Cobomarsen Receives Orphan Drug Designation From the U.S. FDA for the Treatment of T-cell Lymphoma

July 23, 2020

BOULDER, Colo., July 23, 2020 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced that the Food & Drug Administration (FDA) has granted orphan drug designation to cobomarsen, for the treatment of T-cell lymphoma. Cobomarsen is an inhibitor of miR-155 currently being developed by miRagen in two clinical programs to address different types of T-cell lymphoma, including a Phase 2 trial for cutaneous T-cell lymphoma (CTCL) and a Phase 1 trial for adult T-cell leukemia/lymphoma (ATLL).

"This is an important milestone in the development of cobomarsen. We believe the FDA's decision to grant cobomarsen orphan drug designation underscores the need for new treatments for T-cell lymphomas such as ATLL and CTCL," stated miRagen President and Chief Executive Officer William S. Marshall, Ph.D. "In addition to the promising results we've observed for the potential treatment of patients with ATLL and CTCL, we believe that cobomarsen has the potential to be a broad-based therapy for the treatment of cancer patients with elevated levels of miR-155."

T-cell lymphomas are lymphomas that affect T lymphocytes such as ATLL and CTCL, amongst others. Together they make up less than 15% of non-Hodgkin lymphomas in the United States. There are many types of T-cell lymphoma, but they are all fairly rare. Overexpression of microRNA-155 has been associated with poor prognosis in a variety of T-cell lymphomas and other blood cancers.

Orphan drug designation is granted by the FDA to drugs or biologics that demonstrate potential for diagnosis and/or treatment of rare diseases or conditions that affect fewer than 200,000 people in the United States. The designation can provide development and commercial incentives for designated compounds and medicines, including eligibility for seven years of market exclusivity in the U.S. after product approval, FDA assistance in clinical trial design and exemption from FDA user fees.

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 miR-155, which is found at abnormally high levels in malignant cells of several blood cancers. miRagen's clinical product candidate for the treatment of pathological fibrosis, remlarsen, is a replacement for miR-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 miR-92, is miRagen's product candidate 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, 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 and current 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.