# Ultragenyx Reports Fourth Quarter and Full Year 2024 Financial Results and Corporate Update

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*2024 Total Revenue of $560 million, exceeding guidance*
*Crysvita® revenue of $410 million and Dojolvi® revenue of $88 million*

*2025 Financial Guidance: Total Revenue between $640 million to $670 million, Crysvita revenue of $460 million to $480 million, and Dojolvi revenue of $90 million to $100 million*

NOVATO, Calif., Feb. 13, 2025 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultrarare genetic diseases, today reported its financial results for the quarter and full year ended December 31, 2024 and shared financial guidance for 2025.

“We have created a next-generation rare disease company on a pathway to profitability with meaningful revenue growth from multiple global products and a series of potential new drug approvals,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “The major regulatory and clinical catalysts ahead of us this year are the pending PDUFA decision for our gene therapy to treat Sanfilippo syndrome, submission of our second gene therapy biologics license application in Glycogen Storage Disease Type Ia, readout of our pivotal Phase 3 results in osteogenesis imperfecta, and completion of enrollment in our Phase 3 trial in Angelman syndrome."

**Fourth Quarter and Full Year 2024 Selected Financial Data Tables and Financial Results**

*Revenues (dollars in thousands), (unaudited)*

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| Crysvita | | | | |
| Product sales | $ 22,415 | $ 18,379 | $ 134,709 | $ 75,697 |
| Revenue in Profit-Share Territory | 85,534 | 70,124 | 248,966 | 231,574 |
| Royalty revenue in European Territory | 7,473 | 5,612 | 25,849 | 20,783 |
| Total Crysvita Revenue | 115,422 | 94,115 | 409,524 | 328,054 |
| Dojolvi | 31,103 | 23,286 | 88,194 | 70,633 |
| Evkeeza | 10,374 | 2,102 | 32,162 | 3,642 |
| Mepsevii | 7,978 | 7,889 | 30,350 | 30,441 |
| Other | — | — | — | 1,479 |
| Total revenues | $ 164,877 | $ 127,392 | $ 560,230 | $ 434,249 |

*Total Revenues*
Ultragenyx reported $165 million in total revenue for the fourth quarter of 2024, which represents 29% growth compared to the same period in 2023. Fourth quarter 2024 Crysvita revenue was $115 million, which represents 23% growth compared to the same period in 2023. This includes product sales of $22 million from Latin America and Turkey, which represents 22% growth compared to the same period in 2023. Dojolvi revenue in the fourth quarter 2024 was $31 million, which represents 34% growth compared to the same period in 2023. Evkeeza revenue in the fourth quarter 2024 was $10 million.

Total revenue for the year ended December 31, 2024 was $560 million, which represents 29% growth compared to the prior year. Full year 2024 Crysvita revenue was $410 million, which represents 25% growth compared to the prior year. This includes product sales of $135 million from Latin America and Turkey, which represents 78% growth compared to the prior year. Dojolvi revenue in 2024 was $88 million, which represents 25% growth compared to the prior year. Evkeeza revenue in 2024 was $32 million, as demand continues to build following launches in the company’s territories outside of the United States.

*Selected Financial Data (dollars in thousands, except per share amounts), (unaudited)*

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| Total revenues | $ 164,877 | $ 127,392 | $ 560,230 | $ 434,249 |
| Operating expenses: | | | | |
| Cost of sales | 16,894 | 12,051 | 76,728 | 45,209 |
| Research and development | 187,766 | 160,557 | 697,865 | 648,449 |
| Selling, general and administrative | 82,495 | 76,833 | 321,610 | 309,799 |
| Total operating expenses | 287,155 | 249,441 | 1,096,203 | 1,003,457 |
| Net loss | $ (133,385) | $ (123,190) | $ (569,183) | $ (606,639) |
| Net loss per share, basic and diluted | $ (1.39) | $ (1.52) | $ (6.29) | $ (8.25) |

*Operating Expenses*

Total operating expenses for the fourth quarter of 2024 were $287 million, including non-cash stock-based compensation of $40 million. Total operating expenses for the year ended December 31, 2024 were $1,096 million, including $158 million of non-cash stock-based compensation.

*Net Loss*
For the fourth quarter of 2024, Ultragenyx reported net loss of $133 million, or $1.39 per share basic and diluted, compared with a net loss for the fourth quarter of 2023 of $123 million, or $1.52 per share basic and diluted. For the year ended December 31, 2024, Ultragenyx reported net loss of $569 million, or $6.29 per share basic and diluted, compared with a net loss the prior year of $607 million, or $8.25 per share, basic and diluted.

*Cash Balance and Net Cash Used in Operations*
Cash, cash equivalents, and marketable debt securities were $745 million as of December 31, 2024. Net cash used in operations for the year ended December 31, 2024 was $414 million.

