforce Data , and 2025 Annual Report , Item 1. Business – Human Capital
	2-8 Workers who are not employees	8	Information unavailable.
	2-9 Governance structure and composition	5, 16	2025 Impact Report, Corporate Governance . 2025 Impact Report, Corporate Responsibility Oversight .
	2-10 Nomination and selection of the highest governance body	5, 16	2026 Proxy , Nomination of Directors.
	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	2025 Impact Report, Corporate Responsibility Oversight . 2026 Proxy , Board Committees
	2-13 Delegation of responsibility for managing impacts		2025 Impact Report, Corporate Responsibility Oversight .

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2025 Location
	2-14 Role of the highest governance body in sustainability reporting		2025 Impact Report, Corporate Responsibility Oversight .
	2-15 Conflicts of interest	16	2025 Impact Report, Corporate Governance . Corporate Governance Guidelines .
	2-16 Communication of critical concerns		2025 Impact Report, Risk Management . 2025 Annual Report , Item 1A. Risk Factors.
	2-17 Collective knowledge of the highest governance body		2026 Proxy , Building the Right Board .
	2-18 Evaluation of the performance of the highest governance body		Corporate Governance Guidelines .
	2-19 Remuneration policies		2026 Proxy , Executive Compensation . 2026 Proxy , Director Compensation .
	2-20 Process to determine remuneration		2026 Proxy , Executive Compensation . 2026 Proxy , Director Compensation .
	2-21 Annual total compensation ratio		2026 Proxy , CEO Pay Ratio .
	2-22 Statement on sustainable development strategy		2025 Impact Report, Letter From Our CEO . 2025 Impact Report, Impact Report Approach . GRI Index
	2-23 Policy commitments	16	2025 Impact Report, Governance chapter Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-24 Embedding policy commitments		2025 Impact Report, Governance chapter Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-25 Processes to remediate negative impacts		2025 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	2025 Impact Report, Raising A Concern Global Code of Conduct . Global Standard for Suppliers . Human Rights Policy .
	2-27 Compliance with laws and regulations		2025 Impact Report, Ethics and Integrity 2025 Annual Report , Item 3 Legal Proceedings.
GRI 2: General Disclosures 2021 (cont.)	2-28 Membership associations		2025 Impact Report, Collaborations 2025 Impact Report, Public Policy Participation . 2025 Impact Report, Patient and Caregiver Advocacy and Engagement .

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2025 Location
GRI 2: General Disclosures 2021 (cont.)	2-29 Approach to stakeholder engagement		2025 Impact Report, Impact Report Approach .
	2-30 Collective bargaining agreements	8	2025 Annual Report , Item 1. Business – Human Capital.
GRI 3: Material Topics 2021	3-1 Process to determine material topics		2025 Impact Report, Materiality Assessment .
	3-2 List of material topics		2025 Impact Report, Materiality Assessment .
	3-3 Management of material topics		The 2025 Impact Report describes the management of material topics by section. 2025 Annual Report , Item 1A. Risk Factors.
GRI 201: Economic Performance 2016	201-1 Direct economic value generated and distributed	8, 9	2025 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	2025 Impact Report, Reducing Environmental Impacts . TCFD Index
	201-3 Defined benefit plan obligations and other retirement plans		2025 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	2025 Impact Report, Access to our Therapies . 2025 Impact Report, Communities chapter .
	203-2 Significant indirect economic impacts	1, 3, 8	2025 Impact Report, Access to our Therapies . 2025 Impact Report, Communities chapter .
GRI 205: Anti-corruption 2016	205-1 Operations assessed for risks related to corruption	16	2025 Annual Report, Item 1A. Risk Factors. 2025 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	2025 Impact Report, Ethics and Integrity . Global Code of Conduct . Global Standard for Suppliers .
	205-3 Confirmed incidents of corruption and actions taken	16	2025 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	2025 Annual Report , Item 3 Legal Proceedings.
GRI 303: Water and Effluents 2018	303-1 Interactions with water as a shared resource	6, 12	2025 Impact Report, Managing Waste and Water .
GRI 305: Emissions 2016	305-5 Reduction of GHG emissions	13, 14, 15	2025 Impact Report, Reducing Environmental Impacts .
GRI 306: Waste 2020	306-2 Management of significant waste-related impacts	3, 6, 8, 11, 12	2025 Impact Report, Managing Waste and Water .

