Ultragenyx Reports First Quarter 2024 Financial Results and Corporate Update

May 2, 2024 8:00 PM EDT

First quarter total revenue of $109 million, Crysvita® revenue of $83 million and Dojolvi® revenue of $16 million

Reaffirmed 2024 expected total revenue guidance between $500 million to $530 million, Crysvita revenue of $375 million to $400 million, and Dojolvi revenue of $75 million to $80 million

Presented positive interim Phase 1/2 data from GTX-102 Angelman syndrome study demonstrating that Expansion Cohorts showed rapid, clinically meaningful improvement across multiple domains consistent with or exceeding Dose-escalation Cohort data at Day 170

NOVATO, Calif., May 02, 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 March 31, 2024 and reaffirmed its financial guidance for 2024.

"In the first quarter we continued to see rapid revenue growth in the international markets with steady progress globally across our four commercial products. We also just completed enrollment of our Phase 3 program in osteogenesis imperfecta supported by strong enthusiasm from treating physicians and the patient community," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "Last month we shared new data from the GTX-102 program for Angelman syndrome and have received an overwhelming response of excitement and support from the clinical experts and advocates in the Angelman community. We are on track to hold regulatory discussions in mid-2024 with the goal of initiating a Phase 3 trial before the end of the year."

First Quarter 2024 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended March 31,	
	2024	**2023**
Crysvita		
Product sales	$ 36,241	$ 21,234
Revenue in Profit-Share Territory	40,402	49,906
Royalty revenue in European Territory	5,942	4,882
Total Crysvita Revenue	82,585	76,022
Dojolvi	16,362	14,303
Mepsevii	6,611	8,480
Evkeeza	3,275	212
Daiichi Sankyo	—	1,479
Total revenues	$ 108,833	$ 100,496

Total Revenues
Ultragenyx reported $109 million in total revenue for the first quarter of 2024, which represents 8% growth compared to the same period in 2023. First quarter 2024 Crysvita revenue was $83 million, which represents 9% growth compared to the same period in 2023. This includes product sales of $36 million from Latin America and Turkey, which represents 71% growth compared to the same period in 2023. In April 2023, commercialization responsibilities in the Profit-share Territory transitioned to Kyowa Kirin Co and commensurately, revenue in that region transitioned from a profit-share to a royalty ranging from the mid-20's to 30% calculated based on annual revenue tiers. Dojolvi revenue in the first quarter 2024 was $16 million, which represents 14% growth compared to the same period in 2023.

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

	Three Months Ended March 31,	
	2024	**2023**
Total revenues	$ 108,833	$ 100,496
Operating expenses:		
Cost of sales	17,533	12,257
Research and development	178,487	165,698
Selling, general and administrative	78,160	76,646
Total operating expenses	274,180	254,601
Net loss	$ (170,684)	$ (163,972)
Net loss per share, basic and diluted	$ (2.03)	$ (2.33)

Operating Expenses
Total operating expenses for the first quarter of 2024 were $274 million, including non-cash stock-based compensation of $37 million. In 2024, annual operating expenses are expected to be stable or to decrease 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 first quarter of 2024, Ultragenyx reported net loss of $171 million, or $2.03 per share basic and diluted, compared with a net loss for the first quarter of 2023 of $164 million, or $2.33 per share, basic and diluted.

Net Cash Used in Operations and Cash Balance
Net cash used in operations for the quarter ended March 31, 2024 was $191 million. Cash, cash equivalents, and marketable debt securities were $569 million as of March 31, 2024.

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): Enrollment complete for Phase 3 Orbit and Cosmic studies; expect additional Phase 2 data update in second half of 2024
The Phase 3 portion of the *Orbit* study has completed enrollment with 158 patients. *Orbit* is a randomized placebo-controlled study evaluating the effect of setrusumab compared to placebo on the rate of annualized clinical fractures in patients aged 5 to <25 years. Additional longer-term safety and efficacy data from the Phase 2 portion of the *Orbit* study are expected in the second half of 2024.

The Phase 3 *Cosmic* study has also completed enrollment with 66 patients randomized or in screening. *Cosmic* 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 <7 years.