**2025 Financial Guidance**
Revenues are expected to grow approximately 14-20% compared to 2024. The company will continue to prioritize expense management, focusing its investments on the execution of multiple upcoming commercial launches and advancing multiple Phase 3 programs. Together, this is expected to lead to a reduction in 2025 net cash used in operations compared to 2024.

For the full year 2025:

- Total revenue to be in the range of $640 million to $670 million
- Crysvita revenue to be in the range of $460 million to $480 million
- Dojolvi revenue to be in the range of $90 million to $100 million

**Recent Updates and Clinical Milestones**

***UX143 (setrusumab) monoclonal antibody for osteogenesis imperfecta (OI): Phase 3 Orbit study progressing to second interim analysis (IA2) expected in mid-2025***

Patients continue dosing in the ongoing Phase 3 *Orbit* and *Cosmic* clinical trials, which evaluate setrusumab in pediatric and young adult patients with OI. The randomized, placebo-controlled Phase 3 portion of the *Orbit* study is progressing towards the second interim analysis in mid-2025 or a final analysis in the fourth quarter 2025. Conduct of the study is going well and patient safety in the Phase 3 is consistent with the Phase 2. Patients in the *Cosmic* study also are continuing to be treated with either setrusumab or intravenous bisphosphonates (IV-BP) therapy and will be evaluated in parallel with the *Orbit* interim and final analyses.

***GTX-102 an antisense oligonucleotide for Angelman syndrome: Phase 3 study enrolling; expect enrollment completion in second half of 2025***

Enrollment in the global Phase 3 *Aspire* study began in December 2024 and is expected to enroll approximately 120 children ages four to 17 with Angelman syndrome with a genetically confirmed diagnosis of full maternal *UBE3A* gene deletion. Participants will be randomized 1:1 to receive GTX-102 by intrathecal injection via lumbar puncture or to the sham comparator group during the 48-week primary efficacy analysis period. The primary endpoint will be improvement in cognition assessed by Bayley-4 cognitive raw score, and the key secondary endpoint (with a 10% allocation of alpha) will be the Multi-domain Responder Index (MDRI) across the five domains of cognition, receptive communication, behavior, gross motor function, and sleep. Enrollment in the Phase 3 *Aspire* study is expected to complete in the second half of 2025.

The Phase 2/3 *Aurora* study, which will evaluate GTX-102 in other Angelman syndrome genotypes and ages, is expected to initiate in 2025.

***UX111 AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA): Biologics license application (BLA) submitted; expect Prescription Drug User Fee Act (PDUFA) decision on the application and potential launch in second half of 2025***

In December 2024, Ultragenyx submitted a BLA to the U.S. Food and Drug Administration for UX111 supported by the available data, including from the ongoing pivotal *Transpher A* study. New clinical data were recently presented at *WORLDSymposium™ 2025*that demonstrated treatment with UX111 led to a statistically significant improvement in the Bayley-III raw scores for the subdomains of cognition, receptive communication and expressive communication in patients with MPS IIIA compared to natural history data from untreated patients. These clinical endpoints were correlated with substantial and sustained reduction in levels of heparan sulfate in cerebrospinal fluid. A PDUFA decision and potential launch are expected in the second half of 2025.

***DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): BLA filing expected in mid-2025***

Ultragenyx previously announced positive topline results from the Phase 3 *GlucoGene* study for the treatment of patients aged eight years and older. The study achieved its primary endpoint, demonstrating that treatment with DTX401 resulted in a statistically significant and clinically meaningful reduction in daily cornstarch intake compared with placebo at Week 48.

After the 48-week primary efficacy analysis period, crossover patients (previously treated with placebo) were eligible to receive DTX401. These patients were able to titrate cornstarch much more rapidly once they were confirmed to have been treated and had timely direct access to their glucose levels. Patients from the original DTX401 treatment arm who have reached 78 weeks also continued to reduce their daily cornstarch intake, while maintaining glycemic control. DTX401 has demonstrated a consistent and acceptable safety profile with no new safety concerns identified as of the data cut-off.

These results have been discussed with regulatory authorities in a pre-BLA meeting and will be included as part of a BLA submission in mid-2025.

***DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Enrollment in Phase 3 study complete***

Enrollment in the Phase 3 *Enh3ance* study has been completed with a total of 37 patients. The pivotal study enrolled participants 12 years of age and older, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by change in 24-hour ammonia levels and removal of ammonia-scavenger medications and protein-restricted diet. Based on the recently amended protocol, the change in 24-hour ammonia levels will be measured through Week 36 after which the study would unblind and patients will be followed for a total of up to 64 weeks to determine the complete responders able to remove safely both ammonia-scavenger medications and protein-restricted diet control.

***UX701 AAV gene therapy for Wilson Disease: Phase 1/2/3 study ongoing; expect Cohort 4 enrollment completion in second half of 2025***

In Stage 1 of the Phase 1/2/3 *Cyprus2+* study, 15 patients across three sequential dose cohorts were enrolled and demonstrated clinical activity as well as improvements in copper metabolism. Multiple responders completely tapered off their standard-of-care treatment with responses seen in all

three dose cohorts.

