Ultragenyx Reports Fourth Quarter and Full Year 2019 Financial Results and Corporate Update

February 13, 2020

2019 total revenue is $103.7 million; 2019 Crysvita® (burosumab) revenue to Ultragenyx is $87.3 million

2020 Crysvita revenue in Ultragenyx territories guidance of $125 million to $140 million reaffirmed

NOVATO, Calif., Feb. 13, 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 and full year ended December 31, 2019 and reaffirmed its financial guidance for 2020.

“In 2019 we created a substantial foundation based on the strong launch of Crysvita and the validation of our gene therapy platform across multiple clinical programs,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In 2020, we anticipate building on this momentum as we look to the potential approvals of UX007 for LC-FAOD and Crysvita for TIO. We also expect to advance our gene therapy programs into pivotal studies and bring two new programs into the clinic while continuing to exercise financial discipline.”

Fourth Quarter and Full Year 2019 Financial Results

Net Revenues
For the fourth quarter of 2019, Ultragenyx reported $35.6 million in total revenue. Ultragenyx recognized $29.9 million in total Crysvita revenue. This includes $26.1 million in collaboration revenue in the North American[1] profit share territory and $2.2 million in royalty revenue in the European territory from the collaboration and license agreement with Kyowa Kirin Co., Ltd (KKC). Net product sales for Crysvita in other regions were $1.6 million. Mepsevii® (vestronidase alfa) product revenue for the fourth quarter of 2019 was $4.4 million, and UX007 named patient revenue was $1.2 million. Ultragenyx recognized $0.1 million in revenue from its research agreement with Bayer.

Net revenue for the year ended December 31, 2019 totaled $103.7 million. In 2019, Ultragenyx recognized $87.3 million in total Crysvita revenue. This includes $74.9 million in collaboration revenue in the North American[1] profit share territory and $8.1 million in royalty revenue in the European territory from the collaboration and license agreement with KKC. Net product sales for Crysvita in other regions totaled $4.3 million. Mepsevii product revenue for the year ended December 31, 2019 was $12.6 million, and UX007 named patient revenue was $3.3 million. Ultragenyx recognized $0.5 million in revenue from its research agreement with Bayer in the year ended December 31, 2019.

Operating Expenses
Total operating expenses for the fourth quarter of 2019 were $130.0 million compared with $106.6 million for the same period in 2018, including non-cash stock-based compensation of $19.7 million and $21.1 million in the fourth quarter of 2019 and 2018, respectively. Total operating expenses for the year ended December 31, 2019, were $527.9 million, which includes $15.6 million in expenditures for Arcturus collaboration and license amendment and $20 million for the upfront payment on the GeneTx agreement, compared with operating expenses of $422.9 million for the same period in 2018. The operating expenses include non-cash stock-based compensation of $82.0 million and $80.1 million in the full year of 2019 and 2018, respectively. 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 pipeline.

For the fourth quarter of 2019, Ultragenyx reported a net loss of $93.8 million, or $1.62 per share, basic and diluted, compared with a net loss for the fourth quarter of 2018 of $87.8 million, or $1.73 per share, basic and diluted. For the year ended December 31, 2019, the net loss was $402.7 million, or $7.12 per share, basic and diluted, compared with a net loss for the same period in 2018 of $197.6 million, or $3.97 per share, basic and diluted. The loss for the fourth quarter of 2019 and for the year ended December 31, 2019 includes unrealized gains of $1.4 million and $13.4 million, respectively, from the fair value adjustments on the investment in Arcturus equity securities. The loss from the full year 2018 was reduced by the gain on the sale of the Mepsevii priority review voucher (PRV) in January 2018 for net proceeds of $130.0 million and a $40.3 million gain from Ultragenyx’s portion of the sales of the PRV received with the Crysvita approval. The net loss for the full year 2019 reflected cash used in operations of $345.4 million compared to $290.6 million for the same period in 2018.

Cash, Cash Equivalents and Investments
Cash, cash equivalents, and investments were $760.4 million as of December 31, 2019, including proceeds of $320.0 million received from the sale of the company's royalty interest in Crysvita in the European territory.

2020 Guidance

Crysvita Guidance in Ultragenyx Territories
In 2020, the company continues to expect Crysvita revenue in the Ultragenyx territories to be between $125 million and $140 million, which excludes the European territory revenue. Ultragenyx territories include the collaboration revenue from the North American profit share territory (US and Canada) and other regions where revenue from product sales are recognized by Ultragenyx (Latin America, Turkey). In December 2019, the European territory royalty revenue rights were sold to Royalty Pharma. As a result, the company will no longer receive cash payments from the EU territory royalty revenue rights beginning January 1, 2020, until the respective threshold amount is met; however, the company will continue to record the royalty as "non-cash" revenue.

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 the significantly larger growth in revenue.

