



Ultragenyx Underscores Continued Commitment to Rare Disease Innovation with 2024 Impact Report

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NOVATO, Calif., May 14, 2025 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) released its 2024 Impact Report today, which covers the company's approach to corporate responsibility. The report highlights efforts to transform the lives of individuals and families affected by rare diseases, empower its employees, and make a meaningful impact on the communities it operates within and serves.

"Since founding Ultragenyx 15 years ago, I've sought to build a next-generation rare disease company that has a meaningful and sustained impact on the entire rare disease community," said Emil Kakkis, M.D., Ph.D., founder, president, and chief executive officer of Ultragenyx. "Guided by the principle of doing the right thing, we have engineered our own dynamic approach to drug development while working on broader education and policy initiatives that meaningfully support diagnosis, drug development, regulatory pathways, and clinical care. The sustainability and social impact implications of our actions and decisions are consistently considered and reflected in our 2024 Impact Report."

The company's approach to corporate responsibility is structured around six key pillars—**Innovation, Patients, People, Communities, Planet, and Governance**—aligned with its vision to lead the future of rare disease medicine and its mission to transform the lives of people with rare diseases. The 2024 Ultragenyx Impact Report features updates for all six pillars, as well as Sustainability Accounting Standards Board (SASB) and Global Reporting Initiative (GRI) indices. For the full report and more on Ultragenyx's Corporate Responsibility efforts, visit <https://www.ultragenyx.com/ultra-committed/corporate-responsibility/>.

Highlights from the company's 2024 report include:

Innovation:

Ultragenyx is committed to developing and delivering transformative treatments where none exist.

- Successfully transferred the manufacturing process of our DTX401 gene therapy to our Gene Therapy Manufacturing Facility (GTMF) in Bedford, Massachusetts
- Advanced several key clinical programs to treat rare and ultra-rare diseases, including osteogenesis imperfecta (OI), Angelman syndrome, Sanfilippo syndrome type A (MPS IIIA), and glycogen storage disease type Ia (GSDIa)
- Supported more than 80 clinical and non-clinical investigator-sponsored trials (ISTs) globally

Patients:

Ultragenyx is 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.

- Supported more than 650 patients in 50 countries in gaining access to Ultragenyx treatments through various global expanded access and patient assistance programs since 2013
- Successfully advocated for the passage of newborn screening legislation in two more U.S. states
- Hosted the ninth and tenth Rare Bootcamp to help patient families, foundations, and organizations seeking to develop novel treatments for rare diseases

People:

Ultragenyx is committed to maintaining an inclusive, safe, and healthy environment. The company is also committed to fair and equitable compensation practices that are transparent and free from bias.

- Launched the Executive Edge program to support the development of leadership skills at the vice president (VP) and senior VP levels
- Maintained a high engagement score of 88% in our employee engagement survey

Communities:

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

- Approved more than \$3 million in charitable donations, independent medical education, and health-related grants across more than 20 countries
- Hosted 2nd Annual Ultragenyx Global Days of Service, with employees volunteering in nearly 60 service projects

Planet:

Ultragenyx is committed to implementing an environmental strategy that helps minimize its environmental footprint across its business.

- Purchased 100% renewable electricity for our corporate headquarters campus in Novato, Calif.
- Achieved My Green Lab Green certification at our Translational Sciences lab in Novato, Calif.

Governance:

Ultragenyx is committed to strong corporate governance, ethics and integrity, compliance, data protection and security, and responsible procurement.

- More than 90% of responders to the annual compliance culture survey stated that the annual compliance training is adequate for them to confidently execute their responsibilities
- Maintained a formal Global Human Rights policy to hold ourselves and our vendors to its standards

Ultragenyx Receives External Recognition in 2024

Ultragenyx's achievements in 2024 were consistently recognized through several prestigious awards.

In 2024, Ultragenyx was named one of the 100 Most Sustainable Companies by *Barron's* and one of the Top Places to Work in the USA for the second consecutive year, receiving six Cultural Excellence Awards from Top Workplaces. The company also received accolades from *The Boston Globe* and the *San Francisco Chronicle*. Additionally, the company was honored with the MassEcon Gold Award for Economic Impact in the Northeast region, celebrating its significant contributions to the Massachusetts economy, and was recognized as one of the Best Places for Working Parents in Massachusetts.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at: www.ultragenyx.com.

Forward-Looking Statements and Use of Digital Media

This press release and the 2024 Ultragenyx Impact Report and other materials cross-referenced in this press release contain statements that are aspirational or reflective of our views about the company's 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 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 the company believes 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, and certifications, representations or data reviewed or provided by third parties. 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 7, 2025, and its subsequent periodic reports filed with the SEC. Forward-looking statements are aspirational and are not guarantees or promises that goals or targets will be met. Ultragenyx undertakes no obligation to update any forward-looking or other statements, whether as a result of new information, future events, or otherwise, and notwithstanding any historical practice of doing so. Ultragenyx may determine to adjust any goals and targets or establish new ones to reflect changes in its business. The information included in, and any issues identified as material for purposes of, the 2024 Ultragenyx Impact Report is not an indication that they are considered material to Ultragenyx, its investors or other stakeholders, or required to be disclosed in the company's filings, in each case under SEC reporting or any other laws or requirements that may apply to the company. In the context of this report, the term "material" is distinct from, and should not be confused with, such term as defined for SEC or other mandatory reporting purposes.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (<https://ir.ultragenyx.com/>) and LinkedIn website (<https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/>).

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