Ultragenyx Reports Fourth Quarter and Full Year 2023 Financial Results and Corporate Update

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2023 Total Revenue of $434 million, Crysvita® revenue of $328 million and Dojolvi® revenue of $71 million

2024 Financial Guidance: Total Revenue between $500 million and $530 million, Crysvita revenue of $375 million to $400 million, and Dojolvi revenue of $75 million to $80 million

Year-end 2023 cash balance of $777 million and 2024 guidance for Net Cash Used in Operations expected to be less than $400 million

NOVATO, Calif., Feb. 15, 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 and full year ended December 31, 2023 and reaffirmed its financial guidance for 2024.

"Last year, we made significant progress across our pipeline and look forward to multiple catalysts in 2024 with substantive data in Angelman syndrome that could enable a Phase 3 study, pivotal Phase 3 data from our GSDIa gene therapy program, dose-finding data from our seamless study in Wilson disease, and longer-term Phase 2 data in Osteogenesis Imperfecta," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "In 2023, we saw increased demand for both Crysvita and Dojolvi, and achieved multiple regulatory and reimbursement milestones for our commercial products to support continued revenue growth of approximately 20% in 2024."

Fourth Quarter and Full Year 2023 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Crysvita				
Product sales	$ 18,379	$ 7,698	$ 75,697	$ 42,678
Revenue in Profit-Share Territory	70,124	66,903	231,574	215,024
Royalty revenue in European Territory	5,612	6,058	20,783	21,692
Total Crysvita Revenue	94,115	80,659	328,054	279,394
Dojolvi	23,286	16,412	70,633	55,612
Mepsevii	7,889	4,798	30,441	20,637
Evkeeza	2,102	—	3,642	—
Daiichi Sankyo	—	1,479	1,479	7,686
Total revenues	$ 127,392	$ 103,348	$ 434,249	$ 363,329

Total Revenues
Ultragenyx reported $127 million in total revenue for the fourth quarter 2023, which represents 23% growth compared to the same period in 2022. Fourth quarter 2023 Crysvita revenue was $94 million, which represents 17% growth compared to the same period in 2022. This includes product sales of $18 million from Latin America and Turkey, which represents 139% growth compared to the same period in 2022. Dojolvi revenue in the fourth quarter 2023 was $23 million, which represents 42% growth compared to the same period in 2022.

Total revenue for the year ended December 31, 2023 was $434 million, which represents 20% growth compared to the prior year. Full year 2023 Crysvita revenue was $328 million, which represents 17% growth compared to the same period in 2022. This includes product sales of $76 million from Latin America and Turkey, which represents 77% growth compared to the same period in 2022. Dojolvi revenue in 2023 was $71 million, which represents 27% growth compared to the same period in 2022.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Total revenues	$ 127,392	$ 103,348	$ 434,249	$ 363,329
Operating expenses:				
Cost of sales	12,051	5,319	45,209	28,320
Research and development	160,557	170,808	648,449	705,789
Selling, general and administrative	76,833	72,849	309,799	278,139
Total operating expenses	249,441	248,976	1,003,457	1,012,248
Net loss	$ (123,190)	$ (151,833)	$ (606,639)	$ (707,421)
Net loss per share, basic and diluted	$ (1.52)	$ (2.16)	$ (8.25)	$ (10.12)

Operating Expenses
Total operating expenses for the fourth quarter of 2023 were $249 million, including non-cash stock-based compensation of $34 million. Total operating

expenses for the year ended December 31, 2023 were $1,003 million, including $135 million of non-cash stock-based compensation. In 2024, annual operating expenses are expected to be stable or to decrease as the company continues to manage its costs, focus its investment on advancing multiple Phase 3 programs, and execute on commercial product launches.

Net Loss
For the fourth quarter of 2023, Ultragenyx reported net loss of $123 million, or $1.52 per share basic and diluted, compared with a net loss for the fourth quarter of 2022 of $152 million, or $2.16 per share, basic and diluted. For the year ended December 31, 2023, Ultragenyx reported net loss of $607 million, or $8.25 per share basic and diluted, compared with a net loss the prior year of $707 million, or $10.12 per share, basic and diluted.

Net Cash Used in Operations and Cash Balance
Net cash used in operations for the year ended December 31, 2023 was $475 million. Cash, cash equivalents, and marketable debt securities were $777 million as of December 31, 2023.

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

- Total revenue in the range of $500 million to $530 million
- Crysvita revenue in 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 in the range of $75 million to $80 million
- Net Cash Used in Operations to be less than $400 million

Recent Updates and Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 3 portion of Orbit study expected to be fully enrolled around the end of the first quarter of 2024
Patients are being dosed in the late-stage clinical trials, *Orbit* and *Cosmic*, which are evaluating setrusumab in pediatric and young adult patients with OI. The randomized, placebo-controlled Phase 3 portion of the *Orbit* study is expected to enroll approximately 150 patients and be fully enrolled around the end of the first quarter of 2024. Additional longer-term Phase 2 safety and efficacy data from the *Orbit* study are expected in the second half of 2024.

The Phase 3 *Cosmic* study is an active-controlled study evaluating the effect of setrusumab compared to intravenous bisphosphonate (IV-BP) therapy on annualized total fracture rate in patients age 2 to <5 years. *Cosmic* is expected to enroll approximately 50 patients or more at more than 20 global sites and is expected to complete enrollment in the first half of 2024.