The company expects to enroll a fourth cohort in Stage 1 at a moderately increased dose and with an optimized immunomodulation regimen to enhance the efficiency and efficacy of the gene therapy, with the objective of having the majority of patients come off standard-of-care treatment before selecting a dose for the randomized placebo-controlled stage of the study. Enrollment in Cohort 4 is expected to complete in the second half of 2025.

**Conference Call and Webcast Information**

Ultragenyx will host a conference call today, Thursday, February 13, 2025, at 2 p.m. PT/5 p.m. ET to discuss the fourth quarter and full year 2024 financial results and provide a corporate update. The live and replayed webcast of the call will be available through the company's website at https://ir.ultragenyx.com/events-presentations. The replay of the call will be available for three months.

**About Ultragenyx**

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultrarare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, 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**

*Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company's clinical development programs, commercial success of its products and product candidates, continued collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, risks related to serious or undesirable side effects of our product candidates, the company's ability to achieve its projected development goals in its expected timeframes, risks related to reliance on third party partners to conduct certain activities on the company's behalf, our limited experience in generating revenue from product sales, risks related to product liability lawsuits, our dependence on Kyowa Kirin for the commercial supply of Crysvita, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, the transition back to Kyowa Kirin of our exclusive rights to promote Crysvita in the United States and Canada and unexpected costs, delays, difficulties or adverse impact to revenue related to such transition, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and drug candidate. Ultragenyx undertakes no obligation to update or revise any forward-looking statements.*

*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 November 6, 2024, and its subsequent periodic reports filed with the SEC.*

*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-/).*

**Ultragenyx Pharmaceutical Inc.**
**Selected Statement of Operations Financial Data**
**(in thousands, except share and per share amounts)**
**(unaudited)**

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | **2024** | **2023** | **2024** | **2023** |
| **Statement of Operations Data:** | | | | |
| Revenues: | | | | |
| Product sales | $ 71,870 | $ 51,656 | $ 285,415 | $ 180,413 |
| Royalty revenue | 93,007 | 75,736 | 274,815 | 182,652 |
| Collaboration and license | — | — | — | 71,184 |
| Total revenues | 164,877 | 127,392 | 560,230 | 434,249 |
| Operating expenses: | | | | |
| Cost of sales | 16,894 | 12,051 | 76,728 | 45,209 |
| Research and development | 187,766 | 160,557 | 697,865 | 648,449 |
| Selling, general and administrative | 82,495 | 76,833 | 321,610 | 309,799 |
| Total operating expenses | 287,155 | 249,441 | 1,096,203 | 1,003,457 |
| Loss from operations | (122,278) | (122,049) | (535,973) | (569,208) |
| Change in fair value of equity investments | (1,548) | 1,889 | (1,115) | 397 |
| Non-cash interest expense on liabilities for sales of future royalties | (15,522) | (17,328) | (63,041) | (66,004) |

| | | | | |
|---|---|---|---|---|
| Other income, net | 5,944 | 10,596 | 32,543 | 26,351 |
| Loss before income taxes | (133,404) | (126,892) | (567,586) | (608,464) |
| Benefit from (provision for) income taxes | 19 | 3,702 | (1,597) | 1,825 |
| Net loss | $ (133,385) | $ (123,190) | $ (569,183) | $ (606,639) |
| Net loss per share, basic and diluted | $ (1.39) | $ (1.52) | $ (6.29) | $ (8.25) |
| Shares used in computing net loss per share, basic and diluted | 95,681,451 | 81,118,873 | 90,538,118 | 73,543,862 |

**Ultragenyx Pharmaceutical Inc.**
**Selected Activity included in Operating Expense**
**(in thousands)**
**(unaudited)**

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2024 | 2023 | 2024 | 2023 |
| Non-cash stock based compensation | $ 40,190 | $ 33,744 | $ 158,056 | $ 135,213 |
| GTX-102 clinical milestone | $ 30,000 | — | $ 30,000 | — |
| UX143 clinical milestone | — | — | — | $ 9,000 |

**Ultragenyx Pharmaceutical Inc.**
**Selected Balance Sheet Financial Data**
**(in thousands)**
**(unaudited)**

| | December 31, 2024 | December 31, 2023 |
|---|---|---|
| **Balance Sheet Data:** | | |
| Cash, cash equivalents, and marketable debt securities | $ 745,029 | $ 777,110 |
| Working capital | 472,970 | 451,747 |
| Total assets | 1,503,456 | 1,491,013 |
| Total stockholders' equity | 255,297 | 275,414 |

**Contacts Ultragenyx Pharmaceutical Inc.**
**Investors**
Joshua Higa
ir@ultragenyx.com