Ultragenyx Announces Plans to Build Large-scale Gene Therapy Manufacturing Facility to Support Pipeline of Therapies for Rare Diseases

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State-of-the-art facility to be located in Bedford, Massachusetts

NOVATO, Calif., Nov. 09, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for rare and ultra-rare genetic diseases, today announced that it plans to build a new large-scale gene therapy manufacturing facility in Bedford, Massachusetts. The new facility will enable in-house manufacturing of the Company's pipeline of clinical stage adeno-associated virus (AAV)- based gene therapies, including DTX301 for ornithine transcarbamylase (OTC) deficiency, DTX401 for glycogen storage disease type Ia (GSDIa), and UX701 for Wilson disease, as well as other preclinical programs. The company will continue to leverage some contract manufacturing organizations in addition to its own manufacturing facility. Ultragenyx will use both of its gene therapy manufacturing platforms at the new facility: the HeLa producer cell line (PCL) platform which enables large 2,000 liter commercial-scale manufacturing and yields high-quality product from a highly reproducible, highly scalable platform, and the Company's HEK293 transient transfection system.

"We believe that gene therapy is the optimal way to treat many rare and ultra-rare genetic diseases. Our decision to build a state-of-the art manufacturing facility is the logical next step for us as we advance our two clinical-stage programs toward Phase 3 studies, our Wilson Disease program toward IND later this year and make progress in both our HeLa PCL and HEK293 transient transfection manufacturing technology platforms," said Dennis Huang, Chief Technical Operations Officer at Ultragenyx. "Developing internal manufacturing capabilities will allow us to enhance production processes, enabling us to further optimize quality and scale and ultimately reduce the time it takes to bring our gene therapy solutions to patients."

"The Baker-Polito Administration is thrilled that Ultragenyx Pharmaceutical has chosen Massachusetts as home for its new manufacturing facility, bringing new jobs to an already strong life sciences cluster in Bedford," said Secretary of Housing and Economic Development Mike Kennealy, who also serves as co-chair of the Massachusetts Life Sciences Center's Board of Directors. "The Commonwealth remains a global leader in the life sciences because of our talented workforce, our world-class companies and institutions, and our embrace of public-private collaboration. We welcome Ultragenyx and their important work to our ecosystem."

"We are thrilled that Ultragenyx chose Bedford as the location of its new state-of-the-art bio-manufacturing facility, which further strengthens Bedford's life science cluster," said Sarah Stanton, Bedford Town Manager. "We appreciate Ultragenyx's significant investment in our community bringing jobs and economic benefits to Bedford and the region, and look forward to a long-term partnership with Ultragenyx."

The planned Phase I facility will encompass 100,000 square feet and provide important internal capacity to develop and manufacture supply of Ultragenyx's gene therapies for both clinical stage and approved products. The facility will be able to support two independent manufacturing suites with a capacity of 30 runs per year. Construction of the new facility has begun and is expected to be complete in 2023. As the facility becomes fully operational, Ultragenyx expects to hire approximately 100 to 150 full-time employees, over a five-year period across a broad range of functions and skill sets, adding to their existing base of employees in Massachusetts. Ultragenyx already controls land and development rights for an additional 86,000 square feet of Phase II expansion on-site which could be used to double capacity if needed.

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, the company's lack of experience in developing a manufacturing facility, unexpected costs or delays in constructing the facility, the effects from the COVID-19 pandemic on the company's commercialization activities, business and operating results, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, 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, the timing of clinical trial activities and reporting results from same, 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 2, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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