



miRagen Therapeutics Announces New Interim Data From Phase 1 Clinical Trial of Cobomarsen in Mycosis Fungoides

May 8, 2018

- Treatment resulted in durable improved quality of life
- Cobomarsen continued to be generally well tolerated at all dose levels evaluated
- Advancing into a Phase 2 clinical trial in second half of 2018

BOULDER, Colo., May 08, 2018 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, announced new interim data from its Phase 1 clinical trial of cobomarsen in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). The data will be presented May 9, 2018, at the "2015... 2018 T-Cell Lymphomas: we are close to the finalization" medical meeting in Bologna, Italy, by Christiane Querfeld, M.D., Ph.D., Chief of the Division of Dermatology, and Director, Cutaneous Lymphoma Program at the City of Hope in Duarte, California.

"These additional cobomarsen Phase 1 data provide further evidence that microRNA-based therapeutics have the potential to improve the quality of life for patients living with mycosis fungoides," said miRagen President and CEO William S. Marshall, Ph.D. "Cobomarsen continues to be safe and generally well tolerated at all doses tested in the Phase 1 trial, with multiple patients receiving more than a year of therapy with no serious adverse events attributed to the drug. In addition, the improvement in skin disease observed in the trial appears to be durable and is accompanied by improvements in metrics that measure the patients' quality of life. These data support our plans to initiate a Phase 2 clinical trial for cobomarsen in patients with CTCL in the second half of 2018."

The interim results being presented at the conference include safety observations from longer-term dosing of existing patients who have continued participation in the trial and quality of life assessments from 18 patients in the trial. As of April 30, 2018, highlights include the following:

- Cobomarsen treatment resulted in durable improved quality of life, as measured by the Skindex-29 Total Score.
 - 13 of 18 subjects showed an improvement over the first 100 days on cobomarsen.
 - Improvement and stabilization remain durable in four subjects for up to one year, and one subject was stable after more than 400 days on cobomarsen.
- Cobomarsen continued to be generally well tolerated at all dose levels evaluated.
 - Multiple patients received more than a year of therapy (up to 39 grams cumulative dose) with no serious adverse events attributed to cobomarsen.
 - 300 and 600 mg intravenous infusions had similar efficacy and tolerability, offering the most consistent response rate based on skin mSWAT scores, which is a measurement of the severity of skin disease over a patient's entire body.

"These encouraging longer-term data demonstrated that cobomarsen treatment resulted in durable improved quality of life for patients with MF, a disease that in many cases causes painful, disfiguring tumors on the skin, and is in some cases deadly," said Dr. Querfeld. "There is a critical need for a treatment for MF that is efficacious and can be tolerated with long-term dosing, and we believe cobomarsen presents a potential therapeutic option."

Oral Presentation Details

Title: Cobomarsen in CTCL/MF

- **Session:** CTCL: Conventional and Emerging Drugs
- **Date:** May 9, 2018, 11:30 a.m. CET
- **Location:** Royal Hotel Carlton

Cobomarsen is an inhibitor of microRNA-155. In 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.

For additional information, please visit the "2015... 2018 T-Cell Lymphomas: we are close to the finalization" meeting website: www.ercongressi.it.

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.