miRagen Announces First Quarter 2019 Financial Results and Provides Corporate Update

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- Initiated patient dosing in the SOLAR Phase 2 trial for cobomarsen
- Released updated clinical data from the Phase 1 trial of cobomarsen in ATLL patients
- Released data from preclinical studies showing the antifibrotic effects of remlarsen in corneal injury
- $51.0 million in cash, cash equivalents, and short-term investments as of March 31, 2019
- Management to host conference call today at 4:30 p.m. ET

BOULDER, CO, March 3, 2019 – miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company developing proprietary RNA-targeted therapies with a specific focus on microRNAs, today reported financial results for the first quarter ended March 31, 2019 and provided a corporate update.

“Execution on our strategy by the miRagen team over the past few quarters has allowed us to make important advances in the development of cobomarsen, remlarsen, and MRG-110 in 2019, including initial dosing in the SOLAR Phase 2 trial of cobomarsen and the recent release of new data from our Phase 1 trial of cobomarsen in adult T-cell leukemia/lymphoma (ATLL) patients,” said William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics.

“In addition to cobomarsen, we continue to make progress advancing remlarsen and MRG-110. We look forward to releasing additional data from three clinical trials during the remainder of 2019, including our Phase 2 clinical trial of remlarsen in keloid scarring and two Phase 1 trials for MRG-110,” continued Dr. Marshall.

Recent Program Highlights

Cobomarsen

In April 2019, the first mycosis fungoides patients were dosed in the SOLAR Phase 2 trial. The SOLAR trial is designed to evaluate the safety and efficacy of cobomarsen given in 300 mg doses by intravenous infusion in an active control comparison trial versus vorinostat. miRagen has opened a number of clinical sites in the trial and is planning to initiate activities at up to sixty clinical sites in eleven countries worldwide. The primary endpoint of the SOLAR trial is the rate of an objective response 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 with no evidence of disease progression in the blood, lymph nodes, or viscera. Progression-free survival is a secondary endpoint, and miRagen plans to use patient-reported outcomes as additional endpoints to monitor quality of life improvements. Based on discussions with the U.S. Food and Drug Administration (FDA), miRagen believes that primary endpoint data from this clinical trial could allow miRagen to apply for accelerated approval in the United States. The Company expects to report primary endpoint data from this clinical trial in the second half of 2020.

miRagen is also evaluating cobomarsen in certain expansion indications where the disease process appears to be correlated with an increase in miR-155 levels, the target of cobomarsen. The Company recently announced new data from the ATLL cohort of its Phase 1 clinical trial of cobomarsen in an oral presentation at the 19th International Congress HTLV 2019. In the updated data from the trial, five patients with aggressive ATLL subtype in partial remission have been treated with cobomarsen for up to 16 months. Four of the five patients remained stable for up to 16 months and continued dosing. Biological activity of cobomarsen in these patients is indicated by a significant decrease in biomarkers of cell proliferation (e.g., Ki67) and activation (e.g., HLD-R and CD69) in all patients, supporting the observed clinical stabilization. Cobomarsen has been well tolerated over prolonged treatment, with no deaths, dose limiting toxicities, related serious adverse events, related Grade 3 or Grade 4 adverse events, hematological events or discontinuation from trial due to related adverse events.

Remlarsen

miRagen recently announced new preclinical data from a study exploring the antifibrotic effects of remlarsen in the cornea. In the study, remlarsen treatment appeared to accelerate the healing of corneal injury, resulting in a more rapid restoration of epithelial thickness and a reduction in stromal thickness as compared to saline-treated injured eyes. Remlarsen treatment also appeared to reduce the expression of multiple collagens and fibrosis-associated genes from 7-14 days post-injury and to reduce α-SMA protein expression in the epithelium and stroma at 14 days. Lastly, remlarsen treatment appeared to reduce corneal hazing and scarring beginning at 10 days post-burn. Dose

and treatment schedule were optimized in preparation for enabling toxicology studies to support human clinical trials.

