Ultragenyx Pharmaceutical and Arcturus Therapeutics Announce a Research Collaboration and License Agreement to Develop RNA Therapeutics for Rare Diseases

October 29, 2015

NOVATO, CA – October 29, 2015 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, and Arcturus Therapeutics, Inc., a leading RNA medicines company, today announced that they have entered into a research collaboration and license agreement to discover and develop messenger RNA (mRNA) therapeutics to certain rare disease targets using Arcturus' UNA Oligomer™ chemistry and LUNAR™ nanoparticle delivery platform.

Under the terms of the agreement, Ultragenyx will make an upfront payment of $10 million to Arcturus. During the initial phase of the collaboration, Arcturus will design and optimize mRNA therapeutics for two selected rare disease targets. Ultragenyx has the option to add up to eight additional rare disease targets during the collaborative research period. Ultragenyx will be responsible for the development and commercialization of all products under the collaboration. Arcturus will be entitled to preclinical, clinical, regulatory, and sales milestone payments of up to $156 million for each target, as well as reimbursement of all research expenses and mid-single to low double-digit royalties on commercial sales.

"This collaboration with Arcturus will help us address a wider range of rare diseases than is possible with currently available approaches," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. "The joint program combines Arcturus' technology platform, which we believe solves some of the key issues associated with mRNA therapeutics, with Ultragenyx's expertise in rare disease drug development."

The Ultragenyx-Arcturus partnership is an ideal combination to leverage the potential of mRNA therapeutics to provide important new treatments for rare disease patients," said Joseph E. Payne, President and CEO of Arcturus Therapeutics. "Arcturus is thrilled to work with Ultragenyx, one of the premier rare disease companies, to identify treatments for challenging rare disease targets using our messenger RNA therapeutics platform."

About Arcturus
Founded in 2013 and based in San Diego, Arcturus Therapeutics, Inc. is an RNA medicines company with enabling technologies – UNA Oligomer™ chemistry and LUNAR™ nanoparticle delivery. Arcturus' versatile RNA therapeutics platform can be applied toward all types of RNA medicines including small interfering RNA, messenger RNA, antisense RNA, microRNA and gene editing therapeutics. The company owns unlocked nucleomonomer agent (UNA) technology including UNA Oligomers™, which are covered by its patent portfolio (34 patents and patent applications, issued in the U.S. and other countries). Arcturus' proprietary UNA technology can be used to target any gene in the human genome, as well as viral genes, and other species for therapeutic purposes. For more information, visit www.ArcturusRx.com.

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.

Forward-Looking Statements
Except for the historical information contained herein, the matters set forth in this press release, including statements regarding potential milestone and royalty payments and the potential to develop additional rare disease targets pursuant to the collaboration, 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 uncertainties inherent in the drug development process and other matters that could affect 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 August 14, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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