



## MIRAGEN THERAPEUTICS REPORTS THIRD QUARTER 2018 FINANCIAL RESULTS AND PROVIDES CORPORATE UPDATE

- *Continued advancements of three clinical stage microRNA-targeted product candidates currently being evaluated for multiple potential indications in four clinical trials*
- *Global Phase 2 SOLAR clinical trial of cobomarsen in CTCL expected to be initiated in the fourth quarter of 2018*
- *\$70.5 million in cash, cash equivalents, and short-term investments as of September 30, 2018*
- *Conference call and webcast today at 4:30 p.m. ET*

**BOULDER, CO, November 7, 2018** - miRagen Therapeutics, Inc. (NASDAQ: MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today reported third quarter 2018 financial results and provided a corporate update.

“We had a productive quarter in which we advanced preparations to launch our global Phase 2 SOLAR trial of cobomarsen in CTCL. We expect the trial to begin dosing patients in the fourth quarter of 2018,” said William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics. “During the quarter, we announced our collaboration with The Leukemia and Lymphoma Society (LLS) to help support the SOLAR trial, including LLS providing up to \$5.0 million in financial support.”

“We continue to make progress across our microRNA platform and expect to report data from multiple trials in 2019, including the Phase 1 cobomarsen trial in adult T-cell leukemia / lymphoma (ATLL); the Phase 2 proof of mechanism trial of remlarsen in keloids; and the ongoing clinical development of MRG-110 for cardiac and vascular indications in collaboration with Servier. With the needs of patients in mind, we are excited to advance three clinical stage product candidates and provide further evidence supporting the potential of microRNA-based therapeutics as an important new class of targeted therapies.”

### **Cobomarsen Update**

miRagen expects its global Phase 2 SOLAR trial for cobomarsen to begin dosing patients diagnosed with cutaneous T-cell lymphoma (CTCL) during the fourth quarter of 2018. The SOLAR trial will evaluate the safety and efficacy of cobomarsen given by intravenous infusion in an active control comparison trial versus ZOLINZA (vorinostat). Approximately 65 patients per treatment group are expected to enroll in the trial. The primary endpoint of the SOLAR trial is the rate of an objective response, 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, maintained for at least four consecutive months. Progression-free survival will be 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, miRagen believes the results from this clinical trial could allow the Company to apply for accelerated approval in the United States. We expect to report data from this clinical trial in the second half of 2020.

In previously announced Phase 1 trial data, cobomarsen appeared to demonstrate durable responses measured by improvement in total skin tumor burden scoring and quality of life improvement in patients with the mycosis fungoides form of CTCL. Cobomarsen also appeared to be generally well tolerated at all dose levels evaluated. miRagen plans to present complete data from this Phase 1 trial at the 2018 American Society of Hematology (ASH) Annual Meeting in December. The data will include efficacy, safety and tolerability observations from long-term dosing of cobomarsen via various routes of administration in 43 patients who have been enrolled in the trial for up to 22 months.

miRagen is also evaluating cobomarsen in ATLL, diffuse large B-cell lymphoma, and chronic lymphocytic leukemia, for which it has reported preliminary clinical responses in ATLL from its ongoing Phase 1 trial. The Company anticipates announcing additional ATLL data from this clinical trial in the first half of 2019.

### **Remlarsen Update**

The Company is conducting a Phase 2 clinical trial for remlarsen assessing the safety, tolerability, and activity of remlarsen in the prevention or reduction of keloid formation in subjects with a history of keloid scars, a persistent form of hypertrophic scarring. miRagen expects to report data from this clinical trial in the second half of 2019.

miRagen also recently announced data from its preclinical study investigating the anti-fibrotic effects of remlarsen in corneal ulceration. The Company believes the results obtained in the study suggest that topical application of remlarsen may be an effective treatment to inhibit corneal scarring and improve vision in patients suffering from multiple conditions. Corneal scarring remains one of the leading causes of blindness worldwide.

