Ultragenyx Reports First Quarter 2022 Financial Results and Corporate Update

May 5, 2022

First quarter 2022 total revenue of $79.9 million and Crysvita® revenue in Ultragenyx territories[1] of $54.6 million

Reaffirm 2022 Crysvita revenue in Ultragenyx territories guidance of $250 million to $260 million and Dojolvi revenue of $55 million to $65 million

Cash balance of approximately $814 million as of March 31, 2022

NOVATO, Calif., May 05, 2022 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today reported its financial results for the quarter ended March 31, 2022 and reaffirmed its financial guidance for the year.

“Product based revenue continues to grow driven by increased traction for Crysvita in Latin America,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In the clinical portfolio, we are now dosing patients in three of our four concurrent pivotal programs and are on track for updated data from the Phase 1/2 for Angelman syndrome mid-year.”

First Quarter 2022 Financial Results

Net Revenues
For the first quarter of 2022, Ultragenyx reported $79.9 million in total revenue. Ultragenyx recognized $54.6 million in Crysvita (burosumab) revenue in the Ultragenyx territories, which includes $45.2 million in collaboration revenue in the North American profit share territory and net product sales in other regions of $9.4 million. Total royalty revenue related to European Crysvita sales was $4.8 million. Dojolvi (triheptanoin) product sales in the first quarter of 2022 were $12.4 million. Mepsevii (vestronidase alfa) product sales for the first quarter of 2022 were $4.9 million.

Total revenue for the first quarter of 2022 also includes $3.2 million related to technical assistance following the successful completion of technology transfer activities with Daiichi Sankyo. This compares to total revenue in the first quarter of 2021, which includes $42.8 million related to the technology transfer services which were ongoing at the time.

Operating Expenses
Total operating expenses for the first quarter of 2022 were $216.6 million, including non-cash stock-based compensation of $29.4 million.

Net Loss
For the first quarter of 2022, Ultragenyx reported net loss of $152.3 million, or $2.19 per share basic and diluted, compared with a net loss for the first quarter of 2021 of $136.1 million, or $2.03 per share, basic and diluted. Net cash used in operations for the quarter ended March 31, 2022 was $117.5 million.

Cash, Cash Equivalents and Marketable Debt Securities
Cash, cash equivalents, and marketable debt securities were $813.8 million as of March 31, 2022.

2022 Financial Guidance

The company continues to expect 2022 revenue for Crysvita in Ultragenyx territories to be between $250 million and $260 million and Dojolvi revenue to be between $55 million and $65 million.

First Quarter 2022 Revenue and Selected Financial Data Tables

Revenues (dollars in thousands)

	Three Months Ended March 31,	
	2022	2021
Crysvita Collaboration and Product Revenues:		
North America Collaboration	$ 45,164	$ 36,260
ROW Product Sales	9,394	5,872
Crysvita in Ultragenyx Territories	54,558	42,132
EU Royalty Revenue	4,838	3,872
Total Crysvita Revenue	59,396	46,004
Dojolvi	12,429	7,034
Mepsevii	4,861	3,607
Daiichi Sankyo	3,249	42,750
Total Revenue	$ 79,935	$ 99,395

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

	Three Months Ended March 31,	
	2022	2021
Total revenue	$ 79,935	$ 99,395

Operating expense:		
Cost of sales	6,100	5,188
Research and development	143,155	147,518
Selling, general and administrative	67,312	53,258
Total operating expense	216,567	205,964
Net loss	$ (152,320)	$ (136,141)
Net loss per share, basic and diluted	$ (2.19)	$ (2.03)

Recent Updates

Evkeeza® (evinacumab) for Homozygous Familial Hypercholesterolemia (HoFH): European marketing authorization transitioned to Ultragenyx, reimbursement processes initiated
The European Commission has completed transfer of the marketing authorization of Evkeeza from Regeneron. Ultragenyx is preparing reimbursement dossiers for various national health authorities in Europe. Ultragenyx received rights to commercialize and distribute Evkeeza in countries outside the U.S. in January 2022.

UX143 (setrusumab) for Osteogenesis Imperfecta (OI): Dosing has been initiated for the Phase 2/3 *Orbit* study; Phase 2 study in children under age five planned for second half of 2022
Ultragenyx has begun dosing patients in the seamless Phase 2/3 *Orbit* study of UX143 in pediatric and adult patients with OI ages five to <26 years. A dosing update on the Phase 2 portion of the *Orbit* study and transition to Phase 3 is expected in the second half of 2022.

