miRagen Therapeutics Reports Third Quarter 2017 Financial Results and Provides Corporate Update

November 8, 2017

- MRG-106 Phase 1 trial expanded to include additional oncology indications, enrollment commenced
- MRG-201 expected to advance into a Phase 2a trial in cutaneous fibrosis in the first half of 2018
- MRG-110 on track to complete Investigational New Drug (IND) application/Clinical Trial Application (CTA) enabling studies in the fourth quarter of 2017
- Conference call and webcast today at 4:30 p.m. ET

BOULDER, Colo., Nov. 08, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced third quarter 2017 financial results and provided a corporate update.

“miRagen made continued progress across its clinical and pre-clinical programs in the third quarter,” said miRagen President and Chief Executive Officer William S. Marshall, Ph.D. “We are very encouraged by the new MRG-106 clinical data, which showed that 96% of patients treated systemically experienced improvement in total skin disease across all dose levels evaluated. We have expanded the ongoing Phase 1 trial to evaluate MRG-106 in three additional oncology indications, and recently began enrolling subjects. Additionally, we plan to initiate a Phase 2a clinical trial for MRG-201 in the first half of 2018, and advance MRG-110 into clinical development in collaboration with Servier in the first half of 2018.”

Business Highlights and Update

- **Reported new interim MRG-106 Phase 1 clinical trial results:** In October 2017, miRagen announced new interim results from the systemic administration portion of its ongoing Phase 1 clinical trial evaluating the safety, efficacy and pharmacokinetics of MRG-106 in patients with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL). These data were presented at the European Organisation for Research and Treatment of Cancer Cutaneous Lymphoma Task Force Meeting. This part of the trial employed a multiple dose-escalation design to evaluate 300 mg, 600 mg or 900 mg subcutaneous or intravenous administrations of MRG-106. Twenty-two of twenty-three patients (96%) treated systemically showed improvement in total skin disease as measured by the maximal change in each patient’s modified Severity Weighted Assessment Tool (mSWAT) score, which assesses the severity of skin disease over a patient’s entire body. Nine of thirteen patients treated for more than one month showed a 50% or greater maximal improvement in mSWAT scores with five patients, to date, demonstrating a duration of this improvement for four months or longer. MRG-106 has been generally well-tolerated to date at dose levels ranging from 75 mg to 900 mg.

- **Expanded MRG-106 Phase 1 trial to include three new indications:** miRagen is evaluating MRG-106 in additional oncology indications within the current Phase 1 trial. These additional indications include adult T-cell leukemia/lymphoma, diffuse large B-cell lymphoma, and chronic lymphocytic leukemia. In each of these expansion indications, the disease process appears to correlate with an increase in microRNA-155 levels, the sole target of MRG-106. Dosing of subjects in the expansion indications has commenced, and the Company plans to release interim data during 2018.

- **MRG-201 expected to advance into a Phase 2a trial in the first half of 2018:** Earlier this year, miRagen announced results from the double-blind, placebo-controlled, single and multiple dose-escalation Phase 1 trial evaluating MRG-201 in induced cutaneous fibrosis. These data were presented at the American Society for Dermatologic Surgery Annual Meeting and the Society for Investigative Dermatology Annual Meeting. Fifty-four volunteers participated in the clinical trial, and MRG-201 was generally well tolerated at all dose levels evaluated. In addition, miRagen believes the trial provided evidence that MRG-201 may be able to regulate fibrogenesis in humans. In the trial, treatment with MRG-201 appeared to result in a reduction in fibroplasia, a histopathological marker of scar tissue deposition, while not effecting wound healing. In the first half of 2018, the Company intends to initiate a Phase 2a trial to evaluate MRG-201 in subjects with a predisposition for keloid formation. Future indications to be studied for miR-29 replacements could include fibrotic diseases of the lung and eye.

  In September 2017, miRagen also announced new preclinical safety and feasibility data on inhaled delivery of MRG-201, which the Company plans to evaluate for the potential treatment of pulmonary fibrosis. These data were presented at the European Respiratory Society International Congress. These results appeared to demonstrate that MRG-201 can be nebulized with its chemical integrity maintained, and may be administered via nose-only inhalation to rats. The results also showed that exposure to lung tissue after inhalation was high while distribution to tissues tested beyond the lung was minimal, suggesting a potentially effective localized effect and limited systemic impact.

- **MRG-110 expected to advance into clinical trials with Servier in the first half of 2018:** The Company and Servier plan

to initiate two Phase 1 clinical trials to evaluate the safety, pharmacokinetic and pharmacodynamic effects of MRG-110, an inhibitor of microRNA-92a delivered both systemically and intradermally. MicroRNA-92a has been shown in miRagen's preclinical studies and reported in multiple peer reviewed scientific publications to be a regulator of new blood vessel creation. As part of the Phase 1 clinical trials, in addition to safety and pharmacokinetics, miRagen intends to analyze biomarkers that may provide mechanistic proof of concept and support further study of MRG-110 in the treatment of cardiovascular disease and certain other conditions where vascular flow is compromised.

