Ultragenyx Reports Third Quarter 2020 Financial Results and Corporate Update

October 27, 2020

Third quarter 2020 total revenue of $81.5 million and Crysvita[1] revenue of $37.3 million

Increased lower end of Crysvita[1] guidance range; updated full year range is $130 million to $140 million

Dojolvi launch off to a strong start

NOVATO, Calif., Oct. 27, 2020 (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 September 30, 2020 and updated its full year 2020 guidance.

“Crysvita continues to deliver meaningful revenue growth and with the launches of Dojolvi and Crysvita for TIO, we are now generating sales from three products across four indications,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “The Angelman program with GeneTx recently read out very promising, early data for this neurological disorder demonstrating the potential to substantially improve clinical symptoms in that disease. The strategic collaboration with Solid Biosciences we recently announced adds an important potential Duchenne therapy to our preclinical pipeline and a sixth program to our strong gene therapy franchise.”

Third Quarter 2020 Financial Results

Net Revenues
For the third quarter of 2020, Ultragenyx reported $81.5 million in total revenue. Ultragenyx recognized $37.3 million in total Crysvita revenue in the Ultragenyx territories, which includes $34.1 million in collaboration revenue in the North American profit share territory and net product sales in other regions of $3.3 million. Total royalty revenue related to European Crysvita sales were $3.3 million. Mepsevii® (vestronidase alfa) product sales for the third quarter of 2020 was $4.1 million. Dojolvi (triheptanoin) product sales in the initial quarter of our commercial launch were $3.9 million. Total revenue for the third quarter also includes $32.9 million of revenue related to the collaboration and license agreement with Daiichi Sankyo that was executed in March 2020.

Revenue for the nine months ended September 30, 2020 was $179.5 million, including $98.5 million in total Crysvita revenue in the Ultragenyx territories. Crysvita collaboration revenue in the North American profit share territory was $91.1 million and net Crysvita product sales in other regions were $7.4 million. Total royalty revenue related to European Crysvita royalties was $10.9 million, which includes $1.5 million recognized on sales that occurred prior to January 1, 2020. Mepsevii product revenue for the nine months ended September 30, 2020 was $11.7 million. Dojolvi product revenue was $6.6 million. The technology transfer services with Daiichi Sankyo were initiated during the second quarter of 2020. For the nine months ended September 30, 2020, revenue related to this agreement was $51.7 million.

Operating Expenses
Total operating expenses for the third quarter of 2020 were $131.8 million, including non-cash stock-based compensation of $20.3 million. This compares to total operating expenses for the third quarter of 2019, which were $143.8 million and total non-cash stock-based compensation of $19.9 million.

Total operating expenses for the nine months ended September 30, 2020 were $413.5 million, which includes $25.0 million to maintain the option to acquire GeneTx, $7.0 million to license certain vectors from REGENXBIO, and non-cash stock-based compensation of $62.9 million. This is compared with $397.8 million for the same period in 2019, which includes a $15.6 million research and development expense from the Arcturus collaboration amendment and non-cash stock-based compensation of $62.3 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 third quarter of 2020, Ultragenyx reported net loss of $68.8 million, or $1.13 per share basic and diluted, compared with a net loss for the third quarter of 2019 of $113.0 million, or $1.96 per share, basic and diluted. For the nine months ended September 30, 2020, net loss was $162.6 million, or $2.73 per share, basic and diluted, compared with a net loss for the same period in 2019 of $308.9 million, or $5.50 per share, basic and diluted. The net loss for the third quarter of 2020 and the net loss for the nine months ended September 30, 2020 includes an $11.5 million unrealized loss and a $91.3 million unrealized gain for the three and nine months ended September 30, 2020, respectively, from the fair value adjustment on the investment in Arcturus equity. Net cash used in operations for the first nine months of 2020 was $69.8 million, compared to net cash used of $273.3 million for the same period in 2019.

Cash, Cash Equivalents and Investments
Cash, cash equivalents, and investments were $765.5 million as of September 30, 2020.

2020 Financial Guidance

Crysvita Guidance in Ultragenyx Territories[1]
The lower end of the range for 2020 Crysvita revenue in Ultragenyx territories[1] has been increased to $130.0 million from $125.0 million. The updated guidance range is $130.0 million to $140.0 million.

Program Updates and Upcoming Milestones

Dojolvi for Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): Launched on July 22, 2020

- The U.S. FDA approved Dojolvi for the treatment of pediatric and adult patients for all forms of LC-FAOD with a molecularly-confirmed diagnosis. Dojolvi is the first FDA-approved therapy for these lifelong and life-threatening genetic disorders and is now available to patients in the U.S.

- Dojolvi has been submitted for approval with ANVISA in Brazil and has been submitted to Health Canada after being granted priority review. Discussions with EU regulators are ongoing. Requests for named patient access will continue to be supported during the ongoing discussions and review in these countries.