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2025 Location
GRI 401: Employment 2016	401-1 New employee hires and employee turnover	5, 8, 10	2025 Impact Report, Human Capital Development , and 2025 Annual Report, Item 1. Business – Human Capital
	401-2 Benefits provided to full-time employees that are not provided to temporary or part-time employees	3, 5, 8	2025 Impact Report, Employee Compensation and Benefits . Benefits .
	401-3 Parental leave	5, 8	2025 Impact Report, Employee Compensation and Benefits . Benefits .
GRI 403: Occupational Health and Safety 2018	403-1 Occupational health and safety management system	8	2025 Impact Report, Occupational Health and Safety
	403-2 Hazard identification, risk assessment, and incident investigation	8	2025 Impact Report, Occupational Health and Safety
	403-3 Occupational health services	8	2025 Impact Report, Occupational Health and Safety
	403-4 Worker participation, consultation, and communication on occupational health and safety	8, 16	2025 Impact Report, Occupational Health and Safety
	403-5 Worker training on occupational health and safety	8	2025 Impact Report, Occupational Health and Safety
	403-6 Promotion of worker health	3	2025 Impact Report, Occupational Health and Safety
	403-8 Workers covered by an occupational health and safety management system	8	2025 Impact Report, Occupational Health and Safety
	403-9 Work-related injuries	3, 8, 16	2025 Impact Report, Occupational Health and Safety
	403-10 Work-related ill health	3, 8, 16	2025 Impact Report, Occupational Health and Safety
GRI 404: Training and Education 2016	404-2 Programs for upgrading employee skills and transition assistance programs	8	2025 Impact Report, Employee Learning and Development . 2025 Impact Report, Career Development .
	404-3 Percentage of employees receiving regular performance and career development reviews	5, 8, 10	2025 Impact Report, UltraPerformance Management .
GRI 406: Non-discrimination 2016	406-1 Incidents of discrimination and corrective actions taken	5, 8	2025 Impact Report, Raising A Concern .
GRI 408: Child Labor 2016	408-1 Operations and suppliers at significant risk of incidents of child labor	5, 8, 16	2025 Impact Report, Responsible Supply Chain Management Global Code of Conduct . Global Standard for Suppliers .

GRI Index (cont.)

GRI Standard	Disclosure	UN SDG	2025 Location
GRI 409: Forced or Compulsory Labor 2016	409-1 Operations and suppliers at significant risk for incidents of forced or compulsory labor	5, 8	2025 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		2025 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	2025 Impact Report, Public Policy Participation .
GRI 416: Customer Health and Safety 2016	416-1 Assessment of the health and safety impacts of product and service categories		2025 Impact Report, Patient Safety .
	416-2 Incidents of non-compliance concerning the health and safety impacts of products and services	16	2025 Impact Report, Quality . 2025 Impact Report, Patient Safety . FDA Data Dashboard .
GRI 417: Marketing and Labeling 2016	417-1 Requirements for product and service information and labeling	12	2025 Annual Report , Item 1. Business. Crysvita . Mepsevii . Dojolvi . Evkeeza .
	417-2 Incidents of non-compliance concerning product and service information and labeling	16	2025 Annual Report , Item 1A. Risk Factors.
	417-3 Incidents of non-compliance concerning marketing communications	16	2025 Impact Report, Interactions with Patients, Caregivers and Healthcare Professionals . 2025 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	2025 Impact Report, Data Privacy .

TCFD Index

Information presented in this Index references the Task Force on Climate-Related Financial Disclosures (TCFD) voluntary recommended disclosures on climate-related financial risk management. As of 2023, TCFD monitoring is fully incorporated under the IFRS Foundation. Relevant information included below covers the reporting period between January 1, 2025 through December 31, 2025 unless otherwise indicated in specific disclosures.

