ultragenyx

2022 ESG Report

Going beyond every day.™

Forward-Looking Statements & Other Important Legal Information

This document and the materials or websites cross-referenced contain statements that are aspirational or reflective of our views about our future performance that constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are generally identified through the inclusion of words such as "aim," "anticipate," "aspire," "believe," commit," "endeavor," "estimate," "expect," "goal," "intend," "may," "plan," "seek," "strive," "target," "will," vision," "mission," "strategy," "commitment" and "work," or similar statements or variations of such terms and other similar expressions. The forward-looking statements in this document and the materials or websites cross-referenced concern Ultragenyx's goals, progress or expectations with respect to corporate responsibility, sustainability, patients, products, product candidates, employees, environmental matters, policy, and business risks and opportunities. Forward-looking statements inherently involve risks and uncertainties that could cause actual results to differ materially from those predicted in such statements. These statements are based on numerous assumptions that we believe are reasonable, but are open to a wide range of uncertainties and business risks. In addition, these statements may be based on standards for measuring progress that are still developing, controls and processes that continue to evolve, and assumptions that are subject to change in the future. Consequently, actual results may vary materially from what is contained in a forward-looking statement. 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 17, 2023 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 may not be considered material for SEC reporting purposes. In the context of this report, the term "material" is distinct from, and should not be confused with, such term as defined for SEC reporting purposes. 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 document and the materials or websites cross-referenced includes 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.

Cover photo: Sally lives in central Pennsylvania with her husband and two children, ages 1 and 4. She is a classical violinist who teaches violin and is the section leader in a local symphony. Sally, her 1-year-old daughter, Ellie, and Sally's mother live with X-linked hypophosphatemia (XLH). Growing up, Sally experienced bone dislocations, dental problems and years of chronic joint and foot pain. Looking back, she wonders if she should have chosen a different career, because even the healthiest musicians are prone to repetitive stress injuries and pain. But she has learned to manage her XLH fairly well. Sally is hopeful that Ellie's diagnosis and management of XLH at such a young age will mean Ellie will have an easier time and fewer symptoms than Sally or her mother had.

About this Report

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

Our headquarters campus is in Novato, California, and we have offices and laboratories in 13 countries across the United States (U.S.); Europe, the Middle East and Africa (EMEA); Asia Pacific (APAC); and Latin America (LATAM). In the U.S., we have offices and/or laboratories in California, Florida, Massachusetts, Texas and Utah.

Ultragenyx prepares an annual environmental, social and governance (ESG) report. This 2022 report contains ESG disclosures for the period January 1 through December 31, 2022, unless otherwise noted. The scope of our 2022 ESG Report is Ultragenyx's wholly owned operations globally. Third-party manufacturing is not included.

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

Contact

We welcome your feedback. Please contact us at esg@ultragenyx.com with your comments and suggestions about this report or our ESG program.

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Letter From Our CEO

Dear Stakeholders,

A hallmark of 2022 for Ultragenyx was bringing our team members back into the workplace and hosting our first in-person, all-company meeting post-COVID. It was truly special to see the energy and dedication of our team united behind a common mission **to transform the lives of people with rare disease**. We didn't just make it through the COVID-19 pandemic, we grew in number and in strength.

Everyone who was at that meeting, and all of our team members at Ultragenyx, have contributed to the advancement of our business and pipeline and play an instrumental role in developing therapeutics with the potential to help many people around the world. Last year, we achieved meaningful progress on key clinical programs: We shared promising interim Phase 1/2 data in Angelman syndrome, fully enrolled our pivotal gene therapy program in glycogen storage disease type 1a (GSD1a), and dosed the first patients in pivotal studies for Osteogenesis Imperfecta and Wilson disease. We also expanded commercial access to our approved products, with launches of Crysvita® (burosumab-twza) in Argentina and Mepsevii® (vestronidase alfa-vjbk) in Japan.

At the same time, we made strategic investments that further bolster our clinical and commercial pipeline and support our vision of leading the future of rare disease medicine. Specifically, we gained ex-U.S. commercial rights to Evkeeza® for clinical homozygous familial hypercholesterolemia (HoFH), acquired global rights to AAV gene therapy candidate UX111 for the treatment of Sanfilippo syndrome, exercised our option to acquire GeneTx Biotherapeutics following promising interim data from the Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome, and completed the build-out of our gene therapy manufacturing plant in Bedford, Massachusetts, which we expect to begin production this spring. This facility and our manufacturing capabilities are expected to position us to better control our costs and scale the production of our gene therapies, establishing us as a commercial-ready gene therapy company.

In addition to our focus on innovation, we made progress against our other ESG objectives last year. For our team members, we introduced a mandatory education program called "Declaring and Repairing Breakdowns" to further embed inclusion and diversity into our culture. For our communities, we formalized our corporate philanthropy framework, with a focus on four pillars: supporting communities affected by rare diseases, promoting equitable and accessible healthcare for at-risk communities, promoting science, technology, engineering, the arts and math (STEAM) education, and supporting local and at-risk communities. For our planet workstream, we implemented measures for collecting and analyzing our environmental data in order to monitor and reduce our environmental impact. Finally, related to governance, we released our Global Standard for Suppliers, which outlines expectations for suppliers regarding labor standards, human rights and environmental stewardship.

It was gratifying to see Ultragenyx recognized in the top 5% of companies in the biotechnology sector assessed by the S&P Global Corporate Sustainability Assessment based on our inaugural 2021 report. Our company has always had a focus on creating value while also doing good for our communities, and we believe our score reflects that.

On that note, one area where Ultragenyx has invested time and resources is increasing the overall capacity in our industry for ultrarare disease drug development. Initiatives like our Rare Bootcamp are geared at sharing our knowledge and expertise with rare disease foundations embarking on their own R&D efforts. There are thousands of known ultrarare conditions with no treatments and approximately 90% of the rare disease population is waiting for new therapies that can create positive change.

In 2023, we celebrate the 40th anniversary of the Orphan Drug Act (ODA), which created an industry that didn't previously exist in the United States. It enabled the

Above photo: Dr. Emil Kakkis with Kelly, who has X-linked hypophosphatemia (XLH)

formation of companies to develop and commercialize therapies for rare diseases. Without the ODA, Ultragenyx would not be where we are today. Since our founding in 2010, we have achieved four approved therapies in five indications and our medicines have treated over 3,200 patients globally. We now have a pipeline of product candidates with seven clinical programs, five in pivotal studies, and multiple parallel opportunities, supporting our efforts to generate long-term value and transform the lives of many more people with rare disease.

The capability to treat ultrarare diseases has vastly increased over the last 40 years. The scientific advancements that have made this possible have surpassed what the ODA was designed to achieve. As we look ahead, we intend to continue to advocate for policy and regulatory changes to further facilitate rare and ultrarare drug development. Let's push for the next act in rare disease so people living with rare and ultrarare diseases and their families can look forward to the future with hope and optimism.

We encourage our stakeholders to engage with us on our ongoing ESG efforts and on policy initiatives that address the gap between innovation and progress in rare disease.

Sincerely,

Emil D. Kakkis, M.D., Ph.D.
founder, president and CEO

Awards

The San Francisco Business Times named Ultragenyx the **#3 Best Place to Work** in the Bay Area among companies with 500-999 employees and #2 for workplace wellness.

The Boston Globe named Ultragenyx as a **Top Places to Work** in Massachusetts.

Global Health & Pharma named Ultragenyx the **Best Leading Rare Disease Medicines & Therapies Company** in the 2022 Healthcare and Pharmaceutical Awards.

BioSpace named Ultragenyx one of the **Best Places to Work** in the life science industry in 2022.

2022 Drug Highlights

Crysvita® approved in Argentina.

Brazilian Ministry of Health issued guidelines for treatment of pediatric XLH patients with Crysvita to facilitate reimbursement.

First patients obtained commercial access to Crysvita in Costa Rica and Chile.

Product Listing Agreements for Crysvita completed for all 10 Canadian provinces.

Expanded access to Crysvita for tumor-induced osteomalacia (TIO) and XLH adult patients achieved with private insurers in Canada.

First patients obtained commercial access to Dojolvi® in Argentina and Brazil.

Received a positive public health technology assessment (HTA) recommendation for Dojolvi, providing a pathway for public access for patients in Canada with long-chain fatty acid oxidation disorders (LC-FAOD).

Mepsevii® approved for reimbursement in Portugal, launched in Japan and approved in the U.K. for infants.

Evkeeza® obtained marketing authorization for HoFH in the European Economic Area.

Acquired UX111 from Abeona Therapeutics, currently in pivotal Phase 1/2/3 development for Sanfilippo syndrome.

Released interim data results from Phase 1/2 program for GTX-102 for the treatment of Angelman syndrome.

About Us

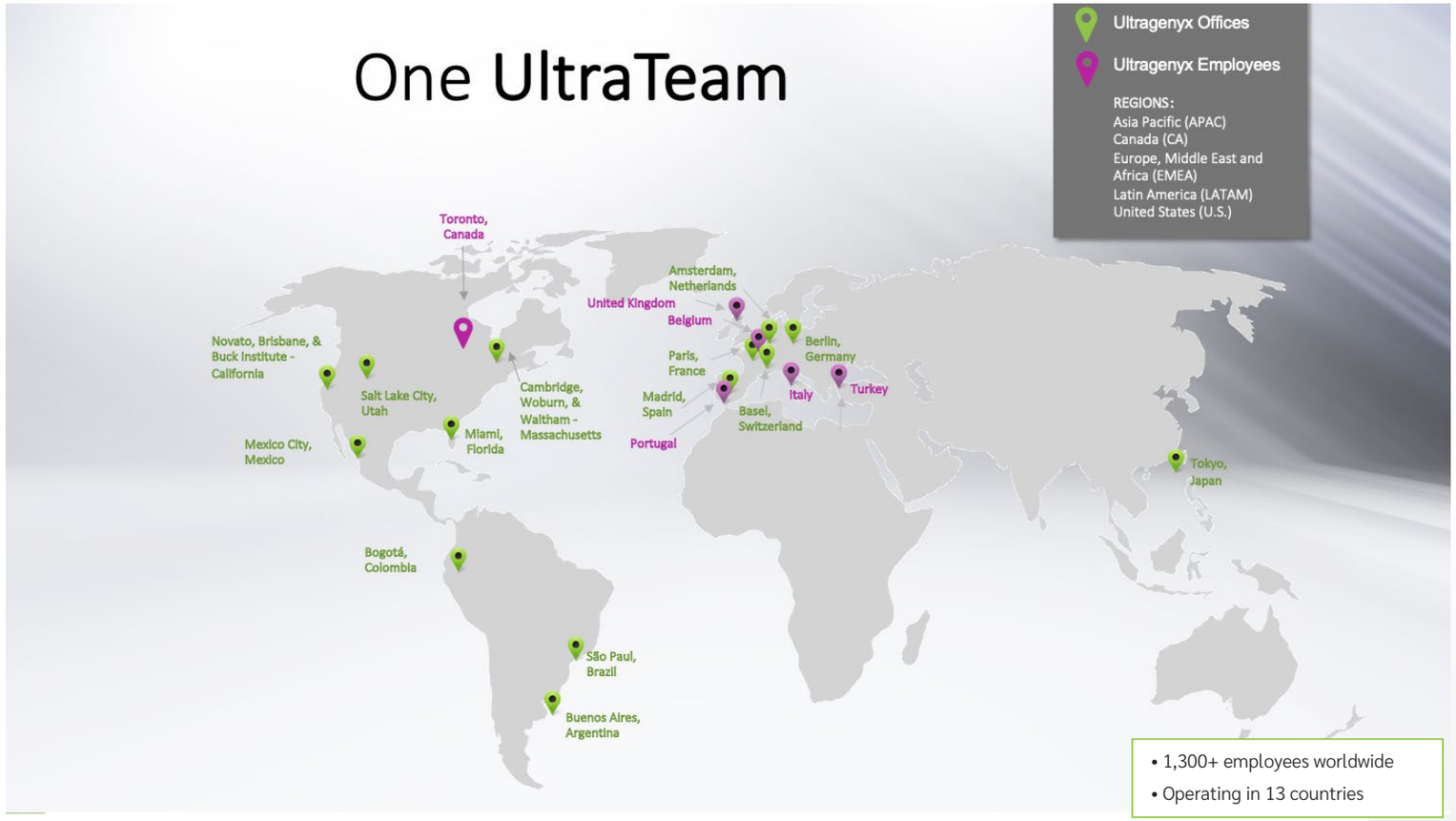
Our vision: Lead the future of rare disease medicine

Our mission: Transform the lives of people with rare disease

About Us

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

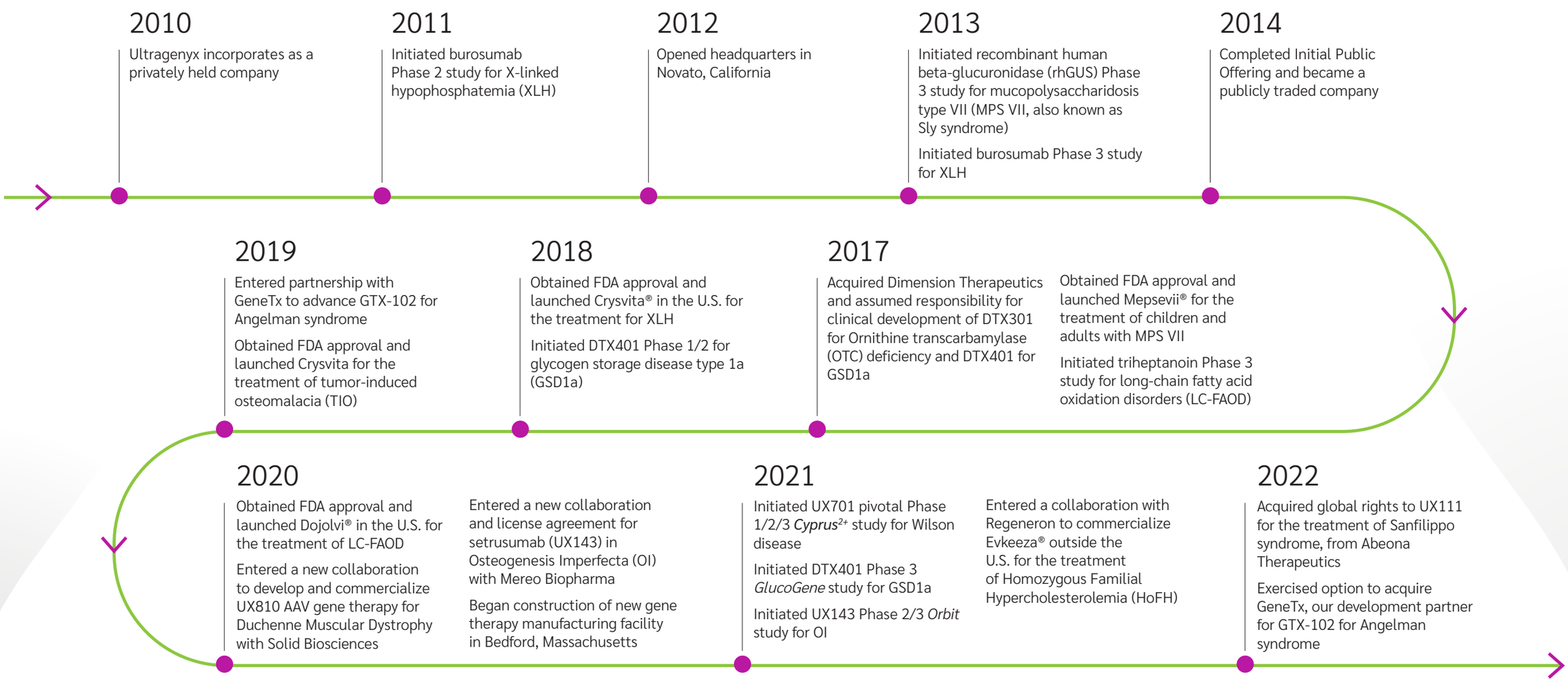
Our purpose is to lead the future of rare disease medicine as we seek to treat individuals afflicted by diseases with limited or no treatment options. We recognize that their lives and well-being are dependent upon our efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care.



“Ultragenyx’s core values are grounded in our dedication to the rare and ultrarare disease community. We have established an innovative drug development approach that leverages a deep understanding of disease biology and the optimal drug modality to target the underlying cause of disease. We also apply insights from the patient experience to support our efforts in developing therapies that can effectively address their needs. We believe it is our responsibility to support equitable care in rare diseases, and so we work to improve access and affordability, and to engage in policy initiatives that benefit all R&D programs for rare and ultrarare diseases.”

Eric Crombez, M.D., executive vice president and chief medical officer

Ultragenyx Celebrates 12 Years



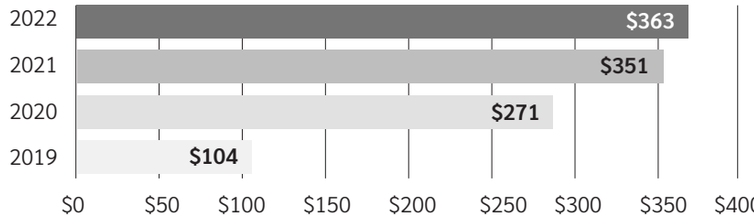
2022 Highlights

- **Four** approved treatments for five different diseases: Crysvita® for XLH and TIO, Mepsevii® for mucopolysaccharidosis type VII (MPS VII), Dojolvi® for LC-FAOD and Evkeeza® for HoFH.
- **3,200+** patients treated through commercial access and expanded use.
- **26** countries where products are sold.
- **200+** clinical trial sites in operation in 24 countries.
- **$705.8 million** in R&D investment.
- **$363.3 million** in annual revenue.
- **Certificate of occupancy and use** received from the town of Bedford, Massachusetts, for our gene therapy manufacturing facility, which is expected to begin production this spring.

THE ULTRAGENYX JAPAN TEAM, ESTABLISHED IN 2022

In Japan, rare diseases are referred to as “intractable diseases.” The term is derived from the word Nanbyo, which translates to “difficult + illness,” reflecting the heavy burden on those affected and their families. Intractable diseases are treated differently in Japan than in the United States and other countries, with most medical costs covered for patients by the government. In addition, Japan implemented a drug price standards list, which sets the price for each approved medication. To date, 788 diseases for children and 331 diseases for adults are designated as intractable diseases in Japan, and all the rare and ultrarare diseases on which Ultragenyx focuses are on this designated list of conditions.

