



Miragen Therapeutics to Present First Cobomarsen Phase 1 Clinical Trial Data From Patients With Adult T-Cell Leukemia/lymphoma and New Interim Data From Patients With Mycosis Fungoides at the 2018 American Society of Clinical Oncology Annual Meeting

June 4, 2018

- First two patients with aggressive forms of adult T-cell leukemia/lymphoma treated with cobomarsen demonstrated greater than five months of clinical response with improvement in objective disease measurements
- New results continue to demonstrate durable response rate, good tolerability and quality of life improvement in patients with mycosis fungoides
- Cobomarsen on track to advance into a global Phase 2 clinical trial for mycosis fungoides in the second half of 2018

BOULDER, Colo., June 04, 2018 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced the first observations on the safety and efficacy of cobomarsen in adult T-cell leukemia/lymphoma (ATLL) patients, a highly morbid T-cell malignancy seen in patients previously infected with the human T-lymphotropic virus type 1 (HTLV1). The Company also released new interim data from its ongoing Phase 1 clinical trial of cobomarsen (also known as MRG-106), a microRNA-155 inhibitor, in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). These data will be presented today, June 4, at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting being held in Chicago, IL.

"We are excited to release the first safety and efficacy observations after cobomarsen dosing in ATLL patients as well as new data from MF patients that continue dosing in the Phase 1 study," said miRagen President and CEO William S. Marshall, Ph.D. "We believe these new data from the trial are compelling and support the continued investigation of cobomarsen in the MF population and other hematological malignancies. We remain on track to initiate the Phase 2 clinical trial of cobomarsen in patients with MF in the second half of 2018. Based on FDA feedback, we believe this Phase 2 clinical trial, if positive, has the potential to be registrational."

Two ATLL patients considered to have aggressive disease at baseline have been treated with cobomarsen; one leukemic patient and one with lymphomatous disease. The first observations of the efficacy, safety and tolerability of cobomarsen in ATLL patients as of May 9, 2018 were as follows:

- Both patients have remained stable on cobomarsen for ≥ 5 months and continue to receive doses of the product candidate;
 - Patients continued to feel well and did not develop new ATLL signs or symptoms
 - Cell activation markers and markers of malignant cell proliferation improved, and the improvements were maintained while on treatment with cobomarsen
 - In the leukemic patient, malignant cell counts decreased with chemotherapy and remained stable for >6 months during treatment with cobomarsen alone
 - In the lymphomatous patient, no evidence of recurrent nodal disease was observed for >5 months with cobomarsen alone
- Cobomarsen was generally safe and well tolerated

"The observed activity of cobomarsen in patients with ATLL is encouraging and the clinical trial is ongoing" said Francine Foss, M.D., professor of medicine in the Section of Medical Oncology at the Yale Cancer Center. "ATLL types such as those seen in the patients treated with cobomarsen are associated with poor prognosis and novel therapies are essential to improve patient outcomes in this devastating disease."

The interim MF results include efficacy, safety and tolerability observations from long-term dosing of cobomarsen via various routes of administration in patients who have been enrolled in the study for up to 17 months. As of April 5, 2018, highlights include the following:

- Cobomarsen appeared to demonstrate durable response rate as measured by mSWAT scores and quality of life improvement, as measured by the Skindex-29 Total Score in patients with MF
 - 29 of 32 subjects (91%) treated systemically with cobomarsen have shown mSWAT score improvement
 - In patients who showed improvements in mSWAT scores, these were observed regardless of whether the patient was receiving stable background medication(s) for CTCL or cobomarsen alone
- 11 of 21 pts (52%) receiving greater than one month of cobomarsen achieved a partial response ($\geq 50\%$ reduction in mSWAT)
 - Mean duration of partial response was 213 days and 8 patients achieved ORR4 (a partial response lasting for ≥ 4 months)
- Cobomarsen continued to be generally well tolerated at all dose levels evaluated with no serious adverse events attributed to cobomarsen

Presentation Details

Abstract title: Phase 1 study of MRG-106, an inhibitor of miR-155, in CTCL.

- **Poster session:** Developmental Therapeutics - Clinical Pharmacology and Experimental Therapeutics
 - **Poster number:** 337
 - **Date:** Monday, June 4, 2018, 8:00 a.m. - 11:30 a.m. CT
 - **Location:** Hall A
- **Oral presentation:** Poster Discussion Session
 - **Date:** Monday, June 4, 2018, 3:00 p.m. - 4:15 p.m. CT
 - **Location:** S406

For additional information, as well as an interactive schedule, please visit the ASCO website at www.asco.org.

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 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), 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, 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, 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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Source: Miragen Therapeutics, Inc.