Ultragenyx Reports Fourth Quarter and Full Year 2020 Financial Results and Corporate Update

February 11, 2021

2020 total revenue of $271.0 million and 2020 Crysvita[1] revenue to Ultragenyx of $138.9 million

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

Strong Dojolvi launch continues with approximately 130 patients on reimbursed commercial therapy in the United States

Six programs in clinical trials in 2021, including three pivotal stage gene therapy trials

NOVATO, Calif., Feb. 11, 2021 (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 and full year ended December 31, 2020 and reaffirmed its financial guidance for 2021.

“Last year was a transformative year as we received two approvals, generated clinically meaningful data in our gene therapy and antisense oligonucleotide programs, and completed several strategic business development transactions,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In addition to growing our global commercial footprint in 2021, we will also have a strong, diverse, late-stage clinical development portfolio. We will initiate three pivotal gene therapy studies, a fourth pivotal study with our recently in-licensed biologic, setrusumab, for osteogenesis imperfecta, make additional progress in the Angelman syndrome study, and bring our first mRNA program to the clinic for glycogen storage disease type III.”

Fourth Quarter and Full Year 2020 Financial Results

Net Revenues
For the fourth quarter of 2020, Ultragenyx reported $91.5 million in total revenue. Ultragenyx recognized $40.4 million in Crysvita (burosumab) revenue in the Ultragenyx territories, which includes $37.5 million in collaboration revenue in the North American profit share territory and net product sales in other regions of $2.9 million. Total royalty revenue related to European Crysvita sales were $3.6 million. Dojolvi (triheptanoin) product sales in the fourth quarter of 2020 were $6.4 million. Mepsevii (vestronidase alfa) product sales for the fourth quarter of 2020 were $3.7 million. Total revenue for the fourth quarter also includes $37.5 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, 2020 was $271.0 million, including $138.9 million in Crysvita revenue in the Ultragenyx territories. Crysvita collaboration revenue in the North American profit share territory was $128.6 million and net Crysvita product sales in other regions were $10.4 million. Total royalty revenue related to European Crysvita royalties was $14.5 million, which includes $1.5 million recognized on sales that occurred prior to January 1, 2020. Dojolvi product revenue for the year ended December 31, 2020 was $13.0 million. Mepsevii product revenue was $15.3 million. For the year ended December 31, 2020, revenue related to our strategic manufacturing partnership with Daiichi Sankyo was $89.2 million.

Operating Expenses
Total operating expenses for the fourth quarter of 2020 were $187.6 million, including non-cash stock-based compensation of $22.8 million. This compares to total operating expenses for the fourth quarter of 2019, which were $130.0 million and total non-cash stock-based compensation of $19.7 million.

Total operating expenses for the year ended December 31, 2020 were $601.1 million, which includes $25.0 million to maintain the option to acquire GeneTx, $13.2 million from a one-time in-process R&D expense related to the strategic collaboration with Solid Biosciences, $7.0 million to license certain vectors from REGENXBIO, and non-cash stock-based compensation of $85.7 million. This is compared with $527.9 million for the same period in 2019, which includes a $15.6 million research and development expense from the Arcturus collaboration amendment, $20.0 million for the upfront payment on the GeneTx agreement, and non-cash stock-based compensation of $82.0 million. The increase in total operating expenses was due to the increase in commercial, on-going clinical and preclinical development, and general and administrative costs as the company commercializes, grows, and advances its portfolio.

For the fourth quarter of 2020, Ultragenyx reported net loss of $24.0 million, or $0.37 per share basic and diluted, compared with

a net loss for the fourth quarter of 2019 of $93.8 million, or $1.62 per share, basic and diluted. For the year ended December 31, 2020, net loss was $186.6 million, or $3.07 per share, basic and diluted, compared with a net loss for the same period in 2019 of $402.7 million, or $7.12 per share, basic and diluted. The net loss for the fourth quarter of 2020 and the net loss for the year ended December 31, 2020 includes a $79.1 million and $170.4 million increase in the fair value of investments in equity securities, respectively. Net cash used in operations for the year ended December 31, 2020 was $132.2 million, compared to net cash used of $345.4 million for the same period in 2019.

Cash, Cash Equivalents and Marketable Debt Securities
Cash, cash equivalents, and marketable debt securities were $1.2 billion as of December 31, 2020.

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 for the North American profit share region and the other regions where product sales are recognized (Latin America and Turkey).

