



miRagen Therapeutics Presents New Preclinical Data Supporting use of MicroRNA-Targeted Therapies for Ophthalmic Diseases

May 2, 2018

- Dosing with microRNA mimics of the microRNA-183/96/182 cluster led to functional improvement of photoreceptors and vision in retinal degeneration model
- MRG-201 dosing inhibited the expression of multiple factors responsible for fibrosis in disease models in both the cornea and retina
- Data to be presented in oral and poster sessions at 2018 Association for Research in Vision and Ophthalmology Annual Meeting

BOULDER, Colo., May 02, 2018 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announces new data from two preclinical studies investigating microRNA mimics of the microRNA-183/96/182 cluster and MRG-201, a microRNA-29 mimic, to potentially treat ophthalmic diseases. The data will be presented today, May 2, at the 2018 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Honolulu, HI.

A summary of preclinical results for microRNAs of the microRNA-183/96/182 cluster to be reported at the ARVO Annual Meeting includes the following:

- Genetic analysis in animals and humans helped validate this microRNA cluster as a potential therapeutic target for retinal degeneration.
- Dosing with mimics of the microRNA-183/96/182 cluster modulated downstream biology controlling several important phototransduction genes.
- microRNA modulators may be functionally delivered to the eye, including the retina and photoreceptors, in animal models.
- microRNAs of the microRNA-183/96/182 cluster led to functional improvement of photoreceptors and vision in a mouse model of retinal degeneration.

"We are excited to unveil this encouraging new program evaluating microRNA mimics of the microRNA-183/96/182 cluster, which have been shown to play an important role in the establishment and maintenance of neurosensory cells, including photoreceptors," said miRagen President and CEO William S. Marshall, Ph.D. "In preclinical mouse studies, dosing with mimics of the cluster showed activity that indicates their therapeutic potential for treating retinal degeneration, a pathological condition characterized by gradual loss of photoreceptors and eventual blindness."

In a second program featured at the ARVO Annual Meeting, data from preclinical work using MRG-201 to address the damaging formation of fibrosis in the eyes include the following highlights:

- MRG-201 was readily taken up into the cornea and all layers of the retina including the retinal pigment epithelial layer after intravitreal injection.
- Pharmacokinetic analysis indicates that dosing into the closed compartment of the eye leads to relatively long tissue exposure in the retina.
- MRG-201 reduced expression of multiple collagens and other microRNA-29 target genes important in fibrogenesis, suggesting that MRG-201 may function as an effective therapeutic to inhibit either corneal or retinal fibrosis.

"We are also very encouraged to report MRG-201 preclinical data demonstrating its potential for the prevention of both corneal and retinal fibrosis," continued Dr. Marshall. "Administration of MRG-201 through various routes of administration resulted in functional uptake of the product candidate and reduced fibrosis and enhanced healing. Patients suffering from ocular fibrosis have limited treatment options and this data demonstrates the potential of MRG-201 to potentially provide therapeutic benefit. Taken together, the results from these two ocular programs highlight an intriguing role for microRNA targeting drugs in diseases leading to vision loss and blindness."

Oral Presentation Details

Title: Restoring miRNAs of the microRNA-183/96/182 cluster results in target gene regulation and ameliorates symptoms of retinal degeneration in a mouse model of retinitis pigmentosa

- **Oral session:** Session 473: Signaling in retinal degeneration
- **Date:** Wednesday, May 2, 2018, 3:30 p.m. – 5:15 p.m. HST
- **Location:** Room 315, Hawaii Convention Center

Poster Presentation Details

Title: Inhibition of ocular fibrosis with a microRNA-29b mimic

- **Poster session:** Session 489: Retina, drugs
- **Poster number:** C0249

- **Date:** Wednesday, May 2, 2018, 3:30 p.m. – 5:15 p.m. HST
- **Location:** Exhibit Hall, Hawaii Convention Center

For additional information, please visit the ARVO Annual Meeting website: www.arvo.org

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 (MRG-106), MRG-201, 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, 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. 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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Source: Miragen Therapeutics, Inc.