miRagen Therapeutics Presents New Data from Phase 1 Clinical Trial of MRG-106 in Mycosis Fungoides at the T-Cell Lymphoma Forum

February 2, 2017

- 90% of evaluable patients show clinical improvement in the ongoing Phase 1 clinical trial of MRG-106.
- 80% of patients treated with 300 mg IV infusion, the anticipated Phase 2 clinical trial dose, demonstrated an objective response.
- Recent FDA meeting informs design of Phase 2 clinical trial of MRG-106 and indicates potential for the trial to result in accelerated approval in the U.S.

BOULDER, Colo., Feb. 02, 2018 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced new interim data from its Phase 1 clinical trial of MRG-106 in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). The data will be presented today, February 2, 2018, at the 10th Annual T-cell Lymphoma Forum in La Jolla, California, 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 that the clinical activity observed with MRG-106 in CTCL continues to demonstrate that patients are experiencing meaningful improvement in total skin disease and that MRG-106 is generally well-tolerated at all doses tested,” said miRagen President and CEO William S. Marshall, Ph.D. “We have identified what we anticipate to be the appropriate therapeutic dose for the Phase 2 clinical trial of MRG-106, and recently discussed the Phase 1 interim results and Phase 2 clinical trial design with the U.S. Food and Drug Administration (FDA). We plan to initiate a Phase 2 clinical trial for MRG-106 in patients with CTCL in the second half of 2018 and, based on discussions with the FDA, believe that the trial could provide data that would potentially support accelerated approval.”

The new data presented at the T-cell Lymphoma Forum include observations from additional patients in the Phase 1 clinical trial, as well as longer term dosing data for existing patients who have continued participation in the trial. As of January 25, 2018, highlights include the following:

- Cohorts were dosed by multiple routes of administration, including subcutaneous injection (SQ), intravenous infusion (IV infusion) and intravenous bolus injection (IV bolus). Efficacy and tolerability were assessed at doses of 300 mg, 600 mg and 900 mg for SQ and IV infusion and at 300 mg for IV bolus.
    - 26 of 29 evaluable patients, or 90%, showed improvement in modified Severity Weighted Assessment Tool (mSWAT) score, which is a measurement of the severity of skin disease over a patient’s entire body.
    - Improvements in mSWAT scores were observed as early as 17 days after a patient’s first dose (the first post-treatment assessment).
    - Best improvement in mSWAT scores were seen after one or more months of dosing.
    - All eight patients who achieved a 50% or greater reduction in mSWAT and received long-term dosing, maintained a durable response for greater than four months. These patients were dosed either via SQ injection or IV infusion at doses ranging from 300 mg to 900 mg.
    - Four of five patients (80%) who were treated with 300 mg IV infusion have achieved a 50% or greater best mSWAT reduction. miRagen anticipates 300mg IV infusion to be the dose in its upcoming Phase 2 clinical trial.

“People suffering from CTCL have few treatment options available and some patients may be intolerant or become resistant to these treatments over time,” said Dr. Querfeld. “We continue to be encouraged by additional data from the Phase 1 clinical trial of MRG-106, providing further evidence that micro-RNA-based therapeutics, if approved, have the potential to significantly improve skin tumor burden for patients living with this disease.”

Based on the outcome of an FDA meeting on January 24, 2018, miRagen anticipates that the Phase 2 clinical trial of MRG-106 in patients with CTCL will employ an open-label, parallel group, randomized design to evaluate the safety and efficacy of 300mg of MRG-106 given by IV infusion versus an active control. This trial will compare numbers of responders in each treatment group with response defined as a 50% or greater improvement in the patient’s mSWAT score maintained for at least four consecutive months (ORR4) with no evidence of disease progression in the blood, lymph nodes or viscera. The ORR4 will be designated as the primary endpoint. Secondary endpoints will include progression free survival and patient reported outcomes measuring improvements in symptoms, such as pain and itching. Expected enrollment will include approximately 65 patients per treatment group. After these discussions with the FDA, miRagen believes that a successful outcome for the primary endpoint of this Phase 2 clinical trial could allow the Company to apply for accelerated approval.

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 tumor growth characteristic of certain types of cancer.

miRagen is currently evaluating a 600 mg IV infusion of MRG-106 in a Phase 1 clinical trial in additional oncology indications in which the disease process appears to be related to an increase in miR-155 levels, including chronic lymphocytic leukemia, diffuse large B-cell lymphoma and adult T-cell leukemia/lymphoma.

Oral Presentation Details
Title: Emerging Rationale for Targeting miRNAs in CTCL

- **Session:** 10th Annual T-Cell Lymphoma Forum, Session 3. CTCL
- **Date:**Friday, February 2, 2018, 9:35 a.m. – 9:50 a.m. PT
- **Location:**Hilton La Jolla Torrey Pines

For additional information, please visit the T-cell Lymphoma Forum website: www.tcellforum.com.

About Mycosis Fungoides

MF is a slow growing form of cancer, and is the most common form of CTCL. Symptoms of MF include rash, tumors, skin lesions and itchy skin. In about 10 percent of cases, the disease can progress to lymph nodes and internal organs. In the U.S., the prevalence of MF is estimated to be around 16,000-20,000 cases, with 3,000 new diagnoses each year.

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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