miRagen Therapeutics Reports Fourth Quarter and Full Year 2017 Financial Results and Provides Corporate Update

March 14, 2018

- Data from Phase 1 clinical trial supports advancing cobomarsen, or MRG-106, into a Phase 2 potentially registrational clinical trial for CTCL (the SOLAR trial)
- Completed Phase 1 clinical trial of MRG-201 supports advancement into a Phase 2 trial in cutaneous fibrosis / scarring
- miRagen-Servier strategic alliance advances with Servier filing a clinical trial authorization to initiate the first of two Phase 1 clinical trials planned for MRG-110 in 2018
- Conference call and webcast today at 4:30 p.m. ET

BOULDER, Colo., March 14, 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 fourth quarter and full year 2017 financial results and provided a corporate update.

“2017 was a remarkable year for miRagen, underscored by the clinical success and advancement of our two lead programs, cobomarsen (MRG-106) and MRG-201, and we believe we are well-positioned to make advances in the development of our product candidates in 2018,” said miRagen President and Chief Executive Officer William S. Marshall, Ph.D. “The cobomarsen Phase 1 results have been encouraging, and we plan to initiate a Phase 2 clinical trial to evaluate cobomarsen in cutaneous T-cell lymphoma (CTCL) this year. We have designed this trial to allow the possibility of accelerated approval in the United States, subject to the outcome of the trial. In 2018, we expect to initiate four clinical trials, including the Phase 2 SOLAR trial evaluating cobomarsen in CTCL, a Phase 2 trial for MRG-201 in cutaneous fibrosis and two Phase 1 trials in collaboration with Servier for MRG-110 to support development in ischemic diseases. Additionally, in 2018 we expect to report interim data from our Phase 1 trial evaluating cobomarsen in multiple hematological malignancies beyond CTCL.”

Business Highlights and Update

- **Data from Phase 1 trial supports advancing cobomarsen (MRG-106) into a Phase 2 potentially registrational trial for CTCL:** In February 2018, miRagen announced interim results from its ongoing Phase 1 clinical trial evaluating the safety, efficacy and pharmacokinetics of cobomarsen in patients with the mycosis fungoides form of CTCL. These data showed 26 of 29 evaluable patients, or 90%, with improved modified Severity Weighted Assessment Tool (mSWAT) scores, a measurement of the severity of skin disease. Four of five patients (80%) who were treated with 300 mg IV infusion achieved a 50% or greater mSWAT reduction, which the Company believes represents an important beneficial clinical response. Cobomarsen has been generally well-tolerated to date at the range of doses tested.

  miRagen intends to initiate a Phase 2 clinical trial for cobomarsen in patients with CTCL in the second half of 2018. Based on recent meetings with the U.S. Food and Drug Administration, miRagen anticipates the trial will employ an open-label, parallel group, randomized design to evaluate the safety and efficacy of 300 mg of cobomarsen given by intravenous infusion versus an active control.

  The Company also began evaluating cobomarsen in three additional oncology indications within the current Phase 1 trial. These additional indications include chronic lymphocytic leukemia, diffuse large B-cell lymphoma, and adult T-cell leukemia/lymphoma. In each of these expansion indications, the disease process appears to correlate with an increase in microRNA-155 levels. The Company plans to release interim Phase 1 data in at least one of these expansion indications in the second half of 2018.

- **Phase 1 trial completed, supports advancing MRG-201 into a Phase 2 trial in the first half of 2018:** miRagen completed a Phase 1 clinical trial evaluating MRG-201 in induced cutaneous fibrosis in 2017. 54 volunteers participated in the clinical trial, and MRG-201 was generally well tolerated at all dose levels evaluated. In addition, miRagen believes the clinical trial provided evidence that MRG-201 regulated fibrogenesis in humans. In the clinical 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 2 trial to evaluate MRG-201 in subjects with a predisposition for keloid formation.

  miRagen also anticipates releasing preclinical in vivo data from lung and ocular fibrosis studies in 2018.

- **miRagen-Servier strategic alliance advances with Servier filing a clinical trial authorization to initiate the first of two Phase 1 clinical trials planned for MRG-110:** In the first half of 2018, Servier plans to initiate a Phase 1 trial to evaluate the safety, tolerability, and pharmacokinetics of MRG-110 (S95010) in a systemic dosing protocol. This Phase 1

trial may allow miRagen and Servier to establish the recommended Phase 2 clinical trial dose for the treatment of patients with heart failure. The Phase 1 clinical trial results will also be analyzed for biomarkers that may provide mechanistic proof of concept and support further potential clinical trials of MRG-110 in the treatment of cardiovascular disease and certain other conditions where vascular flow is compromised.

Also, during the first half of 2018, miRagen plans to initiate a separate Phase 1 trial assessing the safety and tolerability of MRG-110 after intradermal administration in healthy volunteers. The intradermal administration clinical trial is intended to support additional clinical studies necessary to achieve regulatory approval for the use of MRG-110 in other diseases, including dermatological applications, where increased vascularity may result in better healing and improved outcomes.

