



June 7, 2016

Ultragenyx and Takeda enter into a Collaboration to Develop and Commercialize Therapies for Rare Genetic Diseases

Ultragenyx to license and develop one or more product candidates from Takeda

Takeda to make equity investment in Ultragenyx to fund development

NOVATO, Calif. and OSAKA, Japan, June 07, 2016 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare genetic diseases, and Takeda Pharmaceutical Company Limited ([TSE: 4502](#)), today announced a strategic partnership to develop and commercialize therapies to treat rare genetic diseases.

Ultragenyx will initially receive an exclusive license to one preclinical Takeda product candidate in a pre-determined field of use, and will have an exclusive option to co-develop and co-commercialize the product candidate in additional therapeutic areas. The companies have also established a five-year research collaboration in which Ultragenyx will have the option to license up to five additional Takeda product candidates for rare diseases after the parties agree on and conduct initial validation activities under the purview of a Joint Research Committee.

"This broad collaboration provides Ultragenyx with a product opportunity that is approaching clinical-stage development as well as a potential continued source of new product candidates that will help us achieve our goal of bringing a new therapy into the clinic every one to two years," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. "Takeda has an impressive early pipeline of therapies with potential across a number of rare genetic diseases, and we are pleased that Takeda has chosen to partner with us to bring these therapies to patients with rare diseases that have few or no treatment options."

"Ultragenyx is a rapidly emerging rare disease company, led by a highly experienced and successful management team," said Andrew Plump, M.D., Ph.D., Chief Medical and Scientific Officer of Takeda. "This partnership provides Takeda access to Ultragenyx's strong patient-centric development and regulatory capabilities in the rare disease space, and could create significant value for both companies by delivering important new therapies to patients."

Takeda will receive an exclusive option to commercialize any licensed products resulting from the collaboration in Asia, including Japan. In addition, Takeda receives an option to exclusively license one Ultragenyx pipeline product in Japan. Each company will receive potential development and sales milestone payments and royalties on net sales of licensed products by the other party.

Takeda will invest up to \$65 million in Ultragenyx in two tranches, the first of which will comprise a \$25 million stock purchase along with a \$15 million cash premium at closing. This will be followed at Ultragenyx's option, within 12 months, by a second equity purchase of \$25 million with no additional premium. A potential third equity investment by Takeda is contingent upon Ultragenyx achieving a specific development milestone on a second asset. No additional financial details were disclosed.

The completion of the transactions are subject to Hart-Scott-Rodino Antitrust Improvements Act of 1976 (HSR) review and the satisfaction of other customary closing conditions.

About Ultragenyx

Ultragenyx is a clinical-stage 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. 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.

About Takeda

Takeda Pharmaceutical Company Limited (TSE: 4502) is a global, R&D-driven pharmaceutical company committed to bringing better health and a brighter future to patients by translating science into life-changing medicines. Takeda focuses its research efforts on oncology, gastroenterology and central nervous system therapeutic areas. It also has specific development programs in specialty cardiovascular diseases as well as late-stage candidates for vaccines. Takeda conducts R&D both internally and with partners to stay at the leading edge of innovation. New innovative products, especially in oncology, central nervous system and gastroenterology, as well as its presence in emerging markets, fuel the growth of Takeda. More than 30,000 Takeda employees are committed to improving quality of life for patients, working with our partners in health care in more than 70 countries. For more information, visit <http://www.takeda.com/news>.

Ultragenyx Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding the potential to develop the licensed product candidate in additional therapeutic areas, as well as the potential to develop additional rare disease targets pursuant to the collaboration and the ability to bring new therapies to clinic, 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 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 satisfaction of the HSR requirements and the impact on the timing of the closing, whether any products will be successfully developed and commercialized from the collaboration, uncertainties inherent in the drug development process and other matters that could affect the potential for success of the collaboration, including the sufficiency of existing cash, cash equivalents and short-term investments to fund operations. 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on March 10 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Takeda Forward-Looking Statements

This press release contains "forward-looking statements." Forward-looking statements include all statements other than statements of historical fact, including plans, strategies and expectations for the future, statements regarding the expected timing of filings and approvals relating to the transaction, the expected timing of the completion of the transaction, the ability to complete the transaction or to satisfy the various closing conditions, future revenues and profitability from or growth or any assumptions underlying any of the foregoing. Statements made in the future tense, and words such as "anticipate," "expect," "project," "continue," "believe," "plan," "estimate," "pro forma," "intend," "potential," "target," "forecast," "guidance," "outlook," "seek," "assume," "will," "may," "should," and similar expressions are intended to qualify as forward-looking statements. Forward-looking statements are based on estimates and assumptions made by management that are believed to be reasonable, though they are inherently uncertain and difficult to predict. Investors and security holders are cautioned not to place undue reliance on these forward-looking statements.

Forward-looking statements involve risks and uncertainties that could cause actual results or experience to differ materially from that expressed or implied by the forward-looking statements. Some of these risks and uncertainties include, but are not limited to: required regulatory approvals for the transaction may not be obtained in a timely manner, if at all; the conditions to closing of the transaction may not be satisfied; competitive pressures and developments; applicable laws and regulations; the success or failure of product development programs; actions of regulatory authorities and the timing thereof; changes in exchange rates; and claims or concerns regarding the safety or efficacy of marketed products or product candidates in development.

The forward-looking statements contained in this press release speak only as of the date of this press release, and neither Ultragenyx nor Takeda undertakes any obligation to revise or update any forward-looking statements to reflect new information, future events or circumstances after the date of the forward-looking statement. If one or more of these statements is updated or corrected, investors and others should not conclude that additional updates or corrections will be made.

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