Based on this data, miRagen believes that further exploration of the topical application of remlarsen as a treatment to improve vision in patients suffering from multiple conditions resulting in corneal scarring may be warranted. Corneal scarring is one of the leading causes of blindness worldwide.

miRagen is currently conducting a Phase 2 clinical trial of remlarsen assessing the safety, tolerability, and activity of remlarsen in the potential prevention or reduction of keloid formation in subjects with a history of keloid scars, a form of pathological scarring. The trial has completed its enrollment and miRagen expects to report data from this clinical trial in the second half of 2019.

MRG-110

MRG-110 is currently being evaluated in collaboration with Servier in two Phase 1 clinical trials designed to evaluate the safety, tolerability, and pharmacokinetics of MRG-110. The data generated in these clinical trials is expected to provide clinically translatable biomarkers that may support future clinical trials for the treatment of heart failure and other conditions where patients may benefit from increased vascular flow and accelerated healing, such as complicated lacerations in high risk patients and burns. Enrollment has been completed in both Phase 1 clinical trials of MRG-110 and the Company expects to report data in 2019.

Financial Condition and Operating Results

Cash, cash equivalents, and short-term investments were $51.0 million as of March 31, 2019, compared to $62.5 million as of December 31, 2018. Cash used in operating activities was $11.8 million for the first quarter of 2019. miRagen believes that its current cash, cash-equivalents, and short-term investments will be sufficient to fund the Company’s operations for the next twelve months.

Revenue was $0.4 million for the first quarter of 2019, compared to $4.8 million for the first quarter of 2018. The decrease in revenue is primarily due to a $3.7 million development milestone payment miRagen earned under its collaboration agreement with Servier during the first quarter of 2018.

Research and development expenses were $8.8 million for the first quarter of 2019, compared to $6.4 million for the first quarter of 2018. The increase in research and development expenses is primarily due to increases in clinical development activities associated with the Phase 2 SOLAR clinical trial of cobomarsen and personnel-related costs. These increases were partially offset by lower technology license fees in 2019.

General and administrative expenses were $3.4 million for the first quarter of 2019, compared to $3.0 million for the first quarter of 2018. The increase in general and administrative expenses is primarily due to increases in personnel-related costs resulting primarily from the growth of our general and administrative team and higher share-based compensation charges.

The Company's net loss was $11.6 million, or $0.38 per share, for the first quarter of 2019, compared to $4.7 million, or $0.18 per share, for the first quarter of 2018.

Conference Call Information

miRagen will host a conference call today at 4:30 p.m. ET to discuss its financial results for the first quarter of 2019. Participants may access the call by dialing 877-407-0789 in the U.S. or 201-689-8562 outside the U.S. and providing the conference ID number 13689475. 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, remlarsen, 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. 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 in 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, 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 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 March 31,	
	2019	2018
Revenue:		
Collaboration revenue	$ 348	$ 4,756
Grant revenue	24	28
Total revenue	372	4,784
Operating expenses:		
Research and development	8,751	6,413
General and administrative	3,357	2,990
Total operating expenses	12,108	9,403
Loss from operations	(11,736)	(4,619)
Other income (expense):		
Interest and other income	339	167
Interest and other expense	(232)	(209)
Net loss	(11,629)	(4,661)
Change in unrealized loss on investments	5	—
Comprehensive loss	$ (11,624)	$ (4,661)
Net loss	$ (11,629)	$ (4,661)
Net loss per share, basic and diluted	$ (0.38)	$ (0.18)
Weighted-average shares used to compute basic and diluted net loss per share	30,886,085	26,483,112

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

	March 31, 2019	March 31, 2018
Cash and cash equivalents	$ 18,195	$ 32,606
Short-term investments	$ 32,844	$ 29,875
Total assets	$ 54,950	$ 66,147
Note payable, inclusive of current portion	$ 10,392	$ 10,298
Total liabilities	$ 13,994	$ 14,803
Total stockholders' equity	$ 40,956	$ 51,344