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Ultragenyx Reports Fourth Quarter and Full Year 2013 Financial Results

NOVATO, CA – March 24, 2014 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today reported its financial results and business highlights for the fourth quarter and year ended December 31, 2013.

"Ultragenyx completed a landmark year in 2013, during which we achieved multiple milestones in our vision to build a next generation rare disease company," said Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "We have expanded our pipeline to five clinical programs in Phase 1/2 or Phase 2 studies, and with the completion of our initial public offering in early 2014, we believe we are well-positioned to advance multiple programs in parallel to address the significant unmet medical needs of these patients."

Fourth Quarter and Full Year 2013 Financial Results

For the fourth quarter of 2013, Ultragenyx reported a net loss attributable to common stockholders of $18.7 million, or $4.98 per share, basic and diluted, compared with a net loss attributable to common stockholders for the fourth quarter of 2012 of $6.8 million, or $2.83 per share, basic and diluted.

For the year ended December 31, 2013, Ultragenyx reported a net loss attributable to common stockholders of $50.3 million, or $14.87 per share, basic and diluted, compared with a net loss attributable to common stockholders for the year ended December 31, 2012 of $19.6 million, or $14.20 per share, basic and diluted.

Total operating expenses for 2013 were $32.3 million compared with $16.0 million for 2012. Total operating expenses for the fourth quarter of 2013 were $9.5 million compared with $4.7 million for the same period in 2012. The increase in total operating expenses is due to the addition of triheptanoin and KRN23 to Ultragenyx's clinical development pipeline, advancements in the development of sialic acid extended release (SA-ER) and recombinant human beta-glucuronidase (rhGUS), and increased headcount to support the company's growth.

Cash, cash equivalents, and short-term investments were $53.4 million as of December 31, 2013. Based on current operating levels, the company expects that existing cash, cash equivalents and short-term investments, including approximately $121.7 million in net

proceeds received from the initial public offering in February 2014, will be sufficient to fund operations into 2016.

Recent Highlights

KRN23 anti-FGF23 Monoclonal Antibody in X-linked Hypophosphatemia (XLH)

- Results from the Phase 1 single dose study in 38 adult XLH patients were presented at the American Society for Bone and Mineral Research Annual Meeting in October 2013 and published in the *Journal of Clinical Investigation* in February 2014[1]**.** The data demonstrated that KRN23 was well tolerated and increased serum phosphate and vitamin D levels compared to placebo at higher doses.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- In December 2013, we initiated a Phase 1/2 study to evaluate the safety, tolerability, efficacy, and dose of rhGUS in up to five MPS 7 patients between five and 30 years of age.
- In February 2014, results from the treatment of a single patient under an emergency investigational new drug (eIND) application were presented at the Lysosomal Disease Network's 10th Annual World Symposium. The preliminary data showed a reduction in lysosomal storage based on reduced excretion of urinary glycosaminoglycans and a reduction in the size of the enlarged liver and spleen. The patient showed an improvement of pulmonary function and no infusion-associated reactions during the first 14 weeks of treatment.

Triheptanoin in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- In February 2014, we initiated a Phase 2 study in approximately 30 severely affected LC-FAOD patients. A principal goal of the study is to determine the appropriate clinical endpoints and patient population for testing in potential later-stage pivotal studies.
- Multiple investigator-sponsored and compassionate use trials testing triheptanoin in LC-FAOD patients are also ongoing.

Triheptanoin in Glucose Transporter Type-1 Deficiency Syndrome (Glut1 DS)

- In March 2014, we initiated a randomized, double-blind, placebo-controlled, parallel-group Phase 2 study in up to 50 Glut1 DS patients between three and 17 years of age inclusive who are currently not fully compliant with ketogenic diet and continue to have seizures. The primary efficacy objective is the reduction in frequency of seizures compared to placebo.
- Multiple investigator-sponsored and compassionate use trials in Glut1 DS as well as in other indications are also ongoing.

SA-ER in Hereditary Inclusion Body Myopathy (HIBM)

- In December 2013, we released top-line results after 48 weeks of treatment from the Phase 2 randomized study of SA-ER in 47 HIBM patients. The data showed that a modest increase in upper extremity muscle strength composite at the higher dose compared to a decline in the lower dose group was statistically significant. A positive trend was seen in a patient-reported outcome of functional activity. The results were consistent with the 24-week

analysis. SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group.
- We continue to treat patients in an extension study evaluating an increased daily dosage of sialic acid based on the dose dependence observed at weeks 24 and 48 of the Phase 2 study.

