Ultragenyx Reports First Quarter 2020 Financial Results and Corporate Update

May 6, 2020

First quarter 2020 total revenue is $36.3 million

First quarter 2020 Crysvita revenue in Ultragenyx territories is $28.8 million

Maintains full year 2020 guidance based on COVID-19 impact to date

NOVATO, Calif., May 06, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today reported its financial results for the quarter ended March 31, 2020 and maintained its full year 2020 financial guidance.

“The global coronavirus pandemic has created unprecedented challenges for the world at large, and I am proud of the company’s continued efforts to assure an uninterrupted supply of our therapies” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We delivered strong financial results in the first quarter, and our commercial business so far has been manageably impacted and resilient, though the global crisis continues. The FDA reviews for Crysvita for TIO and UX007 for LC-FAOD are on track to be completed in the coming months. Our gene therapy franchise has recently been bolstered by our recent manufacturing collaboration with Daiichi Sankyo, and our two clinical-stage gene therapy programs continue to proceed.”

COVID-19 Update
As the COVID-19 pandemic continues to evolve, the top priority for Ultragenyx is the health and safety of its extended community, including patients, caregivers, healthcare providers, partners, and employees. To date, the company has been able to maintain an uninterrupted supply of medicine to patients around the world as no significant disruptions to manufacturing or distribution activities have been experienced. Even if there were an interruption in the supply chain, the company does not anticipate a shortage of medicine in the near term due to ample levels of inventory currently available.

First Quarter 2020 Financial Results

Net Revenues
For the first quarter of 2020, Ultragenyx reported $36.3 million in total revenue. Ultragenyx recognized $28.8 million in total Crysvita revenue for Ultragenyx territories, which includes $27.2 million in collaboration revenue in the North American profit share territory and net product sales in other regions of $1.6 million. Non-cash royalty revenue related to European Crysvita royalties were $2.6 million. Mepsevii® (vestronidase alfa) product revenue for the first quarter of 2020 was $3.4 million, and UX007 named patient revenue was $1.4 million.

Operating Expenses
Total operating expenses for the first quarter of 2020 were $157.0 million, which includes $25.0 million to maintain the option to acquire GeneTx after the acceptance of the investigational new drug application (IND) for GTX-102, $7.0 million for an exclusive, sublicensable, worldwide license to NAV® AAV8 and AAV9 Vectors from REGENXBIO, Inc and non-cash stock-based compensation of $20.2 million. The cost of sales for the three months ended March 31, 2020 was negative $3.5 million due to a credit with an estimated value of $4.6 million that was agreed to during the quarter with a contract manufacturer in exchange for certain Mepsevii inventory batches that did not meet specified quality standards in prior quarters. Total operating expenses in the first quarter of 2019 were $117.4 million, which includes non-cash stock-based compensation of $20.2 million. The increase in total operating expenses is due to the increase in commercial, development, and general and administrative costs as the company commercializes, grows, and advances its portfolio.

For the first quarter of 2020, Ultragenyx reported a net loss of $119.0 million, or $2.05 per share, basic and diluted, compared with a net loss for the first quarter of 2019 of $96.8 million, or $1.82 per share, basic and diluted. The loss for the first quarter of 2020 includes unrealized gains of $7.7 million from the fair value adjustments on the investment in Arcturus equity securities and non-cash interest expense on the liability related to the sale of future royalties of $8.1 million. The net loss for the first quarter of 2020 reflected cash used in operations of $95.2 million compared to $95.8 million for the same period in 2019.

Cash, Cash Equivalents and Investments

Cash, cash equivalents, and investments were $705.0 million as of March 31, 2020. This includes $75.0 million from the Daiichi Sankyo common stock purchase which closed in March 2020, but excludes the $125.0 million upfront license payment from the Daiichi Sankyo strategic manufacturing partnership which was received in April 2020.

2020 Guidance

Crysvita Guidance in Ultragenyx Territories
The company currently maintains the guidance range for 2020 Crysvita revenue in the Ultragenyx territories to be between $125.0 million and $140.0 million, although it continues to monitor the COVID-19 pandemic situation. 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.

2020 Expected Net Cash Burn Rate
The company also expects a more than 20 percent reduction in net cash burn (net cash used in operations plus capital expenditures) in 2020 compared to 2019 due to a combination of financial discipline in spending with flattening operating expense growth, combined with a significant anticipated growth in revenue.

Corporate Update

Gene Therapy Manufacturing: Strategic partnership and non-exclusive license and technology access agreement with Daiichi Sankyo

- Ultragenyx granted Daiichi Sankyo a non-exclusive license on March 31, 2020 to intellectual property, including know-how and patent applications, with respect to its HeLa PCL and HEK293 transient transfection manufacturing technology platforms for AAV-based gene therapy products. Daiichi Sankyo made an upfront payment in April 2020 of $125.0 million pursuant to the license agreement and in March 2020 closed on the purchase of 1,243,913 shares of Ultragenyx common stock in exchange for $75.0 million, or an effective purchase price of approximately $60 per share. Daiichi Sankyo will pay an additional $25.0 million upon completion of the technology transfer of the HeLa PCL and HEK293 platforms, as well as single-digit royalties on net sales of products manufactured in either system.

