miRagen Therapeutics Reports Second Quarter 2018 Financial Results and Provides Corporate Update

August 7, 2018

- Cobomarsen expansion indication data released; first observations from the ongoing Phase 1 clinical trial on safety and efficacy of cobomarsen in ATLL patients, additional data expected by year end
- New cobomarsen Phase 1 data in CTCL released, Phase 2 trial expected to initiate in the fourth quarter
- Initiated Phase 2 clinical trial to evaluate remlarsen (also known as MRG-201) in cutaneous fibrosis; data expected in 2019
- Initiated Phase 1 clinical trial in collaboration with Servier to evaluate the safety, tolerability and pharmacokinetics of MRG-110
- Recently announced collaboration and stock purchase with the Leukemia and Lymphoma Society; provides up to $5 million in funding
- $76 millionin cash, cash equivalents, and short-term investments as of June 30, 2018
- Conference call and webcast today at 4:30 p.m. ET

BOULDER, Colo., Aug. 07, 2018 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today reported second quarter 2018 financial results and provided a corporate update.

“This is an exciting time for microRNA-based therapeutics and I am very proud of what our team at miRagen has accomplished,” said William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics. “We are focused on building a sustainable company with the potential to deliver multiple product candidates for patients in need. During the quarter, we announced progress on each of our three clinical stage product candidates. We were encouraged by the initial efficacy data for cobomarsen in patients with ATLL and pleased to have initiated two new clinical trials during the quarter, including a Phase 2 trial for remlarsen (also known as MRG-201) and our second Phase 1 trial of MRG-110 in collaboration with Servier. During the fourth quarter of this year, we plan to initiate our Phase 2 SOLAR clinical trial for cobomarsen in patients suffering from CTCL. We look forward to providing updates for each of our product candidates through the year and continuing to advance our clinical programs into later-stage development.”

Clinical Development Update

Cobomarsen

In June, miRagen announced encouraging first observations from the Phase 1 clinical trial on the safety and efficacy of cobomarsen in adult T-cell leukemia/lymphoma (ATLL) patients at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting. Two patients with an aggressive form of the disease have shown clinical response to the treatment. This clinical trial is ongoing and miRagen expects to release additional data at the 2018 American Society of Hematology (ASH) Annual Meeting in December.

In June, an update of clinical results from the cobomarsen Phase 1 clinical trial in patients with the mycosis fungoides form of cutaneous T-cell lymphoma (CTCL) was also presented at ASCO. Cobomarsen appeared to demonstrate durable responses measured by skin tumor burden measurements and quality of life improvement, as measured by the Skindex-29 Total Score. Cobomarsen also continued to be generally well tolerated at all dose levels evaluated. miRagen expects to present final data from this trial at ASH in December.

During the fourth quarter of this year, miRagen expects to initiate its global Phase 2 SOLAR clinical trial for cobomarsen in CTCL. In the SOLAR trial, miRagen is planning to evaluate the safety and efficacy of 300 milligrams of cobomarsen given by intravenous infusion in an active control comparison trial versus ZOLINZA (vorinostat), enrolling approximately 65 patients per treatment group. The primary endpoint of the SOLAR trial is an overall response rate of 50% or greater improvement in the severity of a patient's skin disease over the entire body maintained for at least four consecutive months with no evidence of disease progression in the blood, lymph nodes or viscera. Progression free survival will be a secondary endpoint and miRagen plans to use patient reported outcomes as an exploratory endpoint to monitor quality of life improvements. Based on discussion with the U.S. Food and Drug Administration, miRagen believes the results from this clinical trial could potentially allow miRagen to apply for accelerated approval in the United States.

Remlarsen (also known as MRG-201)

In July, miRagen announced the initiation of a Phase 2 clinical trial to evaluate remlarsen for cutaneous fibrosis in subjects with a predisposition for keloid formation. miRagen believes this intra-patient-controlled clinical trial may provide data that that could facilitate future development of remlarsen in other dermal scarring indications. Data from this trial is expected to be released in 2019.

miRagen also has a robust preclinical development program that aims to provide support for the advancement of remlarsen for the treatment of additional pathological fibrotic conditions. In the second half of 2018, miRagen anticipates releasing preclinical data for remlarsen in lung fibrosis studies.

