Ultragenyx Reports Third Quarter 2023 Financial Results and Corporate Update

November 2, 2023

Third quarter 2023 total revenue of $98.1 million, Crysvita® revenue of $74.9 million and Dojolvi® revenue of $16.6 million

Reaffirmed 2023 expected total revenue guidance between $425 million to $450 million, Crysvita revenue of $325 million to $340 million, and Dojolvi revenue of $65 million to $75 million

Presented positive updates on three lead clinical programs at Analyst Day in October

NOVATO, Calif., Nov. 02, 2023 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultrarare genetic diseases, today reported its financial results for the quarter ended September 30, 2023 and provided its financial guidance for the year.

"We're in a strong financial position due to growing demand for our commercial products, completion of our recent offering, and our disciplined expense and portfolio management," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "We've also made significant progress on our key clinical programs and highlighted new, interim data from UX143 for osteogenesis imperfecta, GTX-102 for Angelman syndrome, and UX701 for Wilson Disease at our Analyst Day in October demonstrating promising therapeutic potential in these larger indications with further updates to come in 2024."

Third Quarter 2023 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Crysvita				
Product sales	$ 19,200	$ 13,184	$ 57,318	$ 34,980
Royalty revenue	35,160	—	64,221	—
Non-cash royalty revenue	20,543	5,373	42,695	15,634
Revenue in profit-share territory	—	51,348	69,705	148,121
Total Crysvita revenue	74,903	69,905	233,939	198,735
Dojolvi	16,553	13,274	47,347	39,200
Mepsevii	5,633	6,045	22,552	15,839
Evkeeza	963	—	1,540	—
Daiichi Sankyo	—	1,479	1,479	6,207
Total revenues	$ 98,052	$ 90,703	$ 306,857	$ 259,981

Total Revenues
Ultragenyx reported $98.1 million in total revenue for the third quarter 2023, which represents 8% growth compared to the third quarter 2022. Third quarter 2023 Crysvita product sales, primarily in Latin America, were $19.2 million, which represents 46% growth compared to the same period in 2022 and 14% growth over the second quarter 2023. Third quarter 2023 also includes Crysvita royalty and non-cash royalty revenue in North America of $50.2 million, which was impacted by a decrease in channel inventory related to Kyowa Kirin Co., Ltd.'s (KKC) change from Ultragenyx labeled product to KKC's labeled product as part of the transition of North America commercialization responsibilities for Crysvita from Ultragenyx to KKC. This one-time change occurred in the third quarter 2023, and the Company expects Crysvita channel inventories to increase to more normal levels at the end of the year. Third quarter 2023 non-cash royalty revenue in Europe was $5.5 million.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Total revenues	$ 98,052	$ 90,703	$ 306,857	$ 259,981
Operating expense:				
Cost of sales	10,987	8,631	33,158	23,001
Research and development	157,245	237,297	487,892	534,981
Selling, general and administrative	74,917	69,841	232,966	205,290
Total operating expense	243,149	315,769	754,016	763,272
Net loss	$ (159,649)	$ (245,106)	$ (483,449)	$ (555,588)
Net loss per share, basic and diluted	$ (2.23)	$ (3.50)	$ (6.81)	$ (7.96)

Operating Expenses
Total operating expenses for the third quarter of 2023 were $243.1 million, including non-cash stock-based compensation of $34.9 million. In 2023, annual operating expenses are expected to decrease compared to 2022, as the company manages headcount and increases operational leverage

while executing on its high-value programs.

Net Loss
For the third quarter of 2023, Ultragenyx reported net loss of $159.6 million, or $2.23 per share basic and diluted, compared with a net loss for the third quarter of 2022 of $245.1 million, or $3.50 per share, basic and diluted.

Cash, Cash Equivalents and Marketable Debt Securities
Cash, cash equivalents, and marketable debt securities were $524.2 million as of September 30, 2023. This excludes net proceeds of $326.5 million from an underwritten public offering of common stock and pre-funded warrants that closed in October 2023.

2023 Financial Guidance
For the full year 2023, the company expects:

- Total revenue in the range of $425 million to $450 million
- Crysvita revenue in the range of $325 million to $340 million. This includes all regions where Ultragenyx will recognize revenue, including the royalties in Europe, which have been ongoing, and the royalties in North America, which began in April 2023.
- Dojolvi revenue in the range of $65 million to $75 million
- Net Cash Used in Operations to be around $425 million

Recent Updates and Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 2 demonstrated 67% reduction in annualized fracture rate and continuous improvements in bone mineral density
At the American Society of Bone and Mineral Research 2023 Annual Meeting (ASBMR), interim data from the Phase 2 portion of the Phase 2/3 *Orbit* study were presented that demonstrated treatment with setrusumab significantly reduced incidence of fractures in patients with OI with at least 6 months of follow-up and continued to demonstrate ongoing and meaningful improvements in lumbar spine bone mineral density (BMD). As of the cut-off date on August 4, 2023 and following at least six months of treatment with setrusumab, the annualized fracture rate across all 24 patients in the Phase 2 portion of the study was reduced by 67%. The median annualized fracture rate of 0.72 in the two years prior to treatment was reduced to 0.00 (n=24, p=0.042) during the mean treatment duration period of nine months. As of the data cut-off, there were no treatment-related serious adverse events (SAEs) observed in the study. Additional longer-term Phase 2 data are expected in 2024.