Program Updates and Upcoming Milestones

UX007 for Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): New Drug Application (NDA) under review by U.S. FDA

- The U.S. FDA is currently reviewing the NDA and has set a Prescription Drug User Fee Act (PDUFA) date of July 31, 2020. The review process is currently on track for a decision by the PDUFA date. The anticipated clinical and manufacturing inspections for UX007 have been successfully completed and, at this time, the FDA has not indicated that any additional inspections would be required for completion of their review.

Crysvita for Tumor-Induced Osteomalacia (TIO): Supplemental Biologics License Application (sBLA) under review by U.S. FDA

- The U.S. FDA accepted for review the sBLA and has set a PDUFA date of June 18, 2020. The review process is currently on track for a decision by the PDUFA date and the FDA has not indicated, at this time, that any inspections would be required for completion of their review.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Positive data from higher dose cohort of Phase 1/2 study; prophylactic steroid cohort planned and Phase 3 study planning underway

- Positive data from this study indicated two confirmed responders and a potential third responder out of three total patients in Cohort 3, as well as a new responder in Cohort 2. There are currently up to six responders of the nine dosed to date with a more consistent response at higher doses. An update with currently available data from the first three cohorts of the study will be presented at the virtual American Society of Gene & Cell Therapy (ASGCT) on May 13.

- A fourth cohort of three patients at the same 1.0 × 10^13 GC/kg dose is planned, using prophylactic steroids. Dosing in this cohort is currently on hold, but data are still expected in the second half of 2020, barring significant further delays related to COVID-19. Ultragenyx is simultaneously planning the Phase 3 study and is currently planning to hold an end-of-phase 2 meeting with the FDA based on the first three cohorts. The Phase 3 study is currently expected to begin enrollment in the first half of 2021.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive data from Phase 1/2 study; enrollment complete in confirmatory cohort and data expected in second quarter 2020

- Enrollment is complete in the confirmatory cohort of three patients at the second dose level (6.0 x 10^12 GC/kg). An update with currently available data from the confirmatory cohort will be presented at the ASGCT meeting on May 15. Certain data may not be available for later patient site visits due to COVID-19 related delays. Depending on the results of the confirmatory cohort, a Phase 3 study could begin in late 2020.

GTX-102 for Angelman Syndrome (AS): Partnered program with GeneTx; First two patients enrolled

- The two patients in the first cohort of the Phase 1/2 study of GTX-102 have been enrolled and continue through dose escalation. Preliminary data are expected in first half of 2021.
- In February 2020, Ultragenyx paid $25.0 million after the acceptance of the IND to maintain its option to acquire GeneTx until the earlier of 30 months from the first dosing of a patient in the Phase 1/2 study (subject to extensions) or 90 days after results are available from that study.
- In May 2020, the U.S. FDA granted Fast Track designation to GTX-102 for the treatment of Angelman syndrome.

UX701 for Wilson Disease: IND anticipated by the end of the year

- An IND application for UX701 gene therapy for Wilson disease, a larger rare metabolic disease, is expected by the end of 2020 barring any delays in nonclinical or manufacturing activities. UX701 will be the second clinical program to utilize the company's HeLa manufacturing system, in addition to the hemophilia A program being developed by our collaborative partner, Bayer.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Wednesday, May 6, 2020, at 2 p.m. PT/ 5 p.m. ET to discuss first quarter 2020 financial results and provide a corporate update. The live and replayed webcast of the call will be available through the company's website at http://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 9455159. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product 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, Ultragenyx's reliance on its third party partner, Kyowa Kirin Co. ,Ltd., for the supply of Crysvita, the effects from the COVID-19 pandemic on the company's business and operating results, smaller than anticipated market opportunities for the company's products and product candidates, the company's evolving commercial infrastructure, uncertainties related to insurance coverage and reimbursement approval for the company's products, manufacturing risks, the uncertainties inherent in the clinical drug development process, including the potential for substantial delays and the risk that earlier study results may not be predictive of future study results, the lack of predictability in the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), 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 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 on February 14, 2020, 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 March 31,	
	2020	**2019**
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 27,215	$ 14,238
Product sales	6,479	3,934
Non-cash collaboration royalty revenue	2,615	—
Total revenues	36,309	18,172
Operating expenses:		
Cost of sales	(3,503)	452
Research and development	112,961	78,105
Selling, general and administrative	47,516	38,829
Total operating expenses	156,974	117,386
Loss from operations	(120,665)	(99,214)
Change in fair value of investment in Arcturus equity securities	7,668	—
Non-cash interest expense on liability related to the sale of future royalties	(8,082)	—
Other income (expense), net	2,463	2,674
Loss before income taxes	(118,616)	(96,540)
Provision for income taxes	(409)	(216)
Net loss	$ (119,025)	$ (96,756)
Net loss per share, basic and diluted	$ (2.05)	$ (1.82)
Shares used in computing net loss per share, basic and diluted	57,995,999	53,209,215

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

	March 31, 2020	**December 31, 2019**
Balance Sheet Data:		
Cash, cash equivalents, and available-for-sale investments	$ 704,989	$ 760,404
Working capital	697,460	747,717
Total assets	1,233,140	1,135,496
Total stockholders' equity	609,146	653,764

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