Ultragenyx Reports Second Quarter 2022 Financial Results and Corporate Update

July 28, 2022

Second quarter 2022 total revenue of $89.3 million and Crysvita® revenue in Ultragenyx territories[1] of $64.0 million

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

Expanded clinical pipeline through acquisition of GeneTx and global rights to UX111 for Sanfilippo syndrome

Completed $500.0 million sale of a capped 30% of royalty interest on the future sales of Crysvita in the United States and Canada

NOVATO, Calif., July 28, 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 June 30, 2022 and provided a corporate update for the year.

“Over the last few months we continued building our commercial footprint, particularly in Latin America, raised $500 million in a capped royalty financing, and acquired GeneTx, all of which puts us in a strong position to generate meaningful value in the coming years,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We have one of the most diverse, late-stage pipelines in rare disease, which includes our fourth pivotal gene therapy, UX111 for Sanfilippo syndrome, our pivotal program in osteogenesis imperfecta, and our promising ASO, GTX-102 in Angelman syndrome.”

Second Quarter 2022 Financial Results

Net Revenues
For the second quarter of 2022, Ultragenyx reported $89.3 million in total revenue. Ultragenyx recognized $64.0 million in Crysvita (burosumab) revenue in the Ultragenyx territories, which includes $51.6 million in collaboration revenue in the North American profit share territory and net product sales in other regions of $12.4 million. Total royalty revenue related to European Crysvita sales was $5.4 million. Dojolvi (triheptanoin) product sales in the second quarter of 2022 were $13.5 million. Mepsevii (vestronidase alfa) product sales for the second quarter of 2022 were $4.9 million.

Total revenue for the second quarter of 2022 also includes $1.5 million related to technical assistance following the successful completion of technology transfer activities with Daiichi Sankyo. This compares to total revenue in the second quarter of 2021, which includes $22.0 million related to the technology transfer services which were ongoing at the time.

Operating Expenses
Total operating expenses for the second quarter of 2022 were $230.9 million, including non-cash stock-based compensation of $35.9 million.

Net Loss
For the second quarter of 2022, Ultragenyx reported net loss of $158.2 million, or $2.26 per share basic and diluted, compared with a net loss for the second quarter of 2021 of $122.4 million, or $1.81 per share, basic and diluted. Net cash used in operations for the six months ended June 30, 2022 was $194.8 million.

Cash, Cash Equivalents and Marketable Debt Securities
As of June 30, 2022, cash, cash equivalents, and marketable debt securities were $706.1 million.

In July 2022, we raised $500.0 million through the sale of 30% of our royalty interest on the future sales of Crysvita in the United States and Canada, capped at $725.0 million or 1.45x the purchase price, and we exercised our option to acquire GeneTx for $75.0 million.

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.

Second Quarter 2022 Revenue and Selected Financial Data Tables

Revenues (dollars in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Crysvita Collaboration and Product Revenues:				
North America Collaboration	$ 51,609	$ 41,756	$ 96,773	$ 78,016
ROW Product Sales	12,402	2,900	21,796	8,772
Crysvita in Ultragenyx Territories	64,011	44,656	118,569	86,788
EU Royalty Revenue	5,423	4,917	10,261	8,789
Total Crysvita Revenue	69,434	49,573	128,830	95,577

Dojolvi	13,497	10,047	25,926	17,081
Mepsevii	4,933	5,399	9,794	9,006
Daiichi Sankyo	1,479	21,956	4,728	64,706
Total Revenue	$ 89,343	$ 86,975	$ 169,278	$ 186,370

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Total revenue	$ 89,343	$ 86,975	$ 169,278	$ 186,370
Operating expense:				
Cost of sales	8,270	3,136	14,370	8,324
Research and development	154,529	113,205	297,684	260,723
Selling, general and administrative	68,137	53,410	135,449	106,668
Total operating expense	230,936	169,751	447,503	375,715
Net loss	$ (158,162)	$ (122,428)	$ (310,482)	$ (258,569)
Net loss per share, basic and diluted	$ (2.26)	$ (1.81)	$ (4.45)	$ (3.84)

Corporate and Program Updates

Partial sale of North America Crysvita royalty

In July 2022, we sold 30% of our royalty interest in Crysvita, in the profit-share territory, to OMERS for $500.0 million, beginning in April 2023. Total payments are capped at $725.0 million, or 1.45x the purchase price, after which payments to OMERS will cease and Ultragenyx will retain the full value of the royalty.

