



## MIRAGEN ANNOUNCES FOURTH QUARTER AND FULL YEAR 2018 FINANCIAL RESULTS AND PROVIDES 2019 OUTLOOK

- *Cobomarsen tested in clinical trials for three different types of blood cancers*
- *Multiple clinical sites open and actively recruiting in the global Phase 2 SOLAR clinical trial of cobomarsen in CTCL*
- *Data from four clinical trials of three product candidates expected to be released in 2019*
- *\$62.5 million in cash, cash equivalents, and short-term investments as of December 31, 2018*
- *Management to host conference call today at 4:30 p.m. ET*

**BOULDER, CO, March 13, 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 and operating results for the fourth quarter and full-year ended December 31, 2018.

“We are encouraged by the advances our team has made in the development of microRNA targeted therapies in 2018 and pleased to begin 2019 with three clinical stage product candidates being evaluated in five clinical trials for the potential to treat patients who suffer from a variety of diseases with high unmet medical need.” said William S. Marshall, Ph.D., President and Chief Executive Officer of miRagen Therapeutics. “In addition to the release of encouraging clinical data for cobomarsen in cutaneous T-cell lymphoma (CTCL), we have also seen exciting preliminary clinical activity for cobomarsen in other blood cancers and have also advanced the development of remlarsen into a Phase 2 clinical trial in cutaneous fibrosis. Further, we are working closely in collaboration with Servier on the clinical development of MRG-110 for cardiovascular disease and other potential indications.”

“As we advance through 2019, we are intensely focused on the achievement of several important clinical milestones, including the enrollment of patients in our global Phase 2 SOLAR trial of cobomarsen in CTCL and releasing clinical data from four additional ongoing clinical trials throughout 2019.”

### Recent Program Highlights and Outlook

#### Cobomarsen

In January 2019, miRagen announced updated durability data for the 300 mg intravenous infusion cohort of the Phase 1 cobomarsen clinical trial, which is the dose and route of administration being used in the SOLAR Phase 2 clinical trial, that showed four of eight patients (50%) achieved an objective response with greater than four months of durability (ORR4). In addition, the topline Phase 1 cobomarsen data 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 believes that data from the Phase 1 clinical trial provides clinical proof-of-concept for cobomarsen in patients with mycosis fungoides. The Company’s global Phase 2 SOLAR trial for cobomarsen in patients diagnosed with mycosis fungoides is in the startup phase with initial dosing expected during early 2019. The SOLAR trial is designed to evaluate the safety and efficacy of cobomarsen given 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, 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 adult T-cell leukemia/lymphoma (ATLL), diffuse large B-cell lymphoma (DLBCL), and chronic lymphocytic leukemia. miRagen has reported preliminary clinical responses in ATLL and DLBCL

from its ongoing Phase 1 clinical trial. The Company expects to release additional Phase 1 ATLL clinical data during the first half of 2019.

## **Remlarsen**

The Company is conducting a Phase 2 clinical trial of 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 studies investigating the antifibrotic 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 improve vision in patients suffering from multiple conditions resulting in corneal scarring, which remains one of the leading causes of blindness worldwide. miRagen expects to report data from preclinical studies in ocular fibrosis during the first 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, as well as surgical incisions in high risk populations, severe lacerations, and severe 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 \$62.5 million as of December 31, 2018, compared to \$47.4 million as of December 31, 2017. Additionally, cash used in operating activities was \$8.2 million for the fourth quarter of 2018 and \$26.8 million for the year ended December 31, 2018. miRagen believes that its current cash, cash-equivalents and short-term investments will be sufficient to fund the execution of its current clinical and operating plan through the first quarter of 2020.

Revenue was \$0.5 million for the fourth quarter of 2018 and \$8.4 million for the year ended December 31, 2018, compared to \$1.2 million and \$4.0 million, respectively, for the comparable periods in 2017. In 2018, miRagen earned a development milestone payment under its collaboration agreement with Servier and recognized \$3.7 million of revenue.

Research and development expenses were \$8.2 million for the fourth quarter of 2018 and \$30.4 million for the year ended December 31, 2018, compared to \$5.0 million and \$19.6 million, respectively, for the comparable periods in 2017. The increase year over year is primarily due to increased clinical development activities associated with the Phase 2 SOLAR clinical trial of cobomarsen, including costs to manufacture cobomarsen, and the initiation of a Phase 1 clinical trial of MRG-110 during 2018, together with an increase in personnel-related costs due to the growth of the Company's research and development team.

General and administrative expenses were \$2.7 million for the fourth quarter of 2018 and \$11.0 million for the year ended December 31, 2018, compared to \$2.5 million and \$10.9 million, respectively, for the comparable periods in 2017. During 2018, the Company's general and administrative personnel-related costs increased due primarily to the growth of the team and increased share-based compensation charges. These increases were offset by lower legal expenses, primarily related to non-reoccurring expenses of a merger that occurred in 2017.

The Company's net loss available to common stockholders was \$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, compared to \$6.4 million, or \$0.29 per share, for the fourth quarter of 2017 and \$26.5 million, or \$1.38 per share, for the year ended December 31, 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 of 2018. 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 13687651. 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. 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](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, 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 December 31,		Year Ended December 31,	
	2018	2017	2018	2017
<b>Revenue:</b>				
Revenue under strategic alliance and	\$ 435	\$ 1,106	\$ 7,373	\$ 3,097
Grant revenue	41	86	1,013	906
Total revenue	476	1,192	8,386	4,003
<b>Operating expenses:</b>				
Research and development	8,234	4,998	30,421	19,623
General and administrative	2,695	2,548	11,049	10,912
Total operating expenses	10,929	7,546	41,470	30,535
Loss from operations	(10,453)	(6,354)	(33,084)	(26,532)
<b>Other income (expense):</b>				
Interest and other income	364	158	1,254	403
Interest and other related expense	(228)	(190)	(873)	(383)
Net loss	(10,317)	(6,386)	(32,703)	(26,512)
Change in unrealized gain (loss) on investments	3	—	(3)	—
Comprehensive loss	\$ (10,314)	\$ (6,386)	\$ (32,706)	\$ (26,512)
Net loss	\$ (10,317)	\$ (6,386)	\$ (32,703)	\$ (26,512)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(5)
Net loss available to common stockholders	\$ (10,317)	\$ (6,386)	\$ (32,703)	\$ (26,517)
Net loss per share, basic and diluted	\$ (0.33)	\$ (0.29)	\$ (1.10)	\$ (1.38)
Weighted-average shares used in computing basic and diluted net loss per share	30,839,099	22,297,302	29,600,332	19,244,605

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

	December 31,	
	2018	2017
Cash and cash equivalents	\$ 32,606	\$ 47,441
Short-term investments	\$ 29,875	\$ —
Total assets	\$ 66,147	\$ 52,481
Note payable, inclusive of current portion	\$ 10,298	\$ 9,922
Total liabilities	\$ 14,803	\$ 13,971
Total stockholders' equity	\$ 51,344	\$ 38,510