miRagen Therapeutics Presents New Data from Phase 1 Clinical Trial Evaluating MRG-106 in Subjects with Mycosis Fungoides

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Improvements in disease severity demonstrated and generally sustained through duration of therapy

BOULDER, Colo., June 05, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced new interim results from its ongoing Phase 1 clinical trial evaluating the safety, efficacy and pharmacokinetics of MRG-106 in subjects with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). The data were presented at the American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago.

The Phase 1 trial consists of two parts. In the first part, from which results were previously reported, subjects were injected with 75 mg of MRG-106 directly into a specific lesion. The second part employed a dose-escalation design to evaluate 300 mg, 600 mg or 900 mg subcutaneous or intravenous administrations of MRG-106. A total of 19 subjects have been dosed over a period of one to 34 weeks and were evaluated based on a Composite Assessment of Index Lesion Severity (CAILS) score, which assesses the severity of one or more lesions on a subject, and/or the modified Severity Weighted Assessment Tool (mSWAT) score, which assesses the severity of skin disease over a subject's entire body. Two subjects participated in both the first and second parts.

A summary of results reported at ASCO from this first-in-human trial includes the following:

- Eighteen of nineteen subjects (95%), independent of administration route, showed improvement in either the individual lesion (first part) or total skin disease (second part) as measured by maximal change in either CAILS or mSWAT. One patient in the 900 mg dose cohort was withdrawn from the trial due to disease progression.
    - All of the four evaluable subjects with lesions directly injected (first part) showed a 50% or greater improvement in CAILS.
    - Despite most of the systemically-treated subjects only being exposed to MRG-106 for only a short duration (median of 29 days on active treatment), 73% showed a >25% improvement, and 40% demonstrated improvement ≥ 50% in mSWAT scoring.
    - In systemically-treated subjects, improvements in mSWAT scores were observed as early as Study Day 19 (the first scheduled post-treatment assessment), and for all but one subject, improvements were maintained throughout the treatment period.
    - The magnitude of mSWAT improvements appeared to correlate with amount of time the subject received MRG-106 treatment. Additionally, some subjects demonstrated mSWAT score worsening upon therapy hiatus, followed by recurrence of mSWAT score improvement upon resumption of MRG-106 dosing.
- MRG-106 has been generally well-tolerated to date.
    - Only one Grade 3 adverse event, pruritus (itchy skin), was considered potentially related to MRG-106 administration.

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.

"We believe the enduring responses seen in this Phase 1 clinical trial of MRG-106 show potential for subjects with MF and other cancers where microRNA-155 is found at abnormally high levels," said Paul Rubin, M.D., miRagen's Executive Vice President of Research and Development. "While preliminary, the improvements appear to correlate with time on MRG-106 treatment, and the results' trends suggest that longer duration of treatment could result in greater efficacy."

"We are pleased to present the new data at the ASCO Annual Meeting from our MRG-106 Phase 1 clinical trial, which indicated that the product candidate has been generally well-tolerated to date. We believe the data presented support MRG-106's intended mechanism of action and provide an example of our foothold clinical development strategy," said miRagen President and CEO William S. Marshall, Ph.D. "We look forward to the continued development of MRG-106 for the potential treatment of MF and believe the experience gained in developing MRG-106 for MF may also allow us to address other indications where microRNA-155 is implicated in disease."

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 Phase 1 clinical trials. 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 several blood cancers. 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, and pulmonary fibrosis, as well as systemic sclerosis. In addition to miRagen's clinical programs, it is developing a pipeline of pre-clinical 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, pharmacodynamics and safety of product candidates in pre-clinical studies. For more information, please visit www.miragenrx.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 results of miRagen's Phase 1 clinical trials 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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