Ultragenyx Reports Fourth Quarter and Full Year 2022 Financial Results and Corporate Update

February 16, 2023

2022 Total Collaboration and Product Revenue of $355.6 million, Crysvita® revenue of $279.4 million and Dojolvi® revenue of $55.6 million

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

Year-end 2022 cash balance of $896.7 million and 2023 guidance for Net Cash used in Operations expected to be less than $400 million

NOVATO, Calif., Feb. 16, 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 and full year ended December 31, 2022 and reaffirmed its financial guidance for 2023.

"In 2022, we continued to reach more patients around the world as we successfully commercialized multiple products to treat rare diseases," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "2023 will be a year of meaningful development progress for us as we focus on data generation from two of our key programs, UX143 in osteogenesis imperfecta and GTX-102 in Angelman syndrome, and the continued advancement of our late-stage gene therapy programs."

Fourth Quarter and Full Year 2022 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands) (unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Crysvita Collaboration and Product Revenues:				
North America Collaboration	$ 66,903	$ 50,211	$ 215,024	$ 171,198
ROW Product Sales	7,698	5,272	42,678	21,422
Crysvita in Ultragenyx Territories[1]	74,601	55,483	257,702	192,620
EU Royalty Revenue	6,058	4,741	21,692	18,195
Total Crysvita Revenue	80,659	60,224	279,394	210,815
Dojolvi	16,412	11,825	55,612	39,560
Mepsevii	4,798	3,111	20,637	16,035
Total Collaboration and Product Revenues	101,869	75,160	355,643	266,410
Daiichi Sankyo	1,479	8,229	7,686	84,996
Total Revenue	$ 103,348	$ 83,389	$ 363,329	$ 351,406

Net Revenues
For the fourth quarter of 2022, Ultragenyx reported $103.3 million in total revenue. Ultragenyx recognized $74.6 million in Crysvita (burosumab) revenue in the Ultragenyx territories[1], which includes $66.9 million in collaboration revenue in the North American profit-share territory and net product sales in other regions of $7.7 million. Total royalty revenue related to European Crysvita sales was $6.1 million. Dojolvi (triheptanoin) revenue in the fourth quarter of 2022 was $16.4 million. Mepsevii (vestronidase alfa) revenue for the fourth quarter of 2022 was $4.8 million. Total revenue for the fourth quarter also includes $1.5 million of revenue related to the collaboration and license agreement with Daiichi Sankyo that was executed in March 2020.

Revenue for the year ended December 31, 2022 was $363.3 million, including $257.7 million in Crysvita revenue in the Ultragenyx territories[1]. Crysvita collaboration revenue in the North American profit-share territory was $215.0 million and net Crysvita product sales in other regions were $42.7 million. Total royalty revenue related to European Crysvita royalties was $21.7 million. Dojolvi product revenue for the year ended December 31, 2022 was $55.6 million. Mepsevii product revenue was $20.6 million. For the year ended December 31, 2022, revenue related to our strategic manufacturing partnership with Daiichi Sankyo was $7.7 million.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Total revenue	$ 103,348	$ 83,389	$ 363,329	$ 351,406
Operating expense:				
Cost of sales	5,319	3,509	28,320	16,008
Research and development	170,808	123,013	705,789	497,153
Selling, general and administrative	72,849	59,431	278,139	219,982
Total operating expense	248,976	185,953	1,012,248	733,143

Net loss	$ (151,833)	$ (122,458)	$ (707,421)	$ (454,025)
Net loss per share, basic and diluted	$ (2.16)	$ (1.79)	$ (10.12)	$ (6.70)

Operating Expenses
Total operating expenses for the fourth quarter of 2022 were $249.0 million, including non-cash stock-based compensation of $29.4 million. Total operating expenses for the year ended December 31, 2022 were $1,012.2 million, which includes $130.4 million of non-cash stock-based compensation and $75.0 million of in-process research and development expense associated with the acquisition of GeneTx in July 2022. In 2023, annual operating expenses are expected to decrease as the company manages headcount and increases operational leverage while executing on high value programs.

Net Loss
For the fourth quarter of 2022, Ultragenyx reported net loss of $151.8 million, or $2.16 per share basic and diluted, compared with a net loss for the fourth quarter of 2021 of $122.5 million, or $1.79 per share, basic and diluted. For the year ended December 31, 2022, net loss was $707.4 million, or $10.12 per share, basic and diluted, compared with a net loss for the same period in 2021 of $454.0 million, or $6.70 per share, basic and diluted.

Cash, Cash Equivalents and Marketable Debt Securities
Cash, cash equivalents, and marketable debt securities were approximately $896.7 million as of December 31, 2022.

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

- 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 will begin in April 2023.
- Dojolvi revenue in the range of $65 million to $75 million
- Net Cash Used in Operations is expected to be less than $400 million

Recent Updates and 2023 Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 2/3 study dosing patients; Phase 2 enrollment complete, data expected in mid-2023
Ultragenyx is currently dosing patients in the Phase 2/3 *Orbit* study of UX143 in pediatric and adult patients with OI aged five to <26 years. Enrollment in the Phase 2 portion of the study is complete and data are expected in mid-2023. The Phase 2 data are expected to include changes in bone biomarkers response and some data on bone mineral density that will be used to establish the dosing algorithm for the Phase 3 portion of the study. Optimizing the dosing across the age range of the study is intended to support meaningful benefit in the reduction of clinically evident fractures in all age groups studied.