Metric	Response
Governance	
Describe the board’s oversight of climate-related risks and opportunities	<p>Ultragenyx is 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, which operates under a written charter setting forth the functions and responsibilities of the committee, regularly reviews and makes recommendations to management and the Board on sustainability and corporate responsibility matters including policies and initiatives.</p> <p>The Board of Directors, its committees and Ultragenyx’s Executive Leadership Team oversees the company’s risk management program. The Nominating and Corporate Governance Committee oversees risks related to corporate responsibility and sustainability and reports on them as appropriate to the Board.</p>
Describe management’s role in assessing and managing climate-related risks and opportunities	<p>The Corporate Responsibility Working Group, a cross-functional team sponsored by executive leadership, is responsible for the execution of Ultragenyx’s corporate responsibility strategy. This group works with topic experts and business leaders to drive implementation and internal stewardship and reports to the Executive Leadership Team on the company’s progress. The Executive Leadership Team is responsible for identifying and assessing risks and implementing appropriate risk management practices.</p>
Strategy	
Describe the climate-related risks and opportunities the organization has identified over the short, medium, and long term	<p>For purposes of its Climate Risk Assessment, Ultragenyx established the following time horizons for assessing climate risks and opportunities and their respective actual and potential impacts on the business:</p> <ul style="list-style-type: none"> • Short term: 0 – 5 years • Medium Term: 6 – 10 years • Long Term: 11 – 25 years <p>Based on these timelines, Ultragenyx identified and assessed potential acute and chronic physical risks, as well as transition risks associated with a transition to a lower-greenhouse gas (“GHG”) emission economy.</p>

TCFD Index (cont.)

Metric	Response
Strategy (cont.)	
Describe the climate-related risks and opportunities the organization has identified over the short, medium, and long term	<p>Ultragenyx leveraged publicly available climate risk assessment tools – FEMA’s National Risk Index, NASA’s Sea Level Projection Tool, USGS’s Climate Change Viewer, and WRI’s Aqueduct – to identify potentially material acute and chronic physical risks at its offices and priority supply chain locations. Based on availability within these tools, this analysis leveraged SSP2-4.5 scenario modeling data.</p> <p>Ultragenyx conducted industry benchmarking and workshops with relevant internal stakeholder groups to qualitatively assess potential transition risks to its direct operations and supply chain based on the following types of transition risks:</p> <ul style="list-style-type: none"> • Policy and legal risk • Technology risk • Market risk • Reputation risk
Describe the impact of climate-related risks and opportunities on the organization’s business, strategy, and financial planning	After these analyses, members of the Corporate Responsibility Working Group reviewed a refined list of what were considered the most probable physical and transition risks and determined that none were currently expected to have a material effect on its present operations, business strategy, and financial planning processes during the time horizons reviewed. These results were consistent with the results of the company’s ongoing risk management progress described below. As of the publication date of this report, Ultragenyx has not experienced material business impacts from climate-related matters.
Describe the resilience of the organization’s strategy, taking into consideration different climate-related scenarios, including a 2°C or lower scenario	Based on its ongoing risk management assessments and qualitative scenario analysis through the 2025 Climate Risk Assessment, Ultragenyx does not anticipate material business impacts from climate change based on its current business model and facility locations. As of the publication date of this report, there have been no material financial effects, and the supply chain has not been identified as being prone to material physical risks. The company has nonetheless invested in energy efficiency and other initiatives to reduce its environmental footprint, as described in its impact reports.
Risk Management	
Describe the organization’s processes for identifying and assessing climate-related risks	As appropriate to comply with applicable reporting obligations, Ultragenyx plans to review actual and potential physical and transition risks through a similar process to the 2025 Climate Risk Assessment.
Describe the organization’s processes for managing climate-related risks	Ultragenyx has determined that climate change does not pose a material risk to its business as of this publication. As a result, it has not implemented formal processes for managing such risks or opportunities. However, if future assessments identify material risks, the company expects to consider appropriate mitigation strategies.
Describe how processes for identifying, assessing and managing climate-related risks are integrated into the organization’s overall risk management	Ultragenyx assesses climate-related risks as part of its overall risk management processes through its Corporate Responsibility Governance structure. The company’s risk management process 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 the company’s 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.
Metrics and Targets	
Ultragenyx is assessing a plan to disclose relevant metrics and targets in the future.	



2025 Ultragenyx Impact Report

[Ultragenyx.com](https://www.ultragenyx.com)