GTX-102 for Angelman Syndrome (AS) Conducted by Partner, GeneTx: Positive interim Phase 1/2 data demonstrating substantial improvements in five out of five patients treated

- Preliminary results from the first five patients treated indicate substantial improvements in all patients in at least two disease domains including communication, behavior, sleep, gross motor function, and fine motor function as measured by the Clinical Global Impression of Improvement Scale for Angelman Syndrome.
- After reaching substantially higher doses as planned per protocol, all five patients experienced a serious adverse event (SAE) of lower extremity weakness believed to be related to local inflammation due to GTX-102. Following these events, the companies paused enrollment and dosing. These serious events were assessed as mild or moderate in severity. Four out of five patients have fully recovered with the fifth nearly fully recovered.
- The study protocol will be amended to reduce the dose-level range and modify the administration process, which is expected to reduce further drug-related SAEs. The companies will obtain agreement on these modifications with the US FDA prior to resuming enrollment and dosing.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Prophylactic steroid cohort is ongoing

- A fourth cohort of three patients at the same cohort 3 dose of 1.0 × 10^13 GC/kg is ongoing using prophylactic steroids. Data from this cohort are expected by the end of 2020. Phase 3 initiation is expected in 2021.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Prophylactic steroid cohort has been added, no impact to timing of Phase 3 study

- A fourth cohort of three patients is ongoing at 6.0 x 10^12 GC/kg, the same dose as cohorts two and three, with a prophylactic steroid regimen. Data from this cohort are not expected to impact the timing of a potential Phase 3 study. Phase 3 initiation is expected in the first half of 2021.

Crysvita for X-Linked Hypophosphatemia (XLH): Approved by European Commission (EC) for treatment of XLH in older adolescents and adults

- The European Commission (EC) approved Crysvita for the treatment of XLH in older adolescents and adults. This expanded approval adds to the prior indication, which included children and adolescents with growing skeletons, to now include adolescents with radiographic evidence of bone disease, regardless of growth status, as well as adults with XLH are now also eligible for treatment with Crysvita.

Crysvita for Tumor-Induced Osteomalacia (TIO): Launched in the United States

- The US FDA approved a second indication for Crysvita in June 2020. Crysvita is now available for the treatment of fibroblast growth factor 23 (FGF23)-related hypophosphatemia in TIO associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adults and pediatric patients 2 years of age and older.

Business Development Update

Pre-clinical Gene Therapy: Strategic Collaboration with Solid Biosciences to Develop and Commercialize New Gene Therapies for Duchenne Muscular Dystrophy

- Strategic collaboration combines Solid's differentiated microdystrophin construct and Ultragenyx's HeLa PCL manufacturing platform for use with AAV8 and variants. Under the terms of the collaboration, Ultragenyx was granted an exclusive license for the use of Solid's proprietary microdystrophin construct with AAV8 and variants. Ultragenyx has made a $40 million investment in Solid and has agreed to pay up to $255 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales at low double digit to mid-teens percentages.

Corporate Update

Executive Team Hires

- Ernie Meyer was hired as Chief Human Resources Officer. In this newly created role, Mr. Meyer leads all aspects of the Global Human Resources and Facilities operations and serves on the Executive Leadership Team.
- Mardi Dier was hired as Chief Financial Officer, following the planned transition of Shalini Sharp. In this role, Ms. Dier is responsible for leading the corporate finance, strategy, investor relations, corporate communications, and information technology functions, and serves on the Executive Leadership Team.

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, Tuesday, October 27, 2020, at 2 p.m. PT/ 5 p.m. ET to discuss the third 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 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 4067577. 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, the effects from the COVID-19 pandemic on the company's commercialization activities, business and operating results, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, uncertainties related to insurance coverage and reimbursement status of the company's newly approved products, the company's evolving integrated commercial organization, 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 July 30, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Joshua Higa
(415) 475-6370

Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 66,924	$ 21,585	$ 144,300	$ 55,070
Product sales	11,215	4,215	25,760	13,051
Non-cash collaboration royalty revenue	3,331	—	9,428	—
Total revenues	81,470	25,800	179,488	68,121
Operating expenses:				
Cost of sales	2,348	2,683	648	3,901
Research and development	87,314	100,144	280,984	274,294
Selling, general and administrative	42,123	41,006	131,891	119,647
Total operating expenses	131,785	143,833	413,523	397,842
Loss from operations	(50,315)	(118,033)	(234,035)	(329,721)
Change in fair value of investments in equity securities	(11,520)	2,166	91,348	11,994
Non-cash interest expense on liability related to the sale of future royalties	(8,582)	—	(25,093)	—
Other income, net	1,885	3,166	6,362	9,527
Loss before income taxes	(68,532)	(112,701)	(161,418)	(308,200)
Provision for income taxes	(313)	(293)	(1,137)	(722)
Net loss	$ (68,845)	$ (112,994)	$ (162,555)	$ (308,922)
Net loss per share, basic and diluted	$ (1.13)	$ (1.96)	$ (2.73)	$ (5.50)
Weighted-average shares used in computing net loss per share, basic and diluted	60,687,177	57,707,694	59,564,163	56,161,996

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

	September 30, 2020	December 31, 2019
Balance Sheet Data:		
Cash, cash equivalents, and available-for-sale investments	$ 765,520	$ 760,404
Working capital	670,817	747,717
Total assets	1,268,385	1,135,496
Total stockholders' equity	666,191	653,764