



## **miRagen Therapeutics Announces Final Safety, Biodistribution and Clinical Efficacy Data From Phase 1 Cobomarsen Clinical Trial in Patients With Mycosis Fungoides**

**Company Release – 12/2/2018 5:00 PM ET**

BOULDER, Colo., Dec. 02, 2018 (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 its Phase 1 clinical trial evaluating the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD) and efficacy of cobomarsen, an inhibitor of miR-155, in mycosis fungoides (MF) patients. These data will be presented today in a poster session at the 60th American Society of Hematology (ASH) Annual Meeting, which is being held in San Diego, CA, from December 1st – 4th.

“We are excited to present final data from our Phase 1 cobomarsen clinical trial, which continue to show that cobomarsen is safe and well-tolerated, has relevant biological and clinical activity in patients and has the potential to impact the quality of life for MF patients,” stated miRagen Executive Vice President, R&D, Paul Rubin. “We believe the Phase 1 data has provided us with the means and the confidence to design and implement a larger controlled trial in MF patients.”

The Phase 1 clinical trial investigated the safety, tolerability, PK, PD and efficacy of cobomarsen in a total of 43 MF patients. Cobomarsen was initially administered via intratumoral injection (75 mg/dose), and then via systemic administration by subcutaneous (SC), intravenous (IV) bolus or IV infusion, at 300, 600 or 900 mg/dose

with loading doses in the first week followed by weekly maintenance dosing. The patients had been in the clinical trial for up to 23 months as of the reporting of these data. Highlights from the trial observations include:

92% of the MF subjects in the systemic administration cohorts had improvement in tumor burden as assessed by modified Severity Weighted Assessment Tool (mSWAT) score independent of concomitant therapies

52% of patients receiving more than six doses achieved a partial response (at least a 50% reduction) in mSWAT score; with 69% of these patients who achieved a partial response maintained the response for at least four consecutive months (ORR4 based on mSWAT). For these patients, the mean duration of response was 259 days at the time of the data cutoff.

Cobomarsen was generally well-tolerated at all doses tested.

“We are pleased with the results from the Phase 1 clinical trial for cobomarsen. Importantly, we also observed an improvement in quality of life that correlated with reductions in tumor burden as assessed by mSWAT score during the treatment phase,” said William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics. “The data from the Phase 1 clinical trial drove our decision to advance the investigation of cobomarsen in the Phase 2 SOLAR clinical trial for patients with MF. Based on our discussions with the U.S. Food and Drug Administration, we believe the results generated in the SOLAR trial could allow us to seek accelerated approval for cobomarsen in the United States.”

### **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](http://www.miragen.com).

For information on clinical trials please visit [www.clinicaltrials.gov](http://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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