miRagen Therapeutics Presents New MRG-201 Preclinical Data for Inhaled MicroRNA-29 Mimic Targeting Pulmonary Fibrosis

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- MRG-201 was nebulized and delivered to the lungs via inhalation in rodents
- Administration of aerosolized microRNA-29 mimic was observed to result in down-regulation of pharmacodynamic and mechanistic biomarkers important in fibrotic tissue deposition

BOULDER, Colo., Sept. 11, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced today new preclinical safety and feasibility data on inhaled delivery of MRG-201, a microRNA-29 mimic, which miRagen plans to evaluate for the potential treatment of pulmonary fibrosis. MicroRNA mimics are molecules intended to replace the native microRNA and re-establish gene expression control over pathway biology. By normalizing the level of microRNA-29, MRG-201 may be able to normalize uncontrolled fibrotic tissue deposition which is a hallmark of pulmonary fibrosis. The inhalation data is being presented at the European Respiratory Society (ERS) International Congress in Milan, Italy.

miRagen's new preclinical data builds upon previous observations on systemically administered microRNA-29 mimic in the bleomycin-induced pulmonary fibrosis in mice. To more effectively assess accurate dose, compound integrity, particle size, and exposure, the Company utilized a nose-only inhalation exposure system with Lovelace Biomedical Research Institute in this study.

A summary of results to be reported at the ERS International Congress from this preclinical study includes the following:

- MRG-201 was nebulized with its chemical integrity maintained, and may be administered via nose-only inhalation to rats.
- Exposure to lung tissue after inhalation was high while distribution to tissues tested beyond the lung was minimal, suggesting a potentially effective localized effect and limited systemic impact.
- Following multiple administrations, aerosolized MRG-201 at 1.0mg/kg was observed to result in down-regulation of intended target genes important in fibrotic tissue deposition induced by bleomycin in rats.

"We are encouraged by these preclinical inhalation feasibility results for MRG-201, which we believe offer an innovative approach for the potential treatment of pulmonary fibrosis," said miRagen President and CEO William S. Marshall, Ph.D. "Patients suffering from pulmonary fibrosis have limited treatment options, and this data demonstrates our commitment to developing a safe and effective therapy to treat this debilitating disease. While we continue to advance MRG-201 in clinical development for the treatment of cutaneous fibrosis, we are excited to further explore its potential utility in pulmonary fibrosis."

MRG-201 is designed to mimic the activity of microRNA-29 and decrease the expression of collagen and other proteins that are involved in fibrous scar formation. Previous studies by miRagen researchers indicated that microRNA-29 may be a regulator of extracellular matrix production, and may be an attractive therapeutic target for the potential treatment of pathological fibrosis. miRagen believes the results from its Phase 1 clinical trial of MRG-201 in induced cutaneous fibrosis, which demonstrated the potential ability of the product candidate to control fibrogenesis in humans, may provide support for the therapeutic approach in other pathological fibrotic conditions.

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's two lead product candidates, MRG-106 and MRG-201, are currently in clinical development. miRagen's clinical product candidate for the treatment of certain cancers, MRG-106, is an inhibitor of microRNA-155, which is found at abnormally high levels in malignant cells of several blood cancers, as well as certain cells involved in inflammation. miRagen's clinical product candidate for the treatment of pathological fibrosis, MRG-201, is a replacement for microRNA-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 systemic sclerosis. miRagen is also developing MRG-110, an inhibitor of microRNA-92, under a license and collaboration agreement with Servier. MRG-110 is being developed 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.

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, future operations, future financial position, future revenue, projected expenses, prospects, plans and objectives of management or the expected features of or potential indications for miRagen's product candidates 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 preclinical and 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 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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