



Ultragenyx Enters into Agreement to Sell Rare Pediatric Disease Priority Review Voucher for \$203 Million

October 14, 2026

Agreement marks the Company's second PRV sale within one week, following the previously announced GENGLYCOS™ PRV transaction

NOVATO, Calif., Oct. 14, 2026 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that it has entered into a definitive agreement to sell a Rare Pediatric Disease Priority Review Voucher (PRV) for \$203 million. Ultragenyx received the PRV upon U.S. Food and Drug Administration (FDA) approval of FAYUVI™ (rebisufligene etisparvovec-hopf), also known as UX111, the first-ever FDA-approved treatment for mucopolysaccharidosis type IIIA (MPS IIIA, Sanfilippo syndrome Type A).

"The sale of this PRV delivers meaningful non-dilutive funding that strengthens our ability to provide innovative treatments for rare and ultra-rare diseases while supporting our objective of achieving profitability," said Howard Horn, chief financial officer and executive vice president, corporate strategy. "FAYUVI itself benefited from capital generated from a previous PRV sale, demonstrating the important role the PRV program plays in helping companies develop transformative therapies for rare disease patients."

The closing of the PRV transaction is subject to customary closing conditions, including expiration of the applicable waiting period under the Hart-Scott Rodino Antitrust Improvements Act.

Jefferies LLC is serving as exclusive financial advisor and Gibson, Dunn and Crutcher LLP is serving as legal counsel to Ultragenyx on this transaction.

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

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, 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 and Use of Digital Media

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 and timing of the PRV transaction, the amount and receipt of the cash consideration, and the anticipated use and benefits of the proceeds, including advancing Ultragenyx's pipeline and supporting its path to profitability, 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 Ultragenyx's 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, risks and uncertainties relating to the satisfaction of the closing conditions for the PRV transaction, including expiration of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, the possibility that the transaction may be delayed or may not close, Ultragenyx's ability to realize the anticipated benefits of the transaction and use the proceeds as anticipated, the sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the uncertainties inherent in clinical drug development and the regulatory approval process, risks related to serious or undesirable side effects of Ultragenyx's products and product candidates, manufacturing risks, market acceptance, uncertainty related to insurance coverage and reimbursement, and the availability or commercial potential of Ultragenyx's products and product 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 (SEC) on August 5, 2026, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (<https://ir.ultragenyx.com/>) and LinkedIn website (<https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/>).

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