MIRAGEN THERAPEUTICS ANNOUNCES POSITIVE DATA FOR COBOMARSEN IN ADULT T-CELL LEUKEMIA/LYMPHOMA PATIENTS WITH RESIDUAL DISEASE

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Cobomarsen-treated adult T-cell leukemia/lymphoma (ATLL) patients with residual disease had a median survival time (MST) of 26 months compared with 7.4 months in a historical cohort

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Median progression free survival (PFS) of 12.5 months compared with 5.4 months in a historical cohort

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Generally safe and well tolerated by ATLL patients and observed to have a favorable safety profile over one year of dosing on a weekly schedule

BOULDER, CO, January 30, 2020 - miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company developing proprietary RNA-targeted therapies with a specific focus on microRNAs, today announced new efficacy and safety data from the ATLL arm of its ongoing Phase 1 clinical trial of cobomarsen, which will be presented at the 12th Annual T-Cell Lymphoma Forum in La Jolla, CA, which is taking place from January 30th to February 1st. Of note are the data from the subtype of aggressive ATLL patients who at study entry had persistent residual disease after chemotherapy or other therapy. In the trial, six patients of this subtype had an MST of 26 months with three patients still on treatment at the time of data analysis (October 17, 2019). The Company was most encouraged, however, by the observed biomarker activity showing that disease stabilization is marked by a decrease in biomarkers of tumor cells activation and proliferation, providing evidence of the biological mechanism effect of cobomarsen on disease stabilization.

"New data from the ATLL arm of our Phase 1 clinical trial offer insights into the potential efficacy and safety of cobomarsen in treating patients with this deadly cancer. We believe that the survival, biomarker and safety data generated by this study is particularly notable as patients with aggressive ATLL historically have a poor prognosis," said miRagen President and CEO William S. Marshall, Ph.D. "We believe these data support the continued development of cobomarsen in ATLL, particularly as a maintenance therapy in those patients with residual disease after front line therapy. This observed anticancer activity in patients with ATLL combined with our previously reported results in cutaneous t-cell lymphoma, further strengthen our view that cobomarsen may be effective in the potential treatment of a variety of cancers where miR-155 is elevated. During the second quarter of 2020, we plan to request a meeting with the U.S. Food and Drug Administration to explore a potential expedited development pathway for cobomarsen in ATLL."

"We believe the results observed in prolonging survival in these types of ATLL patients with cobomarsen treatment are very promising", said Francine Foss, MD, professor of medicine in the Section of Medical Oncology at the Yale Cancer Center. "The prognosis for patients with residual disease after previous therapies is poor with few additional treatment options. Cobomarsen may provide long-term stabilization of disease with minimal side effects."

The Company is evaluating cobomarsen in an ongoing Phase 1 basket trial of cancers where the disease process appears to be correlated with an increase in miR-155 levels, including ATLL, diffuse large B-cell lymphoma, and chronic lymphocytic leukemia. Today's announcement includes results for the first 15 patients with aggressive subtypes of ATLL who were treated with three doses of cobomarsen by intravenous (IV) infusion, including 600, 900 and 1200 mg doses. The preliminary results from this first-in-human Phase 1 clinical trial of the miR-155 inhibitor, cobomarsen, in patients with ATLL was observed to improve disease stabilization and reduce biomarker expression associated with ATLL cellular proliferation and activation in patients with persistent residual disease after chemotherapy and other therapies.

miRagen is also scheduled to participate in the 4th World Congress of Cutaneous Lymphomas, which is taking place from February 12th to 14th in Barcelona, Spain. During the conference, Dr. Francine Foss will provide an encore presentation of this ATLL data in the poster titled, "Phase I Trial of Cobomarsen, a miR-155 Inhibitor, in Patients with Aggressive HTLV-1 Associated ATLL: Disease Stabilization and Biomarker Analysis."

Response in Patients with Residual Disease

Of the 15 ATLL patients treated with cobomarsen, nine were actively relapsing at the time of screening, and six had residual nodal or circulating leukemic disease after chemotherapy or other systemic therapies. For these six patients, the duration of cobomarsen treatment ranged from 4.5 to 23.7 months (median 11.0 months), with three patients still on study as of October 17, 2019. MST of these patients was 26 months, compared with the 7.4 months MST from a retrospective external historical cohort based on a literature search of peer-reviewed papers. The Company compiled a historical external control which includes a series of studies with ATLL patients treated with standard of care over the past 10 years. These studies included more than 6,000 ATLL patients. The Company calculated an MST from diagnosis of 7.4 months for patients with the aggressive ATLL subtypes, regardless of the type of therapy or number of therapies administered. Five of these six patients treated with cobomarsen were alive as of the October 17, 2019 data analysis. In the trial, disease stabilization was marked by an observed decrease in Ki-67, a biomarker of cell proliferation, as well as other biomarkers of cell activation on circulating tumor cells, providing evidence of the biological mechanism effect of cobomarsen on disease stabilization.

For the clinical trial, the Company established a retrospective external historical cohort based on a literature search of peer-reviewed papers which reported MST from diagnosis and PFS on large cohorts of ATLL patients over the last decade. A total of 16 papers describing unique cohorts of patients from Japan, the United States, South America and Europe were included in the MST retrospective analysis, of which 12 included data regarding MST for combined aggressive subtypes (acute and lymphomatous) and eight reported MST for both acute and lymphomatous sub-types separately but not for the combined aggressive subtypes. The number of patients included in each publication varied from 54 to 1,792, for a total of 6,440 patients.

Phase 1 Safety Data for Cobomarsen in ATLL

Chronic administration of cobomarsen has been generally safe and well tolerated with a favorable safety profile over one year of dosing on a weekly basis. There were 196 total reported adverse events (AEs) as of October 17, 2019, with only 22% of the total AEs considered possibly related to study drug, and 86% of the total AEs considered mild or moderate. 14% of the total AEs (occurring in 8 patients) were Grade 3 or 4 and most resolved within 11 days. With no drug related deaths and only two serious adverse events (SAEs) occurring in the same patient and deemed possibly related to the study drug, the observed safety profile of cobomarsen in ATLL through October 17, 2019 appears to be benign and well tolerated with chronic dosing.

About miRagen Therapeutics

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 is developing cobomarsen for the treatment of patients with certain cancers, including cutaneous T-cell lymphoma and adult T-cell leukemia/lymphoma. Cobomarsen, is an inhibitor of microRNA-155, which is found at abnormally high levels in malignant cells of several blood cancers. miRagen is also developing remlarsen and MRG-229, which are product candidates for the treatment of patients with pathological fibrosis. These product candidates are replacements 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 in systemic sclerosis. MRG-110, an inhibitor of microRNA-92, is miRagen's product candidate for the treatment of heart failure and other ischemic disease. 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, anticipated clinical development milestones, 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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