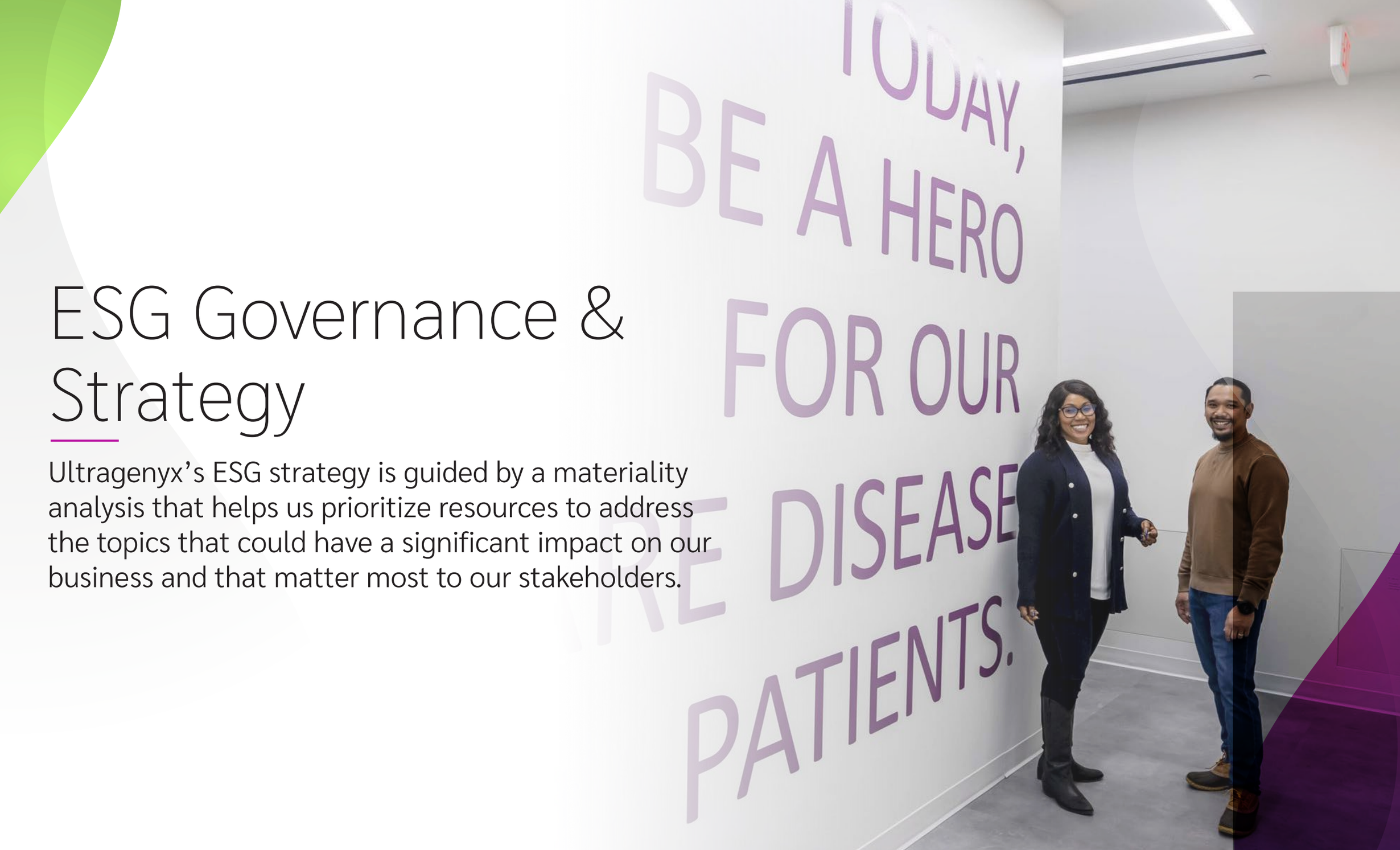
Revenue $ in millions



Our 2022 revenue increased from the prior year due to increased product sales, including an increase in our share of Crysvita collaboration revenue, partially offset by reduced revenue from our collaboration agreement with Daiichi Sankyo due to the substantial completion of the technology transfer in 2022.

ESG Governance & Strategy

Ultragenyx's ESG strategy is guided by a materiality analysis that helps us prioritize resources to address the topics that could have a significant impact on our business and that matter most to our stakeholders.



ESG Governance

Ultragenyx places a strong emphasis on ethics, integrity and corporate responsibility.

This commitment is reflected in our partnerships with the rare disease community, our efforts to improve access to treatments, our focus on creating a people-first culture and our investment in innovation. This commitment is also at the core of our ESG strategy, which was formalized last year and guides our decision-making and policy development.

Our ESG program helps us to identify, manage and communicate the risks and opportunities associated with ESG issues. This standalone ESG report covers a wide range of business practices within our organization, including the development and manufacturing of our medicines with a focus on safety and accessibility, our efforts to create a diverse and inclusive culture for our employees, our support for our communities and our efforts to protect the environment.

The Nominating and Corporate Governance Committee of our board of directors regularly reviews and makes recommendations on our ESG strategy, policies and initiatives. The ESG Working Group regularly reports to the executive leadership team on our progress in the area of ESG.

Nominating and Corporate Governance Committee of the Board of Directors	Oversees Ultragenyx's ESG strategy, policies and initiatives at the board level
Executive Leadership Team	Oversees the development of the ESG strategy and the progress of Ultragenyx's ESG Working Group
ESG Working Group	Responsible for execution of the ESG strategy and works with topic experts and business leaders to drive implementation and internal stewardship

S&P Global ESG Score:

Each year, S&P Global assesses companies across multiple dimensions to determine an ESG score. **In 2022, Ultragenyx's score was in the top 5% of companies in the biotechnology sector.** We are proud of this achievement for the company. We recognize that our ESG successes come from a combination of seeking to align our business decisions with our company culture and values, prioritizing initiatives where we can make a difference for the communities we serve, and empowering our passionate workforce to design and support our broader ESG efforts.

Materiality Analysis

In 2021, we conducted a materiality analysis to determine our ESG priorities.

This process took into account both internal and external perspectives and focused on identifying the ESG topics that could have the greatest impact on our business and that matter most to our stakeholders. By prioritizing these issues, we believe we are able to focus our efforts, communicate more effectively with both internal and external audiences, and allocate our resources towards ESG initiatives that support the creation of long-term value and positive impact for our shareholders and society.

To further strengthen our approach to ESG, we have developed a strategic framework to formalize and structure our initiatives. This integrated and intentional approach is designed to support our efforts to consistently consider the environmental, social and governance implications of our actions and decision-making.

Evaluate relevant ESG topics

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

→

Assess the impact on Ultragenyx

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

→

Topic prioritization taking into account importance to stakeholders

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

MATERIAL ESG TOPICS

Based on our materiality analysis, we developed our ESG framework with **six pillars**: Innovation, Patients, People, Communities, Planet and Governance. These pillars inform the structure of this report and are guiding the development of our ESG strategy, starting with the identification of broad aspirations and objectives, highlighted at the beginning of each chapter. Material topics are listed below each pillar; bolded topics have been identified as higher priority:

Innovation

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

Patients

- **Access & Affordability**
- Patient Advocacy

People

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

Communities

- Community Relations

Planet

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

Governance

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

Evolving Our Strategy & Reporting

In 2015, the United Nations (UN) introduced 17 Sustainable Development Goals (SDGs) as a "shared blueprint for peace and prosperity for people and the planet now and into the future." We highlight the UN SDGs that we believe align most closely with our company vision and mission.

We streamlined our report's structure and strategy in 2022. The "Patients: Innovation & Impact" chapter was split into "Innovation" and "Patients." We relocated the grants section to the "Communities" chapter. Committed to transparent ESG reporting, we incorporated both the GRI and SASB indices. Looking ahead to 2025, our goal is to develop targets that address pillars of our ESG framework, as appropriate, while simultaneously enhancing our data collection and control processes.

UN SDG	Contribution
	• Ultragenyx is focused on advancing health equity by developing therapies for rare and ultrarare diseases that improve the health and quality of life of individuals living with rare disease, and by broadly supporting rare disease communities. • We advocate for broad access to screening and treatment and strive to make our treatments available to as many patients as we can. • We are also committed to advancing employee health, safety and wellness. We continue to provide our employees with wellness offerings and comprehensive benefits to support their financial, familial, physical and mental health.
	• Ultragenyx promotes rare disease education and awareness among healthcare professionals and the rare disease community, and offers employee education and training to support career advancement for our team members. • We are also committed to expanding access to STEAM education opportunities, particularly in economically disadvantaged communities.
	• Ultragenyx promotes gender equality in the workplace by fostering diversity and inclusion at all levels of the organization and is committed to equitable compensation practices.

Looking Ahead...Engaging Employees in Sustainability

At Ultragenyx, we recognize the importance of our employees' passion in fulfilling our own ESG commitments.

In 2023, we plan to offer new opportunities for our employees to engage with sustainability both inside and outside of work. We are also evaluating our existing training programs to pinpoint opportunities for incorporating ESG-related content tailored to the unique needs of individual teams and our organization as a whole. We value our employees' input and will continue to listen to their suggestions and share updates on our sustainability efforts via company events and will explore opportunities to increase environment-related volunteering.

Ultragenyx celebrated Earth Day in our California and Massachusetts offices. During the events, we handed out sunflower seed packets and postcards with a web link and QR code to our 2021 ESG Report. Our Novato, California, office also donated $500 to Rotary District 5150's day of service in honor of Earth Day, making Ultragenyx a "Protecting the River" sponsor.

SPOTLIGHT

In recognition of Earth Month in April 2022, the Ultragenyx team in Massachusetts distributed reusable water bottles to employees and reduced the availability of single-use water bottles in common areas. At several Ultragenyx locations, we have implemented water filtration stations as a means to reduce reliance on single-use plastic water bottles. These efforts are intended to minimize the waste footprint of our offices and raise awareness among employees around the environmental impacts of single-use plastics.

Innovation

Pioneering new approaches to drug development for rare and ultrarare diseases

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

Aspiration

To optimize and accelerate rare disease drug research and development, whether by us or others.

Our Objectives	2022 Progress
Develop industry-leading clinical pipeline in rare and ultrarare diseases that have limited or no treatment options.	• Released interim data from the Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome and exercised option to acquire GeneTx. • Initiated pivotal Phase 2/3 program in Osteogenesis Imperfecta (OI). • Advanced three gene therapies into pivotal studies: DTX401 for GSD1a, UX701 for Wilson disease and DTX301 for Ornithine transcarbamylase (OTC) deficiency. • Secured a license and collaboration agreement to clinically develop, commercialize and distribute Evkeeza® (evinacumab) for HoFH in countries outside of the U.S. • Acquired global rights to AAV gene therapy UX111, currently in a pivotal Phase 1/2/3 study for the treatment of Sanfilippo syndrome type A (MPS IIIA).
Foster industry-wide and community funded development efforts in rare and ultrarare diseases.	• Over 100 participants representing 80 organizations have attended our Rare Bootcamp since 2017. • Participating in six industry consortia to support collaborative drug development.

R&D

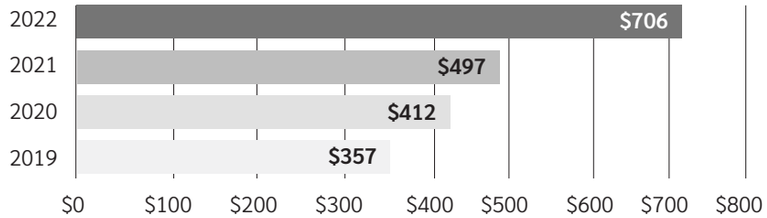
Innovation refers to our processes, initiatives, activities and investments aimed at the efficient and rapid advancement of product candidates that maximize patient health outcomes. Our unique approach to collaboration with the rare disease community at the earliest stages of drug development supports our mission to transform the lives of people with rare disease.

We believe innovation in healthcare is critical for the many individuals living with rare diseases who are waiting for an approved treatment. We make significant investments in research and development (R&D) and have entered into strategic licensing collaborations with other biopharmaceutical companies to innovate and develop new therapies. In 2022, we spent about 70% of our operating expenses on R&D. Please see our 2022 Annual Report for more information.

We seek patent protection in the United States and internationally for our products, product candidates and processes. Our policy is to patent or in-license the technologies, inventions and improvements that we consider important to the development of our business. This policy helps us mitigate risk as well as accelerate the drug development process for the benefit of patients. For more details, please see our 2022 Annual Report. As of December 31, 2022, we own, jointly own or have exclusive rights to more than 225 issued and in-force patents and more than 375 pending patent applications.

Our research aims to advance a new program into clinical development every one to two years. At the end of 2022, we had four rare disease treatments approved by the U.S. Food and Drug Administration (FDA) in five indications and seven programs in clinical trials.

R&D Spend → $ in millions



R&D expenses increased in 2022 due to growth in various programs and areas, including commercial, gene therapy, nucleic acid, biologic and translational research.

Clinical Pipeline

Our current approved therapies and clinical-stage pipeline consist of four modalities: biologics, small molecules, gene therapies and nucleic acid therapies (antisense oligonucleotide [ASO] and messenger RNA [mRNA]).

We have a broad translational research effort that works to turn observations in the laboratory and clinic into interventions that improve the health of individuals with rare and ultrarare diseases. We are advancing clinical and preclinical development programs across multiple rare disease therapeutic areas. In the coming years, we anticipate developing between six and seven clinical therapies with the potential to reach 150,000 patients.

In 2022, we initiated four pivotal studies across our clinical pipeline. We commenced a late-stage clinical development program for UX143 in Osteogenesis Imperfecta (OI), launching the pivotal **Phase 2/3 *Orbit* study** in pediatric patients.

We also advanced three gene therapies into pivotal studies:

The Phase 3 *GlucoGene* study is underway to evaluate the ability of DTX401 to reduce the use of cornstarch while maintaining or improving glucose control as well as the therapy's impact on patients' quality of life. DTX401 has been granted Orphan Drug Designation in the U.S., European Union and U.K., and Regenerative Medicine Advanced Therapy (RMAT) designation and Fast Track designation in the U.S.

The Phase 3 *Enh$_3$ance* study is underway to evaluate the effect of DTX301 on ammonia and its ability to reduce patients' need for ammonia scavenger medication and a protein-restricted diet, the current standard of care. DTX301 has been granted Orphan Drug Designation in the U.S., European Union and U.K. and Fast Track Designation in the U.S.

The seamless *Cyprus^{2+}* Phase 1/2/3 study is underway to evaluate the effect of UX701 on liver copper accumulation, increased ceruloplasmin levels and improved liver pathology in Wilson disease. UX701 leverages Ultragenyx's Pinnacle PCL™ Platform and has been granted Orphan Drug Designation in the U.S. and European Union and Fast Track Designation in the U.S. DTX401 has also been accepted into the European Medicines Agency's (EMA's) Priority Medicines program (PRIME), enabling more frequent interactions with the EMA and the potential for an accelerated approval.

Additionally, we released interim data from the Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome (AS) and exercised the option to acquire GeneTx. The Phase 1/2 study is evaluating the tolerability and safety of GTX-102 and its effect on all major domains of AS in pediatric patients and is currently ongoing in the U.S., U.K. and Canada.

Please see Our Pipeline for further information.

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

Ultragenyx Drug Development Pipeline

Candidate	Description	Pre-Clincal	IND	Phase 1	Phase 2	Phase 3
UX143 (setrusumab)	Anti-Sclerostin Monocional Antibody	Osteogenesis Imperfecta (OI)				
UX111	AAV9 Gene Therapy	Mucopolysaccharidosis Type IIIA (MPS IIIA)				
DTX401	AAV8-G6Pase-a Gene Therapy	Glycogen Storage Disease Type 1a (GSD1a)				
DTX301	AAV8-OTC Gene Therapy	Ornithine Transcarbamlase (OTC) Deficiency				
UX701	AAV9-ATP7B Gene Therapy	Wilson Disease				
UX055	AAV9-CDKL5 Gene Therapy	CDKL5 Deficiency Disorder (CDD)				
UX810	Microdystrophin AAV Gene Therapy		Duchenne Muscular Dystrophy (DMD)			
GTX-102	Antisense Oligonucleotide	Angelman Syndrome				
UX053	mRNA/LNP Oligonucleotide	Glycogen Storage Disease Type III (GSDIII)				

Key: Protein Biologic | Gene Therapy | ASO/mRNA

Rare Disease Facts

There are **10,000+** distinct types of rare and genetic diseases.

Only **5%** of rare diseases have an **FDA-approved drug treatment.**

80% of rare diseases are **genetic.**

Rare diseases affect over **400 million people worldwide**; approximately **50% of people diagnosed with rare diseases are children.**

It is estimated that **1 in 10 people are affected by rare disease.**

30% of children with a rare disease **will not live to see their 5th birthday.**

On average, it takes most rare disease patients **4.8 years to receive an accurate diagnosis**.

Rare diseases impact more people than **cancer and AIDS combined.**

Source: www.alobalgenes.org/rare-facts (last visited April 12, 2023)

Patient-Focused Drug Development

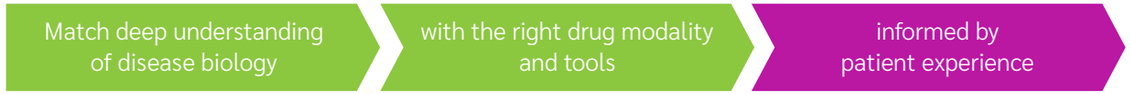
More than 90% of rare diseases do not have available treatments, so we dedicate ourselves every day to the goal of providing treatments as quickly as possible.

Three of our four approved medicines are the only FDA-approved therapy for their respective diseases.

Ultragenyx's Dynamic Development Model (DDM) supports effective decision-making in rare disease drug development through a patient-focused approach that involves collecting direct information and insights from patients. We engage in various activities to help align our programs with the unmet medical needs and expectations of the rare disease community. These activities include informal and structured patient interviews, clinical survey studies, disease monitoring programs, natural history studies and patient engagement plans (PEPs). Engaging early with patients and their caregivers provide 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 disease. The DDM strategy also encourages inventing new approaches and having backup plans to help address unexpected challenges.

For more information on how we partner with the rare disease community and leverage insights, see Patient Advocacy & Engagement.

Our Specialized Approach to Drug Development



Clinical Trials

Our clinical trials are conducted worldwide and are designed to evaluate the safety and efficacy of our product candidates.

