miRagen, Investigators from Goethe University and University Hospital Frankfurt and Servier Publish MRG-110 Phase 1 Pharmacodynamic Biomarker Data in Nucleic Acid Therapeutics

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FRANKFURT, Germany and BOULDER, Colo., July 28, 2020 (GLOBE NEWSWIRE) -- Goethe University, University Hospital Frankfurt and miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced that preliminary results from a Phase 1 trial detailing the pharmacodynamic activity of MRG-110 has been published in the peer-reviewed journal *Nucleic Acid Therapeutics*.

"We are pleased to have these Phase 1 mechanism of action results for MRG-110 published in *Nucleic Acid Therapeutics,"* said William S. Marshall, Ph.D., President and CEO of miRagen Therapeutics, Inc. "These data show that a single systemic dose of MRG-110 reduced detectable miR-92a levels in the peripheral blood of humans and led to the regulation of several well established miR-92a target genes."

"The preliminary characterization of MRG-110 pharmacodynamic activity in the peripheral blood of humans provides important insight into the development of potential blood borne mechanistic biomarkers for miR-92a inhibition," added Prof. Dr. Stefanie Dimmeler, Director of the Institute for Cardiovascular Regeneration at the Center for Molecular Medicine, Goethe University Frankfurt. "We believe these data provide the basis for further development of readily monitorable biomarkers that can be employed in future clinical trials."

The article, titled "*Efficiency and Target Derepression of Anti-miR-92a: Results of a First in Human Study,*" reports mechanistic results from a single ascending dose, double-blind, placebo-controlled, randomized Phase 1 clinical trial. The trial was a study where MRG-110 was administered systemically by intravenous dosing. miR-92a levels were measured in whole blood, circulating endothelial cells, and circulating extracellular vesicles before and after MRG-110 administration. miR-92a levels were found to be significantly reduced in all three of the sample types tested. Importantly, two miR-92a target genes were derepressed after treatment, indicating the intended mechanism of action of MRG-110 in normal healthy volunteers. These data may identify a strategy for measuring pharmacodynamic activity of MRG-110 in peripheral blood in future clinical trials. This would allow for monitoring the intended mechanism of action in blood samples during the conduct of clinical trials in patients, thereby providing an enhanced degree of confidence that the product candidate is affecting the biological pathway that is intended.

Work reported in the article was the result of a collaboration between Goethe University, University Hospital Frankfurt, miRagen Therapeutics and Les Laboratories Servier. Financial support for the studies was also provided by the German Centre for Cardiovascular Research (DZHK).

About miRagen Therapeutics, Inc.
miRagen Therapeutics, Inc. is a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in diseases where there is a high unmet medical need. miRagen has three clinical stage product candidates, cobomarsen, remlarsen, and MRG-110. miRagen's clinical product candidate for the treatment of certain cancers, cobomarsen, is an inhibitor of miR-155, which is found at abnormally high levels in malignant cells of several blood cancers. miRagen's clinical product candidate for the treatment of pathological fibrosis, remlarsen, is a replacement for miR-29, which is found at abnormally low levels in a number of pathological fibrotic conditions, including cutaneous, cardiac, renal, hepatic, pulmonary and ocular fibrosis, as well as in systemic sclerosis. MRG-110, an inhibitor of miR-92, is miRagen's product candidate for the treatment of heart failure and other ischemic disease. In addition to these programs, miRagen is developing a pipeline of preclinical product candidates. The goal of miRagen's translational medicine strategy is to progress rapidly to first-in-human studies once it has established the pharmacokinetics, pharmacodynamic, safety, and manufacturability of the product candidate in preclinical studies. For more information, please visit www.miragen.com. For information on clinical trials please visit www.clinicaltrials.gov.

About the University Hospital Frankfurt
The University Hospital Frankfurt, founded in 1914, is one of the leading academic hospitals in Germany. It offers its patients optimal medical care in 32 medical clinics / institutes. University hospital and faculty of Medicine together operate a total of 20 research institutes. The close connection to research ensures patients the prompt implementation of new knowledge in therapeutic practice. Around 1,500 inpatient as well as day clinic beds are available. Numerous institutes are dedicated to medical-scientific specialized services. Annually we take care of 51,000 inpatients and 227,000 outpatients. The University Hospital Frankfurt has special interdisciplinary competence amongst others in the fields of neuroscience, oncology and cardiovascular medicine. As a specialist for organ and bone marrow transplantation, dialysis and cardiac surgery, the clinic takes a special task of supra-regional medical care. Over 4,500 employees (full-time positions) take care of the patients around the clock.

About the German Centre for Cardiovascular Research (DZHK)
The German Centre for Cardiovascular Research unites high-ranking scientists from all over Germany. It was founded in 2011 with the aim of bundling expertise in cardiovascular research and making it more effective so that research results can be transferred more quickly into clinical application. 1800 researchers from seven partner sites and 30 partner institutions, including university hospitals and non-university research institutions, are involved in the DZHK.

Note Regarding Forward-Looking Statements

This press release may contain 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. All statements contained in this press release other than statements of historical fact, including statements regarding miRagen's strategy, anticipated clinical development milestones, prospects, plans and objectives of management are forward-looking statements. The words "believe," "may," "will," "estimate," "continue," "anticipate," "intend," "plan," "expect," "predict," "potential," "opportunity," "goals," or "should," and similar expressions are intended to identify forward-looking statements. Such statements are based on management's current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation: that miRagen has incurred losses since its inception, and anticipates that it will continue to incur significant losses for the foreseeable future; future financing activities may cause miRagen to restrict its operations or require it to

relinquish rights; miRagen may fail to demonstrate safety and efficacy of its product candidates; miRagen's product candidates are unproven and may never lead to marketable products; miRagen's product candidates are based on a relatively novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all; miRagen's product candidates may cause undesirable side effects or have other properties that could delay or prevent the regulatory approval; and the results of miRagen's clinical trials to date are not sufficient to show safety and efficacy of miRagen's product candidates and may not be indicative of future clinical trial results.

miRagen has based these forward-looking statements largely on its current expectations and projections about future events and trends. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described under the heading "Risk Factors" in miRagen's Annual Report on Form 10-K and subsequent periodic and current reports filed with the Securities and Exchange Commission. Moreover, miRagen operates in a very competitive and rapidly changing environment. New risks emerge from time to time.

It is not possible for its management to predict all risks, nor can it assess the impact of all factors on its business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements it may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this press release may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. miRagen undertakes no obligation to revise or publicly release the results of any revision to such forward-looking statements, except as required by law. Given these risks and uncertainties, readers are cautioned not to place undue reliance on such forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement.

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