Ultragenyx Reports First Quarter 2025 Financial Results and Corporate Update

May 6, 2025

First quarter total revenue of $139 million, Crysvita® revenue of $103 million and Dojolvi® revenue of $17 million

Reaffirm 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

UX111, an investigational treatment for Sanfilippo syndrome, biologics license application (BLA) on track for PDUFA action date of August 18, 2025

NOVATO, Calif., May 06, 2025 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today reported its financial results for the quarter ended March 31, 2025 and reaffirmed its financial guidance for 2025.

"In the first quarter, our commercial team continued expanding our base of revenue around the world, while we also continued to make successful progress for our next potential launch with the review of our first gene therapy BLA for the treatment of Sanfilippo syndrome (MPS IIIA)," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "Patients in the UX143 Phase 3 *Orbit* and *Cosmic* studies have now been on therapy for at least 12 months and will support the interim analyses in mid-2025. We continue to hear encouraging feedback from investigators in the Phase 2 portion of *Orbit*, some with patients on therapy for over 2 years, who noted an excellent risk benefit profile during the open-label Phase 2 portion of the study."

First Quarter 2025 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended March 31,	
	2025	**2024**
Crysvita		
Product sales - Latin America and Türkiye	$ 55,080	$ 36,241
Royalty revenue - U.S. and Canada	40,853	40,402
Royalty revenue - Europe	6,932	5,942
Total Crysvita Revenue	102,865	82,585
Dojolvi	17,009	16,362
Evkeeza	11,031	3,275
Mepsevii	8,387	6,611
Total revenues	$ 139,292	$ 108,833

Total Revenues
Ultragenyx reported $139 million in total revenue for the first quarter of 2025, which represents 28% growth compared to the same period in 2024. First quarter 2025 Crysvita revenue was $103 million, which represents 25% growth compared to the same period in 2024. This includes product sales of $55 million from Latin America and Türkiye, which represents 52% growth compared to the same period in 2024. Dojolvi revenue in the first quarter 2025 was $17 million. Evkeeza revenue in the first quarter 2025 was $11 million as we continue to launch in the Ultragenyx territories outside of the United States.

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

	Three Months Ended March 31,	
	2025	**2024**
Total revenues	$ 139,292	$ 108,833
Operating expenses:		
Cost of sales	28,662	17,533
Research and development	165,772	178,487
Selling, general and administrative	87,797	78,160
Total operating expenses	282,231	274,180
Net loss	$ (151,080)	$ (170,684)
Net loss per share, basic and diluted	$ (1.57)	$ (2.03)

Operating Expenses
Total operating expenses for the first quarter of 2025 were $282 million, including non-cash stock-based compensation of $40 million.

Net Loss
For the first quarter of 2025, Ultragenyx reported net loss of $151 million, or $1.57 per share basic and diluted, compared with a net loss for the first quarter of 2024 of $171 million, or $2.03 per share basic and diluted.

Cash Balance and Net Cash Used in Operations

Cash, cash equivalents, and marketable debt securities were $563 million as of March 31, 2025, which reflects cash payments made during the first quarter of 2025 of $30 million for a GTX-102 Phase 3 study milestone and $15 million for an Evkeeza sales milestone, both achieved in the fourth quarter of 2024. Net cash used in operations for the quarter ended March 31, 2025 was $166 million and includes the payment of annual bonuses and a $30 million cash payment for the GTX-102 development milestone noted above.

2025 Financial Guidance
Ultragenyx reaffirmed its financial guidance for 2025. 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): Next interim analysis for Phase 3 Orbit and Cosmic studies is 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 toward a second interim analysis (IA2) 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 analysis. If *Orbit* progresses to full study completion in the fourth quarter of 2025, *Cosmic* will also continue to a data readout, to align with the *Orbit* readout without spending alpha at the mid-year interim assessment.

