Ultragenyx Reports Third Quarter 2024 Financial Results and Corporate Update

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Third quarter 2024 total revenue grew 42% versus prior year to $139 million, including Crysvita® revenue of $98 million and Dojolvi® revenue of $21 million

Reaffirmed 2024 expected total revenue guidance of $530 million to $550 million

Breakthrough Designation granted for setrusumab (UX143) in osteogenesis imperfecta

DTX401 Phase 3 follow-up data demonstrated higher and faster 62% mean reduction in cornstarch in crossover patients with glycogen storage disease type Ia (GSDIa)

NOVATO, Calif., Nov. 05, 2024 (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 ended September 30, 2024.

“We continue to see substantial year-over-year revenue growth from our commercial portfolio as we expand geographic access to our medicines. This growth could accelerate with up to three near-term BLA submissions and approvals,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “Today, we are also sharing an important update from our Phase 3 study in GSDIa with substantially larger reductions in cornstarch intake in crossover patients treated with DTX401. As in our Phase 2 patients, we observed that patients and their physicians were far more comfortable with aggressive titration of cornstarch once they were confirmed to be treated with the gene therapy and had direct access to timely glucose readings."

Third Quarter 2024 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Crysvita				
Product sales	$ 35,604	$ 19,200	$ 112,294	$ 57,318
Revenue in Profit-Share Territory	55,985	50,230	163,432	161,450
Royalty revenue in European Territory	6,258	5,473	18,376	15,171
Total Crysvita Revenue	97,847	74,903	294,102	233,939
Dojolvi	21,374	16,553	57,091	47,347
Mepsevii	9,616	5,633	22,372	22,552
Evkeeza	10,657	963	21,788	1,540
Daiichi Sankyo	—	—	—	1,479
Total revenues	$ 139,494	$ 98,052	$ 395,353	$ 306,857

Total Revenues
Ultragenyx reported $139 million in total revenue for the third quarter of 2024, which represents 42% growth compared to the same period in 2023. Third quarter 2024 Crysvita revenue was $98 million, which represents 31% growth compared to the same period in 2023. This includes product sales of $36 million from Latin America and Turkey, which represents 85% growth compared to the same period in 2023. Dojolvi revenue in the third quarter 2024 was $21 million, which represents 29% growth compared to the same period in 2023. Evkeeza revenue in the third quarter 2024 was $11 million, as demand continues to build in the company’s territories outside of the United States.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Total revenues	$ 139,494	$ 98,052	$ 395,353	$ 306,857
Operating expenses:				
Cost of sales	21,021	10,987	59,834	33,158
Research and development	170,109	157,245	510,099	487,892
Selling, general and administrative	80,351	74,917	239,115	232,966
Total operating expenses	271,481	243,149	809,048	754,016
Net loss	$ (133,516)	$ (159,649)	$ (435,798)	$ (483,449)
Net loss per share, basic and diluted	$ (1.40)	$ (2.23)	$ (4.91)	$ (6.81)

Operating Expenses
Total operating expenses for the third quarter of 2024 were $271 million, including non-cash stock-based compensation of $42 million. In 2024, annual operating expenses are expected to be similar to 2023, as the company continues to manage its costs and focus its investment on advancing multiple Phase 3 programs and executing on commercial product launches.

Net Loss
For the third quarter of 2024, Ultragenyx reported net loss of $134 million, or $1.40 per share basic and diluted, compared with a net loss for the third quarter of 2023 of $160 million, or $2.23 per share basic and diluted.

Net Cash Used in Operations and Cash Balance
For the three months ended September 30, 2024, net cash used in operations was $67 million and for the nine months ended September 30, 2024 it was $335 million. Cash, cash equivalents, and marketable debt securities were $825 million as of September 30, 2024.

2024 Financial Guidance
For the full year 2024, the company expects:

- Total revenue to be in the range of $530 million to $550 million
- Crysvita revenue to be towards the upper end of the range of $375 million to $400 million. This includes all regions where Ultragenyx will recognize revenue: product sales in Latin America and Turkey, royalties in Europe, which have been ongoing, and royalties in North America, which began in April 2023.
- Dojolvi revenue to be in the range of $75 million to $80 million
- Net Cash Used in Operations of around $400 million

Recent Updates and Clinical Milestones

Dojolvi (triheptanoin) for long-chain fatty acid oxidation disorder (LC-FAOD): Alignment reached on conditional filing requirements with Japan's Pharmaceuticals and Medical Devices Agency (PMDA)
The Company recently received a positive finalized assessment report with agreement to file for Conditional Early Approval (CEA), from the PMDA, based on the currently available global clinical data. With this feedback, the company expects to file a J-NDA in mid-2025.