GTX-102 for Angelman Syndrome

In July 2022, we exercised our option to acquire GeneTx and closed on the acquisition for $75.0 million plus future milestone and royalty payments. Additional milestones include $30.0 million upon achievement of the earlier of a Phase 3 clinical study start or product approvals in Canada and the U.K. We will also pay tiered royalties ranging from a mid-single to low double-digit percentage based on worldwide annual net sales.

In July 2022, we also provided an interim data and program update on patients treated in Canada and the U.K. and the U.S. under each region's amended protocol for the Phase 1/2 study. Further details from this release can be found on the Company website.

We currently expect to provide a program update after we have determined an optimal dose and have gathered substantial data from the expansion cohorts.

UX143 (setrusumab) for Osteogenesis Imperfecta (OI)

Ultragenyx is 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 follow adult patients who were previously treated in the *ASTEROID* study, a Phase 2b study conducted by our partner Mereo.

UX111 for Sanfilippo syndrome type A (MPS IIIA)

Ultragenyx entered into an exclusive license agreement from Abeona Therapeutics for UX111 (formerly ABO-102). Under the terms of the agreement, we assumed responsibility for the UX111 program and in return Abeona is eligible to receive tiered royalties of up to 10% on net sales and commercial milestone payments following regulatory approval.

Interim results from the pivotal *Transpher A* clinical study were presented at the American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting by Abeona. Additional details on this data can be found on the Company website.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Phase 3 study dosing patients

Longer-term Phase 1/2 data were presented at ASGCT 2022 Annual Meeting demonstrating a durable response from all 12 patients lasting up to more than 3.5 years following treatment with DTX401.

Dosing and enrollment of the Phase 3 study of DTX401 is ongoing. The Phase 3 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: Stage 1 of pivotal clinical study dosing patients

The company is dosing patients in the first stage of the *Cyprus2*+ study of UX701. 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. 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 study expected to initiate in second half of 2022

Longer-term Phase 1/2 data were presented at the ASGCT 2022 Annual Meeting showing a response from four out of five patients at the Phase 3 dose and a total of seven responders among the 11 patients enrolled in the study. This response included durable metabolic control lasting up to more

than four years following treatment with DTX301 in patients with OTC deficiency.

Ultragenyx expects to initiate the Phase 3 study of DTX301 in patients with OTC in the second half of 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.

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 is dosing 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 of the study is open label with single-ascending doses. Part 2 is double-blind and will evaluate repeat doses at escalating levels. We currently expect preliminary data from Part 1 of the study and to initiate Part 2 of the study in the second half of 2022.

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, July 28, 2022, at 2 p.m. PT/ 5 p.m. ET to discuss the second 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, please register by clicking on the following link (https://register.vevent.com/register/BIfc019ed9950547e9a1a7e28d5402491f), and you will be provided with dial in details. To avoid delays, we encourage participants to dial into the conference call fifteen minutes ahead of the scheduled start time. 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, 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 May 6, 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 June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 53,088	$ 63,940	$ 101,501	$ 142,950
Product sales	30,832	18,346	57,516	34,859

Non-cash collaboration royalty revenue	5,423	4,689	10,261	8,561
Total revenues	89,343	86,975	169,278	186,370
Operating expenses:				
Cost of sales	8,270	3,136	14,370	8,324
Research and development	154,529	113,205	297,684	260,723
Selling, general and administrative	68,137	53,410	135,449	106,668
Total operating expenses	230,936	169,751	447,503	375,715
Loss from operations	(141,593)	(82,776)	(278,225)	(189,345)
Change in fair value of equity investments	(10,184)	(31,046)	(19,513)	(51,665)
Non-cash interest expense on liability related to the sale of future royalties	(6,052)	(8,517)	(12,636)	(16,935)
Other income (expense), net	(31)	374	752	218
Loss before income taxes	(157,860)	(121,965)	(309,622)	(257,727)
Provision for income taxes	(302)	(463)	(860)	(842)
Net loss	$ (158,162)	$ (122,428)	$ (310,482)	$ (258,569)
Net loss per share, basic and diluted	$ (2.26)	$ (1.81)	$ (4.45)	$ (3.84)
Weighted-average shares used in computing net loss per share, basic and diluted	69,925,358	67,607,752	69,722,141	67,356,443

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Non-cash stock based compensation	$ 35,865	$ 27,142	$ 65,252	$ 51,440
Mereo license and collaboration agreement	—	—	—	50,000

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

	June 30, 2022	December 31, 2021
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 706,096	$ 999,129
Working capital	457,906	675,236
Total assets	1,320,627	1,522,397
Total stockholders' equity	678,073	922,561

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