miRagen Therapeutics Presents New Interim Clinical Data Expanding on Previous Data Suggesting Positive Impact From Systemically Administered MRG-106 on Mycosis Fungoides Form of Cutaneous T-Cell Lymphoma at 2017 American Society of Hematology Annual Meeting

December 11, 2017

- *Nine of fourteen (64%) of patients treated for more than one month showed a 50% or greater improvement in total skin disease*
- *Seven patients have achieved at least a 50% improvement that has been durable for at least four months*
- *MRG-106 continued to be generally well-tolerated at all dose levels evaluated as of December 4, 2017*
- *Initiated dosing in three additional oncology indications including chronic lymphocytic leukemia, diffuse large B-cell lymphoma and adult T-cell leukemia/lymphoma within the ongoing MRG-106 Phase 1 trial*

BOULDER, Colo., Dec. 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 interim results from its ongoing Phase 1 clinical trial of MRG-106 in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). The data are being presented at the 2017 American Society of Hematology (ASH) Annual Meeting in Atlanta, Georgia, by Christiane Querfeld, M.D., Ph.D., Chief of the Division of Dermatology, and Director, Cutaneous Lymphoma Program at the City of Hope in Duarte, California.

“We believe the new MRG-106 Phase 1 data further validate the utility of micro-RNA based therapeutics and provide important insights that may help guide potential Phase 2 clinical trial designs and expanded indication trials,” said miRagen President and CEO William S. Marshall, Ph.D. “We believe that these findings suggest that, if approved, MRG-106 has the potential to offer profound benefits to patients with CTCL. We are currently evaluating MRG-106 in additional hematologic indications within the current Phase 1 trial, and plan to release interim data from these expansion indications in 2018.”

The interim results being presented at ASH include observations from additional patients, as well as longer term dosing data for existing patients who have continued on the trial. Interim data from the second part of the Phase 1 trial are summarized below:

- As of December 4, 2017, a total of twenty-four patients had completed at least one cycle of dosing by systemic administration in the second part of the clinical trial.
  - Nine of fourteen patients (64%) treated for more than one month showed a 50% or greater improvement in total skin disease as measured by the maximal change in each patient’s modified Severity Weighted Assessment Tool (mSWAT) score, which assesses the severity of skin disease over a patient’s entire body.
  - In patients where an initial response was induced by MRG-106, these responses were generally maintained with continued therapy. Additionally, seven patients had maintained a durable response for four months or longer.
  - The magnitude of mSWAT improvements appeared to correlate with amount of time the patient received MRG-106 treatment.
  - In patients who showed improvements, these were observed regardless of whether the patient was receiving stable background medication(s) for CTCL or MRG-106 alone.
- As of December 4, 2017, MRG-106 had been generally well-tolerated for patients evaluated at dose levels ranging from 75 mg to 900 mg.
  - Pain at injection site was reported in some patients after subcutaneous or intralesional administration and was generally minor and self-limited.

“We are pleased with these interim results from our MRG-106 Phase 1 trial, which includes seven patients who maintained a durable response for at least four months,” said Paul Rubin, M.D., miRagen’s Executive Vice President of Research and Development. “We plan to initiate a controlled Phase 2 trial in the second half of 2018 to further evaluate the potential of MRG-106.”

The MRG-106 Phase 1 trial consists of two parts. In the first part, patients were treated with 75 mg injections of MRG-106 directly into a specific lesion. These data, which showed a decrease in lesion size for all injected lesions, have been previously presented. The second part employed a multiple dose-escalation design to evaluate 300 mg, 600 mg or 900 mg subcutaneous or intravenous administrations of MRG-106. Patients were allowed to continue background medication provided these have remained unchanged for one month prior to receiving MRG-106. Patients are monitored for safety, and for improvement in skin disease via mSWAT at regular intervals throughout the trial.

MRG-106 is an inhibitor of microRNA-155. In CTCL, as well as certain other blood cancers, microRNA-155 is present at abnormally high levels, and may play a role in the proliferation of blood and lymph cells. miRagen believes therapeutic inhibition of microRNA-155 may reduce aberrant cell proliferation and the tumor growth characteristic of certain types of cancer.

Oral Presentation Details
Abstract title: Phase 1 Study of the Safety and Efficacy of MRG-106, a Synthetic Inhibitor of microRNA-155, in CTCL Patients

- **Session:** 624. Hodgkin Lymphoma and T/NK Cell Lymphoma—Clinical Studies: T-Cell Lymphoma Clinical Studies
- **Date:**Monday, December 11, 2017, 4:30 p.m. – 6:00 p.m. ET

- **Location:** Building A, Level 4, Marcus Auditorium

For additional information, please visit the ASH Annual Meeting website: www.hematology.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'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 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.