



miRagen Announces New Clinical Data in Patients With Three Different Types of Blood Cancers Treated With Cobomarsen

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- 50% of cutaneous T-cell lymphoma patients treated with 300mg IV infusion achieved objective response lasting for greater than four months (ORR4)
- Evidence of disease stabilization in five adult T-cell leukemia/lymphoma patients
- Early indication of response seen in a patient with diffuse large B-cell lymphoma
- Data to be presented at 11th Annual T-Cell Lymphoma Forum

BOULDER, Colo., Jan. 10, 2019 (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 and efficacy of cobomarsen, an inhibitor of microRNA-155, in cutaneous T-cell lymphoma (CTCL) and in adult T-cell leukemia/lymphoma (ATLL). The Company will also discuss initial clinical experience in treating diffuse large B-cell lymphoma (DLBCL) patients with cobomarsen. The data will be presented at the 11th Annual T-Cell Lymphoma Forum, which is being held in La Jolla, CA, from January 10th-12th.

"The ATLL clinical data shows that cobomarsen provided sustained disease stabilization in five patients for up to 13 months after completing chemotherapy or experimental treatment and an improvement in the levels of normal circulating blood cells. In addition, patients did not report any significant side effects attributed to cobomarsen. We believe these data are particularly encouraging, as the patients in this study had previously failed on other therapies and the median survival for patients with acute disease typically ranges from four to ten months after diagnosis," stated Paul Rubin M.D. Executive Vice President, R&D, at miRagen. "We are encouraged by our early experience in the ABC subtype of DLBCL, as we have observed an improvement in a patient that had long-standing disease and had previously been on multiple chemotherapeutic regimens."

Phase 1 Data for Cutaneous T-cell Lymphoma (CTCL)

Updated durability data for the 300mg IV infusion cohort of the Phase 1 cobomarsen clinical trial, which is the dose and route of administration being used in the ongoing SOLAR Phase 2 clinical trial, showed that four of eight patients (50%) achieved an objective response with greater than four months of durability (ORR4).

The Phase 2 SOLAR trial will evaluate the safety and efficacy of cobomarsen given by intravenous infusion in an active control comparison trial versus ZOLINZA (vorinostat) in patients with CTCL. ORR4 is the primary endpoint that will be used in the SOLAR trial. Based on discussions with the U.S. Food and Drug Administration, miRagen believes the results from the SOLAR trial could allow the Company to apply for accelerated approval in the United States.

Phase 1 Data for HTLV-1 Associated Adult T-cell Leukemia/Lymphoma (ATLL)

Data from the ongoing Phase 1 clinical trial in ATLL has shown that cobomarsen had a favorable safety and tolerability profile with no serious adverse events attributed to the drug candidate in the clinical trial and no documented opportunistic infections, which are common in patients with the disease. Four patients – two lymphomatous and two acute – who demonstrated a partial response after chemotherapy have maintained their responses while on cobomarsen monotherapy. Two of these patients – one from each subtype – have been stable for more than a year. There is evidence of disease stabilization in five patients on cobomarsen, as shown in both peripheral blood and lymph nodes, without negatively impacting the number of normal immune cells. One of these lymphomatous patients with significant adenopathy prior to enrollment has remained stable on cobomarsen, as measured by CT scans, for six months. This includes an objective improvement in three out of four measurable abnormal nodes since initiating cobomarsen therapy.

Phase 1 Trial of Cobomarsen in Patients with Diffuse large B-cell lymphoma (DLBCL)

To date, three DLBCL patients of the ABC subtype have received cobomarsen. One patient has seen a complete reduction in one of two measured lymph nodes and stabilization in the second lymph node after six weeks of therapy. This patient remains on cobomarsen. Two of the three patients discontinued therapy after less than one month due to lack of immediate response. Previously, all three patients had relapsed after multiple cycles of treatments with other therapies received over 12-56 months from diagnosis. Prior treatments for these patients ranged from standard of care to experimental chemotherapy.

William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics, stated, "Cobomarsen continues to be generally well-tolerated, has had durable clinical activity in responding patients, and has the potential to improve the quality of life for patients with a variety of hematological malignancies. These clinical responses, combined with the favorable tolerability profile observed across patients with three different types of malignancy, suggests that cobomarsen may combat cancers that overexpress microRNA-155 in a targeted manner."

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

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 adult T-cell leukemia/lymphoma (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 the lymphomatous version. 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 DLBCL

According to the Lymphoma Research Foundation, diffuse large B-cell lymphoma (DLBCL) is the most common type of non-Hodgkin lymphoma (NHL) in the United States and worldwide, accounting for up to one-third of patients with newly diagnosed NHL in the United States. DLBCL is an aggressive (fast-growing) NHL that affects B-lymphocytes. Lymphocytes are one type of white blood cell. B-cells are lymphocytes that make antibodies to fight infections and are an important part of the lymphatic system. DLBCL can develop in the lymph nodes or in "extranodal sites" (areas outside the lymph nodes) such as the gastrointestinal tract, testes, thyroid, skin, breast, bone, brain, or essentially any organ of the body. It may be localized (in one spot) or generalized (spread throughout the body).

Approximately 40% of patients have refractory disease or disease that will relapse after an initial response, and the majority of patients with relapsed DLBCL will succumb to the disease. There are two major biologically distinct molecular subtypes of DLBCL: germinal center B-cell (GCB) and activated B-cell (ABC). ABC DLBCL is associated with substantially worse outcomes when treated with standard chemoimmunotherapy (Nowakowski & Czuczman 2015). One key molecular distinction between the two subtypes is the understanding that nuclear factor (NF)- κ B, a prosurvival and antiapoptotic molecule, is constitutively expressed and may be a key contributor to chemotherapy resistance in the ABC subgroup (Khan & Fisher, Blood 2015). Cobomarsen has been shown to inactivate the (NF)- κ B pathway in a Phase 1 human clinical trial.

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 (MRG-106), remlarsen (MRG-201), 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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