### **MRG-110 Update**

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 several clinically translatable biomarkers that may support future clinical trials for the treatment of heart failure, as well as surgical incisions in high risk populations, severe lacerations, and chronic wounds. miRagen expects to report data from these clinical trials in 2019.

### **Third Quarter 2018 Financial Results**

miRagen had \$70.5 million in cash, cash equivalents and short-term investments as of September 30, 2018. Cash used in operating activities was \$6.8 million for the third quarter of 2018 and \$18.7 million for the first nine months of the year. miRagen had 30.8 million shares of common stock outstanding as of September 30, 2018.

The Company reported a net loss available to common stockholders of \$9.0 million, or (\$0.29) per share (basic and diluted) for the third quarter of 2018 as compared to \$5.8 million, or (\$0.27) per share (basic and diluted) for the third quarter of 2017. The increase in net loss was primarily due to higher research and development expenses in 2018. Additionally, net loss available to common stockholders for the third quarter of 2018 was impacted by a \$0.7 million decrease in revenue of \$0.9 million, compared to \$1.6 million for the same period of 2017.

Research and development expenses totaled \$7.4 million for the third quarter of 2018 as compared to \$5.0 million for the third quarter of 2017. The increase in research and development expenses is primarily due to increased clinical development activities associated with the Phase 2 SOLAR clinical trial of cobomarsen, including costs to manufacture cobomarsen together with an increase in personnel-related costs due to the growth of the Company's research and development team.

General and administrative expenses totaled \$2.7 million for the third quarter of 2018 as compared to \$2.5 million for the third quarter of 2017. The increase in general and administrative expenses is primarily due to increased personnel related costs as miRagen added to its team in 2018. These increases were partially offset by lower legal fees related to intellectual property during the third 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 third quarter 2018. Participants may access the call by dialing 855-327-6837 in the U.S. or 631-891-4304 outside the U.S. and providing the conference ID number 10005752. The call will also be webcast and can be accessed from the Investors and Media section of miRagen's website at [www.miragen.com](http://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, as well as certain cells involved in inflammation. 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 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](http://www.miragen.com).

For information on clinical trials please visit [www.clinicaltrials.gov](http://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; 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
<b>Revenue:</b>				
Collaboration revenue	\$ 814	\$ 1,493	\$ 6,938	\$ 1,991
Grant revenue	130	138	972	820
Total revenue	944	1,631	7,910	2,811
<b>Operating expenses:</b>				
Research and development	7,399	5,018	22,187	14,625
General and administrative	2,696	2,502	8,354	8,364
Total operating expenses	10,095	7,520	30,541	22,989
Loss from operations	(9,151)	(5,889)	(22,631)	(20,178)
<b>Other income (expense):</b>				
Interest and other income	362	113	890	245
Interest and other expense	(222)	(58)	(645)	(193)
Net loss	(9,011)	(5,834)	(22,386)	(20,126)
Change in unrealized loss on investments	(10)	—	(6)	—
Comprehensive loss	\$ (9,021)	\$ (5,834)	\$ (22,392)	\$ (20,126)
Net loss	\$ (9,011)	\$ (5,834)	\$ (22,386)	\$ (20,126)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(5)
Net loss available to common stockholders	\$ (9,011)	\$ (5,834)	\$ (22,386)	\$ (20,131)
Net loss per share, basic and diluted	\$ (0.29)	\$ (0.27)	\$ (0.77)	\$ (1.11)
Weighted-average shares used to compute basic and diluted net loss per share	30,723,704	21,572,498	29,182,872	18,215,857

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

	September 30, 2018	December 31, 2017
Cash and cash equivalents	\$ 31,700	\$ 47,441
Short-term investments	\$ 38,827	\$ —
Total assets	\$ 76,553	\$ 52,481
Note payable, inclusive of current portion	\$ 10,205	\$ 9,922
Total liabilities	\$ 15,835	\$ 13,971
Total stockholders' equity	\$ 60,718	\$ 38,510

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