Corporate Highlights
- In February 2014, we closed our initial public offering of 6,624,423 shares of common stock at an initial public offering price of $21.00 per share, which includes the exercise in full by the underwriters of their option to purchase up to an additional 864,054 additional shares of common stock. Net proceeds from the offering were approximately $121.7 million, after deducting underwriting discounts and commissions, estimated offering expenses, and a dividend paid to preferred stockholders.
- In January 2014, concurrent with the pricing of our initial public offering, we appointed Clay B. Siegall, Ph.D. and Matthew K. Fust to our board of directors. Dr. Siegall is President, Chief Executive Officer, and Chairman of the Board of Seattle Genetics, Inc. Mr. Fust is former Executive Vice President and Chief Financial Officer of Onyx Pharmaceuticals, Inc.

Anticipated 2014 Program Milestones

KRN23 in XLH
- Release data from the Phase 1/2 adult repeat-dose studies completed by our collaborative partner Kyowa Hakko Kirin Co., Ltd. (KHK).
- Initiate a Phase 2 study in pediatric XLH patients following discussions with multiple regulatory agencies on our pediatric study design.

rhGUS in MPS 7
- Release early urinary glycosaminoglycans data on the first three MPS 7 patients dosed with rhGUS in our Phase 1/2 study in March 2014 at the Annual Clinical Genetics Meeting (ACMG).
- Release interim 12-week data from our Phase 1/2 study of rhGUS in patients with MPS 7 in the second half of 2014.
- Initiate a pivotal Phase 3 study of at least 12 patients in the second half of 2014 if the 12-week Phase 1/2 data are positive.

Triheptanoin in LC-FAOD
- Continue to enroll and treat patients in the ongoing Phase 2 study of triheptanoin in approximately 30 severely affected LC-FAOD patients. We expect that data from this study should be available in 2015.

Triheptanoin in Glut1 DS
- Continue to enroll and treat patients in the ongoing Phase 2 study of triheptanoin in up to 50 Glut1 DS patients. We expect to release data from this trial in 2015.

SA-ER in HIBM

- Present detailed data from the 48-week randomized, controlled Phase 2 study of SA-ER in 47 HIBM patients at the American Academy of Neurology (AAN) Annual Meeting in April. The abstract was one of ten chosen for presentation in a late-breaking news session called the Emerging Sciences session. A brief topline presentation of the results will take place on April 30, 2014 at approximately 6:30pm ET and will be followed by a poster session.
- Release data from the ongoing extension study of SA-ER at a higher daily dosage in late 2014.

About Ultragenyx

Ultragenyx is a development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

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.

[1]Carpenter TO, et al. Randomized trial of the anti-FGF23 antibody KRN23 in X-linked hypophosphatemia. J Clin Invest. 2014. doi:10.1172/JCI72829.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's plans, potential opportunities, expectations, projections, goals, objectives, milestones, strategies, product pipeline, clinical studies, product development, release of data and the potential benefits of its products under development 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, 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 uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings and other matters that could affect the availability or commercial potential of our 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 the Company in general, see Ultragenyx's prospectus filed with the Securities and

Exchange Commission on January 31, 2014, and its future periodic reports to be filed with the Securities and Exchange Commission.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2012	2013	2012	2013
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 3,775	$ 8,204	$ 12,641	$ 27,829
General and administrative	903	1,321	3,344	4,451
Total operating expenses	4,678	9,525	15,985	32,280
Loss from operations	(4,678)	(9,525)	(15,985)	(32,280)
Other expense, net	(253)	(1,792)	(349)	(2,790)
Net loss	$ (4,931)	$ (11,317)	$ (16,334)	$ (35,070)
Net loss attributable to common stockholders	$ (6,813)	$ (18,665)	$ (19,561)	$ (50,289)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.83)	$ (4.98)	$ (14.20)	$ (14.87)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	2,404,852	3,744,525	1,377,207	3,382,489

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

	As of December 31,	
	2012	2013
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 86,190	$ 53,377
Working capital	83,257	49,304
Total assets	88,316	59,649
Convertible preferred stock warrant liability	518	3,419
Convertible preferred stock	111,387	124,930
Deficit accumulated during the development stage	(27,058)	(74,836)
Total stockholders' deficit	(27,047)	(74,821)