Ultragenyx to Present Corporate Update at Analyst and Investor Day on April 17 in New York

April 11, 2019

NOVATO, Calif., April 11, 2019 (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 announced that management and external experts will provide an update to the investment community on the global commercial launch of Crysvita® (burosumab), the company's gene therapy programs and platform including manufacturing capabilities, and preclinical data on the next three Investigational New Drug (IND) applications the company plans to submit to the U.S. Food and Drug Administration. The presentations will be held in New York on Wednesday, April 17, from 8:00 a.m. to 12:15 p.m. ET.

"Ultragenyx is at a great point in its history. The company has launched two products globally and plans to submit an NDA for a third product candidate, has advanced two clinical gene therapy programs, and has moved three more early-stage programs towards INDs in 2020," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "The progress in our pipeline has given us the opportunity to change the lives of patients affected by nine different diseases in nine years since the company was founded."

Ultragenyx Analyst Day Discussion Topics:

Vision, Mission, and Strategy

- Emil Kakkis, M.D., Ph.D., Chief Executive Officer
- Shalini Sharp, Chief Financial Officer

Commercial Operations, Crysvita Launch, and Physicians Perspectives on X-linked hypophosphatemia (XLH) and Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- Vlad Hogenhuis, M.D., Chief Operating Officer
- Erik Harris, Senior Vice President, North America Commercial Operations
- Camille Bedrosian, M.D, Chief Medical Officer, Ultragenyx
- Anthony Portale, M.D., University of California San Francisco; expert on XLH and Crysvita study investigator
- Gerard Vockley, M.D., Ph.D., UPMC Children's Hospital of Pittsburgh; expert on LC-FAOD and UX007 study investigator

Gene Therapy Platform Deep Dive including Manufacturing and Physician Perspectives on Glycogen Storage Disease Type Ia (GSDIa) and Wilson Disease

- Eric Crombez, M.D., Chief Medical Officer, Ultragenyx Gene Therapy
- Sam Wadsworth, Ph.D., Chief Scientific Officer, Ultragenyx Gene Therapy
- Paul Wickman, Ph.D., J.D., Vice President, Intellectual Property
- David Weinstein, M.D., M.Sc., University of Connecticut and Connecticut Children's Medical Center; expert on GSDIa and DTX401 study investigator
- Frederick K. Askari M.D., Ph.D., Michigan Medicine Hepatology Clinic; expert on Wilson Disease

Early Pipeline Review with Focus on Progress in Glycogen Storage Disease III and Creatine Transporter Deficiency and Physician Perspective

- Arjun Natesan, Ph.D., Vice President, Translational Research
- Ton DeGrauw, M.D., Ph.D., Emory University School of Medicine; expert on Creatine Transporter Deficiency

Webcast Details
Ultragenyx will host its Analyst and Investor Day on Wednesday, April 17, 2019, from 8:00 a.m. to 12:15 p.m. ET. The live and replayed webcast will be available through the company's website at http://ir.ultragenyx.com/events.cfm. The replay of the webcast will be available for one year.

About Ultragenyx Pharmaceutical Inc.
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 regarding the potential timing and success of filings for regulatory approvals, 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, such as the regulatory approval process, the timing of regulatory filings, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our 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 20, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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