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Breakthrough Therapy Designation granted by the U.S. Food and Drug Administration (FDA)
In October 2024, UX143 received Breakthrough Therapy Designation (BTD) from the FDA. This designation is based on preliminary clinical evidence including the positive 14-month results from the Phase 2 portion of the *Orbit* study, which demonstrated a rapid and clinically meaningful 67% (p=0.0014) decrease in fracture rate in patients. It is also based on data from the completed Phase 2b *ASTEROID* study. BTD aims to expedite the development and review of drugs that are intended to treat serious or life-threatening diseases and whose preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies.

GTX-102 antisense oligonucleotide for Angelman syndrome: Program update planned for the upcoming Foundation for Angelman Syndrome Therapeutics (FAST) 17th Annual Global Science Summit and the 53rd Child Neurology Society (CNS) Annual Meeting
Longer-term interim data from the Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome are expected to be presented at the upcoming FAST Science Summit, taking place November 8-9, and the CNS Annual Meeting, taking place November 11-14. Data are expected to include an update on patients from the dose-escalation and expansion cohorts who have been on therapy for at least 338 days and will build on previously presented safety and efficacy data that showed patients demonstrated rapid and clinically meaningful improvements across multiple domains with an acceptable safety profile.

The company continues to be on track to initiate the pivotal Phase 3 *Aspire* study by the end of 2024 and the *Aurora* study in 2025.

UX111 AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA): Successful completion of a pre-BLA meeting with FDA
In October 2024, Ultragenyx participated in a successful pre-BLA meeting with the FDA during which the company aligned on the details of its BLA that is expected to be filed around the end of 2024.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Phase 3 data in crossover patients previously treated with placebo demonstrated 62% mean reduction in cornstarch at Week 30 post-treatment with DTX401 and patients in original treatment arm showed continued reductions in cornstarch
In May 2024, Ultragenyx 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. Twelve crossover patients have reached Week 30 post-treatment and have had a substantial 62% mean reduction of daily cornstarch at this early timepoint, double the rate of decrease when compared to patients in the original DTX401 treatment arm (n=20) that showed a 41% mean reduction at the end of 48-weeks. 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 are continuing 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 will be discussed with regulatory authorities to support a BLA submission in mid-2025.

UX701 AAV gene therapy for Wilson disease: Stage 1 update demonstrated clinical activity as well as improvements in copper metabolism
In October 2024, Ultragenyx shared that the Phase 1/2/3 *Cyprus2+* study demonstrated clinical activity as well as improvements in copper metabolism for patients treated in Stage 1. Multiple responders completely tapered off their standard-of-care treatment with responses seen in all three dose cohorts. UX701 has been well tolerated, with no unexpected, related treatment-emergent adverse events and no significant immunologic safety events as of the data cut-off.

The company expects to enroll an additional 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 of standard-of-care treatment before selecting a dose for the randomized placebo-controlled stage of the study.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients; expect enrollment to be completed in the next few months
Ultragenyx is randomizing and dosing patients in the ongoing Phase 3 study. The pivotal, 64-week study will include up to 50 patients, 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.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, November 5, 2024, at 2 p.m. PT/5 p.m. ET to discuss the third quarter 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 one year.

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 our clinical development programs, 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 candidates. 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 August 2, 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-/mycompany/).

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Statement of Operations Data:				
Revenues:				
Product sales	$ 77,251	$ 42,349	$ 213,545	$ 128,757
Royalty revenue	62,243	55,703	181,808	106,916
Collaboration and license	—	—	—	71,184
Total revenues	139,494	98,052	395,353	306,857
Operating expenses:				
Cost of sales	21,021	10,987	59,834	33,158
Research and development	170,109	157,245	510,099	487,892
Selling, general and administrative	80,351	74,917	239,115	232,966
Total operating expenses	271,481	243,149	809,048	754,016
Loss from operations	(131,987)	(145,097)	(413,695)	(447,159)
Change in fair value of equity investments	678	(1,419)	433	(1,492)
Non-cash interest expense on liabilities for sales of future royalties	(15,712)	(17,665)	(47,519)	(48,676)
Other income, net	13,808	5,182	26,599	15,755
Loss before income taxes	(133,213)	(158,999)	(434,182)	(481,572)
Provision for income taxes	(303)	(650)	(1,616)	(1,877)
Net loss	$ (133,516)	$ (159,649)	$ (435,798)	$ (483,449)
Net loss per share, basic and diluted	$ (1.40)	$ (2.23)	$ (4.91)	$ (6.81)
Shares used in computing net loss per share, basic and diluted	95,493,996	71,664,493	88,811,157	70,987,801

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Non-cash stock-based compensation	$ 41,569	$ 34,877	$ 117,866	$ 101,469
UX143 clinical milestone	—	—	—	$ 9,000

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

	September 30, 2024	December 31, 2023
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 824,694	$ 777,110
Working capital	514,600	451,747
Total assets	1,538,363	1,491,013
Total stockholders' equity	346,827	275,414

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