miRagen Reports Second Quarter 2020 Financial Results and Provides Business Updates

August 5, 2020

- Progress on next generation microRNA-29 mimic for use in pulmonary fibrosis (MRG-229) includes new preclinical data; additional NIH grant funding triggered
- Exploratory preclinical safety and pharmacokinetic data for MRG-229 expected before year end
- Plan to receive guidance from the FDA on proposed regulatory pathway for cobomarsen in ATLL before year end
- A majority of patients in the Phase 2 SOLAR trial of cobomarsen in CTCL have continued to receive uninterrupted treatment and are being evaluated for clinical response
- Reported $30.6 million in cash and cash equivalents as of June 30, 2020; anticipate cash runway into Q3 2021

BOULDER, Colo., Aug. 05, 2020 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of proprietary RNA-targeted therapies with a specific focus on microRNAs, today reported financial results for the second quarter ended June 30, 2020 and provided business updates.

“The miRagen team continues to execute on our strategy across multiple product opportunities, by pursuing guidance from the FDA on a development pathway for cobomarsen in ATLL, delivering preclinical data for MRG-229 in pulmonary fibrosis models that triggered NIH funding to support large animal safety and pharmacokinetic studies, and working closely with our clinical centers to ensure our ability to deliver topline data from the planned interim analysis in the Phase 2 SOLAR trial,” said William S. Marshall, Ph.D., miRagen’s President and Chief Executive Officer.

“We are encouraged by the preclinical and clinical safety and efficacy data we have generated across all of our programs and energized about the potential of microRNA-based therapies to bring benefit to patients in need.”

Summary of Program Highlights this Quarter

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).*

- CTCL: As a result of the COVID-19 pandemic, the Company has seen an impact on clinical activities at some sites where the SOLAR trial is being conducted. However, with a majority of patients in the SOLAR trial continuing to receive uninterrupted treatment and evaluation for clinical response, the impact has been limited to a small number of patients. As of July 17, 2020, 34 of the 37 CTCL patients are being followed for a response, and 32 have continued to receive uninterrupted treatment.

- ATLL**:** Based on the clinical data released earlier this year, during the second quarter of 2020, the Company requested a meeting with the FDA to discuss the development path for cobomarsen in ATLL and plans to meet with the FDA and receive guidance on the Company’s proposed clinical path in this indication before the end of 2020.

MRG-229: *MRG-229 is a miR-29 mimic, or replacement for miR-29, a microRNA that is found at abnormally low levels in a number of pathologic fibrotic conditions.*

- Idiopathic Pulmonary Fibrosis (IPF): In June 2020, the Company hosted a key opinion leader (KOL) webcast to discuss the current and increasing unmet medical need in treating patients with IPF and reported additional preclinical rodent safety and *in vitro* human efficacy data for MRG-229. In summary, the latest preclinical observations were:
  - Next-generation targeted miR-29 mimics demonstrated down-regulation of targeted genetic pathways and anti-fibrotic effects including decreased collagen secretion in diseased lung fibroblasts *in vitro*, highlighting functional activity in addition to molecular readouts.
  - Anti-fibrotic activity of next-generation miR-29 mimics in mouse bleomycin-induced pulmonary fibrosis was observed for both intravenous and subcutaneous routes of administration.
  - Next-generation targeted miR-29 mimics demonstrated efficacy in *ex vivo* profibrotic-induced human precision cut lung slices.
  - Exploratory toxicity studies in rodents showed that MRG-229 treatment resulted in no adverse effects on organ histology, hematology, clinical chemistries, coagulation, or urinalysis.

Based on these results, the Company has initiated a non-human primate toxicology study and expects to report additional preclinical safety and efficacy data before the end of 2020.

Financial Results

- **Cash Position and Runway:** Cash and cash equivalents were $30.6 million as of June 30, 2020, compared to cash, cash equivalents, and short-term investments of $26.8 million as of December 31, 2019. Net cash used in operating activities

was $6.8 million for the second quarter of 2020 as compared to $7.2 million during the second quarter of 2019. miRagen believes that its current cash and cash equivalents will be sufficient to fund the Company's operations into the third quarter of 2021.

- **Revenue:** Revenue was $0.2 million for the second quarter of 2020, compared to $2.5 million for the second quarter of 2019. The decrease in revenue was primarily due to a decrease in research and development activities reimbursable to the Company under a prior license and collaboration agreement.

- **Research and Development Expenses:** Research and development expenses were $3.8 million for the second quarter of 2020, compared to $8.6 million for the second quarter of 2019. The $4.8 million decrease in research and development expenses was primarily attributable to a decrease in clinical and related manufacturing development activities associated with the Phase 2 SOLAR clinical trial of cobomarsen, personnel-related costs, and other miscellaneous expenses during the second quarter of 2020.

- **General and Administrative Expenses:** General and administrative expenses were $2.7 million for the second quarter of 2020, compared to $2.9 million for the second quarter of 2019. The decrease in general and administrative expenses was due primarily to decreased personnel-related costs, which were partially offset by increases in legal costs during the second quarter of 2020.

- **Net Loss:** The Company's net loss was $6.4 million, or $0.12 per share, for the second quarter of 2020, compared to $8.9 million, or $0.29 per share, for the second quarter of 2019.

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 second quarter 2020. To access the call, please dial 877-407-0789 (domestic) or 201-689-8562 (international) and provide the passcode 13706539. 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.

Investor/Media Contact:
Dan Ferry
LifeSci Advisors
(617) 430-7576
daniel@lifesciadvisors.com

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,	
	2020	**2019**	**2020**	**2019**
Revenue:				
Collaboration revenue	$ —	$ 2,498	$ 681	$ 2,846
Grant revenue	168	16	315	40
Total revenue	168	2,514	996	2,886
Operating expenses:				
Research and development	3,836	8,599	9,939	17,350
General and administrative	2,706	2,857	5,429	6,214
Total operating expenses	6,542	11,456	15,368	23,564
Loss from operations	(6,374)	(8,942)	(14,372)	(20,678)
Other income (expense):				
Interest and other income	33	275	128	614
Interest and other expense	(94)	(229)	(235)	(461)
Net loss	(6,435)	(8,896)	(14,479)	(20,525)
Change in unrealized gain on investments	—	9	—	14
Comprehensive loss	$ (6,435)	$ (8,887)	$ (14,479)	$ (20,511)
Net loss	$ (6,435)	$ (8,896)	$ (14,479)	$ (20,525)
Net loss per share, basic and diluted	$ (0.12)	$ (0.29)	$ (0.29)	$ (0.66)
Weighted-average shares used to compute basic and diluted net loss per share	53,077,348	30,983,918	49,276,843	30,935,272

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

	June 30, 2020	December 31, 2019
Cash and cash equivalents	$ 30,582	$ 24,846
Short-term investments	$ —	$ 1,999
Total assets	$ 33,485	$ 30,262
Notes payable, inclusive of current portion	$ 8,797	$ 8,304
Total liabilities	$ 12,314	$ 14,508
Total stockholders' equity	$ 21,171	$ 15,754

Source: miRagen Therapeutics, Inc.