Ultragenyx Reports Second Quarter 2021 Financial Results and Corporate Update

August 2, 2021

Second quarter 2021 total revenue of $87.0 million including Crysvita[1] revenue to Ultragenyx of $44.7 million

2021 Crysvita revenue in Ultragenyx territories[1] guidance of $180 million to $190 million reaffirmed

Strong Dojolvi launch continues with approximately 220 patients on reimbursed commercial therapy in the United States

Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome to begin enrolling patients in Canada and the U.K.; U.S. pending protocol revisions

NOVATO, Calif., Aug. 02, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today reported its financial results for the second quarter 2021 and reaffirmed its financial guidance for 2021.

"In the first half of 2021 we have advanced all our commercial, clinical, and regulatory priorities. This has led to meaningful revenue growth in the second quarter and has positioned us well as we enter a period of significant execution on our clinical programs in the second half of this year," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We have one of the most robust late-stage pipelines in both gene therapy and other biologics for rare diseases and are on track to initiate four pivotal clinical trials over the next six months."

Second Quarter 2021 Financials

In the second quarter 2021, Crysvita revenue in Ultragenyx territories[1] increased 38% versus the second quarter 2020. This increase is driven by demand from pediatric and adult patients with X-linked hypophosphatemia (XLH) and patients with tumor induced osteomalacia (TIO), which became Crysvita's second approved indication in the United States in June 2020.

Dojolvi revenue in the second quarter 2021 continued to build on the launch momentum after the U.S. Food and Drug Administration (FDA) approval in June 2020. As of the end of the second quarter 2021, the company has received approximately 270 completed start forms from approximately 130 unique prescribers. This has led to approximately 220 patients on reimbursed therapy as of the end of June 2021.

Second quarter 2021 revenue included $22.0 million related to the technology transfer as part of the Daiichi Sankyo strategic manufacturing partnership around the HeLa PCL and HEK293 technologies. This revenue is expected to taper significantly through the end of this year as these activities come to a close.

Total operating expenses of $169.8 million in the second quarter 2021 increased 36% or $45.0 million versus the second quarter 2020, primarily driven by pipeline advancements including four registrational studies. For the year, total operating expenses are expected to increase modestly as the company continues the commercial launch of Dojolvi and supports six clinical programs, including four pivotal studies.

Net cash used in operations for the six months ended June 30, 2021 was $224.7 million, compared to net cash used of $7.8 million for the same period in 2020 which included approximately $135 million of operating cash received in 2020 from Daiichi Sankyo related to the collaboration and license agreement. Cash, cash equivalents, and marketable debt securities were $973.8 million as of June 30, 2021.

2021 Financial Guidance

Crysvita Guidance in Ultragenyx Territories
The company reaffirms the 2021 guidance range for Crysvita that was provided at the beginning of the year. This range is $180 million to $190 million and includes the North American profit share region and the other regions where product sales are recognized.

Second Quarter 2021 Revenue and Selected Financial Data Tables

Revenues (dollars in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Crysvita Collaboration and Product Revenues:				
North America Collaboration	$ 41,756	$ 29,806	$ 78,016	$ 57,021
ROW Product Sales	2,900	2,549	8,772	4,159
Crysvita in Ultragenyx Territories	44,656	32,355	86,788	61,180
EU Royalty Revenue	4,917	4,980	8,789	7,595
Total Crysvita Revenue	49,573	37,335	95,577	68,775
Dojolvi	10,047	1,332	17,081	2,776
Mepsevii	5,399	4,185	9,006	7,610
Daiichi Sankyo	21,956	18,857	64,706	18,857
Total Revenue	$ 86,975	$ 61,709	$ 186,370	$ 98,018

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Total revenue	$ 86,975	$ 61,709	$ 186,370	$ 98,018
Operating expense:				
Cost of sales	3,136	1,803	8,324	(1,700)
Research and development	113,205	80,709	260,723	193,670
Selling, general and administrative	53,410	42,252	106,668	89,768
Total operating expense	169,751	124,764	375,715	281,738
Net income (loss)	$ (122,428)	$ 25,315	$ (258,569)	$ (93,710)
Net income (loss) per share:				
Basic	$ (1.81)	$ 0.42	$ (3.84)	$ (1.59)
Diluted	$ (1.81)	$ 0.41	$ (3.84)	$ (1.59)

Program Updates and Upcoming Milestones

Crysvita for the treatment of X-Linked Hypophosphatemia, or XLH

- The European Commission (EC) approved Crysvita for self-administration as a treatment option when recommended by the treating physician, for pediatric and adult patients with XLH.

GTX-102 for the treatment of Angelman Syndrome, partnered with GeneTx

- Health Canada (HC) cleared a protocol amendment and the U.K. Medicines and Healthcare Products Regulatory Agency (MHRA) approved a Clinical Trial Application to initiate the Phase 1/2 study in Canada and the U.K, respectively.
- Following a productive meeting with the FDA, GeneTx submitted an amended protocol. The companies have received feedback on the amended protocol and will make additional revisions in order to resume the study in the U.S.
- The Phase 1/2 study is expected to begin in the U.K. and Canada in the second half of 2021, with early clinical data from some patients in the study expected before the end of the year.

