miRagen Reports Fourth Quarter and Full Year 2019 Financial Results

March 11, 2020

- *Completed enrollment of 37 patients in the Phase 2 SOLAR trial of cobomarsen in CTCL; expects to report topline data in Q3 2020*
- *Completed initial planned enrollment of the Phase 1 trial of cobomarsen in ATLL patients; reported additional data in January 2020; expects to meet with the FDA in Q3 2020 to explore a potential expedited development path*
- *Entered into a $20.0 million stock purchase agreement in December 2019; $5.1 million funded to date*
- *Closed $15.0 million equity offering in February 2020 extending cash runway into Q3 2021*
- *Reported $26.8 million in cash, cash equivalents, and short-term investments as of December 31, 2019*
- *Management to host conference call today at 4:30 p.m. ET*

BOULDER, Colo., March 11, 2020 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ: MGEN), today reported operating highlights and financial results for the quarter and full-year ended December 31, 2019 and provided an outlook for 2020.

“In 2019, we announced a series of changes intended to streamline our focus and operations. As a result, we enter 2020 with the opportunity to deliver several important milestones. We started the year by announcing positive new data for cobomarsen in ATLL patients in January and completed a $15.0 million equity financing in February which we believe extends our cash runway into the third quarter of 2021,” stated miRagen President and Chief Executive Officer, William S. Marshall, Ph.D.

“In the next few quarters, we look forward to meeting with FDA to discuss a potentially expedited development path for cobomarsen in ATLL, reporting topline data from the Phase 2 cobomarsen SOLAR trial in CTCL, and reporting additional preclinical data for MRG-229, our idiopathic pulmonary fibrosis (IPF) therapy product candidate. We believe this program provides us an exciting opportunity to deliver a potentially differentiated approach for the treatment of patients with IPF.”

Summary of Program Highlights

Cobomarsen: Cobomarsen is currently being evaluated for the potential treatment of patients with miR-155 elevated hematological malignancies, including Cutaneous T-Cell Lymphoma (CTCL) and Adult T-Cell Leukemia/Lymphoma (ATLL).

- **Cutaneous T-Cell Lymphoma:** In December 2019, the Company announced plans to stop the enrollment of new patients in the SOLAR trial and conduct an analysis of topline clinical response. This analysis will provide controlled data to assess the benefit of cobomarsen based on disease response in the skin in comparison to vorinostat. A total of 37 patients have been enrolled and will continue to be evaluated for safety and clinical response in the coming months. miRagen plans to assess the rate of an objective response in the skin, that is durable for four months, defined as 50% or greater improvement in the severity of a patient’s skin disease over the entire body (mSWAT). This change from assessing overall response to skin response was driven by the fact that patients allowed into the study only have skin disease and are verified not to have blood, nodes, or visceral involvement at study entry. Improvements in skin disease are thus intended to reflect efficacy of the drug whereas progression in skin disease reflect lack of efficacy. Follow up analysis for blood, nodes or visceral disease may be conducted based on the results obtained using mSWAT. The Company believes that obtaining controlled clinical data from this cohort of patients may allow for a better assessment of the clinical potential of cobomarsen as compared to data from its Phase 1 trial. The Company intends for this controlled clinical data to form the basis of determining what additional clinical investigation of cobomarsen in CTCL is warranted, if any, and what would be required to potentially obtain regulatory approval. Topline data from this amended trial is expected to be announced in the third quarter of 2020.

- **Adult T-Cell Leukemia/Lymphoma:** In January 2020, the Company announced positive data for cobomarsen in ATLL patients with residual disease from this first-in-human Phase 1 clinical trial. In this trial, cobomarsen was observed to improve disease stabilization and reduce cellular proliferation and activation biomarker expression associated with ATLL cellular proliferation and activation in patients with persistent residual disease after chemotherapy and other therapies. Based on these results, the Company announced that it is focusing its cobomarsen expansion indication efforts on ATLL and expects to meet with the FDA in the third quarter of 2020 to explore a potential expedited development path for cobomarsen in ATLL.