GTX-102 antisense oligonucleotide for Angelman syndrome: Phase 1/2 fully enrolled; expansion data expected in the first half of 2024
Enrollment in the expansion cohorts was completed in December 2023 with a total of 53 new patients enrolled. A total of 74 patients are enrolled in the Phase 1/2 study including the dose-escalation/extension study patients. The expansion cohorts will evaluate many of the same safety, pharmacokinetic, and efficacy measures as the previously enrolled dose-escalation/extension cohorts plus some new evaluations. The next safety and efficacy update is expected in the first half of 2024 and is planned to include data from at least 20 expansion cohort patients with a minimum of Day 170 data.

In January 2024, GTX-102 was accepted into the Priority Medicines (PRIME) program by the European Medicines Agency (EMA). PRIME is granted by the EMA to medicines that show the potential to benefit patients with unmet needs based on early clinical data. Through PRIME, the EMA offers early and proactive support to optimize development plans and the generation of robust data on a medicine's benefits and risks, and enables accelerated assessment of medicines applications.

UX701 AAV gene therapy for Wilson disease: Last patient in Cohort 3 dosed; expect interim Stage 1 data in mid-2024
All patients in the three dose-escalation cohorts of Stage 1 have been dosed. During Stage 1, the safety and efficacy of UX701 will be evaluated and a dose will be selected for further evaluation in Stage 2, the pivotal, randomized, placebo-controlled stage of the study. Data from Stage 1 are expected in mid-2024, which will be followed by dose selection and initiation of Stage 2 in the second half of 2024.

UX111 AAV gene therapy for Sanfilippo syndrome (MPS IIIA): Updated data from pivotal Transfer A study presented at WORLDSymposium™
New positive data from the ongoing pivotal *Transfer A* study evaluating the safety and efficacy of UX111 in children with MPS IIIA were presented at the 20th Annual WORLD*Symposium*™ earlier this month showing that a single infusion of UX111 can substantially correct the underlying metabolic disease and maintain cognitive function in nearly all patients. The presentation also showed that the observed reductions of heparan sulfate exposure in cerebrospinal fluid can predict improved long-term cognitive function in patients with MPS IIIA following treatment with UX111. With these data and other data, discussions are ongoing with the FDA seeking an accelerated review path for UX111.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Dosing in Phase 3 study complete; Phase 3 data readout expected in the first half of 2024
In May 2023, Ultragenyx announced the last patient had been dosed in the Phase 3 study. The 48-week study has fully enrolled patients eight years of age and older, randomized 1:1 to DTX401 or placebo. The primary endpoint is the reduction in oral glucose replacement with cornstarch while maintaining glucose control. Phase 3 safety and efficacy data are expected in the first half of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients; expect enrollment to be completed in the first half of 2024
Ultragenyx is randomizing and dosing patients in the ongoing Phase 3 study. The pivotal, 64-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by removal of ammonia-scavenger medications and protein-restricted diet and change in 24-hour ammonia levels. Enrollment is currently expected to be completed in the first half of 2024.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, February 15, 2024, at 2 p.m. PT/5 p.m. ET to discuss the fourth quarter and full year 2023 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. To participate in the live call, please register by clicking on the following link (https://register.vevent.com/register/BI177d5c166d3045ddaa4dcd3b3ec136ba) and you will be provided with dial-in details. 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 November 3, 2023, 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/).

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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,	
	2023	2022	2023	2022
Statement of Operations Data:				
Revenues:				
Product sales	$ 51,656	$ 28,908	$ 180,413	$ 118,927
Royalty revenue	75,736	6,058	182,652	21,692
Collaboration and license	—	68,382	71,184	222,710
Total revenues	127,392	103,348	434,249	363,329
Operating expenses:				
Cost of sales	12,051	5,319	45,209	28,320
Research and development	160,557	170,808	648,449	705,789
Selling, general and administrative	76,833	72,849	309,799	278,139
Total operating expenses	249,441	248,976	1,003,457	1,012,248
Loss from operations	(122,049)	(145,628)	(569,208)	(648,919)
Change in fair value of equity investments	1,889	1,840	397	(19,299)
Non-cash interest expense on liabilities for sales of future royalties	(17,328)	(15,874)	(66,004)	(43,015)
Other income, net	10,596	6,378	26,351	9,508
Loss before income taxes	(126,892)	(153,284)	(608,464)	(701,725)
Benefit from (provision for) income taxes	3,702	1,451	1,825	(5,696)
Net loss	$ (123,190)	$ (151,833)	$ (606,639)	$ (707,421)
Net loss per share, basic and diluted	$ (1.52)	$ (2.16)	$ (8.25)	$ (10.12)
Shares used in computing net loss per share, basic and diluted	81,118,873	70,152,192	73,543,862	69,914,225

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

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
Non-cash stock based compensation	$ 33,744	$ 29,355	$ 135,213	$ 130,368
In-process research and development expense from GeneTx acquisition	—	$ (201)	—	$ 75,033
UX143 clinical milestone	—	—	$ 9,000	—

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

	December 31, 2023	December 31, 2022
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 777,110	$ 896,732
Working capital	451,747	622,689
Total assets	1,491,013	1,545,444
Total stockholders' equity	275,414	352,494