miRagen Announces New Clinical Data for Cobomarsen in ATLL Patients at the 19th Annual HTLV Congress in Lima, Peru

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- Continued evidence of disease stabilization observed in ATLL patients treated with cobomarsen for up to 16 months
- Cobomarsen allowed for rapid bone marrow recovery following chemotherapy
- In most patients, biomarkers that have been associated with poor prognosis were reduced following cobomarsen treatment
- Cobomarsen continues to show good tolerability with no serious adverse events deemed related to trial drug even after 16 months of treatment

BOULDER, CO, April 26, 2019 – miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced incremental new data from its Phase 1 clinical trial evaluating the safety, tolerability and efficacy of cobomarsen, an inhibitor of microRNA-155, in adult T-cell leukemia/lymphoma (ATLL) in an oral presentation at the 19th International Congress HTLV 2019, which is being held from April 24 – 26, 2019, in Lima, Peru.

Previously published data suggest that patients with aggressive forms of ATLL typically succumb to their disease within 4-10 months on average from the time of diagnosis. In updated data from the cobomarsen Phase 1 trial, five patients with an aggressive ATLL subtype in partial remission have been treated with cobomarsen for up to 16 months. Four of 5 patients remained stable for up to 16 months and continue on the trial. Biological activity of cobomarsen in these subjects is indicated by a significant decrease in biomarkers of cell proliferation (e.g., Ki67) and activation (e.g., HLD-R and CD69) in all patients, supporting the observed clinical stabilization. Cobomarsen has been well tolerated over prolonged treatment, with no deaths, DLTs, related serious adverse events,

related Grade 3 or Grade 4 adverse events, hematological events or discontinuation from trial due to related adverse events.

“We are encouraged by the additional data announced today from our Phase 1 cobomarsen trial in ATLL patients,” stated Paul Rubin, M.D., Executive Vice President, R&D, at miRagen. “Patients with the aggressive subtypes of ATLL have a very poor prognosis with few potential long-term treatment options. The continued durability of disease stabilization observed in patients treated with cobomarsen, combined with the favorable tolerability profile, supports our belief that cobomarsen may be a meaningful potential treatment option for patients with aggressive forms of ATLL.”

About cobomarsen

Cobomarsen is an inhibitor of microRNA-155. In cutaneous T-cell lymphoma (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 characteristics of certain types of cancer. The Company is currently evaluating cobomarsen in three oncology indications within the current Phase 1 trial, including ATLL, diffuse large B-cell lymphoma (DLBCL) and chronic lymphocytic leukemia (CLL).

About ATLL

ATLL is a blood cell malignancy that develops in patients after prolonged infection with the virus, HTLV1. Literature suggests that the infection with HTLV1 as well as the subsequent malignancies may be associated with elevation in the expression of microRNA-155, the target of cobomarsen. The disease presents in multiple forms, but the most lethal include the acute leukemic form and lymphomatous forms. Although the disease is rare, these two manifestations lack good treatment options, and once the diagnosis is made, average life expectancy is approximately 4 months for the acute leukemic form and approximately 10 months for the lymphomatous variety.

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.

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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