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Ultragenyx Sells Priority Review Voucher for \$130 million

NOVATO, Calif., Dec. 18, 2017 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced that it has entered into a definitive agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV) to Novartis for \$130,000,000. Ultragenyx was awarded the voucher under a U.S. Food and Drug Administration (FDA) program intended to encourage the development of treatments for rare pediatric diseases. The Company received the PRV in November when MEPSEVII™ (vestronidase alfa) was approved by the U.S. FDA for the treatment of children and adults with Mucopolysaccharidosis VII (MPS VII, Sly syndrome).

"The sale of the PRV provides us with an important source of non-dilutive capital to help advance our pipeline of rare and ultra-rare therapies, and accelerates the availability of these potential therapies to patients," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx.

Under the agreement, Ultragenyx will receive a lump sum payment of \$130 million upon the closing of the transaction, which is subject to customary closing conditions including antitrust review.

About the Rare Pediatric Disease Priority Review Voucher Program

This U.S. FDA program is intended to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a PRV that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor receives the PRV upon approval of the rare pediatric disease product application and it can be sold or transferred.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. The Company has rapidly built and advanced 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.

Forward Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements relating to Ultragenyx's expectations regarding the consummation of the transaction and the potential benefits of the transaction, 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 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 3, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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