Remlarsen and MRG-229: Remlarsen and MRG-229 are miR-29 mimics, or replacements for miR-29, a microRNA that is found at abnormally low levels in a number of pathologic fibrotic conditions.

- **Cutaneous Fibrosis (Remlarsen):** During the fourth quarter of 2019, the Company reported interim data from a Phase 2 clinical trial assessing remlarsen for safety, tolerability, and activity in the potential prevention or reduction of keloid formation in patients with a history of keloid scars, a form of pathological scarring. These data suggest that remlarsen was

generally safe and well tolerated and treatment had no negative effect on healing reported. In addition, the Company observed initial volume reductions in treated keloids compared to placebo in a subset of patients. Based on these data, the Company has decided to continue its analysis of patient data at the one-year primary endpoint of the clinical trial. With these data, the Company may seek a collaboration partner for the future development of remlarsen.

- **Ocular Fibrosis (Remlarsen):** The Company is also evaluating remlarsen in ocular fibrotic indications, such as corneal injury and keratitis. In April 2019, miRagen presented data in preclinical studies testing remlarsen's ability to penetrate injured corneas and reduce fibrosis after a corneal injury. Topical administration of remlarsen to an injured rat cornea resulted in faster healing of the cornea and reduced scarring/hazing. Remlarsen has also been observed in in vitro studies to regulate miR-29 pharmacodynamic biomarkers in the cornea.

- **Idiopathic Pulmonary Fibrosis (MRG-229):** In December 2019, the Company announced that its preclinical pipeline development efforts will be primarily focused on the development of MRG-229 as a potential treatment for patients with IPF. The Company believes that the efficacy and safety profile of MRG-229 positions it as a potentially differentiated approach for IPF. This program is supported in part by a grant in collaboration with the National Institutes of Health and Yale University. miRagen expects to report additional preclinical safety and efficacy data for MRG-229 during the second quarter of 2020.

MRG-110:MRG-110 is an inhibitor of miR-92, a microRNA expressed in endothelial cells, which has been observed in preclinical studies to be a regulator of new blood vessel creation and other wound healing processes.

- **Tissue Repair:** During the fourth quarter of 2019, the Company announced data from two Phase 1 clinical trials of MRG-110 in normal human volunteers, in which administration of MRG-110 was observed to increase angiogenesis, as demonstrated by increased perfusion and histological markers of neoangiogenesis, as well as reduce alpha-smooth muscle actin (α-SMA) expression, which has been shown to correlate with activation of myofibroblasts. A total of 65 subjects were exposed for up to three weeks. MRG-110 was shown to be generally safe and well tolerated, with no evidence of unwanted distal angiogenesis, acute inflammatory toxicities, or significant abnormalities in liver, kidney, or blood, and no injection site reactions. The Company believes that MRG-110 may have the potential to be used for the treatment of heart failure and other conditions where patients may benefit from increased vascular flow and accelerated healing in indications such as burns, skin flaps, grafts, or laparotomy or sternotomy incisions in patients with high risk of poor wound closure.

Financial Results

- **Cash Position and Runway:** Cash, cash equivalents, and short-term investments were $26.8 million as of December 31, 2019, compared to $62.5 million as of December 31, 2018. Net cash used in operating activities was $7.8 million for the fourth quarter of 2019 and $36.1 million for the year ended December 31, 2019. Based on the Company's cash, cash-equivalents, and short-term investments as of December 31, 2019 and after giving effect to proceeds received after year end through the date of this release, the Company believes that its current cash, cash equivalents and short term investments will be sufficient to fund its operations into the third quarter of 2021.

- **Revenue:** Revenue was $0.9 million for the fourth quarter of 2019 and $4.5 million for the year ended December 31, 2019, compared to $0.5 million and $8.4 million, respectively, for the comparable periods in 2018. In 2018, miRagen earned a development milestone payment under a collaboration agreement and recognized $3.7 million of revenue.