We are committed to conducting trials in an ethical manner and to following internal procedures and policies, the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use/ Good Clinical Practice (ICH/GCP) principles, and national and local regulations, as applicable, for trial design and conduct.

We also seek to protect patient safety and well-being through appropriate informed consent procedures and good clinical practices. Our policy is to regularly monitor for safety throughout the course of clinical trials and to discuss with participating investigators. We document and report relevant safety information and adverse events (AEs), such as any new or worsening conditions, to the relevant regulatory authorities.

In 2022:

Three new protocols registered by Ultragenyx on www.clinicaltrials.gov.

First patient dosed in pivotal Phase2/3 *Orbit* study of UX143 (setrusumab) for the treatment of Osteogenesis Imperfecta (OI).

First patient dosed in pivotal Phase 3 *GlucoGene* study of DTX401 for glycogen storage disease type 1a and completed enrollment of the last patient into the baseline screening period.

Initiated and dosed patients in pivotal, seamless Phase 1/2/3 *Cyprus*$^{2+}$ study of UX701 for Wilson disease.

23 patients from the U.S., U.K. and Canada were evaluated in the Phase 1/2 study of GTX102 for Angelman syndrome across various clinical measurements, with 10 patients having six to 12 months and five with over 12 months exposure.

Released long-term Phase 1/2 data on DTX301 (Ornithine transcarbamylase [OTC] deficiency) gene therapy that demonstrated durable metabolic control and sustained responses lasting over four years following treatment.

Over 200 clinical trial sites in operation across **24** countries in 2022

GlucoGene™
Gene Therapy for the Treatment of GSDIa

Cyprus^{2+}™
Gene Therapy for the Treatment of Wilson Disease

Enh$_3$ance™
Gene Therapy for the Treatment of OTC Deficiency

Recruitment & Site Selection

In our commitment to ethical and responsible clinical research, we endeavor to make our clinical trials inclusive and diverse – available in multiple countries to patients regardless of gender, ethnicity or socioeconomic status.

We understand that many of the diseases we are studying are rare and have no approved treatments and as such, we strive to make these opportunities accessible to a broad range of patients. To help minimize the financial burden on patients, we cover all expenses necessary for clinical trial participation, such as travel expenses.

We also prioritize reducing the burden of participation through home visits and phone calls when possible. 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. Furthermore, we 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.

Over
50
clinical investigator sponsored trials (ISTs)
approved and **465+** clinical participants enrolled globally since 2012

Matthew, who has MPS VII, celebrated his 21st birthday with his mother, Helen.

Data Transparency

Data transparency is essential to fostering trust with patients, healthcare professionals, regulatory agencies and medical researchers.

We recognize this importance and support the overall principles of greater clinical trial data transparency as part of our patient-focused drug development model. We endeavor to make clinical trial information and results public in a timely manner while protecting essential proprietary information and patient privacy.

We follow the standards and principles for clinical trial data transparency set forth by international industry organizations, such as as the International Federation of Pharmaceutical Manufacturers & Associations (IFPMA), the European Federation of Pharmaceutical Industries and Associations (EFPIA), the Japan Pharmaceutical Manufacturers Association (JPMA) and the 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.

Ultragenyx registers protocol information for company-sponsored clinical trials of investigational and marketed products in accordance with applicable laws and regulations. In the U.S., protocol information is registered at www.clinicaltrials.gov.

Ultragenyx discloses the results of company-sponsored clinical trials in accordance with applicable laws and regulations. We also publish results – regardless of outcome – in peer-reviewed journals or at scientific meetings. We publish the results of both successful and failed trials to advance scientific learning.

Our medical writing follows industry standards, such as the Good Publication Practice guidelines (GPP3) published by the International Society for Medical Publication Professionals, and Recommendations for the Conduct, Reporting, Editing and Publication of Scholarly work in Medical Journals published by the International Committee of Medical Journal Editors (ICMJE).

We also prepare plain language summaries of results to share with patients and the public. Additionally, we make reasonable efforts to address clinical data requests for legitimate medical or scientific research purposes from qualified researchers in the interest of improving patient care and helping advance medical science.

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

In 2022:

- **16** peer-reviewed manuscripts published
- **106** abstracts submitted
- **147** oral and poster presentations delivered at medical and scientific congresses

Data Protection, Anonymization & 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 and to not contain names or other personally identifiable information (PII). 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 Protection & Privacy in this report for more information.

Disease Monitoring Programs & Post-Marketing Commitments

We utilize disease monitoring programs (DMPs) to evaluate the 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 medications. 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 disease monitoring program. A DMP 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 drug – or not.

DMPs go beyond clinical trials by enhancing the understanding of the disease and its treatments for the benefit of patients, physicians, payers and the company. DMPs provide progress reports, broader patient population data analysis, high-quality disease information and supportive long-term outcome data, and promote therapy science advancement and research.

As part of a DMP, the pharmaceutical sponsor may partner with an academic institution and a patient advocacy group, if applicable, on the ownership and management of data. DMPs can also support the generation and transparent sharing of high-quality, Good Clinical Practice (GCP)-compliant data with patients, physicians, sponsors and the rare disease community. We provide participants in our DMPs with the opportunity to receive their own data in easy-to-understand language.

To date, we have initiated eight DMPs, including several for Crysvita® for the treatment of X-linked hypophosphatemia (XLH). Our in-clinic DMP for XLH is our largest study, with over 780 patients in its fifth year. Our most recent DMP, for Dojolvi, was initiated in 2021.

DMP Highlights:

- **First** DMP launched in 2012
- **1,350+** patients enrolled* at nearly **80** sites in **28** countries

*Enrollment as of December 31, 2022

Safety

Our company's reputation is dependent on the trust that patients, healthcare professionals and the general public place in us.

We are committed to complying with applicable laws, regulations and international standards dealing with Good Clinical Practices, Good Distribution Practices, Good Laboratory Practices, Good Manufacturing Practices and Good Pharmacovigilance Practices (collectively "GxP"), and our employees undergo annual GxP training.

In 2022, we launched an internal patient safety podcast platform to increase employee awareness on all aspects of safety and pharmacovigilance.

In 2022:

- **Zero** units recalled
- **Zero** product recalls

COUNTERFEIT DRUGS

At Ultragenyx, we take the safety and effectiveness of our products 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 our 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.

Employees at our gene therapy manufacturing facility in Bedford, Massachusetts

Quality

Ultragenyx is committed to delivering quality medicines to our patients.

We seek to maintain an engaged quality culture that prioritizes the safety, efficacy and reliability of our medicines.

These principles are reflected in the research, development and manufacturing of our products. Our leaders are committed to a responsible quality mindset and a quality management system to support sound science and the right behaviors for the development, manufacture and distribution of our medicines to our patients.

As it pertains to patient safety, product quality and data integrity, we are committed to:

- Complying with applicable laws, regulations, international standards and the collective "GxP" good practices as governed by health authorities.
- Cultivating a peer-driven commitment to quality, compliance and innovation as guided by our mission and core values.

We require our suppliers and business partners to adhere to similar standards when performing work for or on behalf of the company, and we require that they comply with the Drug Supply Chain Security Act (DSCSA).

Our quality system continues to evolve to support business and quality requirements, growth and scale. We developed the Quality System Maturity Model to better assess the current maturity level of our core processes and systems and where we believe we need to be (aspired maturity level). The levels span from initial (level 1) to excellence (level 5). In 2022, we established a master plan describing the end-to-end process to conduct maturity assessments and completed assessments on critical global quality systems. In 2023, we expect to continue our assessments and implement actions that are relevant to continual improvements, increased performance and aspired maturity.

Ultragenyx directors and senior leaders touring our gene therapy manufacturing facility in Bedford, Massachusetts, during construction in 2022

Strategic Collaborations

At Ultragenyx, we are working to develop a robust and diverse pipeline of candidates to support our mission to transform the lives of people with rare disease.

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

In 2022, Ultragenyx closed several strategic acquisitions and licensing agreements. We exercised an option to acquire GeneTx, our collaborator since 2019 on GTX-102 for Angelman syndrome (for more on our partnership with GeneTx, see the story on A Parent's Journey). We also added two new programs to our portfolio. We secured a license and collaboration agreement with Regeneron to clinically develop, commercialize and distribute Evkeeza® (evinacumab) in countries outside of the United States, and we acquired global rights to adeno-associated virus (AAV) gene therapy UX111 from Abeona Therapeutics, currently in a pivotal Phase 1/2/3 study for the treatment of Sanfilippo syndrome type A (MPS IIIA).

Ultragenyx has a successful history of partnering with other biotech companies to develop and commercialize rare disease therapeutics. Since 2013, Ultragenyx and Kyowa Kirin have been partners in the successful development and worldwide commercialization of Crysvita (burosumab-twza) across two indications.

For more information on our partnerships and collaborations, please see our 2022 Annual Report.

Mason is living with Angelman syndrome.

A PARENT'S JOURNEY TO DRUG DEVELOPER & PARTNERSHIP WITH ULTRAGENYX

Ainsley Evans was born in 2004. Nine months later, she was diagnosed with Angelman syndrome, a genetic disorder that causes developmental disabilities and nerve-related symptoms. Her prognosis consisted mostly of things she would not do – she wouldn't walk, speak, go to school or attend college; she would never have a job, get married or have a family of her own. At the time, there were no treatments and there was no hope for a cure.

> Angelman syndrome is a devastating neurodevelopmental disorder that affects about **500,000** people worldwide and has no approved treatment.
>
> Source: https://cureangelman.org/

In 2008, Ainsley's mother Paula started the Foundation for Angelman Syndrome Therapeutics (FAST) with modest donations, a group of like-minded parents and a fierce determination to rewrite the future for these children. The foundation initially focused on raising the funding necessary to either replace the maternal gene causative of Angelman syndrome, or activate the normally silent, paternal copy.

This fundraising allowed FAST to create a virtual center of excellence comprising a cross-functional group of specialists called the "FIRE team" (Fast Integrated Research Environment). Dr. Scott Dindot at Texas A&M University was a member of the FIRE team, and FAST had been funding his research program for more than five years when Scott figured out how to potentially restore the missing protein in the brains of children with Angelman syndrome using an antisense oligonucleotide (ASO) therapeutic.

With this promising development, FAST launched GeneTx Biotherapeutics in late 2017 to further develop the ASO program and ready it for the clinic. In 2019, the company participated in Ultragenyx's RARE Bootcamp, where they became part of a community of other parent-led organizations working to develop first-ever treatments for their own children. That same year, GeneTx began looking for a partner to support its clinical program and tapped Ultragenyx to partner on the development of GTX-102.

In July 2022, Ultragenyx and GeneTx provided a program update on GTX-102 for the treatment of Angelman syndrome, including encouraging interim data from the Phase 1/2 study in pediatric patients. Ultragenyx acquired GeneTx at that time and continues to develop the potentially transformative treatment for individuals with Angelman syndrome.

Paula Evans with her daughter, Ainsley, and husband, Michael

Industry Participation

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

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

- Founding sponsor of **BeginNGS™** (newborn genomic sequencing), a public-private coalition led by Rady Children's Institute that is piloting a program to use genetic testing technology to screen newborns for rare genetic diseases – with an ultimate goal of testing for up to 1,000 disorders and sequencing 3.7 million newborns in the United States annually. See Engaging Policymakers for more information on our support of newborn screening.

- Member of the Accelerating Medicines Partnership® (AMP®) **Bespoke Gene Therapy Consortium**, a public-private partnership between the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), and multiple public and private organizations to create a standardized approach to help reduce upfront costs and lower barriers to developing new gene therapies for rare and ultrarare diseases.

- Member of the **LouLou Foundation CDKL5 Deficiency Disorder Consortium**, which is directing the ClinicalAssessment of NeuroDevelopmental measures In CDD (CANDID), a three-year observational study for the development of disease modifying therapeutics for CDD. In October 2022, the first patient was enrolled in the study. CANDID study results will be shared with the entire community to aid CDD clinical trial design and inform therapeutic development for related neurodevelopmental disorders.

- Supporter of the **"Living with Osteogenesis Imperfecta: Understanding Experiences Based on Community Insight and Experience" (IMPACT) Survey**, the largest collection of data about 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 upport from Mereo BioPharma, Ultragenyx's partner in the development of UX143 (setrusumab) for the potential treatment of OI. Initial self-reported data and insights from the survey were presented at the American Society for Bone and Mineral Research (ASBMR) 2022 Annual Meeting.

- Partner of the **n-Lorem Foundation** to bolster its mission to discover, develop and provide experimental antisense oligonucleotide (ASO) medicines for nano-rare patients (1 to 30 patients worldwide) for free, for life. As of December 2022, more than 80 patients have been accepted into n-Lorem's drug development program and three patients have received treatment.

Rare Bootcamp

We host a recurring Rare Bootcamp, supported by a number of contributing sponsors, where we share our knowledge, expertise, insights and connections to help patient families, foundations and other organizations seeking to develop novel treatments for rare diseases. Our bootcamp is designed for incredibly determined patient families and advocates who have started funding their own rare disease research and are looking to better coordinate and build a structure around their efforts. In collaboration with our sponsors, we work towards making a meaningful impact in the rare disease community.

Our bootcamp curriculum evolves after each event. What began as a half day meeting in 2017 is now a three-day event. 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-ones so that experts can share their advice. We sometimes invite participants to return to share their progress and learnings.

In 2022, we added lectures on the pros and cons of gene therapy and a panel discussion on intellectual property and contracting.

Bootcamp Attendance:

- In 2022, we hosted two bootcamps with over **50** participants from over **40** organizations attending.
- Since 2017, Ultragenyx has held six bootcamps with over **100** participants from **80** organizations in attendance.

"We are in a position to share what we've learned from bringing multiple drugs to market with rare disease start-ups early in their own R&D cycle. Making the process easier for these organizations aligns with our goal of treating as many rare disease patients as possible. Our aim is to empower these organizations with guidance and tools and help facilitate their development of life-changing rare disease treatments."

Arjun Natesan, senior vice president of Translational Research

CURE SPG50: A STORY OF HOPE & DETERMINATION IN THE FIGHT AGAINST AN ULTRARARE DISEASE

SPG50, or Spastic Paraplegia Type 50, is known to affect only around 80 people worldwide. It is an ultrarare disease caused by a mutated gene and typically starts with low muscle tone, microcephaly (small head) and epilepsy, then goes on to affect the whole body. Most patients experience lower body paralysis by age 10 with severe mental disability, and many never walk or speak.

After Terry and Georgia Pirovolakis' son, Michael, was diagnosed with this devastating disorder, they founded Cure SPG50, a nonprofit organization working to fund gene therapy to cure or halt disease progression. Terry attended our Rare Bootcamp for the first time in 2019 and has come to every Bootcamp we've hosted since then, while raising $4.5 million to develop and produce the first AAV gene therapy to treat SPG50. The therapy is known as Melpida, using the first initial of Michael's name and the word "elpida," which is Greek for "hope." Michael finally received a dose of Melpida in March 2022. Ultragenyx is proud to have provided support and mentorship to Terry and his team throughout their journey.

From left to right: Dr. Yael Weiss, founder of Mahzi Therapeutics, Dr. Allyson Berent, chief science officer of FAST, Dr. Emil Kakkis, CEO of Ultragenyx, and Terry Pirovolakis, founder of Cure SPG50.

Ultragenyx sponsored an award at the Precision Medicine World Conference (PMWC) in January 2023 for Terry and Allyson Berent, chief science officer of FAST – the PMWC 2023 Luminary Award – which recognizes the impact of these parent advocates on R&D innovation in rare disease.

SPG50,
or Spastic Paraplegia Type 50, is known to affect only around **80** people worldwide.

Patients

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

We are **committed** to supporting the rare disease community through our efforts to develop novel therapies, share our science and expertise, achieve broad access to screening and treatment, and partner with policymakers for meaningful change.

Aspiration

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

Our Objectives	2022 Progress
Achieve majority access through responsible pricing and support services.	• In 2022, our U.S. price increases remained aligned with the Consumer Price Index for all of our commercially available products. • Over 500 patients in over 40 countries have been approved for access to Ultragenyx treatments through various global expanded access and patient assistance programs since 2013. • Over 3,200 patients have received Ultragenyx treatments through commercial access and expanded use.
Use our expertise to amplify the voices of the rare disease community to inform and influence key decision-makers in the field of rare disease.	• In recognition of Rare Disease Day 2022, Ultragenyx developed a campaign to support education and access to newborn screening and provided matched donations to two nonprofit organizations doing critical work in this area: Baby's First Test and Rare Disease Innovation Institute.
Incorporate the perspectives and experiences of patients and caregivers in our decision-making, with a focus on addressing unmet needs for basic necessities and improving quality of life.	• Convened three meetings of the Gene Therapy Advisory Committee and identified key opportunities for 2023. • Hosted four townhalls with over 575 participants.

Photo on previous page: Kesha and her son, Elias, live with X-linked hypophosphatemia (XLH), a rare genetic condition. Despite dealing with severe joint pain, bow-leggedness and undergoing 14 surgeries by the age of 16, Kesha remains strong and positive. Her parents always inspired her to think beyond her limitations and stay as active as she can. Now that Kesha is a mother, she believes it's important to provide that same support to her son so he can thrive and grow into the person he wants to be.

Access & Affordability

The greatest impact we can have on the lives of individuals with rare disease is to make our treatments accessible and affordable, and to engage and support rare disease families along every step of their journey.

