Ultragenyx and Arcturus Therapeutics Expand Existing Research Collaboration and License Agreement to Develop Additional Nucleic Acid Therapies for Rare Diseases

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- *Scope of the collaboration expands to include up to 12 rare disease targets and includes Arcturus nucleic acid technologies to enable mRNA, DNA, and siRNA therapeutics*
- *$30 million of upfront payments to Arcturus, including $6 million cash for collaboration agreement amendment and a $24 million equity investment at $10 per share*
- *Ultragenyx becomes Arcturus' largest shareholder; Karah Parschauer, J.D., General Counsel of Ultragenyx, joining Arcturus' Board of Directors and Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx, joining as Arcturus Board Observer*

NOVATO, Calif. and SAN DIEGO, June 19, 2019 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical, Inc. (Nasdaq: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases and Arcturus Therapeutics Holdings Inc. (Nasdaq: ARCT), a leading messenger RNA medicines company, today announced that they have expanded their collaboration to discover and develop mRNA, DNA and siRNA therapeutics for up to 12 rare disease targets.

"This expanded collaboration further solidifies our mRNA platform by adding additional targets and expanding our ability to potentially treat more diseases," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are pleased with the progress of our ongoing collaboration. Our most advanced mRNA program, UX053 for the treatment of Glycogen Storage Disease Type III, is expected to move into the clinic next year, and we look forward to further building upon the initial success of this partnership."

"The expansion of the collaboration with Ultragenyx underscores the partnership's early successes and ongoing commitment," said Joseph Payne, President and Chief Executive Officer of Arcturus. "Our expertise in the discovery, early development and manufacturing of RNA medicines aligns well with Ultragenyx's proven clinical development and commercial experience in rare diseases." He further added, "We look forward to utilizing Ultragenyx's seasoned experience and presence on our Board of Directors to advance mRNA therapeutics as a new class of genetic medicines, and build Arcturus into a leading, independent biopharmaceutical company that delivers value to patients and shareholders."

In connection with the amendment to the license agreement, Ultragenyx made a $6 million cash upfront payment to Arcturus. Ultragenyx is also purchasing 2,400,000 shares of Arcturus' common stock at a stated value of $10 per share. Ultragenyx has an option to purchase an additional 600,000 shares of Arcturus' common stock at $16 per share. Ultragenyx will become Arcturus' largest shareholder with Karah Parschauer, J.D., General Counsel of Ultragenyx, joining Arcturus' Board of Directors and Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx, joining as an Arcturus Board Observer.

Arcturus is entitled to preclinical, clinical, regulatory, and sales milestone payments for each product developed under the collaboration. Under the amended license agreement, certain early-stage milestone payments are reduced and the total potential milestone payments are increased due to the expanded number of targets. Arcturus is also entitled to reimbursement of related research expenses and royalties on commercial sales.

The original collaboration and license agreement between Ultragenyx and Arcturus was signed in October 2015. The two companies have been working together to develop mRNA therapeutic candidates for certain rare disease targets. The first disclosed indication under the collaboration is Glycogen Storage Disease Type III, and an Investigational New Drug (IND) application for this mRNA therapeutic program, UX053, is expected to be filed in 2020.

About Ultragenyx Pharmaceutical, Inc.
Ultragenyx is a biopharmaceutical company committed to bringing 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.

About Arcturus Therapeutics Holdings Inc.
Founded in 2013 and based in San Diego, California, Arcturus Therapeutics Holdings Inc. (NASDAQ: ARCT) is an RNA medicines company with enabling technologies – LUNAR® lipid-mediated delivery and Unlocked Nucleomonomer Analog (UNA) chemistry – and mRNA drug substance along

with drug product manufacturing. Arcturus' diverse pipeline of RNA therapeutics includes programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, Glycogen Storage Disease Type 3, Hepatitis B, and non-alcoholic steatohepatitis (NASH). Arcturus' versatile RNA therapeutics platforms can be applied toward multiple types of nucleic acid medicines including small interfering RNA, messenger RNA, replicon RNA, antisense RNA, microRNA, DNA, and gene editing therapeutics. Arcturus technologies are covered by its extensive patent portfolio (154 patents and patent applications, issued in the U.S., Europe, Japan, China and other countries). Arcturus' commitment to the development of novel RNA therapeutics has led to partnerships with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, Ultragenyx Pharmaceutical, Inc., Takeda Pharmaceutical Company Limited, Synthetic Genomics Inc. and the Cystic Fibrosis Foundation. For more information, visit www.Arcturusrx.com.

Ultragenyx 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 regarding 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, and investment, in Arcturus, 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 approvals (including whether such approvals can be obtained), 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 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 filed on Form 10-Q with the Securities and Exchange Commission on May 7, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Arcturus Forward-Looking Statements
This press release contains forward-looking statements that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. Any statements, other than statements of historical fact, included in this press release regarding strategy, future operations, collaborations, future financial position, prospects, plans and objectives of management, the likelihood of success of the Company's technology or potential development of any products, the status of the preclinical development program for any of the clinical development programs of Arcturus, the status of IND-enabling studies and early clinical development related to any of the clinical development programs of Arcturus, the sufficiency of any drug substances or drug products of the Company to meet the Company's current clinical goals or expectations, the date that an IND may be filed with the FDA, the potential market or success for the clinical development programs of Arcturus, current standards of care, and the Company's future cash and financial position are forward-looking statements. Arcturus may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in any forward-looking statements such as the foregoing and you should not place undue reliance on such forward-looking statements. Actual results and performance could differ materially from those projected in any forward-looking statements as a result of many factors, including without limitation, an inability to develop and market product candidates, inability to generate positive verifiable data, unexpected clinical results, unforeseen expenses and general market conditions that may prevent such achievement or performance. Such statements are based on management's current expectations and involve risks and uncertainties, including those discussed under the heading "Risk Factors" in Arcturus' Annual Report on Form 10-K for the fiscal year ended December 31, 2018, filed with the SEC on March 18, 2019 and in subsequent filings with, or submissions to, the SEC. Except as otherwise required by law, Arcturus disclaims any intention or obligation to update or revise any forward-looking statements, which speak only as of the date they were made, whether as a result of new information, future events or circumstances or otherwise.

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