GTX-102 an antisense oligonucleotide for Angelman syndrome: Phase 3 study is enrolling, expect 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. Phase 3 study site startup and enrollment are going well. Participants are 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 is improvement in cognition assessed by Bayley-4 cognitive raw score, and the key secondary endpoint (with a 10% allocation of alpha) is 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): BLA accepted; U.S. Food and Drug Administration (FDA) granted Priority Review with a Prescription Drug User Fee Act (PDUFA) action date of August 18, 2025

In February 2025, the FDA accepted for review the BLA seeking accelerated approval for UX111. The FDA granted the BLA Priority Review with a PDUFA action date of August 18, 2025 and also informed the company that they are not currently planning to hold an advisory committee meeting to discuss this application. The FDA review currently continues to progress as expected with a mid-cycle review recently completed and multiple clinical and manufacturing inspections scheduled and underway. Based on available information, the company continues to expect the FDA to meet its stated timeline with a PDUFA decision on August 18, 2025 that would be followed by a potential launch in the second half of 2025.

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

As previously disclosed by Ultragenyx in May 2024, the Phase 3 *GlucoGene* study for the treatment of patients aged eight years and older with GSDIa 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. At Week 48, patients entered a 48-week Crossover Period where patients previously treated with placebo were treated with DTX401. During the Crossover Period, patients demonstrated even greater reductions in total daily cornstarch at their last visit compared to baseline in both the ongoing DTX401 group (-60%) and the Crossover Placebo to DTX401 group (-64%). Glycemic control was maintained in patients treated with DTX401 despite significant reductions in daily cornstarch intake. DTX401 has demonstrated a consistent and acceptable safety profile with no new safety signals identified as of the data cut-off.

Process Performance Qualification (PPQ) runs have been successfully completed in the Company's U.S. gene therapy manufacturing facility in preparation for upcoming submissions. The latest clinical results and data from PPQ runs will be included as part of a BLA submission expected in mid-2025.

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

Enrollment has begun in the fourth cohort evaluating a 4.0e13 GC/kg dose in the ongoing, dose-finding, stage of the pivotal *Cyprus2+* study of UX701 for the treatment of Wilson disease. The company expects to enroll five patients in Cohort 4 who will receive immunomodulation therapy with rituximab and tacrolimus, in addition to the prophylactic oral corticosteroid regimen patients in Cohorts 1 through 3 received, prior to being dosed with UX701. Enrollment in Cohort 4 is expected to complete in the second half of 2025.

The protocol for the pivotal, Stage 2 portion of *Cyprus2+* was amended to a 52-week, randomized, open-label, active-controlled design, evaluating the safety and efficacy of UX701 following dose selection in Stage 1. The Stage 2 primary endpoints include comparisons between the UX701 and active control groups of change in 24-hour urinary copper from Baseline at Week 52 and percent reduction in SOC medication by Week 52. Enrollment in this stage is expected following dose selection in Stage 1.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, May 6, 2025, at 2 p.m. PT/5 p.m. ET to discuss the first quarter 2025 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 ultra-rare 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 commercialization of Crysvita in certain major markets, including the U.S. and Canada, and for our commercial supply of Crysvita in those markets, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, , smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, our ability to successfully manage the expansion of our company, competition from other therapies or products, market acceptance of our products, uncertainty related to insurance coverage and reimbursement, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 19, 2025, and its subsequent periodic reports filed with the SEC.

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 March 31,	
	2025	**2024**
Statement of Operations Data:		
Revenues:		
Product sales	$ 91,507	$ 62,489
Royalty revenue	47,785	46,344
Total revenues	139,292	108,833
Operating expenses:		
Cost of sales	28,662	17,533
Research and development	165,772	178,487
Selling, general and administrative	87,797	78,160
Total operating expenses	282,231	274,180
Loss from operations	(142,939)	(165,347)
Change in fair value of equity investments	(157)	3,746
Non-cash interest expense on liabilities for sales of future royalties	(14,342)	(15,847)
Other income, net	7,668	7,219
Loss before income taxes	(149,770)	(170,229)
Provision for income taxes	(1,310)	(455)
Net loss	$ (151,080)	$ (170,684)
Net loss per share, basic and diluted	$ (1.57)	$ (2.03)
Shares used in computing net loss per share, basic and diluted	96,288,650	84,286,292

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

	Three Months Ended March 31,	
	2025	2024
Non-cash stock-based compensation	$ 39,910	$ 36,934

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

	March 31, 2025	December 31, 2024
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 563,003	$ 745,029
Working capital	407,487	472,970
Total assets	1,311,927	1,503,456
Total stockholders' equity	144,246	255,297

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