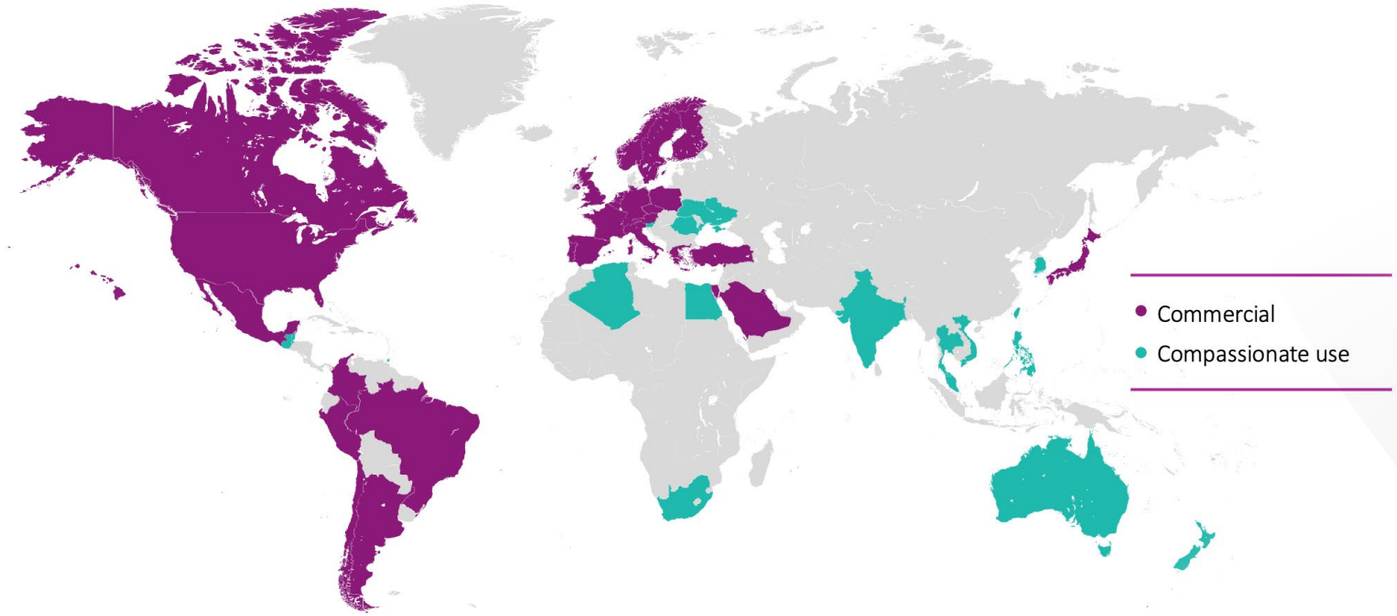
More than 3,200 patients have benefited from Ultragenyx treatments via commercial access and expanded use, demonstrating our dedication to making therapies accessible.

We strive for majority access to our therapies. We believe that pricing should not be a barrier to accessing treatment and are committed to pricing our medicines responsibly. We set our drug prices with global pricing in mind and, in the U.S., we use our best efforts to help make it possible that no patient foregoes treatment for financial reasons.

In 2022, our U.S. price increases remained aligned with the Consumer Price Index for all of our commercially available products.

As we look ahead to our next-generation gene therapies, we have invested in our proprietary Pinnacle PCL™ gene therapy technology platform and a gene therapy manufacturing facility in Bedford, Massachusetts. Gene therapy manufacturing issues have been a known barrier to treatment access, and our goal with these investments is to support control over scalability and cost of production.

Reaching Patients Around the World With Ultragenyx Therapies



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 www.ultracaresupport.com for more information.

Compassionate Use

Our organization remains dedicated to supporting patients and their families in their quest to explore available treatment options following a rare disease diagnosis. Our commitment to developing new medicines for both children and adults with rare and ultrarare diseases is matched by our unwavering efforts to make our commercial therapies accessible to patients via approved 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, we offer our investigational therapies on a compassionate use basis to qualified patients worldwide via our early access program. Our evaluation of requests for individual patients to receive investigational therapies outside of a clinical study is conducted on a case-by-case basis.

Given the urgency associated with rare disease patients, we aim to respond to compassionate use requests within 24 hours and to ship therapies within 48 hours wherever feasible. We are committed to providing timely support to patients and their families.

Making our Treatments Accessible:

- Over **500** patients in over **40** countries have been approved for access to Ultragenyx treatments through various global expanded access and patient assistance programs since 2013.
- Over **120** patients were treated through compassionate use in 2022.

SPOTLIGHT

Dojolvi Since Launch in 2020:

- Approved in **4** countries.
- Over **670** patients are receiving Dojolvi through commercial access and expanded use.
- Approximately **95,000** bottles have been filled and shipped worldwide.

Patient Advocacy & Engagement

Ultragenyx was built from the ground up with rare disease community collaboration in mind. We strive to partner with patients and their families from the earliest stage of drug development through clinical research, and to support patients and their families throughout the treatment life cycle.

We also have the opportunity to listen to first-hand experiences from invited speakers living with rare disease, which allows us to see the real-world impact that our work can provide for patients and families.

The role of patient advocacy at Ultragenyx is to:

1) Partner with patient organizations to gather diverse perspectives, support patients and explore potential collaborations for new therapies that address unmet needs and disease impact.

2) Educate by sharing information about rare and ultrarare diseases and their impact.

3) Engage key policymakers to amplify the voices of the rare disease community.

4) Support patients, their families and the broader 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.

Patient Engagement Plans

Patient engagement plans (PEPs) are collaboratively developed by multiple teams to explore and address all aspects of a disease with patients, caregivers, the affected community and specific care landscapes, including the healthcare environment and services. Since physicians and disease experts may not have comprehensive insight into how patients perceive the disease's impact on their quality of life, the PEP process involves regularly reassessing existing sources of patient experience data. Patient involvement is critical to making informed decisions in drug development. As an example, the UX143 (setrusumab) PEP aims to incorporate the experiences and insights of OI patients and caregivers, with participation from Patient Advocacy and various departments at Ultragenyx. Regular meetings with patients and caregivers provide valuable insights into their experiences, such as the tendency to avoid emergency rooms for every suspected bone fracture.

Partnering with Patient Organizations

The Ultragenyx Patient Advocacy and Patient Engagement team is dedicated to advancing global rare disease advocacy through inclusive patient engagement and partnership. To further this purpose, we hosted four townhalls in 2022, reaching over 575 participants, with content that included research updates on the UX055 program for CDKL5 Deficiency Disorder, and nutrition and gene therapy updates related to Wilson disease.

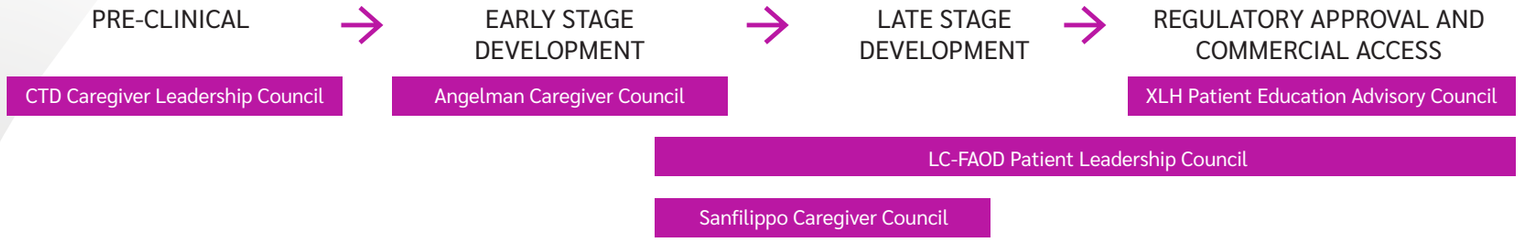
Ultragenyx also launched leadership and advisory councils intended to gather feedback from the patient community, build authentic and enduring relationships with advocates, caregivers and people living with rare disease, and identify opportunities for appropriate collaboration. These councils meet regularly, and the insights gleaned through these efforts help to inform our strategies and decision-making throughout the product lifecycle.

GLOBAL GENE THERAPY ADVISORY COUNCIL

In 2021, Ultragenyx launched a Global Gene Therapy Advisory Council with nine patient advocacy leaders from the U.S., Mexico, Netherlands, U.K. and Sweden, representing various rare disease communities. The goals of the council are to 1) garner insight from council members on priority challenges, knowledge gaps and unmet needs related to gene therapy within rare disease communities, 2) increase community awareness, knowledge and understanding of gene therapy, and 3) identify potential collaboration opportunities to address unmet community needs regarding gene therapy.

The council met three times in 2022. Ultragenyx has identified two key opportunities for 2023: developing educational resources on gene therapy in partnership with relevant organizations, and gaining a deeper understanding of geographic differences and commonalities in unmet needs related to gene therapy across different regions worldwide.

Advisory Councils Across the Therapeutic Life Cycle



Providing Educational Resources

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

- www.mpsviiinfocus.com (available to the MPS VII community in English, Spanish, Portuguese, Italian, Polish, Romanian, Hungarian and Croatian)
- www.OneXLHvoice.pt (available to the XLH community in Mexico and Brazil)
- https://faodinfocus.com (provides education and resources to the LC-FAOD community)
- https://hofhdisease.ca/ (provides education and resources to the HoFH community in Canada)
- www.ultraclinicaltrials.com (a trial recruitment website for caregivers and people living with rare diseases)

Our Rare Journey Resources, available at www.ultrarareadvocacy.com, are designed to provide access to information for many of the common phases of the “rare” journey and empower patients and their caregivers to connect and get involved.

We also prepare plain language summaries of clinical trial and DMP results. See Clinical Trials for more information.

FROM RARE DISEASE PATIENT TO PATIENT ADVOCATE

At the young age of 1.5 years old, Isabel Bueso was diagnosed with Maroteaux-Lamy syndrome, also known as mucopolysaccharidosis type VI (MPS VI). Her body was missing an enzyme required for the breakdown of a complex sugar, causing the sugar to accumulate in her organ tissues, with the threat of severe damage to her heart valves, skeleton, liver, cornea and spleen. The most disheartening part for her parents was to learn that there was no approved drug treatment for MPS VI. The emotional challenge they faced was enormous.

However, for both of her parents, her diagnosis was not the end of the road, but the beginning of their advocacy journey.

Isabel was born in Guatemala and was only three weeks old when she began to suffer from a series of health ailments. For an accurate diagnosis, her parents brought her to Miami, where doctors confirmed her diagnosis of MPS VI. A bone marrow transplant (BMT) was the only treatment available. A hospital in Memphis offered to provide the transplant at no cost. For five years, her parents traveled twice a year from Guatemala to Tennessee with the hope of hearing from doctors about a date for the BMT. Sadly, the hospital never found a good match.

One day, Isabel's grandmother came to their house with the latest issue of Reader's Digest. The magazine featured the article "Saving Ryan," about a little boy named Ryan Dant with MPS type I who had received an enzyme replacement therapy developed by then-UCLA researcher, Dr. Emil Kakkis.

Filled with hope, her parents connected with the National MPS Society and learned about a clinical trial underway in California for another treatment in development by a team led by Dr. Kakkis – specifically for MPS VI.

Isabel's parents became her non-stop advocates and successfully enrolled her in the third clinical trial to evaluate the effectiveness of the treatment. The trial was a success and the treatment was approved by the FDA.

Isabel was inspired to start sharing her own MPS story, which led to new opportunities to build her experience as an advocate and raise awareness, not only for MPS, but also for other rare diseases. She started by organizing Rare Disease Day events for students and staff in her high school and at her university, where she graduated with honors with a degree in sociology.

After graduating, she participated in an internship with the Patient Advocacy team at a biopharmaceutical company, where she found her calling. She knew she wanted to be part of a team focused on improving the lives of others.

Isabel was hired as a patient advocacy specialist at Ultragenyx in March 2021, and in this role, she has been able to combine her personal passion for the rare disease community with the values of relentlessness and advocacy that were instilled in her from a young age. She knows first-hand the obstacles families face in accessing treatments and services, and she is happy to have the chance to be part of the support system that helps them go beyond these challenges.

In December 2022, the U.S. Senate passed legislation that was signed by President Joe Biden granting Isabel and her family permanent citizenship so she may continue to receive treatment for her rare, life-threatening disease.

"Today, it feels like my journey has come full circle – the experiences that led me to this point started in 2003 with the clinical trial for the MPS VI treatment developed by Dr. Kakkis, and now I'm proud to be part of the organization he founded to support the rare disease community."

Engaging Policymakers

Policy and regulation are critical to the cost-effective and timely development and commercialization of treatments for the one in 10 Americans living with rare diseases. Our Global Policy Committee determines priority policy areas for our engagement and advocacy. The goal of the committee is to educate and inspire key decision-makers in the field of rare disease with the aim of enabling all people living with a rare disease to receive an accurate diagnosis, quality care and available therapy as quickly as possible.

We are a founding member of the Rare Disease Company Coalition (RDCC), a coalition of 21 companies that are collectively investing over $15 billion annually in R&D and have over 200 treatments either approved or in development. RDCC educates policymakers on the distinct considerations of life science companies operating in the rare disease space and focuses on three priorities: supporting robust development and innovation, ensuring accessibility and enabling earlier diagnosis. Betsy Ricketts, our vice president of Policy, Government and Public Affairs, was a founding chair in 2021 and elected to serve as secretary to RDCC's Executive Committee in 2022 and 2023.

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

Patient Experience: Prioritizing patients in development and commercialization, including patient experience data in drug applications and labels.

Market Access: Ensuring rare diseases are a priority in global markets.

Pricing & Reimbursement: Proactively engaging with payers to ensure majority access to products while advocating for responsible pricing and flexible payment models for innovative therapies.

Diagnostic Odyssey: Shortening the timeline from diagnosis to treatment access through initiatives like newborn screening and genomic sequencing.

Gene Therapy: Engaging policymakers in innovative approaches to clinical and manufacturing development while characterizing unmet needs and burden of illness in peer-reviewed literature.

Accelerated Development: Streamlining drug development timelines, including biomarker and endpoint development, to speed up assessment and approval by global health authorities.

Emerging Markets: Raising awareness of ultrarare diseases, encouraging local definitions and educating on advanced therapies like gene therapy and mRNA.

40TH ANNIVERSARY OF THE ORPHAN DRUG ACT

January 4, 2023 marked the 40th anniversary of the Orphan Drug Act (ODA). This legislation has encouraged the development of drugs for rare diseases and resulted in more than 1,100 FDA approvals of new treatments, greatly improving the lives of patients with rare diseases. Notwithstanding the success of the ODA, there are still tremendous regulatory and commercial barriers to developing treatments for diseases with extremely small patient populations (ultrarare diseases). Many companies have had to cease development of such therapies, resulting in nonprofit organizations, often those run by parents of a child with an ultrarare disease, to develop therapies on their own. Ultragenyx advocates for a new framework that will address barriers to ultrarare disease drug development and create a clinical and regulatory framework that keeps pace with scientific advancements.

Advocating for Newborn Screening

Newborn screening (NBS) is a vital part of treating rare diseases. With early detection, affected infants can receive prompt treatment that can help prevent permanent disability, developmental delay and death.

NBS programs in the U.S. are state-run public health programs that identify newborns with certain genetic, metabolic, hormonal or functional disorders. Because NBS programs are state run, there are major discrepancies in which states screen for which diseases. The total number of conditions included in screening ranges from 33 to 67, and no state screens for all 37 core conditions and all 26 secondary conditions on the Recommended Uniform Screening Panel (RUSP). Ultragenyx advocates for these state-run programs to screen for all conditions on the RUSP so that infants with rare disease can receive prompt access to treatment, no matter where they're born in this country.

In recognition of Rare Disease Day 2022, Ultragenyx elevated awareness and contributed $50,000 while our partner organizations contributed $25,000 in matched funds to two nonprofit organizations doing critical work to engage and equip the rare disease community and to expand access to newborn screening:

baby's first test

Genetic Alliance

Genetic Alliance & Baby's First Test Centered on real experiences navigating newborn screening, the Genetic Alliance created the Baby's First Test program to provide up-to-date information, support and services for families today and every day.

Rare Disease Innovations Institute

Rare Disease Day Is Every Day

Rare Disease Innovation Institute (RDII) Through grass roots education and policy, Rare Disease Innovations Institute (RDII) accelerates diagnosis, enables access to treatments and improves the quality of life for those living with rare disease with newborn screenings being a primary focus.

See also Industry Participation for information on Ultragenyx joining BeginNGS consortium, a Rady Children's initiative to develop genomic analysis tools for hospitals to significantly increase newborn screening and early diagnosis of rare diseases.

Living with Wilson Disease

Amanda was in her early thirties, working in the fashion industry making upscale health wear, when she began experiencing symptoms of Wilson disease. Prior to the onset of disease, Amanda's life was predictable; she loved her job and valued her independence. Wilson disease was as unexpected as it was painful and disruptive.

Wilson disease is a genetic disorder that leads to copper buildup in the liver and brain. As the mineral accumulates, patients can experience extreme pain and body tremors, among other debilitating symptoms. If left untreated, Wilson disease can lead to organ failure and fatal copper poisoning.

Amanda's symptoms were severe and her quality of life deteriorated as she faced chronic pain and tremors. Her doctors initially dismissed the symptoms, attributing them to socio-emotional stressors. When she finally received a diagnosis, the information was both challenging and relieving.

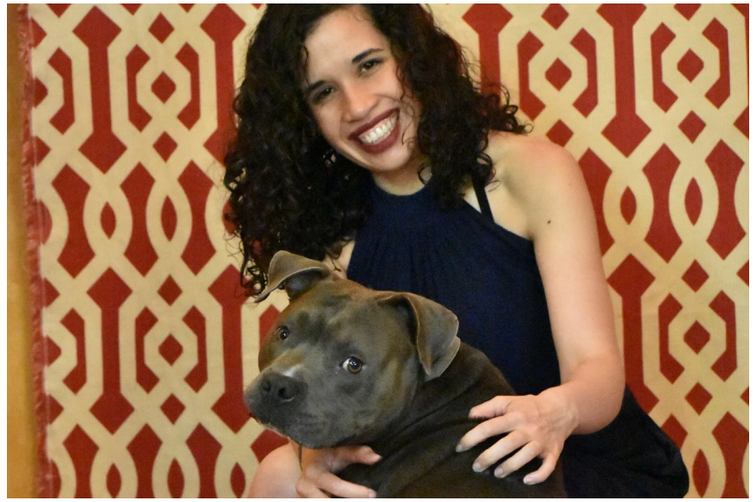
Amanda's management strategy is multifold. Like many, she used chelating agents to remove the heavy metal from her body and takes zinc daily.

She wears a heated wristband to relieve muscle tension in her wrist and arm and uses compression wear and a weighted vest to relax muscles in her shoulders and core.

The weighted vest Amanda purchased did not meet her aesthetic standards, so she applied her fashionable health wear expertise and developed one with a new "fly" design.

Amanda now has some days with minimum pain and few tremors. While her life is not as carefree as it once was, she can enjoy the activities that always brought her joy, like making clothes and spending time with family. Her dog Ellis is a constant source of fun and reminds her that she can rely on others to provide support. Amanda is hopeful about the future. She is optimistic about new research that allows for earlier diagnosis of Wilson disease and the potential for gene therapy as a durable treatment. Now that she has a management plan to go forward, she also feels she wouldn't trade her experience for anything else.