- **Research and Development Expenses:** Research and development expenses were $8.4 million for the fourth quarter of 2019 and $34.8 million for the year ended December 31, 2019, compared to $8.2 million and $30.4 million, respectively, for the comparable periods in 2018. The year over year increase was primarily due to increased clinical development activities associated with the Phase 2 SOLAR clinical trial of cobomarsen and increased personnel-related costs, including restructuring charges, partially offset by decreases in technology license fees and other miscellaneous expenses in 2019.

- **General and Administrative Expenses:** General and administrative expenses were $2.5 million for the fourth quarter of 2019 and $11.6 million for the year ended December 31, 2019, compared to $2.7 million and $11.0 million, respectively, for the comparable periods in 2018. During 2019, the Company's general and administrative costs increased as compared to 2018 due primarily to increased personnel-related costs, including restructuring costs, and increased legal expenses.

- **Restructuring Costs:** In 2019, the Company began implementing two phases of a cost restructuring plan to streamline its operations, reduce costs, and direct resources to advance cobomarsen and miR-29 mimics, while reducing investments in new discovery research. The restructuring plan identified approximately 44 positions for elimination, or approximately 50% of the Company's workforce, primarily associated with research and development and corresponding project, general, and administrative support. Restructuring charges of $2.0 million were recorded during the year ended December 31, 2019, of which $1.7 million was recorded in research and development expenses and $0.3 million recorded in general and

administrative expenses. Additional restructuring costs of approximately $0.2 million are expected to be incurred in the first half of 2020.

- **Net Loss:** The Company's net loss was $10.1 million, or $0.31 per share, for the fourth quarter of 2019, and $41.9 million, or $1.34 per share, for the year ended December 31, 2019, compared to $10.3 million, or $0.33 per share, for the fourth quarter of 2018, and $32.7 million, or $1.10 per share, for the year ended December 31, 2018.

Conference Call Information

The miRagen Therapeutics management team will host a conference call and webcast today at 4:30 p.m. ET to provide and discuss its financial results for the fourth quarter and full year ended 2019 and its outlook for 2020. To access the call, please dial 877-407-0789 (domestic) or 201-689-8562 (international) and provide the passcode 13699475. A live webcast of the call will be available on the Investors section of the miRagen Therapeutics website at www.miragen.com and a replay of this conference call will be available approximately one hour after its completion.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2019	2018	2019	2018

Revenue:				
Collaboration revenue	$ 837	$ 435	$ 4,308	$ 7,373
Grant revenue	43	41	153	1,013
Total revenue	880	476	4,461	8,386
Operating expenses:				
Research and development	8,417	8,234	34,794	30,421
General and administrative	2,534	2,695	11,646	11,049
Total operating expenses	10,951	10,929	46,440	41,470
Loss from operations	(10,071)	(10,453)	(41,979)	(33,084)
Other income (expense):				
Interest and other income	123	364	941	1,254
Interest and other expense	(170)	(228)	(835)	(873)
Net loss	(10,118)	(10,317)	(41,873)	(32,703)
Change in unrealized gain (loss) on investments	(3)	3	3	(3)
Comprehensive loss	$ (10,121)	$ (10,314)	$ (41,870)	$ (32,706)
Net loss	$ (10,118)	$ (10,317)	$ (41,873)	$ (32,703)
Net loss per share, basic and diluted	$ (0.31)	$ (0.33)	$ (1.34)	$ (1.10)
Weighted-average shares used to compute basic and diluted net loss per share	32,380,418	30,839,099	31,336,409	29,600,332

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

	December 31,	
	2019	**2018**
Cash and cash equivalents	$ 24,846	$ 32,606
Short-term investments	$ 1,999	$ 29,875
Total assets	$ 30,262	$ 66,147
Note payable, inclusive of current portion	$ 8,304	$ 10,298
Total liabilities	$ 14,508	$ 14,803
Total stockholders' equity	$ 15,754	$ 51,344

Source: miRagen Therapeutics, Inc.