Recent Updates and Upcoming Milestones

Crysvita for X-linked Hypophosphatemia: Non-dilutive $320 million sale of future European royalties

- In December 2019, Ultragenyx sold to Royalty Pharma for $320 million its royalty interest in Crysvita in the European territory, where it is being commercialized by Kyowa Kirin Co., Ltd.

Crysvita for Tumor-Induced Osteomalacia (TIO): Supplemental Biologics License Application (sBLA) submitted

- Ultragenyx submitted the sBLA to the U.S. FDA on December 18, 2019 and expects to hear back from FDA on submission acceptance and review designation in February 2020.

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

- The U.S. FDA accepted for review the New Drug Application (NDA) and has set a Prescription Drug User Fee Act (PDUFA) date of July 31, 2020. The FDA has indicated that it is not currently planning to hold an advisory committee meeting to discuss the application. The review process is on track for a decision by the PDUFA date.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Positive data from higher dose cohort of Phase 1/2 study; data from prophylactic steroid cohort in second half 2020

- Recent positive data from Cohort 3 indicated two confirmed responders and a potential third responder out of three total patients, 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.
- Ultragenyx is initiating a fourth cohort (n=3) using prophylactic steroids at the same dose as Cohort 3. Data from the prophylactic steroid cohort are expected in the second half of 2020.

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

- Enrollment is complete in the confirmatory cohort of three patients at the second dose cohort, with data expected in the first half of 2020. Following the results of the confirmatory cohort, a Phase 3 study could begin in the second half of 2020.

GTX-102 for Angelman Syndrome (AS): Partnered program with GeneTx; IND active and enrollment of first patient expected in first half 2020

- An investigational new drug (IND) application is now active and GeneTx has received Institutional Review Board (IRB) approval from the first study site. Enrollment in the Phase 1/2 study of GTX-102 is expected to begin in the first half of 2020.
- In February 2020, Ultragenyx paid $25 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.

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

- An IND application is expected by the end of 2020 for a new gene therapy for Wilson disease, a larger rare metabolic disease. UX701 will be the company's second clinical program to utilize its HeLa manufacturing system.

DTX201 / BAY 2599023 for Hemophilia A: Partnered with Bayer; Cohort 2 data presented at European Association for Haemophilia and Allied Disorders (EAHAD)

- Three cohorts with two patients each, six patients in total, have been dosed with AAVhu37 (DTX201 / BAY 2599023), using material from Ultragenyx's proprietary HeLa manufacturing platform. Longer term dose cohort 1 and new dose cohort 2 data presented at EAHAD showed three of four patients have achieved clinically meaningful FVIII levels. One patient in Cohort 1 has achieved clinically meaningful FVIII levels and has experienced four bleeds since receiving treatment down from 99 bleeds the prior year. Both patients in dose cohort 2 achieved clinically meaningful FVIII levels out to 24 and 30 weeks. Patient 4 has been off treatment and bleed free for seven months as of the data cut-off date. The second patient in dose cohort 2 had mild ALT/AST elevations that was managed with a short tapering course of corticosteroids.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, February 13, 2020, at 2 p.m. PT/ 5 p.m. ET to discuss fourth quarter and full year 2019 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 5068548. The replay of the call will be available for one year.

1: Corrected to North American from U.S.

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, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 6, 2019, 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 December 31,		Year Ended December 31,	
	2019	**2018**	**2019**	**2018**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 28,423	$ 12,797	$ 83,493	$ 41,693
Product sales	7,170	3,464	20,221	9,802
Total revenues	35,593	16,261	103,714	51,495
Operating expenses:				
Cost of sales	5,107	507	9,008	1,146
Research and development	83,061	71,618	357,355	293,998
Selling, general and administrative	41,877	34,476	161,524	127,724
Total operating expenses	130,045	106,601	527,887	422,868
Loss from operations	(94,452)	(90,340)	(424,173)	(371,373)
Gain from sale of priority review vouchers	—	—	—	170,322
Change in fair value of investment in Arcturus equity securities	1,419	—	13,413	—
Non-cash interest expense on liability related to the sale of future royalties	(1,135)	—	(1,135)	—
Other income (expense), net	2,924	2,640	12,451	3,954
Loss before income taxes	(91,244)	(87,700)	(399,444)	(197,097)
Provision for income taxes	(2,561)	(126)	(3,283)	(514)
Net loss	$ (93,805)	$ (87,826)	$ (402,727)	$ (197,611)
Net loss per share, basic and diluted	$ (1.62)	$ (1.73)	$ (7.12)	$ (3.97)
Shares used in computing net loss per share, basic and diluted	57,808,025	50,694,007	56,576,885	49,775,223

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

	December 31, 2019	December 31, 2018
Balance Sheet Data:		
Cash, cash equivalents, and investments	$ 760,404	$ 459,706
Working capital	747,717	447,644
Total assets	1,135,496	719,558
Total stockholders' equity	653,764	608,908

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