Servier is responsible for leading the global clinical development and potential commercialization of MRG-110 in all countries except the United States and Japan, where miRagen retains all rights.

Anticipated Milestones

- Cobomarsen (blood cancers)
  - Presentation of additional Phase 1 CTCL data, including response rates from longer-term duration of treatment (1H 2018)
  - Phase 1 interim clinical data release in at least one potential expansion indication (2H 2018)
  - Initiation of a Phase 2 clinical trial in CTCL (2H 2018)
  - Presentation of Phase 2 CTCL clinical trial data (2H 2020)

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

- MRG-110 (ischemic disease)
  - Initiation of two Phase 1 trials (1H 2018)

Financial Results

- **Cash and cash equivalents** at December 31, 2017 were $47.4 million, compared to $22.1 million at December 31, 2016. Total net cash used in operations was approximately $7.2 million and $28.2 million for the quarter and year ended December 31, 2017, respectively. In February 2018, miRagen completed an underwritten public offering of 7,414,996 shares of its common stock. Gross proceeds to miRagen from the offering were $40.8 million before deducting underwriting discounts, commissions and other offering expenses. As a result of this financing and based on miRagen's current clinical development plans, the company expects its cash runway will extend into early 2020.

- **Net loss** available to common stockholders for the year ended December 31, 2017 was $26.5 million, or $1.38 per share, and $6.4 million, or $0.29 per share, for the fourth quarter of 2017, as compared to a net loss of $17.3 million, or $28.21 per share, for the year ended December 31, 2016, and $6.0 million, or $9.20 per share, for the fourth quarter of 2016.

- **Revenue** was $4.0 million for the year ended December 31, 2017, and $1.2 million for the fourth quarter of 2017, as compared to $3.5 million for the year ended December 31, 2016, and $0.5 million for the fourth quarter of 2016. The increase in revenue was primarily driven by increases in research and development activity reimbursable under miRagen's agreement with Servier.

- **Research and development expenses** were $19.6 million for the year ended December 31, 2017, and $5.0 million for the fourth quarter of 2017, as compared to $13.7 million for the year ended December 31, 2016 and $3.9 million for the fourth quarter of 2016. The increase in research and development expenses was driven primarily by higher clinical trial and related outsourced manufacturing costs to support expanded development stage programs together with increased personnel costs as the Company added to its research and development team in 2017.

- **General and administrative expenses** were $10.9 million for the year ended December 31, 2017, and $2.5 million for the fourth quarter of 2017, as compared to $6.8 million for the year ended December 31, 2016 and $2.5 million for the fourth quarter of 2016. The increase in general and administrative expenses in 2017 was due primarily to increases in insurance, 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 personnel related costs, due primarily to an increase in share-based compensation charges, as well as an increase in general and administrative employees during 2017.

Conference Call Information

miRagen will host a conference call today at 4:30 p.m. ET to discuss its financial results for the fourth quarter and full year 2017. Participants may access the call by dialing (800) 289-0438 in the U.S. or (323) 994-2083 outside the U.S. and providing the conference ID number 5740056. The call will also be webcast and can be accessed from the Investors and Media section of the Company'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's two lead product candidates, cobomarsen (MRG-106) and MRG-201, are currently in clinical development. miRagen's clinical product candidate for the treatment of certain cancers, cobomarsen (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 is also developing MRG-110, an inhibitor of microRNA-92, under a license and collaboration agreement with Servier. MRG-110 is being developed for the treatment of heart failure and other ischemic diseases. 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, 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.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2017	2016	2017	2016
Revenue:				
Collaboration revenue	$ 1,106	$ 335	$ 3,097	$ 2,814

Grant revenue	86	174	906	663
Total revenue	1,192	509	4,003	3,477
Operating expenses:				
Research and development	4,998	3,906	19,623	13,692
General and administrative	2,548	2,517	10,912	6,772
Total operating expenses	7,546	6,423	30,535	20,464
Loss from operations	(6,354)	(5,914)	(26,532)	(16,987)
Other income (expense):				
Interest and other income	158	16	403	39
Interest and other expense	(190)	(76)	(383)	(326)
Net loss	(6,386)	(5,974)	(26,512)	(17,274)
Accretion of redeemable convertible preferred stock to redemption value	—	(12	(5)	(49)
Net loss available to common stockholders	$ (6,386)	$ (5,986)	$ (26,517)	$ (17,323)
Net loss per share, basic and diluted	$ (0.29)	$ (9.20)	$ (1.38)	$ (28.21)
Weighted-average shares used to compute basic and diluted net loss per share	22,297,302	650,799	19,244,605	614,017

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

	December 31,	
	2017	2016
Cash and cash equivalents	$ 47,441	$ 22,104
Total assets	$ 52,481	$ 24,760
Notes payable, inclusive of current portion	$ 9,922	$ 4,789
Total liabilities	$ 13,971	$ 9,705
Redeemable convertible preferred stock	$ —	$ 76,976
Total stockholders' equity (deficit)	$ 38,510	$ (61,921)

Source: Miragen Therapeutics, Inc.