MRG-110

In May, following the initiation of a Phase 1 clinical trial of MRG-110 by Servier in the first quarter of 2018, miRagen announced the initiation of a second Phase 1 clinical trial of MRG-110. Both trials are designed to evaluate the safety, tolerability and pharmacokinetics of MRG-110. The first trial is a systemic dosing protocol that is intended to support the development of MRG-110 for the treatment of heart failure. In the second trial, MRG-110 is

being administered by intradermal injection in healthy volunteers receiving induced wounds through biopsy. In addition to assessing the tolerability of MRG-110 after local administration, the second clinical trial is designed to provide data that can validate MRG-110's intended mechanism of action in humans. The data generated in this trial is expected to provide several clinically translatable biomarkers that may support future clinical studies for the treatment of heart failure, as well as surgical incisions in high risk populations, severe lacerations and chronic wounds.

Second Quarter 2018 Financial Results

miRagen had $76 million in cash, cash equivalents, and short-term investments as of June 30, 2018. Cash used in operating activities was $4.5 million for the second quarter of 2018 and $11.9 million for the first half of the year. During the second quarter, miRagen received a $3.7 million development milestone payment from Servier, which was recognized as revenue in the first quarter of 2018. miRagen had 30.6 million shares of common stock outstanding as of June 30, 2018.

The Company reported a net loss available to common stockholders for the second quarter of 2018 of $8.7 million, or ($0.29) per share (basic and diluted), and $13.4 million, or ($0.47) per share for the first half of 2018. The net loss for the second quarter of 2017 was $7.3 million, or ($0.34) per share and $14.3 million or ($0.87) per share (basic and diluted) for the first half of 2017. Net loss for the second quarter and first half of 2018 included revenue of $2.2 million and $7.0 million, respectively, compared to $0.7 million and $1.2 million for the same periods of 2017. The decrease in net loss year over year was primarily due to higher revenue recognized under miRagen's collaboration with Servier partially offset by increases in research and development expenses in 2018.

Research and development expenses totaled $8.4 million for the second quarter of 2018, and $14.8 million for the first half of 2018, compared to $5.5 million for the second quarter of 2017, and $9.6 million for the first half of 2017. The increase year over year is primarily due to increased clinical development activities associated with each of miRagen's three clinical stage product candidates together with an increase in personnel-related costs as miRagen has grown its research and development team.

General and administrative expenses totaled $2.7 million for the second quarter of 2018, and $5.7 million for the first half of 2018, compared to $2.6 million for the second quarter of 2017 and $5.9 million for the first half of 2017. In 2018, miRagen incurred increased personnel and board of director related costs as miRagen added to its team. These increases were offset by lower legal fees in 2018 when compared to the higher merger-related legal expenses incurred in 2017 and, to a lesser extent, lower consulting and contract labor expenses.

Conference Call Information

miRagen will host a conference call today at 4:30 p.m. ET to discuss its financial results for the second quarter 2018. Participants may access the call by dialing 800-289-0438 in the U.S. or 323-794-2423 outside the U.S. and providing the conference ID number 2516510. The call will also be webcast and can be accessed from the Investors and Media section of miRagen's website at www.miragen.com. A replay of this conference call will be available on miRagen's website approximately one hour after the event.

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.

Miragen Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue:				
Collaboration revenue	$ 1,368	$ 488	$ 6,124	$ 498
Grant revenue	814	230	842	682
Total revenue	2,182	718	6,966	1,180
Operating expenses:				
Research and development	8,375	5,487	14,788	9,607
General and administrative	2,668	2,581	5,658	5,862
Total operating expenses	11,043	8,068	20,446	15,469
Loss from operations	(8,861)	(7,350)	(13,480)	(14,289)
Other income (expense):				
Interest and other income	361	102	528	132
Interest and other expense	(214)	(64)	(423)	(135)
Net loss	(8,714)	(7,312)	(13,375)	(14,292)
Change in unrealized gain on investments	4	—	4	—
Comprehensive loss	$ (8,710)	$ (7,312)	$ (13,371)	$ (14,292)
Net loss	$ (8,714)	$ (7,312)	$ (13,375)	$ (14,292)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(5)
Net loss available to common stockholders	$ (8,714)	$ (7,312)	$ (13,375)	$ (14,297)
Net loss per share, basic and diluted	$ (0.29)	$ (0.34)	$ (0.47)	$ (0.87)
Weighted-average shares used to compute basic and diluted net loss per share	30,295,200	21,409,708	28,399,687	16,509,719

Miragen Therapeutics, Inc.
Selected Financial Information
Condensed Consolidated Balance Sheet Data
(amounts in thousands)
(unaudited)

	June 30, 2018	December 31, 2017
Cash and cash equivalents	$ 43,402	$ 47,441
Short-term investments	$ 32,860	$ —
Total assets	$ 81,887	$ 52,481
Notes payable, inclusive of current portion	$ 10,111	$ 9,922
Total liabilities	$ 14,216	$ 13,971
Total stockholders' equity	$ 67,671	$ 38,510

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Source: Miragen Therapeutics, Inc.