GTX-102 antisense oligonucleotide for Angelman syndrome: Positive interim Phase 1/2 data shared in April; expect End of Phase 2 meeting with Food and Drug Administration (FDA) in mid-2024
New interim data from the Phase 1/2 study were presented at a company presentation and at the 76th Annual American Academy of Neurology Meeting. Patients in Expansion Cohorts A & B treated with a set dose and regimen of GTX-102 showed rapid and clinically meaningful improvement across multiple domains consistent with or exceeding Dose Escalation Cohorts 4-7 data at Day 170. Treatment of the Dose Escalation Cohorts 4-7 showed long-term increasing and sustained clinical benefit far exceeding Natural History data at Day 758.

As previously reported and as of the data cut-off on April 5, 2024, there were two patients who had mild or moderate lower extremity weakness across 53 patients who completed the loading phase in the Expansion Cohorts A-E. Both were in Cohorts A & B and none reported in Cohorts C–E. The lower extremity weakness resolved rapidly without sequelae and patients remain in the study without ongoing safety concerns and are expected to continue dosing. The FDA and other regulatory agencies were notified and raised no issues nor required additional actions. There were no other unexpected serious adverse events.

We expect to share additional long-term safety and efficacy data from the Phase 1/2 study and plan to continue to provide routine safety updates with efficacy updates. We currently expect to have an End of Phase 2 meeting with the FDA in mid-2024 and meetings with other regulatory agencies in the second half of 2024.

UX701 AAV gene therapy for Wilson disease: Last patient in Cohort 3 dosed; expect interim Stage 1 data in the second half of 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 the second half of 2024, which will be followed by dose selection and initiation of Stage 2.

UX111 AAV gene therapy for Sanfilippo syndrome (MPS IIIA): Discussions ongoing with FDA, seeking accelerated approval path
In February 2024, Ultragenyx participated in a workshop hosted by the Reagan-Udall Foundation for the FDA that brought together FDA representatives, patient advocates, scientists, and industry leaders to discuss the use of CSF heparan sulfate as a relevant biomarker to enable accelerated approval in neuronopathic mucopolysaccharidoses (MPS). Discussions are ongoing with the FDA in seeking an accelerated review path for UX111.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Dosing in Phase 3 study complete; expect Phase 3 data readout in the second quarter of 2024
In May 2023, Ultragenyx announced the last patient was dosed in the Phase 3 study. The 48-week study enrolled 49 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 second quarter of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients; expect enrollment to be completed in the second 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 second half of 2024.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, May 2, 2024, at 2 p.m. PT/5 p.m. ET to discuss the first 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. To participate in the live call, please register by clicking on the following link (https://register.vevent.com/register/BI7c62f78f6df84941acb020e8220f4d81) 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 21, 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/).

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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 March 31,	
	2024	**2023**
Statement of Operations Data:		
Revenues:		
Product sales	$ 62,489	$ 44,229
Royalty revenue	46,344	4,882
Collaboration and license	—	51,385
Total revenues	108,833	100,496
Operating expenses:		
Cost of sales	17,533	12,257
Research and development	178,487	165,698
Selling, general and administrative	78,160	76,646
Total operating expenses	274,180	254,601
Loss from operations	(165,347)	(154,105)
Change in fair value of equity investments	3,746	(334)
Non-cash interest expense on liabilities for sales of future royalties	(15,847)	(15,636)
Other income, net	7,219	6,598
Loss before income taxes	(170,229)	(163,477)
Provision for income taxes	(455)	(495)
Net loss	$ (170,684)	$ (163,972)
Net loss per share, basic and diluted	$ (2.03)	$ (2.33)
Shares used in computing net loss per share, basic and diluted	84,286,292	70,368,478

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

	Three Months Ended March 31,	
	2024	2023
Non-cash stock-based compensation	$ 36,934	$ 31,939

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

	March 31, 2024	December 31, 2023
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 568,661	$ 777,110
Working capital	350,392	451,747
Total assets	1,307,080	1,491,013
Total stockholders' equity	140,264	275,414