In addition, in the first half of 2023, Ultragenyx intends to initiate a randomized study in OI in children under age five with serious bone disease, comparing bisphosphonates to UX143. Younger pediatric patients with OI often have a much higher fracture rate than other age groups and a greater medical need, driving clinical urgency for better treatment options. Total fractures is expected to be the primary endpoint in the study.

GTX-102 antisense oligonucleotide for Angelman syndrome: Phase 1/2 continues dose exploration
As of January 2023, 23 patients had received loading doses ranging from 2 mg to 10 mg, with maintenance dosing ranging up to 10 mg to 14 mg. Ten patients have had between six and twelve months of exposure to GTX-102 and five patients have been on continuous therapy for more than one year. Based on the current protocols, the dose-finding cohorts in the U.K. and Canada will be followed by two, larger expansion cohorts with approximately 20 patients in each age range. Discussions are currently ongoing with the FDA to harmonize the three regions.

Based on the ongoing observations of encouraging clinical activity in the dose escalation cohorts, screening began for patients in expansion Cohort A (ages 4 to <8 years) and expansion Cohort B (ages 8 to <18 years) in February 2023. Each expansion cohort will enroll approximately 20 patients and will evaluate the same safety, pharmacokinetic, and efficacy measures as the dose escalating cohorts.

There continues to be dose and time dependent clinical activity seen across the study population, including three of the original U.S. patients who have been re-dosed, under the amended or expanded access protocols. The next data update, based on a larger number of patients in the program, is expected later this year.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Enrollment in Phase 3 study complete
The Phase 3 study has a 48-week primary efficacy analysis period, and the company has enrolled 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. The last patient has entered the baseline screening phase of the Phase 3 study of DTX401, with Phase 3 data expected in the first half of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study enrolling patients
Ultragenyx has randomized and dosed the first patient in the Phase 3 study with additional patients in the approximate 4- to 8-week baseline screening period. 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.

UX701 AAV gene therapy for Wilson Disease: Stage 1 of pivotal clinical study dosing patients; expect interim Stage 1 enrollment completion in mid-2023
The company is dosing patients in the first stage of the pivotal UX701 study under a recently amended protocol that removes placebo from the dose finding stage and enrolls five patients per cohort. During this 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. Completion of Stage 1 enrollment is expected in mid-2023 with data expected in early 2024 that would include safety and potentially initial signs of clinical activity.

UX111 AAV gene therapy for UX111 for the treatment of Sanfilippo syndrome type (MPS IIIA): Discussions ongoing with FDA to determine a plan to file for accelerated approval
In May 2022, Ultragenyx announced an exclusive license agreement with Abeona Therapeutics for UX111, formerly ABO-102. Under the terms of the agreement, Ultragenyx assumed responsibility for the UX111 program in exchange for Abeona's right to receive tiered royalties of up to 10% on net sales, and milestone payments upon the attainment of certain commercial revenue milestones.

A meeting with the FDA to discuss a plan to file for accelerated approval is expected to occur in the first half of 2023.

UX053 mRNA for glycogen storage disease type III (GSDIII): Phase 1/2 single ascending dose cohort enrolled; data in 1H23
Dosing in the single ascending dose stage of the Phase 1/2 study of UX053 for the treatment of GSDIII has been completed. Data from this cohort are expected in the first half of 2023. Based on these analyses and other work, we will then review our plans for the next steps in the program.

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 is 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, February 16, 2023, at 2 p.m. PT/ 5 p.m. ET to discuss the fourth quarter and full year 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, please register by clicking on the following link (https://register.vevent.com/register/BI4de832f7ef8946e486c3198ed80e759f), 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 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, including under our collaboration agreement with Kyowa Kirin, 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, 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 December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 68,382	$ 58,440	$ 222,710	$ 256,438
Product sales	28,908	20,208	118,927	77,017
Non-cash collaboration royalty revenue	6,058	4,741	21,692	17,951
Total revenues	103,348	83,389	363,329	351,406
Operating expenses:				
Cost of sales	5,319	3,509	28,320	16,008
Research and development	170,808	123,013	705,789	497,153
Selling, general and administrative	72,849	59,431	278,139	219,982
Total operating expenses	248,976	185,953	1,012,248	733,143
Loss from operations	(145,628)	(102,564)	(648,919)	(381,737)
Change in fair value of equity investments	1,840	(16,100)	(19,299)	(42,063)

Non-cash interest expense on liabilities for sales of future royalties	(15,874)	(3,804)	(43,015)	(29,422)
Other income, net	6,378	30	9,508	241
Loss before income taxes	(153,284)	(122,438)	(701,725)	(452,981)
Benefit from (provision for) income taxes	1,451	(20)	(5,696)	(1,044)
Net loss	$ (151,833)	$ (122,458)	$ (707,421)	$ (454,025)
Net loss per share, basic and diluted	$ (2.16)	$ (1.79)	$ (10.12)	$ (6.70)
Shares used in computing net loss per share, basic and diluted	70,152,192	68,570,546	69,914,225	67,795,540

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

	Three Months Ended December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Non-cash stock based compensation	$ 29,355	$ 26,549	$ 130,368	$ 104,979
Mereo license and collaboration agreement	—	—	—	50,000
In-process research and development expense from GeneTx acquisition	(201)	—	75,033	—

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

	December 31, 2022	December 31, 2021
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 896,732	$ 999,129
Working capital	622,689	675,236
Total assets	1,545,444	1,522,397
Total stockholders' equity	352,494	922,561

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