After her diagnosis with Wilson disease and enrolling in a gene therapy investigational trial, Amanda made a pact with herself not to let the work of managing her disability quench her love for fashion.



"I think you should let whatever you were good at before shine during this time, too. See how it could make your new life maybe that much better."

In 2022, the first patient was dosed in the *Cyprus*$^{2+}$ study, a one-time intravenous (IV) infusion of UX701, an investigational gene therapy for the treatment of Wilson disease. Ultragenyx is sponsoring this global study to determine if UX701 gene therapy is a safe and effective treatment for adults with Wilson disease. This adeno-associated virus (AAV) gene therapy has the potential to restore ATP7B function, which may improve copper metabolism and distribution.

Watch the interview with Amanda by Rhonda Rowland, a former CNN reporter also living with Wilson disease.

People

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

We are **committed** to maintaining a diverse, inclusive, safe and healthy environment. We are also committed to fair and equitable compensation practices that are transparent and free from bias.

Aspiration

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

Our Objectives	2022 Progress
Maintain a positive workforce culture by achieving a total turnover rate below the industry average and continuing to have high employee engagement.	• Total turnover was 13.3%, which is below the U.S. and global averages for our industry (according to Aon Radford's Salary Increase and Turnover Studies). • Maintained a high engagement score of 86% in our employee engagement survey. • Shifted from an annual survey to an 18-month survey, complemented by two or three focused "Pulse" surveys that emphasize the most pertinent and significant subjects for our organization.
Support internal career and leadership development through our significant investment in customized employee programs that build core competencies and bring our company values and culture to life.	• On average, each employee attended approximately six hours of development workshops, with 95% attending at least one of the 70 development workshops we offered in 2022. • Expanded career coaching program to all employees. • Hosted over 45 interns during our summer program.
Continue to strengthen and expand diversity and inclusion through intentional talent acquisition and management efforts including candidate pipelining, interview processes, ongoing education, awards, promotions and succession planning.	• 57% of global employees are women. • Launched Declaring and Repairing Breakdowns, a mandatory employee education program. • Launched a new I&D Employee Resource Group (ERG), UltraAPAC. • Launched a partnership with Disability Solutions on outcomes for talent with disabilities, and Ultragenyx's jobs became directly available on the Disability Solutions' Career Center.
Implement a robust and comprehensive health and safety management system framework and audit process.	• Implemented a new process to track key performance indicators for injury rates, safety observations, ergonomic assessments and others. • Delivered 19 Environmental, Health & Safety (EHS) training courses. • Reviewed over 940 on-site chemicals to characterize industrial hygiene risks. • Completed 130 ergonomic hazard assessments.

Culture & Values

At Ultragenyx, we intentionally built and have consistently nourished our company culture so our employees can feel a sense of purpose and fulfillment in their work and feel connected each day to the bigger impact we have on the rare disease community.

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

“Our vision is for Ultragenyx to be a place not only where people can do the best and most meaningful work in their careers, but also where they will experience profound personal and professional growth. For me, as a mother and stepmother, this means being a place where I would be proud for my kids to work, and where my colleagues of all backgrounds feel the same about the young people in their lives. To me, leading the future of rare disease medicine also means leading the future of workplace culture.”

Bria Martin
vice president of Culture and Organizational Strategy

Our Cultural Values were carefully crafted to empower our team members, allow for their voices to be heard and encourage them to strive to make a difference.

Generous

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

Courageous

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

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 our patients - together always searching for solutions.

Dynamic

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

Human Capital Development

Ultragenyx has over 1,300 employees globally. We are dedicated to building a global and diverse team; maintaining a healthy, inclusive company culture where employees feel respected and valued; and providing opportunities for learning, personal growth and career advancement.

We strive to provide employees with a workplace and work environment where they do their best work and where they want to stay long term. Our voluntary turnover was 11.7% in 2022, which is below the U.S. and global averages for our industry (according to Aon Radford's 2022 Salary Increase and Turnover Studies).

At Ultragenyx, we push each other to perform at our very best to support our mission to transform the lives of people with rare disease. To enhance camaraderie and continuous recognition, we have an internal platform called "U Earned It," where employees at all levels can recognize colleagues for their contributions and award them with points that they can redeem for rewards.

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 diversity strategies.

We conduct **formal employee reviews** twice per year and set expectations with our managers to have regular conversations with their employees to further support their development. Additionally, managers meet regularly with each employee to provide broader visibility of their strengths, career aspirations and performance contributions, and to identify opportunities to accelerate career development.

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

Turnover in 2022

Total Turnover Rate	**13.3%**
Voluntary Turnover Rate	**11.7%**
VP or above	1.0%
Mid-level managers	3.1%
Professionals	7.6%

In 2022:

25% of new hires came through our employee referral program.

Employee Learning

Our culture is constantly growing and evolving, and we believe that each employee plays an important role in shaping and sustaining it. That's why we are deeply invested in the personal and professional growth of our employees. Our fit-for-purpose customized learning and employee development programs are designed and delivered by a dedicated in-house team and seek to reinforce our values by building and strengthening the specific skills and competencies that bring our culture to life. Foundational to all of our employee development and leadership programs is deepening awareness and compassion of self and others while fostering an inclusive and collaborative environment.

We offer a selection of both required and optional workshops. Some of our required workshops include:

Dynamic Feedback – Seek, Offer, Receive: Employees learn to seek, offer and receive feedback that is clear and kind and supports a dynamic culture of continual growth.

Empowered Mindset: Employees learn how to recognize the powerful connection between mindset, actions and results. This empowers them to see choices and options that will lead to better, more fulfilling outcomes.

Managing at Ultragenyx: This three-part experiential workshop is designed to equip managers with tools to communicate effectively, manage skillfully and support their reports' growth and development. Through completing this course, participants receive training on specific, practical strategies that are designed to enhance their ability to achieve great results with their teams. Access to our online resource library addressing career growth and development tools is included with the workshop.

How to Effectively Manage Declining Performance: This 90-minute training is designed to give managers the skills to manage employees who are failing to meet the expectations of their role.

Fostering High Performance: This training is designed to place clear expectations on the role of an effective people manager. Managers are provided tools with the aim to set a vision and outline priorities for effective development of their people and learn to manage performance.

Leadership Development Program (LDP): This program is designed to provide a deeper awareness of personal leadership styles and potential, build greater capacity for creating thriving working relationships, and improve leadership impact, enrich relationships with fellow leaders and strengthen commitment to our company culture.

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

High Performing Teams: Our customized model and approach to help teams move quickly from forming and storming to performing.

Cultivating Resilience in Everyday Life: Provides research based tools to stay strong and promote well-being.

Energy Management Wins - PeopleFuel: Helps employees think about energy as a key resource to meet demands, take an assessment of how energized they are across areas of their life, and discuss managing emotions for increased resilience, performance and well-being.

Insights 101: Introduction to the Colors: Enhances self awareness and ability to understand others by learning about the insights working style assessment and the four color energies.

Mindfulness for Stress Relief: Equips participants with simple tools for stress relief. Includes access to online recordings and an overview of scientific research on how mindfulness meditation positively impacts the brain and nervous system.

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

Additionally, we sponsor programs such as Ignite, a forum for exploring employees' unique and creative ideas, and UltraTalks, our version of TED Talks, designed to bring new perspectives and insights to the organization. These experiences build employee morale, stimulate innovation and invest employees in company improvement. Some of our past speakers have included Abby Wambach, Patrisse Cullors, Chip Conley and Phil Hawking.

In 2022:

- We delivered **70** employee development workshops, of which **55** were delivered by our in-house Organizational Development team.
- On average, each employee attended approximately six hours of development workshops, with **95%** attending at least one workshop.
- The average rating for in-house delivered workshops was **94** out of **100**.

ULTRALEADERSHIP SUMMIT

In 2020, we launched an annual UltraLeadership Summit to bring our most senior leaders together to introduce a corporate 2025 vision and strategy, further enhance their leadership skills, and engage in conversations around inclusion, diversity and belonging. Since then, we have hosted the UltraLeadership Summit annually with great success. The Summit allows our leaders to continue their development journey together and further enhance their skills in modeling authenticity, courage and inclusion as one global leadership team. During the 2022 Summit, leaders had the opportunity to gain insights and hear from external speakers and experts on topics such as maximizing individual and organizational capacity, how to create a culture that embraces diversity and champions inclusion, and how to awaken the creative genius within each of us.

As Ultragenyx continues to grow, the Summit also serves as an important opportunity for global leaders to come together and deepen their connections to each other and their shared commitment to leading the next chapter for the company.

Career Development

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

In 2022, women received 55% of total promotions, which is representative of our gender balance, and 7% of total open positions were filled by internal candidates.

Ultra-Orbit

Ultra-Orbit, a new program introduced in 2022, provides an opportunity for employees at all levels interested in developing or enhancing their program and project management skills while still staying in their current role. In this program each participant engages with a Program Manager mentor with measurable outcomes and timelines. A new manager training tool has also been provided to foster performance, belonging and well-being for employees.

Career Coaching

To enhance career growth and development, we expanded our career coaching program in 2022 to all employees. This program offers 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 2022, nearly 18% of our global workforce participated in the coaching program. Participants rated the quality of sessions 4.8 out of 5 stars. Initial feedback shows:

- **92%** of participants feel more positive about their issue.
- **92%** of participants are more likely to address their situation.
- **84%** of participants learned a new skill or strategy.

“Cannot express how grateful I am for [the career] coaching. It has helped me navigate managing difficult conversations, relationships, feedback – but most importantly, to be an advocate for myself and communicate what I need to do my job. My confidence and self-esteem have improved.”

“Both of my sessions have left me feeling stronger than when I started, have given me tools that I could immediately use, and have boosted my confidence to take the next step.”

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 is to build the next level of leaders that will help us evolve our organization and support our culture as we grow.

Started in 2018, each of the four LEAD Cohorts has been cross-functional and diverse as well as balanced geographically, with the aim of being inclusive of remote employees. The program uses workshops, business simulations, mentorship and executive leadership talks to help equip participants with the skills to activate change throughout the organization. In the first three LEAD cohorts, 78% of participants were promoted within one year of completing the program.

2022 LEAD Cohort Highlights:

- **56%** were women.
- **11%** were based outside the U.S.
- **36%** self-reported as members of ethnic/racial minorities.

Emerging Talent Programs

As an advocate of early scientific careers, Ultragenyx is committed to providing emerging talent with a range of opportunities to learn from a diverse team of world-leading experts at the cutting edge of research. During the summer of 2022, we grew our paid internship program to host over 45 interns across our U.S. and Latin American locations.

In an effort to increase inclusion and diversity in the industry, we hosted our first-ever high school interns in Translational Sciences via a partnership with JCYC, a nonprofit in San Francisco. Additionally, we continued our partnership with Middlesex Community Colleges' Learn & Earn program in Middlesex, Massachusetts. In this program, students can gain college credit while earning a competitive wage and learning skills in demand within Technical Operations. Our Emerging Talent program participants engage in fireside chats and panel discussions with leadership and complete a final poster presentation to highlight their lessons and accomplishments. In 2023, we look forward to expanding emerging talent opportunities in the U.S., LATAM and EMEA regions.

In addition to our intern program, we offer:

Six-month seasonal co-ops: This program provides supplemental training and experience to students with the aim of advancing their education and enhancing excitement around a career in life science/biotechnology. We support hands-on projects that allow students the opportunity to be embedded in meaningful work.

Post-doctoral programs: This program allows postdoctoral candidates the opportunity to study the scientific questions that impact Ultragenyx patients the most. While developing the skills to thrive as a scientific investigator, participants have the chance to grow in a curious, collaborative environment under the mentorship of leaders within life sciences/biotechnology.

Hosted lab tours: This experience allows students an inside look at our new Novato lab and the opportunity to hear from speakers about their journeys and receive career tips. For example, members of our UltraGiving team volunteered at the Novato High School/University of California Davis MBA STEAM Day at our headquarters campus, where 50 students experienced the working environment at a biopharma company, including the lab with its high-tech instrumentation and scientists at work, to help them discover, understand and possibly motivate them into a STEAM career path.

As part of our efforts to prepare the next generation of talent and strengthen university relations, we also began working with Stanford University in 2022 to engage with students, share our company culture and highlight career path options through sponsorship of their Biomedical & Bioscience Industry Expo (BBIE). Team members also participated in Q&As, panel discussions and on-site career fairs.

Summer interns from JCYC High School

Employee Engagement

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

In order to implement feedback more effectively and enhance corporate policies from our "YourVoice" Employee Engagement Survey, we restructured our approach from an annual survey to an 18-month survey, with between two and three new "Pulse" surveys that focus on a limited set of topics, including our corporate goals and key strategic initiatives and efforts. We made this change with the goal of creating a more real-time listening platform so we can develop and deploy solutions to address opportunity areas and to get a more holistic voice-of-the-employee that leads to meaningful improvements.

Moving forward, we expect to send out this limited version in between the 18-month "YourVoice" surveys to all full-time employees who have been with Ultragenyx for at least 90 days. "Pulse" results are utilized by the human resources team and executive leadership to support our employees' voices being heard. "YourVoice" results are expected to continue to be discussed with our board of directors and used as part of the board's oversight of the general organizational health of the company.

Beyond the engagement survey, team meetings are held regularly to help enable interaction with senior leaders and managers. During these meetings, employees are encouraged to be inquisitive and data-driven so that decision-making is responsive to their inputs.

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

- An increase in connectivity and collaboration in our new hybrid working environment.
- Development of additional strategies to improve the health and well-being for our employees.
- Updates to our compensation philosophy and benefits programs.
- A more refined and enhanced communication of our philosophy and criteria for promotion and advancement opportunities.

Employee Recognition

The Ultra Leadership Recognition and Ultra Sharp Award Programs are dedicated to recognizing the top leaders at Ultragenyx on an annual basis. These programs help us acknowledge and celebrate the exemplary leadership that has a significant and positive impact on Ultragenyx team members, our vision, mission, values and culture. In 2022, **19** Ultra Leaders were recognized.

EMPLOYEE ENGAGEMENT SCORE

Enhancing employee engagement is a corporate goal at Ultragenyx. A subset of the employee engagement survey – focused on employee pride in our company, whether an employee would recommend Ultragenyx as a good place to work, if employees experience feeling a personal accomplishment from their work, and their intent to remain with us – is used to calculate an employee engagement score. We maintained a high employee engagement score of over 85% in our employee engagement survey.

According to our 2022 internal employee engagement pulse survey:

- Over **90%** of employees* reported they are proud to work at Ultragenyx. Pride in Ultragenyx continues to be the highest item in engagement, four years in a row.
- Over **85%** of employees feel like they can build and maintain strong working relationships with their colleagues no matter where they are working.

* Refers to employees that participated in the survey.

Inclusion & Diversity

Ultragenyx is dedicated to maintaining a healthy and inclusive company where employees feel respected and valued and experience a sense of belonging from the start.

Respect is a fundamental value that we require and expect from all our employees. This is core to our corporate culture and in alignment with our company goals to foster a thriving culture based on inclusion and diversity (I&D).

"We at Ultragenyx want to be known for our inclusive culture and values. We aim to attract, hire and retain diverse candidates, service diverse communities and foster accountability throughout our organization, creating a people-first ethos that puts communities and our employees at the center of our approach and mission."

Kenyatta Parker, director of Inclusion & Diversity

We have taken several steps to bolster I&D within our company, including expanding workshop and engagement opportunities. In 2022, 90% of employees participated in the I&D curriculum. Highlights of our accomplishments during 2022 include the following:

- Hired a dedicated I&D leader to expand our efforts and lead the I&D action team comprised of I&D champions across the company.
- Conducted an external cultural audit to gain additional perspectives on opportunities for growth and enhancement of our I&D program.
- Hosted our first annual Employee Resource Group (ERG) Summit exhibiting how ERGs play a vital role in driving the growth of our business and our overall culture of inclusion. The summit provided us the opportunity to share experiences, learn, collaborate, problem-solve and network with one another.
- Continued our UltraTalks series, with featured guest speakers on equality and inclusion, fat shaming awareness, team collaboration and other topics.
- Launched the Power + Privilege diversity education workshop for EMEA and LATAM customized to those regions.
- Launched the new Dispelling Fear and Stigma workshop to develop a greater understanding of the disability community, the wide array of disabilities people may face and how to shape disability-inclusive workplaces. This workshop is designed to help challenge preconceived notions about what it means to be a person with a disability and to help equip attendees to be better allies.
- Increased ongoing I&D dialogues and engagement through our internal ERGs.
- Continued our participation in the Healthcare Businesswomen's Association (HBA) and the National Sales Network (NSN).

According to our 2022 internal employee engagement pulse survey, our I&D scores continue to be strong:

- **90%** of our employees* feel that their managers support I&D in the workplace.
- Over **85%** feel that their managers model inclusive behaviors.
- Over **85%** feel that their managers create an environment where people feel they belong.

* Refers to employees that participated in the survey.

NEW DECLARING & REPAIRING BREAKDOWNS PROGRAM

In 2022, we launched a company-wide mandatory employee education called Declaring and Repairing Breakdowns, a program designed to develop employees' ability to declare and repair both business and interpersonal breakdowns, including things like micro-aggressions. Participants learn to foster breakthroughs that lead to greater trust, empowerment, high performance and aligned execution.

"I have to admit, coming from big pharma where I had attended so many similar trainings, I was skeptical when I started. But the conversations so far have been interesting and inspiring and have applicability to my day-to-day meetings."

An Ultragenyx employee

90% of employees participated in 2022, resulting in ~4,800 hours of training completed.

For information on diversity in our board of directors, see Corporate Goverance.

Workforce Data (All data is as of December 31, 2022.[1])

Number of Total Employees	**1,311**
% of employees who are women	56.8%
% of employees with a disability	3.4%

Age Breakdown (Total Employees)	
<30 years of age	7.3%
30-50 years of age	58.7%
>50 years of age	33.9%

Number of Employees on the Executive Leadership Team (XLT)	**8**[2]
Number of XLT members who are women	2
Number of XLT members who self-reported identified as racially or ethnically diverse	3

Number of Women in Management Positions	**222**
% of women in all management positions (as % of total management positions[3])	50.5%
% of women VP and above (as % of total management positions)	45.6%

Number of U.S. Employees	**1,155**
% of U.S. employees who are women	57.1%
% of U.S. employees who self-reported identified as racially or ethnically diverse	44.9%[4]
Asian	25.9%
Black/African American	6.5%
Hispanic or Latino	8.6%
White	54.5%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	3.5%
Not Specified	1.1%

Number of U.S. Employees in Management Positions	**396**
Asian	23.2%
Black/African American	4.0%
Hispanic or Latino	8.6%
White	60.6%
American Indian/Alaskan Native, Native Hawaiian or Other Pacific Islander, Two or More Races	3.3%
Not Specified	0.3%

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

[2] The role of CFO was not filled at the end of 2022. Once filled, there will be nine XLT members.