In addition, Ultragenyx intends to initiate an additional study in children with OI under age 5 years in the second half of 2022 and will continue to evaluate adult patients who were previously treated in the *ASTEROID* study, a Phase 2b study conducted by our partner Mereo.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Phase 3 *GlucoGene* study dosing patients
Dosing and enrollment of the Phase 3 study of DTX401 is ongoing. The pivotal *GlucoGene* study has a 48-week primary efficacy analysis period and the company plans to enroll approximately 50 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.

UX701 for Wilson Disease: *Cyprus2*+ pivotal Phase 1/2/3 study dosing patients
The company is dosing patients in the Phase 1/2 stage of the seamless Phase 1/2/3 *Cyprus2*+ study of UX701. During the first stage of the study, safety and efficacy of up to three dose levels of UX701 will be evaluated and a dose will be selected for further evaluation in Stage 2. In Stage 2, a new cohort of patients will be randomized 2:1 to receive the selected dose of UX701 or placebo. The primary efficacy endpoints are change in 24-hour urinary copper concentration and percent reduction in standard of care medication by Week 52.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 *eNH$_3$ance* study expected to initiate in mid-2022
Ultragenyx expects to initiate the Phase 3 *eNH$_3$ance* study of DTX301 in patients with OTC in mid-2022. The 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.

GTX-102 for Angelman Syndrome: Patients continue to be treated in the Phase 1/2 study in Canada and the U.K. and under a separate protocol in the U.S.
Dosing is ongoing in cohorts 4 and 5 of the Phase 1/2 study in the U.K. and Canada, as well as for four additional patients in the U.S. under a separate protocol. To date, no treatment-related serious adverse events or lower extremity weakness adverse events have been observed in these patients. An interim update on the safety and efficacy of GTX-102 is planned for mid-2022.

UX053 for Glycogen Storage Disease Type III (GSDIII) Debrancher Deficiency: Phase 1/2 study currently dosing patients; Preliminary data from first part of study and initiation of second part of study anticipated in second half of 2022
Ultragenyx has begun to dose patients in the two-part Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of UX053 in adults age 18 years and older with GSDIII. Part 1 is open label and will enroll up to 10 patients who will receive a single ascending dose of UX053 administered via intravenous infusion. Part 2 is double-blind and will evaluate five repeat doses at escalating dose levels in up to 16 patients across four cohorts randomized 3:1 to UX053 or placebo. Preliminary data from the Part 1 single ascending dose phase of the study is expected in the second half of the year.

1: Ultragenyx territories include the collaboration revenue from the North American profit share territory (U.S. and Canada) and other regions where revenue from product sales are recognized by Ultragenyx (Latin America, Turkey). This excludes the European territory revenue, which is recognized as non-cash royalty revenue since the rights were sold to Royalty Pharma in December 2019.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, May 5, 2022, at 2 p.m. PT/ 5 p.m. ET to discuss the first quarter 2022 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 by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 7951356. 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 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 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 effects from the COVID-19 pandemic on the company's clinical and commercial activities and business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, the company's ability to achieve its projected development goals in its expected timeframes, risks and uncertainties related to the regulatory approval process, 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-K filed with the Securities and Exchange Commission (SEC) on February 15, 2022, 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 March 31,	
	2022	**2021**
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 48,413	$ 79,010
Product sales	26,684	16,513
Non-cash collaboration royalty revenue	4,838	3,872
Total revenues	79,935	99,395
Operating expenses:		
Cost of sales	6,100	5,188
Research and development	143,155	147,518
Selling, general and administrative	67,312	53,258
Total operating expenses	216,567	205,964
Loss from operations	(136,632)	(106,569)
Change in fair value of equity investments	(9,329)	(20,619)
Non-cash interest expense on liability related to the sale of future royalties	(6,584)	(8,418)
Other income (expense), net	783	(156)
Loss before income taxes	(151,762)	(135,762)
Provision for income taxes	(558)	(379)
Net loss	$ (152,320)	$ (136,141)
Net loss per share, basic and diluted	$ (2.19)	$ (2.03)
Weighted-average shares used in computing net loss per share, basic and diluted	69,516,668	67,102,342

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

	Three Months Ended March 31,	
	2022	**2021**
Non-cash stock based compensation	$ 29,387	$ 24,298

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

	March 31,	**December 31,**

	2022	2021
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 813,847	$ 999,129
Working capital	540,288	675,236
Total assets	1,384,029	1,522,397
Total stockholders' equity	796,424	922,561

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