Anticipated Milestones

- MRG-106 (hematological malignancies)
  - Phase 1 CTCL data presentation at American Society of Hematology Annual Meeting (Q4 2017)
  - Phase 1 data release in expansion indications (2018)
  - Initiation of a Phase 2 trial in CTCL/Non-Hodgkin's lymphoma (2H 2018)
  - Presentation of Phase 2 CTCL data (2019/2020)

- MRG-201 (pathologic fibrosis)
  - Initiation of a Phase 2a trial in cutaneous fibrosis (1H 2018)
  - Preclinical safety and efficacy lung fibrosis data release (2018)
  - Presentation of Phase 2a cutaneous fibrosis trial data (2019)

- MRG-110 (ischemic disease)
  - Completion of IND/CTA enabling studies (Q4 2017)
  - Initiation of Phase 1 trial(s) (1H 2018)

Upcoming Events

miRagen plans to present at the following upcoming conferences:

- Anti-Fibrotic Drug Development Conference, Boston, MA, November 13-14, 2017
- Stifel Healthcare Conference, New York, NY, November 14-15, 2017
- Jefferies London Healthcare Conference, London, UK, November 15-16, 2017
- Piper Jaffray Healthcare Conference, New York, NY, November 28-29, 2017
- Evercore ISI Biopharma Catalyst/Deep Dive Conference, Boston, MA, November 29-30, 2017
- American Society of Hematology Annual Meeting, Atlanta, GA, December 9-12, 2017

Third Quarter 2017 Financial Results

Revenue was $1.6 million for the third quarter of 2017, compared to $0.9 million for the third quarter of the prior year. The increase in revenue was primarily driven by increases in research and development activity reimbursable under our agreement with Servier, which was amended in 2017.

Research and development expenses increased to $5.0 million during the third quarter of 2017 from $3.0 million during the same period in 2016. The increase was driven primarily by increased personnel costs as the Company grew its research and development team and higher clinical trial and related outsourced manufacturing costs to support its expanding development stage programs.

General and administrative expenses increased to $2.5 million during the third quarter of 2017 from $2.1 million during the same period in 2016. The increase was due primarily to increases in consulting, professional fees and board compensation, related to the Company's expanded operations since becoming a public company in February 2017. The Company also incurred higher share-based compensation charges and increased legal costs related to patent filing, prosecution and enforcement. These increases were partially offset by a decrease in legal expenses attributable to merger related activities during the third quarter of 2017, as compared to the third quarter of 2016.

Net loss attributable to common stockholders was $5.8 million for the third quarter of 2017, compared to $4.2 million for the third quarter of 2016.

Cash and cash equivalents at September 30, 2017 were $42.8 million, compared to $22.1 million at December 31, 2016. Total net cash used in operations was $20.9 million for the nine months ended September 30, 2017. Total net cash flows provided by financing activities for the nine months ended September 30, 2017 of $40.6 million included $39.5 million in net proceeds from the February 2017 financing.

miRagen's principal use of capital continues to be research and development activities aimed at advancing its development stage programs and pipeline of preclinical programs, as well as general and administrative expenses to support its public company compliance and administrative obligations. Based on the Company's research and development plans, it expects that the cash and cash equivalents as of September 30, 2017, as well as amounts funded by Servier under the Company's collaboration agreement, will enable miRagen to fund its operating expenses and capital expenditures through the end of 2018.

Conference Call & Webcast

miRagen's senior management will host a conference call and live audio webcast today at 4:30 p.m. ET to discuss its third quarter 2017 financial results and provide a corporate update. The conference call is being webcast and can be accessed from the miRagen website, www.miragen.com, under Investors & Media. A replay of the webcast will be available for 90 days. The conference call can also be accessed by dialing 800.500.0311 (U.S./Canada) or 719.457.2617 (international) and providing the passcode 5433113.

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's two lead product candidates, MRG-106 and MRG-201, are currently in clinical development. 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 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. miRagen also is developing MRG-110, an inhibitor of microRNA-92, under license and collaboration agreement with Servier. MRG-110 is being developed for the treatment of heart failure and other ischemic disease. In addition to these programs, miRagen is developing 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, pharmacodynamic, safety and manufacturability of the product candidate in pre-clinical 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, and as a result miRagen cannot guarantee that it will be able to start, or cause its clinical trials to progress, on the schedule it currently anticipates; 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 results of miRagen's Phase 1 clinical trials 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
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	**2016**	**2017**	**2016**
Revenue:				
Collaboration revenue	$ 1,493	$ 715	$ 1,991	$ 2,479
Grant revenue	138	221	820	489
Total revenue	1,631	936	2,811	2,968
Operating expenses:				
Research and development	5,018	2,965	14,625	9,786
General and administrative	2,502	2,053	8,364	4,255
Total operating expenses	7,520	5,018	22,989	14,041
Loss from operations	(5,889)	(4,082)	(20,178)	(11,073)
Other income (expense):				
Interest and other income	113	7	245	23
Interest and other related expense	(58)	(78)	(193)	(250)
Net loss	(5,834)	(4,153)	(20,126)	(11,300)
Accretion of redeemable convertible preferred stock to redemption value	—	(13)	(5)	(37)

Net loss available to common stockholders	$ (5,834)	$ (4,166)	$ (20,131)	$ (11,337)
Net loss per share, basic and diluted	$ (0.27)	$ (6.92)	$ (1.11)	$ (18.84)
Weighted-average shares used to compute basic and diluted net loss per share	21,572,498	601,667	18,215,857	601,667

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

	September 30, 2017	December 31, 2016
Cash and cash equivalents	$ 42,805	$ 22,104
Total assets	$ 47,081	$ 24,760
Notes payable, inclusive of current portion	$ 3,374	$ 4,789
Total liabilities	$ 8,096	$ 9,705
Redeemable convertible preferred stock	$ —	$ 76,976
Total stockholders' equity (deficit)	$ 38,985	$ (61,921)

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Source: Miragen Therapeutics, Inc.