[3] Total management positions includes all management positions at Ultragenyx, including employees in manager roles.

[4] Due to rounding, the percent breakdowns add up to >100%.

Employee Resource Groups & Community Groups

I&D ERGs are voluntary, employee-led groups that aim to foster a diverse and inclusive workplace by creating a sense of community and belonging for members.

These groups aim to create and promote cultural awareness and education for the company. Each ERG has a distinct impact on culture at Ultragenyx and is equipped with a charter, objective, mission and support team. In 2022, Ultragenyx welcomed a new I&D ERG, UltraAPAC.

The UltraMosaic ERG hosted an inaugural Juneteenth Celebration to honor this day of freedom and recognize the importance of this significant moment in history for Black Americans. Employees at our Novato and Brisbane, California, offices celebrated with food, games, team building activities and networking opportunities.

Employee Resource Groups

LatinX: Seeking to empower the LatinX community at Ultragenyx to realize its fullest potential and encouraging the next generation of LatinX talent to pursue careers in science and biotechnology.

UltraAPAC: Dedicated to recognizing and celebrating the unique and diverse cultures within the many Asian and Pacific Islander (APAC) communities across the globe.

UltraMosaic: A mix of ethnic groups and cultures that coexist with society and here at Ultragenyx—recognizing, celebrating and using our gifts, abilities and resources to support one another and the company while also creating a safe space and support network for Black, Indigenous and People of Color to thrive.

UltraProud: Focuses on fostering a culture of equality and belonging at Ultragenyx so that LGBTQIA+ employees can thrive in their careers and lives and achieve greater impact in the world.

X2 Women in Biotech: Seeking to create a supportive culture to encourage women, diversity and equity in leadership and science.

Xtended Office: Seeks to enhance Ultragenyx's remote working experience to ensure that employees feel a sense of belonging even while working from home.

Community Groups

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

UltraFun: Planning for events, including fun pop-ups, happy hours, watch parties and annual company parties.

UltraGiving: Connecting employees with opportunities to support nonprofit organizations assisting local, at-risk communities and promoting STEAM education.

I&D in Recruitment

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

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

In 2022, we launched a partnership with Disability Solutions (DS) on outcomes for talent with disabilities, and Ultragenyx's jobs became live on the Disability Solutions' Career Center. The DS Career Center works with Ultragenyx to drive traffic to our job postings by actively recruiting candidates with disabilities from their large network of local and national workforce community partners.

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

79%

of 2022 new hires
self-reported as members of diverse populations.

"Ultragenyx remains committed to bringing together people with different perspectives, backgrounds and experiences to fuel our collective innovation and value creation. Our hiring practices advance inclusion and representation while supporting a diverse global workforce so we can achieve our ambitious mission together."

Ernie Meyer
executive vice president and chief human resources officer

Occupational Health, Safety & Wellness

The health and safety of our workforce is a key priority. Our health and safety management system is vital to a safe and healthy work environment and includes several elements, such as Global Environmental, Health, Safety and Sustainability (EHSS) standards, site-specific standard operating procedures, incident and safety observation reporting, hazard identification and risk assessments, job safety analyses, ergonomic assessments and industrial hygiene evaluations.

The system is implemented based on the principles of ISO 45001:2018, the International Organization for Standardization (ISO) standard for occupational health and safety management, and is designed to help us comply with applicable statutory and regulatory requirements and Ultragenyx policies; proactively identify and prioritize occupational health and safety risks and potential mitigation options for our operations; and drive continued improvement across our global operations.

At Ultragenyx, we continuously search for opportunities to improve. In 2022, as part of our efforts to advance health and safety management across our business, we focused on the on-going development, improvement and effective implementation of our health and safety management system.

Notable achievements this year included:

Strengthening of key Global EHSS Standards for incident reporting and investigation, performance reporting, emergency response and preparedness, biological safety, chemical management, ergonomics and respiratory protection.

Implementation of a new process designed to track key performance indicators for injury rates, safety observations, incident investigation completion, corrective action closure, risk and ergonomic assessments, and EHSS training.

Improvements to our EHSS training process and training content, including Global EHSS Orientation, Site EHSS Orientation, Laboratory Safety, Hazard Communication, Bloodborne Pathogens and Respiratory Protection training. In all, 19 EHSS training courses were delivered in 2022.

Review of over 940 on-site chemicals to help characterize industrial hygiene risks. We plan to prioritize follow-on exposure assessments and exposure monitoring during 2023.

Offering of the Hepatitis B vaccination to all laboratory personnel with potential for exposure to bloodborne pathogens.

Implementation of a Global Safe Work Manual designed to guide the safe completion of all construction work, capital projects and maintenance at Ultragenyx facilities. Global Safe Work training was provided to over 50 Ultragenyx employees that manage contractors and vendors.

Provided evacuation procedure training to over 650 on-site personnel and conducted evacuation drills at each of our San Francisco Bay area and Boston area locations, including the gene therapy manufacturing facility.

Completion of 130 ergonomic hazard assessments for Ultragenyx employees intended to identify and mitigate potential injuries due to awkward postures, repetitive motion and forceful exertion.

Moving forward, we plan to enhance our safety culture by encouraging employees to report safety observations, expand proactive ergonomic assessments, and identify the top chemicals of concern and complete hazard assessments.

2022 Safety Data

Lost Time Incident Frequency Rate (LTIFR)* Employees and Contractors	0.397
Number of Fatalities – Employees	0
Number of Fatalities – Contractors	0

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

Return to Office

In April 2022, after two years of working from home, we introduced a newly defined work model and welcomed many employees back to our offices. Offering more flexibility over how and where our employees work, this new hybrid environment is designed to balance productivity, flexibility and work-life integration while deepening connections to support team members to do their best work, regardless of location.

Creating and maintaining a safe work environment for our employees remains a top priority. To support the well-being of others, we require anyone experiencing cold, flu or COVID-19 related symptoms to stay home and we continue to enforce our established health and safety protocols, which cover both in and out of office activities.

Protocols are communicated to our employees regularly so they remain top of mind and include guidance on wearing masks, conducting in-person meetings or events, vaccination requirements, traveling, what to do in the event of a positive test or direct exposure to COVID-19 and testing as well as other details related to specific roles. Our established safety protocols also extend to those still working from home. As our return to office approach continues to evolve, we welcome feedback and encourage our employees to share their experiences, good or bad, along the way.

PREPARING FOR A SAFE STARTUP AT OUR GENE THERAPY MANUFACTURING FACILITY

In 2022, as part of strengthening our management system and preparing for the opening of our new gene therapy manufacturing facility, we focused on putting health and safety procedures and practices in place to help support a safe startup of our facility. We developed procedures on emergency preparedness and response, ergonomics, performance reporting, incident reporting and investigation, and respiratory protection.

In an endeavor to improve the safety of construction workers at the site, we organized a mental well-being day featuring a stress management talk and a lunch-and-learn session on addiction and recovery by the Massachusetts Building Trade Council.

Wellness

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

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

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

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

Caring for U provides an annual stipend to support things like fitness classes and gym membership, nanny and childcare, backup childcare, summer camp, eldercare services, meal delivery service to a dependent family member and pet walking.

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

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

Caring for U Benefit

In 2022, we launched Caring for U, a global reimbursement program designed to help employees adopt and maintain a well-rounded healthy lifestyle. The program also supports those with caregiving responsibilities. The program covers expenses for a variety of wellness and caregiving activities. Employees may receive up to $1,200 a year (in their local currency) in wellness credits for eligible expenses.

ULTRAGENYX'S RESPONSE TO THE U.S. SUPREME COURT DECISION OVERTURNING ROE V. WADE

In light of the U.S. Supreme Courts' decision to overturn Roe v. Wade, Ultragenyx has adopted policies so that our U.S. employees have access to reproductive care regardless of where they live. We believe that healthcare decisions and family planning are personal. We also believe that increasing access to women's healthcare is important to advancing healthcare equity for all.

In coordination with Blue Shield of California, we have developed an extended healthcare benefit that is intended to provide travel reimbursements (for example, transportation, hotel accommodations, meals and companion expenses) for U.S. team members living in restricted states that no longer offer access to reproductive care. Critically, any use of these services does not require any disclosure to the company or approvals by Ultragenyx employees and is handled directly by Blue Shield.

Employee Compensation & Benefits

Ultragenyx employees bring their best to work every day. We honor their dedication by offering competitive compensation and benefits packages designed to attract, retain and motivate talented people.

We are committed to fair and equitable compensation practices within a pay-for-performance framework and believe these principles are critical in supporting our culture and achieving our mission. We regularly review our programs and practices as well as our employees' pay with the goal of providing pay and opportunities that are equitable, transparent and free from bias. Our compensation decisions are based on role, performance, location, external and internal peer data, relevant experience, professional and personal contributions, and senior leadership review.

Ultragenyx is committed to investing in our employees' career growth and rewarding great performance.

We continue to extend our compensation policies, increase benefit offerings and communicate criteria for promotions. In 2022, in our endeavor to enable all employees to reach their full potential by equal access to opportunities for growth and advancement, we reset our compensation peer group from broad life sciences to focus on biopharmaceutical and biotechnology advanced compensation ranges, which increased our compensation ranges and increased the annual salary budget.

More information can be found on our Careers webpage.

For information on median compensation and CEO pay ratio, see the Proxy.

"At Ultragenyx, we believe in rewarding our employees for their contributions to the company with both financial and non-financial incentives. Our competitive compensation and benefits packages are designed to attract top talent and keep our employees motivated and engaged. We believe this approach helps to drive improved productivity and better business results."

Andrea Tuck, vice president of Global Rewards & Human Resources Operations

SPOTLIGHT

Pay Transparency

Ultragenyx is in compliance with the new California pay transparency law requiring employers to include pay ranges in job postings. We also comply with these requirements for all remote positions as well as those listed in the states of New York, Washington and Colorado.

Our Benefits Programs

Our benefit programs provide employees and their families with access to a suite of innovative programs that enhance their physical, emotional, familial, financial and social well-being, plus other perks to support employees 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 many other programs that support balancing work with life. Eligible employees participate in our annual short-term and equity-based, long-term incentive programs, which provide opportunities to share in our company's success.

Some of our employees' favorite benefits

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

2022 Enhancements

- Enhanced the **U.S. 401(k) retirement plan** and increased the company match.
- Launched **ID theft benefits** that help protect U.S. employees from identification theft.
- Added a **referral bonus** for temporary positions as part of our expanded Employee Referral Program.
- Rolled out a new **career coaching benefit** to all employees.
- Evolved the **Total Rewards Program** by introducing a long-term incentive plan that grants performance stock options with a vesting period of three years upon the achievement of strategic performance goals.

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, STEAM education, and local, at-risk communities.

Aspiration

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

Our Objectives	2022 Progress
Support charitable organizations that contribute meaningfully to the health and well-being of the communities where we operate and beyond.	• Approved $4.27 million in company-sponsored charitable donations and grants. • Provided funding to EURORDIS, Razom for Ukraine and Save the Children to support relief efforts in Ukraine and the broader refugee crisis. • Our Ultragiving team and employee resource groups (ERGs) sponsored more than 10 employee-directed giving and volunteer campaigns to support a variety of local community organizations.
Expand opportunities for employee volunteerism.	• Partnered with Life Science Cares to provide volunteer opportunities to Ultragenyx employees in support of local public health and wellness initiatives in the San Francisco Bay and Greater Boston areas. • Launched a volunteer mentorship program in Latin America, where Ultragenyx volunteers dedicate their time, attention, experience and counseling to children ages 14 to 19 who live in vulnerable social environments. • Created an International Day of Service for employees in Latin America to give back to the communities in which we live and work.

Charitable Giving

Our charitable giving mission at Ultragenyx builds on our company's broader mission to transform the lives of people with rare disease.

We direct our charitable giving to our local communities and beyond, and recognize that healthy lives are supported by healthy communities and environments, including education and access to healthcare. During 2022, we formalized our corporate philanthropy framework, focusing on the following priorities:

Rare disease community support

Equitable healthcare

Science, Technology, Engineering, the Arts and Mathematics (STEAM) education

Initiatives benefiting local, at-risk communities

We are committed to supporting initiatives that we believe provide impactful resources for each of these priorities. We also expanded the representation and reach of our charitable giving programs from the U.S. to include EMEA, LATAM and APAC regions.

In 2022:

Ultragenyx approved **$4.27 million** in company-sponsored charitable donations, independent medical education grants and health-related grants.

EMPLOYEE GIVING & VOLUNTEERING

Ultragenyx offers employees the opportunity to take two paid volunteer days each year (16 hours) so they can spend time giving back to our communities and contribute to local initiatives. In 2022, employees donated time 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 a community group called UltraGiving committed to connecting employees with opportunities to support nonprofit organizations assisting local, at-risk communities and promoting STEAM education. Once the connections are made, employees can choose to use their paid volunteer time, volunteer on their own or make donations. In some cases, the organizations are the same as those supported by corporate giving; in others, employees identify different organizations they wish to support. UltraGiving has chapters in the San Francisco Bay and Greater Boston areas, Utah and Latin America.

UltraGiving Latin America runs a volunteer mentorship program. The program includes a volunteer mentor guide and training to all participants. In 2022, 23 Ultragenyx volunteers dedicated their time, attention, experience and counseling to 23 children ages 14 to 19 who live in high-risk environments.

Rare Disease Community Support

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

In 2022, Ultragenyx proudly supported:

Camp Korey is a medically safe camp experience that offers children and families an escape from the endless medical treatments that often overshadow childhood. The objective is to create empowering and adaptive year-round programs for children and their families living with life-altering medical conditions, free of charge. Ultragenyx funded the medical oversight, program and activity supplies for eight sessions of residential Summer Camp serving more than 350 children living with serious illnesses and their family members.

Ultragenyx made a contribution to **Parent to Parent USA**, a nonprofit organization dedicated to offering emotional and informational support to families with children who have special needs. The donation was directed towards supporting the P2P USA's Leadership Institute, which is a unique national conference that specifically addresses the challenges of running a Parent to Parent program. During this conference, leaders from various programs across the country gather to share best practices and strategies for meeting the evolving needs of families with disabilities and special healthcare needs.

In recognition of Rare Disease Day 2022, Ultragenyx donated to **Baby's First Test**, a newborn screening education resource center for families and health professionals in the U.S. Newborn screening, a state public health service designed to identify individuals in a population who may be at an increased risk of a certain disease, allows a condition to be identified and treated before a problem occurs. Ultragenyx is advocating on behalf of patients and communities for the continued growth and improvement of the newborn screening system. See our blog for a guest post by Natasha Bonhomme, chief strategy officer at Genetic Alliance and director at Baby's First Test, for her perspective on the life-changing impact of newborn screening.

Ultragenyx supported Evening of Hope, a fundraiser for **Boston Children's Hospital**, which treats children with rare diseases and complex conditions and is dedicated to improving and advancing the health and well-being of children around the world through its life-changing work in clinical care, biomedical research, medical education and community engagement.

In recognition of Rare Disease Day 2022, Ultragenyx donated to **Rare Disease Innovations Institute (RDII)**, a global nonprofit focused on educating, engaging and equipping the rare disease community. RDII promotes the development of rare disease advisory councils (RDACs), which give the rare disease community a stronger voice in state government. To date, RDII has empowered people in 20 states to establish RDACs and another 13 states are working to form their own RDACs. See our blog for a guest post by Tara Britt, founder and president of RDII, on supporting grassroots advocacy and health equity in rare disease.

Little Wishes, a nonprofit founded by two pediatric oncology/pediatric intensive care unit nurses, helps prevent patients from losing their identity to illness by tapping into their passions. Young patients may identify what makes them happy and wish for something that will fill their hearts and brighten their darkest days. Ultragenyx donations enabled the Little Wishes team to begin granting wishes on the oncology floor at Arkansas Children's Hospital. With support from partners like Ultragenyx, Little Wishes granted its 20,000th wish in 2022.

SPOTLIGHT

Lizzie was the first cancer patient to receive a Little Wish at Arkansas Children's. Lizzie is 11 years old and loves slime, games, cats and the color pink, all of which helped her create her very first Little Wish.

Ultragenyx donated to the **European Organisation for Rare Diseases (EURORDIS),** a nongovernmental patient-driven alliance of patient organizations and individuals active in the field of rare diseases, that promotes research on rare diseases and commercial development of orphan drugs. The donation supported the EURORDIS Rare Response Fund for Ukraine, which developed a Rare Disease Online Resource Centre to 1) help Ukrainians living with rare diseases navigate the different health and social care policies available across Europe to access care; 2) support patient and humanitarian aid organizations in Ukraine and neighboring countries to deliver frontline response to Ukrainians living with a rare disease; and 3) support displaced Ukrainians living with rare diseases to secure free, short-term housing outside of Ukraine.

Grants

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

Ultragenyx works side by side with organizations that provide educational resources and support for those affected by rare diseases. We partner with patient advocacy groups (PAGs) listed on our website to provide education, support and periodic updates of our clinical programs.

We provide grant support for the rare disease community for:

Independent Medical Education: We provide educational grants for accredited and nonaccredited clinical, technical and scientific programs and continuing medical education (CME) activities focused on rare diseases for healthcare providers.

Health-Related Grant Funding: We provide sponsorships and grants for nonprofit patient organizations and for-profit health institutions in support of patient advocacy focused initiatives, scientific meetings (non-accredited), disease awareness campaigns, research programs, medical publications, fellowships and sponsorships.

Please see our website for areas currently being considered for funding.

In 2022:

- Over **70** patient advocacy organizations located in **21** countries received health-related grants totaling almost **$2 million**.
- Over **40** organizations in **9** countries received independent medical grants totaling approximately **$2 million**, with over **60,000** healthcare professionals educated through these supported independent medical education programs.