Patients are being dosed in the late-stage clinical trials, *Orbit* and *Cosmic*, which evaluate setrusumab in pediatric and young adult patients with OI. The Phase 3 portion of the *Orbit* study is targeting to enroll up to 195 patients at more than 50 sites across 12 countries. 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 aged 2 to <5 years. *Cosmic* is targeting to enroll approximately 65 patients at more than 20 global sites.

GTX-102 antisense oligonucleotide for Angelman syndrome: data from extension cohorts in Phase 1/2 study showed clinically meaningful improvements in multiple domains
In October 2023, interim data from the extension cohorts (Cohorts 4-7) in the ongoing Phase 1/2 for GTX-102 in Angelman syndrome were presented at an Analyst Day event. The data showed improvements across multiple domains compared to natural history data, where available, and clinical changes were associated with quantitative changes in EEG. Long-term data showed patients who stopped and restarted treatment reacquired previously gained developmental skills when they were re-dosed with the current regimen. As of the data cut-off, there have been no additional treatment-related SAEs, including lower extremity weakness, since November 2022.

Globally, sites are dosing patients in the expansion cohorts (Cohorts A-E), which will evaluate the same safety, pharmacokinetic, and efficacy measures as the extension cohorts. Data from at least 20 patients enrolled in the expansion cohorts, who have been on therapy for six months or more, are currently expected in the first half of 2024.

UX701 AAV gene therapy for Wilson Disease: Stage 1 of pivotal clinical study dosing patients; expect Stage 1 enrollment completion around the end of the year
In October 2023, interim data from the first dose cohort (5.0 x 10^12 GC/kg) in the ongoing *Cyprus2*+ study for UX701 in Wilson disease were presented at an Analyst Day event. The company announced four out of five patients in Cohort 1 had reductions in urinary copper and were tapering off of chelators and/or zinc therapy, including two of three earlier treated patients in the cohort that are now completely off standard of care therapy. As of the data cut-off, UX701 had been generally well-tolerated with no treatment-related SAEs.

Dosing in the second of three dose escalation cohorts in the pivotal study has been completed. The data safety monitoring board (DSMB) is scheduled to meet and will review the available safety data from Cohort 2 before making a recommendation on escalating to Cohort 3 of Stage 1 in this study. Stage 1 is currently on track to complete enrollment around the end of the year and these data are expected in the first half of 2024. During this stage, the safety and efficacy of UX701 will be evaluated and a dose will be selected for further evaluation in the pivotal, randomized, placebo-controlled stage of the study.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Dosing in Phase 3 study complete
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 data are expected in the first half of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients
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, November 2, 2023, at 2 p.m. PT/5 p.m. ET to discuss the third quarter 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://edge.media-server.com/mmc/p/t97kpsfo), 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 August 4, 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/).

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,	
	2023	2022	2023	2022
Statement of Operations Data:				
Revenues:				
Product sales	$ 42,349	$ 32,503	$ 128,757	$ 90,019
Royalty revenue	55,703	5,373	106,916	15,634
Collaboration and license	—	52,827	71,184	154,328
Total revenues	98,052	90,703	306,857	259,981
Operating expenses:				
Cost of sales	10,987	8,631	33,158	23,001
Research and development	157,245	237,297	487,892	534,981
Selling, general and administrative	74,917	69,841	232,966	205,290
Total operating expenses	243,149	315,769	754,016	763,272
Loss from operations	(145,097)	(225,066)	(447,159)	(503,291)
Change in fair value of equity investments	(1,419)	(1,626)	(1,492)	(21,139)
Non-cash interest expense on liability related to the sale of future royalties	(17,665)	(14,505)	(48,676)	(27,141)
Other income, net	5,182	2,378	15,755	3,130
Loss before income taxes	(158,999)	(238,819)	(481,572)	(548,441)
Provision for income taxes	(650)	(6,287)	(1,877)	(7,147)
Net loss	$ (159,649)	$ (245,106)	$ (483,449)	$ (555,588)
Net loss per share, basic and diluted	$ (2.23)	$ (3.50)	$ (6.81)	$ (7.96)
Weighted-average shares used in computing net loss per share, basic and diluted	71,664,493	70,054,173	70,987,801	69,834,037

Ultragenyx Pharmaceutical Inc.
Selected Activity included in Operating Expenses

(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Non-cash stock-based compensation	$ 34,877	$ 35,761	$ 101,469	$ 101,013
In-process research and development expense from GeneTx acquisition	—	$ 75,234	—	$ 75,234
UX143 clinical milestone	—	—	$ 9,000	—

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

	September 30, 2023	December 31, 2022
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 524,165	$ 896,732
Working capital	345,210	622,689
Total assets	1,238,140	1,545,444
Total stockholders' equity	31,714	352,494

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

Media
Jeff Blake
media@ultragenyx.com