Ultragenyx Announces Exercise of Option to Purchase Additional Stock of Arcturus Therapeutics

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NOVATO, Calif. and SAN DIEGO, May 21, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical, Inc. (Nasdaq: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases and Arcturus Therapeutics Holdings Inc. (Nasdaq: ARCT), a leading clinical-stage messenger RNA medicines company focused on the discovery, development and commercialization of therapeutics for rare diseases and vaccines, today announced that Ultragenyx has exercised its option to purchase 600,000 shares of Arcturus common stock at $16.00 per share.

“We are encouraged by the advancement of Arcturus’s broad nucleic acid platform across multiple therapeutic areas, including their self-replicating mRNA-based COVID-19 vaccine candidate,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We are also pleased with the progress of our preclinical UX053 mRNA candidate for Glycogen Storage Disease Type III and other earlier-stage opportunities we are exploring under the collaboration.”

“The new investment from Ultragenyx is a testament to the strength of our long-term collaboration in developing nucleic acid therapies,” said Andrew Sassine, Chief Financial Officer of Arcturus. “The additional funding will support our clinical programs, including our efforts to move our COVID-19 vaccine candidate into clinical testing this summer.”

After completion of the new equity purchase, Ultragenyx will own 3,000,000 shares, or 14.6%, of Arcturus outstanding common stock and will continue to be its largest shareholder. The purchase was made pursuant to the equity purchase agreement between the parties entered into in June 2019 in connection with the amendment to the research collaboration and license agreement between the two companies focused on nucleic acid therapies for rare diseases that was initiated in 2015. 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 2021.

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 a clinical-stage mRNA medicines and vaccines company with enabling technologies: (i) LUNAR® lipid-mediated delivery, (ii) STARR™ mRNA Technology and (iii) mRNA drug substance along with drug product manufacturing expertise. Arcturus’ diverse pipeline of RNA therapeutic candidates includes programs to potentially treat Ornithine Transcarbamylase (OTC) Deficiency, Cystic Fibrosis, Glycogen Storage Disease Type 3, Hepatitis B, non-alcoholic steatohepatitis (NASH) and a self-replicating mRNA vaccine for SARS-CoV-2. Arcturus’ versatile RNA therapeutics platforms can be applied toward multiple types of nucleic acid medicines including messenger RNA, small interfering RNA, replicon RNA, antisense RNA, microRNA, DNA, and gene editing therapeutics. Arcturus’ technologies are covered by its extensive patent portfolio (187 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 collaborations with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, Ultragenyx Pharmaceutical, Inc., Takeda Pharmaceutical Company Limited, CureVac AG, Synthetic Genomics Inc., Duke-NUS, Catalent 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), the effects from the COVID-19 pandemic on our clinical trial activities, business and operations 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, 2020, 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, including those regarding Arcturus' expected performance, Arcturus' efforts to develop a vaccine against COVID-19, the forecasted safety, timing, efficacy, reliability or availability of a vaccine against COVID-19 were one to be successfully developed by Arcturus, the timing and the potential initiation of clinical trials of a vaccine against COVID-19 by Arcturus, 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. 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, 2019, filed with the SEC on March 16, 2020 and in subsequent filings with, or submissions to, the SEC. No assurances can be given that any results reported in pre-clinical studies can be replicated in further studies or in human beings, or that a vaccine can or will ever be developed or approved using Arcturus' technology. 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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