DTX401 for the treatment of Glycogen Storage Disease Type Ia, or GSDIa

- Longer-term Phase 1/2 data were presented at the American Society of Gene & Cell Therapy (ASGCT) 2021 Annual Meeting demonstrating a durable response from all nine patients lasting up to more than 2.5 years following treatment with DTX401.
- Operations are underway to initiate a Phase 3 study with a 48-week primary efficacy analysis period with the plan to enroll approximately 50 patients 8 years of age and older, randomized 1:1 to DTX401 (1.0 x 10^13 GC/kg dose) or placebo. The coprimary endpoints are the reduction in oral glucose replacement with cornstarch while maintaining or improving glucose control assessed by continuous glucose monitoring.
- The first patient in the Phase 3 study is expected to be dosed in the second half of 2021.

DTX301 for the treatment of Ornithine Transcarbamylase, or OTC, Deficiency

- Longer-term Phase 1/2 data were presented at the ASGCT 2021 Annual Meeting showing a response from all three patients at the Phase 3 dose and a total of 6 of 9 responders in the first three cohorts of patients enrolled. This response included durable metabolic control lasting up to more than three years following treatment with DTX301 in patients with OTC deficiency.
- Operations are underway to initiate a Phase 3 study that will include a 64-week primary efficacy analysis period and enroll approximately 50 patients 12 years of age and older, randomized 1:1 to DTX301 (1.7 x 10^13 GC/kg dose) or placebo. The co-primary endpoints are the percentage of patients who achieve a response as measured by discontinuation or reduction in baseline disease management and 24-hour plasma ammonia levels.
- The first patient in the Phase 3 study is expected to be dosed in the second half of 2021.

UX701 for the treatment of Wilson Disease

- Operations are underway to initiate a seamless, single-protocol Phase 1/2/3 study. The first part of the study will enroll approximately 27 patients (nine per cohort), randomized 2:1 to DTX701, manufactured using the company's proprietary HeLa 2.0 producer cell line (PCL) process at the 2,000 liter scale, or placebo. The dose cohorts will be enrolled sequentially using ascending doses. The patients will be followed for 52 weeks before transitioning to long-term follow-up and selecting a pivotal dose. The dose will be determined based on the safety profile, changes in biomarkers of copper metabolism, and the reduction in the use of the current standard of care.
- The first patient in the Phase 1/2/3 study is expected to be dosed in the second half of 2021.

UX053 for the treatment of Glycogen Storage Disease Type III, or GSDIII

- UX053 was granted Orphan Drug Designation (ODD) by the FDA and European Medicines Agency (EMA), highlighting the significant unmet need for patients with GSDIII.
- The first patient in the Phase 1/2 study is expected to be dosed in the second half of 2021.

1: Ultragenyx territories include the collaboration revenue from the North American profit share territory and other regions where revenue from product sales are recognized by Ultragenyx. 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, Monday, August 2, 2021, at 2 p.m. PT/ 5 p.m. ET to discuss the second quarter 2021 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.cfm. To participate in the live call by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 3654725. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel therapies 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 treatment, 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

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 activities, 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, 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 on May 5, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission

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,	
	2021	2020	2021	2020
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 63,940	$ 50,161	$ 142,950	$ 77,376
Product sales	18,346	8,066	34,859	14,545
Non-cash collaboration royalty revenue	4,689	3,482	8,561	6,097
Total revenues	86,975	61,709	186,370	98,018
Operating expenses:				
Cost of sales	3,136	1,803	8,324	(1,700)
Research and development	113,205	80,709	260,723	193,670
Selling, general and administrative	53,410	42,252	106,668	89,768
Total operating expenses	169,751	124,764	375,715	281,738
Loss from operations	(82,776)	(63,055)	(189,345)	(183,720)
Change in fair value of equity investments	(31,046)	95,200	(51,665)	102,868
Non-cash interest expense on liability related to the sale of future royalties	(8,517)	(8,429)	(16,935)	(16,511)
Other income (expense), net	374	2,014	218	4,477
Income (loss) before income taxes	(121,965)	25,730	(257,727)	(92,886)
Provision for income taxes	(463)	(415)	(842)	(824)
Net income (loss)	$ (122,428)	$ 25,315	$ (258,569)	$ (93,710)
Net income (loss) per share:				
Basic	$ (1.81)	$ 0.42	$ (3.84)	$ (1.59)
Diluted	$ (1.81)	$ 0.41	$ (3.84)	$ (1.59)
Weighted-average shares used in computing net income (loss) per share:				

Basic	67,607,752	59,995,617	67,356,443	58,996,278
Diluted	67,607,752	61,146,231	67,356,443	58,996,278

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,	
	2021	2020	2021	2020
Non-cash stock based compensation	$ 27,142	$ 22,409	$ 51,440	$ 42,581
GeneTx purchase option and extension	—	—	—	25,000
REGENXBIO license agreement	—	—	—	7,000
Mereo license and collaboration agreement	—	—	50,000	—

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

	June 30, 2021	December 31, 2020
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 973,787	$ 1,212,039
Working capital	822,425	1,105,695
Total assets	1,512,444	1,759,555
Total stockholders' equity	972,171	1,154,375

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