SUPPORTING PATIENT ADVOCACY IN MEXICO

Through a PAG in Mexico, a mother and daughter were fortunate to receive support that led them to a rare disease diagnosis. Itzel and her daughter Isabella live with XLH. With XLH, the body doesn't retain enough phosphorus—a mineral that's important for healthy bones. This can cause bones to weaken over time (a condition called osteomalacia). In May 2021, Isabella, at the age of 3, became the first patient to be treated with a biologic treatment for the disease and to receive proper management for her disease in Mexico. Itzel and Isabella consider themselves fortunate for the support network that led them to the diagnosis and treatment. They have dedicated physicians who searched for answers and advocated for them, and they have access to private healthcare insurance. But they know this is not the story of every XLH family, and that's why they are now strong advocates of the XLH patient advocacy group in Mexico (XLH y otros raquitismos México*). They are sharing their story and raising awareness with the goal that every XLH patient can have adequate access to early diagnosis and the best care, both in the private and public healthcare systems.

* XLH y otros raquitismos México receives financial support from Ultragenyx

Equitable Healthcare

We believe that equitable access to healthcare is key to a thriving and sustainable society.

We support organizations that work to improve public health and increase access to care for at-risk communities. Our equitable healthcare giving targets public health initiatives, access to services and wellness for these communities.

In 2022:

Partnered with Life Science Cares to provide volunteer opportunities to Ultragenyx employees in support of local public health and wellness initiatives in the San Francisco Bay and Greater Boston areas.

Donated to WheeLog, a nonprofit created to support wheelchair users and people with mobility challenges in Japan. WheeLog is an app with an interactive map and user community that leverages crowdsourced information to share information on the accessibility of public and commercial facilities and the routes wheelchair users and others with limited mobility can take. The award winning app launched in 2017 with funding from an MIT competition and various family-based foundations, and Ultragenyx funding is expected to support expansion into multiple languages. This should help tourists in Japan who have limited mobility and will allow current app users (across 30+ countries and territories) in places like Taiwan and the U.S. to begin crowdsourcing information in their own geographies.

Supported Expedicionários da Saúde (Health Expeditionary Operation), a nonprofit in Brazil that brings specialized medical care, especially surgery, to indigenous populations living in hard-to-reach areas in the Brazilian Amazon. These efforts are also intended to prevent deforestation by keeping indigenous people in their homes within the rainforest. Ultragenyx funded an expedition to São Gabriel da Cachoeira in the state of Amazon in November 2022.

Support for Ukraine

RAZOM

Donated to **Razom for Ukraine**, an emergency response program focused on providing medical supplies for critical situations.

Save the Children

Supported **Save the Children**, an organization that broadly focuses on humanitarian aid for refugee children across the globe. Our donation to their Ukraine crisis-relief fund helped get food, water, hygiene kits, cash and psychosocial support to children and families.

SPOTLIGHT

The X2 Women in Biotech ERG sponsored a Women's Hygiene Kit Drive for The Ritter Center. Employees in the San Francisco Bay area donated $2,900 worth of hygiene kit supplies. The ERG also sponsored a Diaper Drive to support Horizons for Homeless Children. Employees from the greater Boston area donated over 5,700 diapers and 1,000 wipes.

STEAM Education

To help inspire and advance the development of the next generation of science, technology, engineering, the arts and mathematics (STEAM) leaders, we support locally implemented initiatives and organizations.

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

2022 Highlights

- Donated to **Biotech Partners** to support students interested in STEAM careers.
- Supported 7th and 8th grade science after-school programs in Sonoma County, California, through **Expanding Your Horizons.**
- Donated STEAM funding to **Buck Institute** and participated in the **North Bay Science Discovery Day** by hosting a student-focused exhibit to spark wonder and curiosity about science, technology, engineering and mathematics for children, teenagers and their families.
- Held back-to-school supply drives at our offices in Boston to benefit the **East End House** and in San Francisco to benefit **Homeward Bound** and **The Ritter Center**. Employees donated over $3,000 worth of backpacks and school supplies and volunteered to help assemble backpacks by age range to support students from elementary through high school.
- Supported Life Sciences Education and Workforce Development Programs by donating to **BoStem**, an initiative led by United Way of Massachusetts Bay and Merrimack Valley in partnership with Boston Afterschool and Beyond and Boston Public Schools, to inspire the next generation of STEAM professionals in the greater Boston area.
- Donated $3,500 and built 350 STEAM kits to support the **Science Club for Girls** in Boston.

Members of the LatinX ERG attended the 2022 **Society for Advancing Chicanos/ Hispanics and Native Americans in Science (SACNAS)** diversity in STEM conference in Puerto Rico and served as mentor judges to 24 undergraduate and graduate students for the scientific poster presentations.

Local & At-Risk Communities

At Ultragenyx, we are committed to improving local communities and creating a positive impact on the lives of those in need while fostering a closer-knit community.

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

2022 Highlights

- Ultragenyx provided a grant to the **Heroes and Helpers** Program, run by North Marin Community Services (NMCS) in partnership with the Novato Police Department. The program enabled 61 children from the NMCS Child Development or Case Management Programs to purchase holiday gifts for their family members. The children were dropped off at NMCS, then brought to Target on a school bus. Upon arriving, each child was given a $100 gift card to purchase gifts and matched with a police officer or a fire fighter to help them with their shopping. The children brought the gifts they purchased home to give to family members.
- Employees from the UltraAPAC ERG organized a drive to support the **San Francisco Asian Women's Shelter**, a nonprofit organization founded to address the urgent and unmet needs of survivors of domestic violence and human trafficking, especially those who are immigrant or refugee women, children, LGBTQ+ and/or youth. In support of their mission of eliminating domestic violence by promoting the social, economic and political self determination of women and all survivors of violence and oppression, our San Francisco Bay Area and Boston area offices as well as the UltraMosaic ERG donated 12 space heaters, over 60 pairs of slippers, 30 pajama sets, $600 in gift cards, and over 200 toiletries and personal hygiene items to the shelter's drive.
- Ultragenyx team members led a year-end toy drive to support the work of **Wonderfund**, an organization that serves children engaged with the Massachusetts Department of Children and Families (DCF). Wonderfund works directly with social workers to meet the individual needs of children and helps to create magical holiday moments for children throughout the state. Ultragenyx employees were able to provide holiday gifts for 138 local families and make a few wishes come true.
- Employees in our Utah office braided and donated 100 jump ropes made from t-shirts to **Lifting Hands International** for distribution to Ukrainian refugees.
- Employees in the San Francisco region contributed to bringing meals to people in need through volunteering and hosting food drives for the **Redwood Empire Food Bank** and the **San Francisco-Marin Foodbank**.
- Employees from Ultragenyx's Culture and Organizational Strategy team donated over $700 to **The Ritter Center** to purchase holiday gifts for children and families in need.
- Employee teams across the LATAM region (which includes the regional headquarters in Miami, Florida) coordinated volunteer initiatives with the goal of creating an **International Day of Service** to give back to the communities in which we live and work. Employees contributed to a range of causes, including building meal kits for families in need in Mexico, volunteering at a food bank in Colombia, giving blood and building meal kits in Brazil, beautifying a neighborhood school in Argentina, and removing invasive plant species from a state park in Florida. The Day of Service resulted in 78% employee participation.

Planet

Reducing our environmental impact and promoting sustainability

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

Aspiration

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

Our Objectives	2022 Progress
Implement continuous improvements to reduce our environmental footprint.	• Purchased 1,988 megawatt-hours (MWh) of renewable electricity, which avoided an estimated 465 MT of carbon dioxide equivalent (CO_2e). • Installed additional electric vehicle charging ports, bringing our total to 11. • Partnered with our waste services vendor to launch a medical waste takeback program.
Develop an environmental strategy.	• Initiated the collection of environmental data across our facilities using our newly developed procedures and policies.

Photo on previous page: Our Ultragenyx Brazil teammates traveled to the Amazon rainforest with the Brazilian Health Expeditionary and helped support the doctors who bring specialized care to indigenous populations. See page 66 for more information.

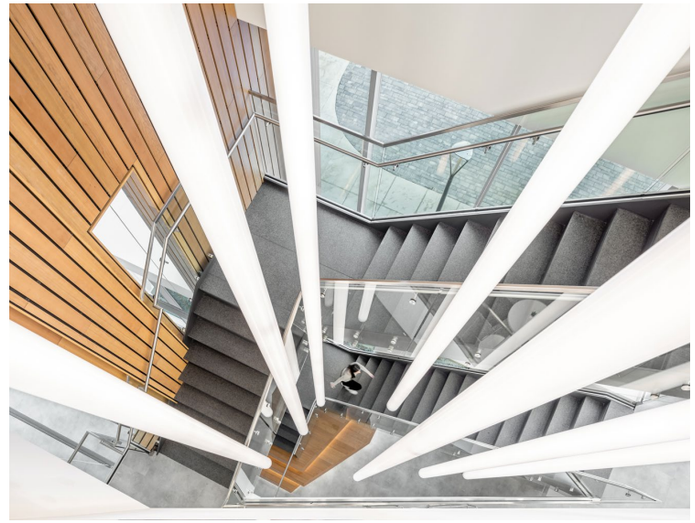
Reducing Environmental Impacts

Ultragenyx is working to reduce the environmental impact of our business by enhancing and promoting sustainable practices across our office, laboratory and manufacturing spaces, both leased and owned, and designing our facilities with sustainability in mind.

In 2022, we developed policies and procedures related to environmental data collection. The policies include standards for tracking and reporting environmental information to support analysis of our environmental impacts by location. We also developed a reporting protocol to support comparability and reliability of metrics in future years.

In 2023, we plan to automate data collection and implement a dashboard to assist in monitoring our impact. Coupled with benchmarking and analytics efforts, we expect these programmatic improvements to allow our teams to develop environmental initiatives more efficiently.

Ultragenyx's 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 the Ultragenyx Global Standard for Suppliers.



Inside our gene therapy manufacturing facility in Bedford, Massachusetts

Minimizing GHG Emissions

Ultragenyx recognizes the importance of minimizing greenhouse gas (GHG) emissions as part of our commitment to sustainability. As such, we have introduced several initiatives to reduce the company's footprint.

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

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 eliminating GHG emissions associated with our electricity use. Purchasing renewable electricity supports the City of Novato's Climate Change Action Plan, which outlines strategies for the city to achieve a GHG reduction target of 40% below 2005 levels by 2035. In 2022, Ultragenyx purchased 1,988 MWh of renewable electricity through this initiative, which avoided an estimated 465 MT CO_2e.

Electric vehicle (EV) charging stations are offered at the Novato and Brisbane, California, office complexes to support employees who drive electric vehicles. There are 11 EV charging connections across these two complexes, including two that comply with the Americans with Disabilities Act. More EV charging is also planned, including for our new gene therapy manufacturing facility in Massachusetts.

Ultragenyx offers comprehensive **commuter reimbursement benefits** to our employees around the world to encourage utilization of public transportation. All of our locations in Massachusetts are located near public transit options, including train and bus stations. We encourage employees to utilize these options when commuting. Taking public transportation to and from work reduces the number of vehicles on the roads, which in turn should reduce GHG emissions from employee commuting.

The Laboratory Operations team implemented a **management system** in 2022 to support tracking and review of frozen sample inventory. While working cross functionally with scientists and study leads to improve this process, the team was also able to increase space efficiency and recover nearly a freezer worth of additional storage. The team is also working to replace obsolete sample freezers with newer, more energy-efficient models. Combined, these initiatives are expected to lead to energy use reductions over time.

Managing Waste & Water

We are committed to complying with applicable federal, state and local requirements for the handling of hazardous and non-hazardous wastes. We have procedures in place, along with training and compliance audits, to promote appropriate handling and disposal of our waste streams.

Practicing sustainability in our labs is important to Ultragenyx and our employees. For example, we partner with Polycarbin, a company dedicated to circular solutions designed to transform today's laboratory waste into tomorrow's laboratory products, to recycle pipette tip boxes. Polycarbin's innovative mailbox program follows a closed-loop recycling approach, where they receive lab plastics that may otherwise go to landfill or downcycling, and remanufacture the waste into new lab products. Since launching this program in our labs, we estimate that Ultragenyx has diverted 950 pounds of plastic waste from landfill, including circularizing 75% into new lab products. Furthermore, we have implemented a single-stream recycling program across our sites.

TRANSFORMING REGULATED MEDICAL WASTE INTO PRODUCTIVE VALUE

In the U.S., most regulated medical waste is rendered non-infectious, then incinerated or landfilled.

As we expand our operations into manufacturing and operate additional laboratories, the associated amount of regulated medical waste is expected to grow. In 2022, we partnered with our waste services vendor to launch a medical waste takeback program.

Working with our vendor, the program is expected to allow most of Ultragenyx's medical waste to be sent to private processing facilities. There, it will be sterilized, processed and repurposed into plastic lumber, which has multiple applications including for landscaping, truck toppers and pallets. The treatment process itself follows green chemistry principles. It uses no air or water. It is designed to render source materials non-infectious and they become inert when absorbed into the plastic lumber end-product.

Ultragenyx employees have been trained in the new procedures under the takeback program. We also installed recycling receptacles that are intended to allow for the separation of waste into various categories to enable its efficient processing. This program has been initiated at the new gene therapy manufacturing facility, and performance will be monitored and reported. Expansion of this program is planned at other locations.

Managing Water:

Although water usage is relatively minimal in our industry, we continue to be conscious of our water footprint and operate responsibly. Our teams are studying updated wastewater methods in order reduce impacts from our operations. Additionally, we have upgraded common area toilets and sinks to low-flow models in an effort to conserve water. In the future, we plan to conduct water stewardship assessments at facilities located in water scarce areas in the U.S.

Governance

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

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

Aspiration

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

Our Objectives	2022 Progress
Act responsibly and with integrity and provide annual, targeted training to our workforce on Ultragenyx's ethical standards.	• 100% of full-time employees provided written or digital acknowledgment of the Global Code of Conduct. • 100% of full-time employees have received training on the Global Code of Conduct within the last three years.
Maintain a high compliance culture and adherence to all applicable legal requirements.	• Ultragenyx's Healthcare Compliance Manual was translated into Spanish, Portuguese, French and German. • All reported complaints related to potential breaches to our Code of Conduct and incidents of discrimination or harassment were investigated and promptly addressed.
Maintain a high rate of third-party due diligence of our suppliers.	• We became a member of Rx-360 (The International Pharmaceutical Supply Chain Consortium) and began to participate in the Rx-360 Joint Audit Program.

Photo on previous page: Members of Ultragenyx's board of directors and executive leadership team with employees and members of the construction crew at our new gene therapy manufacturing facility in Bedford, Massachusetts.

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 we periodically review our practices.

Our Global Code of Conduct (Code) 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

The board of directors (the board) 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 that represent a diversity of talents, skills, backgrounds and expertise.

Our board currently has a standing Audit Committee, Compensation Committee, Nominating and Governance Committee, and Research and Development Committee. Each of these committees operates under a written charter setting forth the functions and responsibilities of the committee, a copy of which is available on our website.

Board Diversity:

- **Three** are women.
- **Three** self-identify as racial or ethnic minority.
- **One** self-identifies as LGBTQ+.
- Average age is **60.9 years**.
- Average tenure is **6.4 years.**

* As of April 1, 2023, our board consisted of nine directors, eight of whom are independent.

For more information on our directors and corporate governance, please see our Proxy Statement. See also ESG Governance & Strategy for information on our ESG governance approach.

Examples of Good Corporate Governance

Best Practices

- Ongoing shareholder engagement program
- Diverse board
- Board oversight of ESG
- Minimum stock ownership requirements for directors and named executive officers
- At least 90% attendance of board and committee meetings in 2022 by our current directors
- Director Overboarding Policy

Independence

- Strong and active independent board chairman
- Other than our president and CEO, all other directors are independent
- 100% independent directors in Audit Committee, Nominating and Corporate Governance Committee, and Compensation Committee

Accountability

- Director Resignation Policy for directors that receive less than majority support in uncontested elections
- Clawback Policy
- 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 risk and discusses with management the appropriate level of risk for the company.

In 2022, the board and the committees reviewed with management various risks and mitigation strategies, including those related to:

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

The board delegates oversight of certain risks to each board committee, and each member of the executive leadership team is responsible for certain risk areas. Executive leadership is responsible for establishing our business strategy, identifying and assessing the related risks, and implementing appropriate risk management practices. For a summary of risks and uncertainties, see Item 1A Risk Factors in our 2022 Annual Report.

Risk Management Oversight

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.

Board of Directors

Has overall responsibility for the oversight of risk management

Executive Leadership Team

Is responsible for establishing our business strategy, identifying and assessing risks, and implementing appropriate risk management practices

Board Committees

Nominating and Corporate Governance

Oversees risks related to board composition, evaluation and succession planning, ESG and sustainability, and compliance

Compensation

Oversees risks related to human capital, including compensation, senior management succession planning, inclusion and diversity, and pay equity matters

Audit

Oversees risks related to financial, data privacy and cybersecurity

Research and Development

Oversees risks related to the company's pipeline, investment and R&D activities

Ethics & Integrity

Ultragenyx is committed to maintaining high standards of honest and ethical business conduct. We have policies designed to prevent, deter and detect bribery, fraud and other corrupt business practices.

In 2022, Ultragenyx did not have any material monetary losses as a result of legal proceedings associated with corruption and bribery (please refer to our 2022 Annual Report).

Our Global Code of Conduct (Code) establishes principles and expectations that apply globally to our workforce, officers and directors regardless of position or tenure. The Code 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. The Code also makes clear when and how individuals should raise concerns and documents our no-retaliation policy. We enforce our policies and requirements with appropriate disciplinary actions, when necessary, and we take a zero-tolerance approach to any violation of law or policy.

The company reinforces compliance with the Code's expectations through training that we provide to our employees. On an annual basis, employees are expected to receive and acknowledge understanding of the Code. Additionally, all full-time, U.S.-based employees must acknowledge our anti-harassment and anti-discrimination policy and complete the company-offered training within the first several months of employment. All U.S. employees, including part-time and temporary employees, must take a harassment prevention training course every year.

In 2022:

- **100%** of full-time employees provided written or digital acknowledgment of the Global Code of Conduct.
- **100%** of full-time employees have received training on the Global Code of Conduct within the last three years.

Ethical Treatment of Animals

Ultragenyx is committed to the ethical treatment of animals used in the development of potential new and life-changing therapies for patients with rare disease. Our company is committed to the "3Rs":

- **Replace** – Use non-animal methods for experiments whenever possible, such as simulations and computational tools or in vitro systems.
- **Reduce** – Use the minimum number of animals in each study in order to achieve valid results and objectives.
- **Refine** – Use procedures that decrease the potential for pain and limit distress for animals.