Program Updates and Upcoming Milestones

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

- Five months into the United States launch of Dojolvi for LC-FAOD, the company has received approximately 190 completed start forms from approximately 90 unique prescribers. This has led to approximately 130 patients on reimbursed therapy as of the end of December, a 117% increase over the prior quarter.
- In Canada, Dojolvi has been granted priority review by Health Canada and a new drug submission was filed in August 2020, with a decision expected soon. Dojolvi has been submitted to the Brazilian Health Regulatory Agency (ANVISA) seeking marketing authorization. Discussions with the European Medicines Agency (EMA) are ongoing. Responses to named patient treatment requests continue in the EU.

Three global, pivotal stage gene therapy clinical studies are expected in 2021

- DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Following Scientific Advice with the EMA and an End of Phase 2 (EOP2) meeting with the U.S. Food and Drug Administration (FDA), the company currently expects to initiate a Phase 3 study in the first half of 2021.
- DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Based on initial Scientific Advice from the EMA and following a planned EOP2 meeting with the FDA, the company currently expects to initiate a Phase 3 study in the second half of 2021.
- UX701 for Wilson disease: The Investigational New Drug (IND) application has cleared and the program recently received Fast Track Designation from the FDA. The company currently expects to initiate a seamless, single-protocol Phase 1/2/3 study in the first half of 2021.

GTX-102 for Angelman Syndrome, partnered with GeneTx Biotherapeutics: Study expected to resume in first half 2021 with additional data anticipated in second half of 2021

- GeneTx, with Ultragenyx support, submitted to the FDA a substantial information amendment to the IND including follow-up safety information for the five patients dosed and toxicology data in nonhuman primates that demonstrate no evidence of this safety issue at higher repeat dosing. GeneTx has received feedback and questions from the FDA based on this submission and GeneTx and Ultragenyx have filed a response to FDA and are working on an amendment to the protocol.
- A Clinical Trial Application (CTA) has been submitted in Canada and a protocol and information amendment similar to what has been proposed to FDA will be submitted. GeneTx, with Ultragenyx support, is in the process of expanding the study to other countries using the amended dosing and administration plan.
- The companies currently expect the Phase 1/2 study to resume enrollment and dosing in the first half of 2021, following resolution of FDA requests and approval to proceed.

UX143 (setrusumab) for Osteogenesis Imperfecta, in collaboration with Mereo BioPharma: Phase 2/3 in pediatric patients anticipated in second half of 2021

- The companies expect to conduct a Phase 2/3 study in pediatric patients that first focuses on determining the optimal dose based on increases in bone production via changes in serum P1NP levels over two months and an acceptable safety profile. Following determination of the dose, the study is intended to transition into a pivotal Phase 3 study, evaluating

fracture reduction over an estimated 15 to 24 months. Final study design and endpoints are pending regulatory review.

UX053 for Glycogen Storage Disease Type III or Debrancher deficiency: IND on track in the first half of 2021

- An IND for the company's first mRNA program, UX053, for the treatment of GSDIII debrancher deficiency is on track for the first half of 2021, with a Phase 1/2 study expected to initiate in the second half of 2021.

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, February 11, 2021, at 2 p.m. PT/ 5 p.m. ET to discuss the fourth quarter and year 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 6391931. 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 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 October 27, 2020, and its subsequent annual and periodic reports filed with the Securities and Exchange Commission.

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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,	
	2020	**2019**	**2020**	**2019**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 75,015	$ 28,423	$ 219,315	$ 83,493
Product sales	12,960	7,170	38,720	20,221
Non-cash collaboration royalty revenue	3,567	—	12,995	—
Total revenues	91,542	35,593	271,030	103,714
Operating expenses:				
Cost of sales	5,481	5,107	6,129	9,008
Research and development	131,100	83,061	412,084	357,355
Selling, general and administrative	51,042	41,877	182,933	161,524
Total operating expenses	187,623	130,045	601,146	527,887
Loss from operations	(96,081)	(94,452)	(330,116)	(424,173)
Change in fair value of equity investments	79,055	1,419	170,403	13,413
Non-cash interest expense on liability related to the sale of future royalties	(8,198)	(1,135)	(33,291)	(1,135)
Other income (expense), net	1,283	2,924	7,645	12,451
Loss before income taxes	(23,941)	(91,244)	(185,359)	(399,444)
Provision for income taxes	(70)	(2,561)	(1,207)	(3,283)
Net loss	$ (24,011)	$ (93,805)	$ (186,566)	$ (402,727)
Net loss per share, basic and diluted	$ (0.37)	$ (1.62)	$ (3.07)	$ (7.12)
Shares used in computing net loss per share, basic and diluted	64,661,831	57,808,025	60,845,550	56,576,885

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

	December 31, 2020	December 31, 2019
Balance Sheet Data:		

Cash, cash equivalents, and marketable debt securities	$ 1,212,039	$ 760,404
Working capital	1,105,695	747,717
Total assets	1,759,555	1,135,496
Total stockholders' equity	1,154,375	653,764