



miRagen Presents Preclinical Data in Pulmonary Fibrosis Using Second Generation microRNA-29 Replacement Product Candidate at the 2019 American Thoracic Society International Conference

SECOND GENERATION MICRORNA-29 REPLACEMENT SHOWS EFFICACY IN BLUNTING FIBROSIS IN THE BLEOMYCIN MODEL OF PULMONARY FIBROSIS WHEN DOSED SYSTEMICALLY

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BOULDER, CO, May 21, 2019 – miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced today that it will report new data demonstrating that systemic administration of its second-generation microRNA-29 mimic, the Company's pre-clinical micro-RNA targeted therapeutic candidate for idiopathic pulmonary fibrosis ("IPF"), efficiently reduced extracellular matrix deposition in a series of preclinical studies. The data will be presented today at the [2019 American Thoracic Society \("ATS"\) Conference](#) in Dallas, TX.

"We are encouraged by the data presented from several in vivo studies of our second-generation microRNA-29 mimic. These data demonstrate efficacy in blunting the fibrotic response in the bleomycin model of pulmonary fibrosis in mice," said Paul Rubin, M.D., Executive Vice President, R&D, of miRagen Therapeutics. "Considering the data from these studies, we believe that our lead compound has the potential to be efficacious in the treatment of IPF when administered systemically at considerably lower doses compared to other compounds we have tested in previous pre-clinical studies."

“These data coupled with previous observations in humans with IPF support the role of microRNA-29 in pathologic fibrosis, as well as the use of microRNA-29 replacements as potential therapeutics in pulmonary fibrosis. I am very excited about the potential role of miRagen’s microRNA-29 mimics, which is being facilitated by our collaboration through the NIH-NHLBI funded CADET program,” stated Naftali Kaminski, M.D., Boehringer-Ingelheim Endowed Professor of Internal Medicine, Chief of Pulmonary, Critical Care and Sleep Medicine, Department of Medicine, Yale School of Medicine.

In these studies, a chemically modified microRNA-29 replacement was studied in mice with bleomycin-induced fibrosis in their lungs, which is a well-established model of IPF. The compound was evaluated in both therapeutic and prophylactic dosing paradigms. Data from the studies demonstrated a down-regulation of certain pro-fibrotic genes that were previously established as targets of microRNA-29. These effects extended to downstream targets involved in the pro-fibrotic response, including connective tissue growth factor (CTGF). The downregulation of these mechanistic biomarkers translated to the significant inhibition of the pro-fibrotic response as measured by quantitative histopathological analyses of whole lung scans. This resulted in an approximately 50% inhibition of collagen deposition. Additionally, animals treated with the compound showed normal alveoli architecture, which was not present in control mice.

The poster will be available on the scientific publications page of the Company’s website ([click here](#)) following the presentation at the 2019 ATS Conference.

About microRNA-29

microRNA-29 is a fibroblast-enriched miRNA family that is downregulated in fibrotic diseases, including IPF in humans, thereby leading to a coordinate increase of many extracellular matrix genes. microRNA-29 family targets multiple extracellular matrix proteins and profibrotic molecules. Reamlarsen, a first-generation microRNA-29 mimic, has been observed to prevent fibrotic activity in human skin but is not optimized for systemic delivery.

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, reamlarsen, and MRG-110. miRagen’s clinical product candidate for the treatment of certain cancers, cobomarsen, 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, reamlarsen, 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. MRG-110, an inhibitor of microRNA-92, is being developed under a license and collaboration agreement with Servier 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 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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