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

Compliance Program

We maintain a high compliance culture by demanding ethical behavior, holding each individual accountable for compliance, fostering effective communication and working together to make good decisions.

We comply with applicable laws and regulations while maintaining patient safety and leadership accountability. Our compliance program has been developed in accordance with the laws applicable to our industry, the Program Guidance for Pharmaceutical Manufacturers published by the Office of the Inspector General of the U.S. Department of Health and Human Services, and the PhRMA Code of Interactions with Healthcare Professionals.

Ultragenyx's compliance program includes:

A compliance officer and compliance committee responsible for developing, operating and monitoring the compliance program and with authority to report directly to the board and our CEO.

Written standards of conduct, policies and practices that document the company's commitment to compliance and requirements to strictly follow fraud and abuse laws.

Education and training programs that are easy to understand, effective and readily available for all employees.

Open lines of communication and partnership between the compliance officer and workforce to ethically achieve our company mission and goals.

An audit and monitoring program that is continually enhanced to identify and address risks.

Enforcement of compliance obligations through guidelines that include disciplinary action for noncompliance.

Mechanisms to investigate and respond promptly and properly to reports of noncompliance, including processes to initiate corrective measures.

Compliance Hotline

Ultragenyx's compliance hotline, which also serves as the Confidential and Anonymous Financial Concern Hotline, allows employees or anyone else to report any potential or actual violations of Ultragenyx's Global Code of Conduct, company policies and procedures, and applicable laws and regulations. Any employee can provide comments using the hotline. Messages may be submitted anonymously using a secure web form, email or telephone.

Complaints or other messages left on the compliance hotline are anonymously sent to our chief legal officer and the chairperson of our Audit Committee, who then take the necessary next steps. All hotline reports are required to be promptly handled and any identified issues are required to be addressed. If an employee makes a complaint of discrimination or harassment, regardless of where the complaint is made, Ultragenyx's policy is to conduct a timely and thorough investigation and take appropriate action. Investigations are required to be assessed and conducted based on Ultragenyx's internal investigations protocol and are required to be conducted by the appropriate personnel depending on the issue. If and when issues arise, we are required to identify root causes and, in a timely and efficient manner, implement measures to stop repeat occurrences. In 2022, all reported complaints related to potential breaches to our Code of Conduct and incidents of discrimination or harassment were investigated and promptly addressed.

Interactions with Patients, Caregivers & Healthcare Professionals

We respect the doctor-patient relationship and the privacy rights of patients. We strive to interact with patients and caregivers in an appropriate manner and in compliance with applicable laws, regulations and our internal healthcare compliance manual. Ultragenyx has adopted policies and practices consistent with the PhRMA Code and other applicable industry standards that govern interactions with healthcare professionals. These policies include:

- Support for medical education as well as collaboration with healthcare professionals to provide services to the company as researchers, consultants and speakers.
- Provision of business courtesies.
- Providing grants and charitable contributions so that such funds are not conditioned, expressly or implied, on any agreement to prescribe, purchase, recommend, influence or provide favorable formulary status for any Ultragenyx product.
- Promotion of Ultragenyx products in compliance with the FDA's regulatory framework as well as regulatory requirements in other jurisdictions regarding promotion of pharmaceutical products.

Our compliance program is overseen by a management-level compliance committee that provides guidance with respect to healthcare law compliance. The committee meets at least quarterly.

In 2022:

Ultragenyx's Healthcare Compliance Manual was translated into **Spanish, Portuguese, French and German**. The manual will be translated into additional languages in the future.

Intellectual Property

We rely on patent protection, trade secrets, know-how and continuing innovation to develop and maintain our competitive position.

Our policy is not to tolerate any unlawful use or activity that violates the intellectual property rights of others, as highlighted in the Ultragenyx Global Code of Conduct. We also expect suppliers with which we conduct business to respect the intellectual property rights of others.

Data Protection & Privacy

We are committed to compliance with all global privacy laws and maintain a privacy program, including a global privacy policy, comprehensive training, and system operating procedures and controls.

We expect our workforce to be accountable, to protect personal data – which we may acquire or maintain during the ordinary course of our business operations – and to process such data responsibly in accordance with company policy and any applicable laws.

In 2022:

- Ultragenyx did not have any material data privacy breaches.
- 100% of employees received data protection, cybersecurity and social media training.

Cybersecurity

Ultragenyx has an information security program with policies and procedures to guide our security and data protection decision-making process. We regularly update our systems in an effort to quickly remediate any potential vulnerabilities. We also purchase a fixed amount of cybersecurity and crime insurance coverage to help mitigate some of the risk and potential liability from cybersecurity breaches.

We provide information security training to employees 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 conduct phishing exercises three times per year, focusing on users with repeated simulation failures and implementing corrective actions. We also perform an annual internal and external full penetration test. In 2022, we conducted an internal cloud security audit and remediated all critical findings.

We have consolidated information security and cyber resiliency activities under the leadership of the senior director of information security and chief information officer (CIO). The CIO regularly reviews the company's cybersecurity program and risks, processes and procedures with the board's Audit Committee.

In 2022, we mapped and inventoried our systems in a central asset management tool and developed more robust operating procedures. We also deployed cloud security, endpoint data loss prevention and privileged access management solutions.

We have also begun updating our disaster recovery plans for our information technology systems.

Responsible Procurement

To complement and supplement the Global Code of Conduct, we released the Global Standard for Suppliers in early 2022.

The Standard applies to all suppliers, manufacturers, distributors, vendors, contractors, subcontractors, agents, consultants and providers of goods and services and their employees, or anyone working on behalf of Ultragenyx (collectively referred to as Suppliers), and includes expectations of Suppliers on the topics of integrity and compliance with laws, fair marketing and sales practices, conflicts of interest, labor standards (including forced employment, slavery and human trafficking), human rights, environmental stewardship, global pandemic preparedness and community involvement.

Developing strategic, long-lasting and mutually beneficial relationships with Suppliers is critical to our company's success. We seek to engage with Suppliers that share our values and our commitment to ethics and compliance. Before engaging a new Supplier, we screen for various legal and compliance risks in accordance with company policy. Before payment is issued, we confirm that all third parties are not on watchlists maintained by the U.S. Department of Treasury's Office of Foreign Assets Control (OFAC). We conduct appropriate due diligence on new Suppliers, which includes screening for economic sanctions, anti-bribery and anti-corruption activity, and other review and analysis.

In 2022, we automated certain elements of the vendor approval process, aiming to make it more efficient. We also upgraded our enhanced supplier quality management system. Our teams spent months conducting requirements gathering sessions, collaborative design workshops and iterative configuration cycles in order to monitor more effectively the quality of our suppliers and to support future integrations.

Also in 2022, we became a member of Rx-360 (The International Pharmaceutical Supply Chain Consortium), a nonprofit international consortium that seeks to protect patient safety by sharing information and developing processes related to the integrity of the healthcare supply chain and the quality of its material. Ultragenyx participates in the Rx-360 Joint Audit Program. In 2022, Ultragenyx licensed nine audit reports to satisfy the audit requirements for 10 supplier sites (three in the U.S. and seven internationally).

Rx-360 facilitates and manages the complete life cycle of the audit to help support a seamless experience and a consistent result. Audit checklists are based on accepted international standards, including ISO standards, such as those on active pharmaceutical ingredients (APIs), excipients, basic chemicals/raw materials, primary packaging and supply chain security. In addition to participating in the Joint Audit Program, Ultragenyx personnel have also participated in the Data Integrity and Cell and Gene Therapy Working Groups.

SASB Index

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

ACTIVITY METRIC	CODE	RESPONSE / REFERENCE / REPORT LOCATION
Number of patients treated	HC-BP-000.A	>3,200. See our 2022 ESG Report, About Us.
Number of drugs 1) in portfolio and 2) in research and development (Phases 1-3)	HC-BP-000.B	4 drugs in portfolio; 7 drugs in development Phases 1-3. See our current Pipeline.

TOPIC	ACCOUNTING METRIC	CODE	RESPONSE / REFERENCE / REPORT LOCATION
Safety of Clinical Trial Participants	Discussion, by world region, of management process for ensuring quality and patient safety during clinical trials	HC-BP-210a.1	See our 2022 ESG Report, Quality and Safety.
	Number of FDA Sponsor Inspections related to clinical trial management and pharmacovigilance that resulted in: (1) Voluntary Action Indicated (VAI) and (2) Official Action Indicated (OAI)	HC-BP-210a.2	None
	Total amount of monetary losses as a result of legal proceedings associated with clinical trials in developing countries	HC-BP-210a.3	None
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	While not listed as a company in scope for the 2022 Access to Medicine Index, we are committed to increasing access to their medicines and pricing responsibly. Our commercial products and clinical pipeline do not cover any priority countries or diseases listed in the Access to Medicine Index. For more information, see our 2022 ESG Report, Access & Affordability.
	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 does not have any products that qualify for the WHO List of Prequalified Medicinal Products at this time.

TOPIC	ACCOUNTING METRIC	CODE	RESPONSE / REFERENCE / REPORT LOCATION
Affordability & Pricing	Number of settlements of Abbreviated New Drug Application (ANDA) litigation that involved payments and/or provisions to delay bringing an authorized generic product to market for a defined time period	HC-BP-240b.1	This metric does not currently apply to Ultragenyx's business.
	Percentage change in: (1) average list price and (2) average net price across U.S. product portfolio compared to previous year	HC-BP-240b.2	See our 2022 ESG Report, Access & Affordability.
	Percentage change in: (1) list price and (2) net price of product with largest increase compared to previous year	HC-BP-240b.3	See our 2022 ESG Report, Access & Affordability.
Drug Safety	List of products listed in the Food and Drug Administration's (FDA) MedWatch Safety Alerts for Human Medical Products database	HC-BP-250a.1	Our therapies are not listed in the FDA MedWatch. Please visit the FDA FAERS MedWatch website for more information.
	Number of fatalities associated with products as reported in the FDA Adverse Event Reporting System	HC-BP-250a.2	None. Please visit the FDA FAERS MedWatch website for more information.
	Number of recalls issued, total units recalled	HC-BP-250a.3	See our 2022 ESG Report, Access & Affordability.
	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 (a) expire before use and are returned, (b) are found to be unsuitable for release or (c) are subject to a recall or withdrawal notice, Ultragenyx does not reintroduce them again for reuse. They get disposed of using regulated and monitored incineration processes.
	Number of FDA enforcement actions taken in response to violations of current Good Manufacturing Practices (cGMP), by type	HC-BP-250a.5	None
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 2022 ESG Report, Safety, section on Counterfeit Drugs.
	Discussion of process for alerting customers and business partners of potential or known risks associated with counterfeit products	HC-BP-260a.2	Ultragenyx has implemented a comprehensive process designed to address counterfeit product risks, including a Field Action procedure and security features like tamper-evident seals and serialization of product labeling. See our 2022 ESG Report, Safety, section on Counterfeit Drugs.
	Number of actions that led to raids, seizure, arrests, and/or filing of criminal charges related to counterfeit products	HC-BP-260a.3	None. See our 2022 ESG Report, Safety, section on Counterfeit Drugs.

TOPIC	ACCOUNTING METRIC	CODE	RESPONSE / REFERENCE / REPORT LOCATION
Ethical Marketing	Total amount of monetary losses as a result of legal proceedings associated with false marketing claims	HC-BP-270a.1	None. For more information, see our 2022 Annual Report.
	Description of code of ethics governing promotion of off-label use of products	HC-BP-270a.2	More information can be found in 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 2022 ESG Report, People.
	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	For information on turnover rates, see our 2022 ESG Report, Human Capital Development.
Supply Chain Management	Percentage of (1) entity's facilities and (2) Tier I suppliers' facilities participating in the Rx-360 International Pharmaceutical Supply Chain Consortium audit program or equivalent third-party audit programs for integrity of supply chain and ingredients	HC-BP-430a.1	See our 2022 ESG Report, Responsible Procurement.
Business Ethics	Total amount of monetary losses as a result of legal proceedings associated with corruption and bribery	HC-BP-510a.1	None. See our 2022 ESG Report, Ethics & Integrity.
	Description of code of ethics governing interactions with health care professionals	HC-BP-510a.2	See our 2022 ESG Report, Interactions with Patients, Caregivers & Healthcare Professionals.

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

GRI Index

Statement of use	Ultragenyx has reported the information cited in this GRI content index for the period January 1 to December 31, 2022, with reference to the GRI Standards listed below.
GRI 1 used	GRI 1: Foundation 2021

GRI STANDARD	DISCLOSURE	2022 LOCATION
GRI 2: General Disclosures 2021	2-1 Organizational details	2022 ESG Report, About Us 2022 Annual Report
	2-2 Entities included in the organization's sustainability reporting	2022 ESG Report, About This Report
	2-3 Reporting period, frequency and contact point	2022 ESG 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	2022 Annual Report, Item 1 2022 ESG Report, About Us
	2-7 Employees	2022 ESG Report, Inclusion & Diversity
	2-9 Governance structure and composition	2022 ESG Report, ESG Governance 2022 ESG Report, Corporate Governance
	2-10 Nomination and selection of the highest governance body	2023 Proxy, Nomination of Directors
	2-11 Chair of the highest governance body	Daniel G. Welch, Chairperson of the Board
	2-12 Role of the highest governance body in overseeing the management of impacts	2022 ESG Report, ESG Governance
	2-13 Delegation of responsibility for managing impacts	2022 ESG Report, ESG Governance

GRI STANDARD	DISCLOSURE	2022 LOCATION
GRI 2: General Disclosures 2021	2-19 Remuneration policies	2023 Proxy, Executive Compensation 2023 Proxy, Director Compensation
	2-20 Process to determine remuneration	2023 Proxy, Executive Compensation 2023 Proxy, Director Compensation
	2-21 Annual total compensation ratio	2023 Proxy, CEO Pay Ratio
	2-22 Statement on sustainable development strategy	2022 ESG Report, Letter From Our CEO
	2-23 Policy commitments	Global Code of Conduct Global Standard for Suppliers
	2-24 Embedding policy commitments	2022 ESG Report, Ethics & Integrity 2022 ESG Report, Responsible Procurement
	2-26 Mechanisms for seeking advice and raising concerns	2022 ESG Report, Compliance Program
	2-27 Compliance with laws and regulations	2022 ESG Report, Compliance Program 2022 Annual Report, Item 3 Legal Proceedings
	2-28 Membership associations	2022 ESG Report, Industry Participation 2022 ESG Report, Patient Advocacy & Engagement
GRI 3: Material Topics 2021	3-1 Process to determine material topics	2022 ESG Report, Materiality Analysis
	3-2 List of material topics	2022 ESG Report, Materiality Analysis
	3-3 Management of material topics	The 2022 ESG Report describes the management of material topics by section.

GRI STANDARD	DISCLOSURE	2022 LOCATION
GRI 201: Economic Performance 2016	201-1 Direct economic value generated and distributed	2022 Annual Report, Item 8 Financial Statement
GRI 205: Anti-corruption 2016	205-1 Operations assessed for risks related to corruption	2022 ESG Report, Responsible Procurement
	205-2 Communication and training about anti-corruption policies and procedures	2022 ESG Report, Ethics & Integrity
	205-3 Confirmed incidents of corruption and actions taken	2022 ESG Report, Ethics & Integrity
GRI 206: Anti-competitive Behavior 2016	206-1 Legal actions for anti-competitive behavior, anti-trust, and monopoly practices	2022 Annual Report, Item 3 Legal Proceedings
GRI 303: Water and Effluents 2018	303-1 Interactions with water as a shared resource	2022 ESG Report, Managing Waste & Water
GRI 305: Emissions 2016	305-5 Reduction of GHG emissions	2022 ESG Report, Minimizing GHG Emissions
GRI 306: Waste 2020	306-2 Management of significant waste-related impacts	2022 ESG Report, Managing Waste & Water
GRI 401: Employment 2016	401-1 New employee hires and employee turnover	2022 ESG Report, Human Capital Development
	401-2 Benefits provided to full-time employees that are not provided to temporary or part-time employees	2022 ESG Report, Employee Compensation & Benefits Our Benefits
	401-3 Parental leave	2022 ESG Report, Employee Compensation & Benefits

GRI STANDARD	DISCLOSURE	2022 LOCATION
GRI 403: Occupational Health and Safety 2018	403-1 Occupational health and safety management system	2022 ESG Report, Occupational Health, Safety & Wellness
	403-2 Hazard identification, risk assessment, and incident investigation	2022 ESG Report, Occupational Health, Safety & Wellness
	403-4 Worker participation, consultation, and communication on occupational health and safety	2022 ESG Report, Occupational Health, Safety & Wellness
	403-5 Worker training on occupational health and safety	2022 ESG Report, Occupational Health, Safety & Wellness
	403-6 Promotion of worker health	2022 ESG Report, Wellness
	403-9 Work-related injuries	2022 ESG Report, Occupational Health, Safety & Wellness
GRI 404: Training and Education 2016	404-1 Average hours of training per year per employee	2022 ESG Report, Employee Learning
	404-2 Programs for upgrading employee skills and transition assistance programs	2022 ESG Report, Employee Learning 2022 ESG Report, Career Development
	404-3 Percentage of employees receiving regular performance and career development reviews	2022 ESG Report, Human Capital Development

GRI STANDARD	DISCLOSURE	2022 LOCATION
GRI 405: Diversity and Equal Opportunity 2016	405-1 Diversity of governance bodies and employees	2022 ESG Report, Inclusion & Diversity (overall diversity data) 2022 ESG Report, Board Diversity (board diversity)
GRI 406: Non-discrimination 2016	406-1 Incidents of discrimination and corrective actions taken	2022 ESG Report, Compliance Hotline
GRI 413: Local Communities 2016	413-1 Operations with local community engagement, impact assessments, and development programs	2022 ESG Report, Communities Includes information on local community engagement, including corporate philanthropy and volunteering.
GRI 416: Customer Health and Safety 2016	416-1 Assessment of the health and safety impacts of product and service categories	2022 ESG Report, Safety
	416-2 Incidents of non-compliance concerning the health and safety impacts of products and services	2022 ESG Report, Safety
GRI 418: Customer Privacy 2016	418-1 Substantiated complaints concerning breaches of customer privacy and losses of customer data	2022 ESG Report, Data Protection & Privacy