



## miRagen Therapeutics Receives Orphan-Drug Designation for MRG-106 for the Treatment of Mycosis Fungoides

March 31, 2017

BOULDER, Colo., March 31, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (Nasdaq:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of microRNA-targeted therapies, today announced that the U.S. Food and Drug Administration ("FDA") has granted orphan-drug designation to miRagen's product candidate, MRG-106, for the treatment of mycosis fungoides. Mycosis fungoides is the most common form of a type of blood cancer called cutaneous T-cell lymphoma ("CTCL"). CTCL occurs when certain types of T-cells become cancerous and cause debilitating tumors in the skin and in other parts of the body.

Paul Rubin, M.D., miRagen's Executive Vice President of Research and Development, said, "We see the FDA's granting of orphan-drug designation for MRG-106 as underscoring the medical need for novel therapies in the treatment of mycosis fungoides, and we believe it should facilitate our ability to continue developing this product candidate for potential use in these patients."

"This designation is an important step forward for our MRG-106 program as we continue to advance our clinical and regulatory strategy in areas of high unmet medical need," added William S. Marshall, Ph.D., President and CEO of miRagen. "Patients suffering from mycosis fungoides have few treatment options available, and MRG-106 could be an alternative approach to battling the disease."

### About Miragen Therapeutics, Inc.

Miragen Therapeutics, Inc. is a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapeutics with a specific focus on microRNAs and their role in diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression or activity and play a vital role in influencing the pathways responsible for many disease processes. miRagen believes its experience in microRNA biology and chemistry, drug discovery, bioinformatics, and translational medicine provide it with a potential competitive advantage to identify and develop microRNA-targeted drugs designed to regulate gene pathways to result in disease modification. miRagen uses its expertise in systems biology and oligonucleotide chemistry to discover and develop a pipeline of product candidates. miRagen's two lead product candidates, MRG-106 and MRG-201, are currently in Phase 1 clinical trials. miRagen's clinical product candidate for the treatment of certain cancers, MRG-106, is an inhibitor of microRNA-155, which is found at abnormally high levels in several blood cancers. miRagen's clinical product candidate for the treatment of pathological fibrosis, MRG-201, is a replacement for miR-29, which is found at abnormally low levels in a number of pathological fibrotic conditions, including cardiac, renal, hepatic, and pulmonary fibrosis, as well as systemic sclerosis. In addition to miRagen's clinical programs, it continues to discover and develop a pipeline of pre-clinical 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 (the movement of drug into, through, and out of the body), pharmacodynamics (the effect and mechanism of action of a drug), safety and manufacturability of the product candidate in preclinical studies. For more information, please visit [www.miragentherapeutics.com](http://www.miragentherapeutics.com).

For information on clinical trials please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

### Note Regarding Forward-Looking Statements

This press release contains 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 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, has a limited operating history on which to assess its business, and anticipates that it will continue to incur significant losses for the foreseeable future; miRagen has never generated any revenue from product sales and may never be profitable; raising additional capital may cause dilution to miRagen's stockholders, restrict its operations or require it to relinquish rights; miRagen may be unsuccessful in maintaining orphan-drug designation for its product candidates because even after an orphan drug is approved, the FDA can subsequently approve a different drug for the same indication if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care; clinical trials are costly, time consuming and inherently risky, and miRagen may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities; the approach it is taking to discover and develop novel therapeutics using microRNA is unproven and may never lead to marketable products; miRagen's microRNA therapeutic 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; to date, no microRNA therapeutics have been approved for marketing in the United States; miRagen may not be able to develop or identify technology that can effectively deliver MRG-106, MRG-201 or any other of miRagen's microRNA-targeted product candidates to the intended diseased cells or tissues, and any failure in such delivery technology could adversely affect and delay the development of MRG-106, MRG-201 and miRagen's other product candidates; and miRagen's product candidates may cause undesirable side effects or have other properties that could delay or prevent the regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.

miRagen has based these forward-looking statements largely on its current expectations and projections about future events and trends that it believes may affect its financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in under the heading "Risk Factors" in miRagen's Annual Report on Form 10-K and any 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 these 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.