miRagen Therapeutics Announces New Data Supporting the Potential Use of Remlarsen to Prevent Corneal Fibrosis at the 2019 ARVO Meeting

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- Preclinical data indicate that remlarsen accelerated the healing of corneal injury when compared to placebo
- miRagen believes the study's results support that remlarsen may offer a novel therapeutic for prevention of corneal scarring and hazing following injury or ulceration
- Corneal scarring after injury is one of the leading causes of blindness globally and there are currently no approved pharmacological agents available for treatment of the disorder

BOULDER, Colo., April 29, 2019 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced data from a preclinical study of remlarsen, a microRNA-29 replacement, exploring the anti-fibrotic effects of remlarsen in the cornea of rats after corneal ulceration. The data will be delivered today in a poster presented at the 2019 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting at the Vancouver Convention Centre in Vancouver, British Columbia.

Remlarsen was administered topically to the rat cornea for up to 28 days following an induced chemical burn. Eyes were scored for corneal haze, then evaluated histologically for corneal thickness and expression of α-SMA (alpha smooth muscle actin), a marker of epithelial-to-mesenchymal transition (EMT) and fibroblast-to-myofibroblast transition (FMT). Expression of collagen and other fibrosis-associated genes was assessed using quantitative reverse transcription-polymerase chain reaction.

Highlights from the study observations include:

- Remlarsen treatment accelerated the healing of corneal injury, resulting in a more rapid restoration of epithelial thickness and a reduction in stromal thickness as compared to saline-treated injured eyes.
- Remlarsen treatment appeared to reduce the expression of multiple collagens and fibrosis-associated genes from 7-14 days post-injury and to reduce α -SMA protein expression in the epithelium and stroma at 14 days.
- Remlarsen treatment appeared to reduce corneal hazing and scarring beginning at 10 days post-burn. Dose and treatment schedule were optimized in preparation for enabling toxicology studies to support human clinical trials.

"We are pleased to report today that, in this preclinical rat study, remlarsen treatment accelerated repair and inhibited EMT, FMT, extracellular matrix expression and corneal hazing and scarring compared to saline treated controls. These new data support our continued belief that the topical application of remlarsen may be an effective treatment to inhibit corneal fibrosis and scarring," stated Paul Rubin M.D., Executive Vice President, R&D, at miRagen.

Anterior surface injury (e.g., trauma or burn) or corneal ulceration due to infection or neuropathy can result in EMT, keratocyte activation, FMT, and can culminate in aberrant production of collagens and other extracellular-matrix molecules. This process is termed corneal fibrosis and may lead to hazing and vision loss if located centrally or to irregular astigmatism and visual distortions if located peripherally. No approved pharmacological agents are currently available to prevent or treat corneal fibrosis. According to the World Health Organization, corneal scarring is one of the leading causes of blindness worldwide, and it estimates that nearly 4.9 million individuals worldwide are blind as a result of corneal scarring from trachoma infections alone, with an additional 1.5 to 2.0 million new cases of unilateral blindness each year resulting from ocular trauma and corneal ulceration.

miR-29b is an antifibrotic microRNA that inhibits EMT, FMT and collagen expression in multiple organs and tissues. microRNA-29 is expressed at abnormally low levels in numerous pathological fibrotic conditions. Remlarsen, a synthetic oligonucleotide replacement of microRNA-29, has been previously observed to be pharmacodynamically active as measured by both target gene regulation and inhibition of fibrosis in skin wounds in a double-masked, placebo-controlled Phase 1 human clinical trial.

miRagen is currently conducting a Phase 2 clinical trial of remlarsen, assessing the safety, tolerability, and activity of remlarsen in the potential prevention or reduction of keloid formation in subjects with a history of keloid scars, a form of pathological scarring. miRagen expects to report data from this clinical trial in the second half of 2019.

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 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, remlarsen, 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; remlarsen may not be effective in the treatment of ophthalmological indications; remlarsen may not advance into additional human clinical ; 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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