



miRagen Therapeutics Reports Second Quarter 2017 Financial Results and Provides Corporate Update

August 11, 2017

- New MRG-106 Phase 1 cutaneous T-cell lymphoma clinical trial data released
- Expansion of the MRG-106 trial to include additional potential indications
- Two new independent members added to its Board of Directors
- Conference call and webcast today at 4:30 p.m. ET

BOULDER, Colo., Aug. 11, 2017 (GLOBE NEWSWIRE) -- miRagen Therapeutics, Inc. (NASDAQ:MGEN), a clinical-stage biopharmaceutical company focused on the discovery and development of RNA-targeted therapies, today announced second quarter 2017 financial results and provided a corporate update.

"The second quarter of 2017 has been marked by a number of important development milestones as we position the company for additional clinical opportunities," said miRagen President and Chief Executive Officer, William S. Marshall, Ph.D. "In addition to reporting positive new interim Phase 1 clinical trial data underscoring our belief in the potential of the MRG-106 program, we completed our MRG-201 Phase 1 clinical trial and established a potential path forward for expanding both development programs into additional indications. The results generated in both clinical trials represent significant strides forward in execution of our foothold clinical development strategy. We believe that the continued application of the foothold clinical approach may allow us to expand opportunities for our microRNA targeted therapeutic candidates in the future."

Business Highlights and Update

- **Reported new interim MRG-106 Phase 1 clinical trial results, and announced plans to expand the program to include additional potential indications:** New interim results from the Company's ongoing Phase 1 clinical trial of MRG-106 in subjects with the mycosis fungoides (MF) form of cutaneous T-cell lymphoma (CTCL) were presented at the American Society of Clinical Oncology (ASCO) Annual Meeting in June 2017. Eighteen of nineteen subjects (95%), independent of administration route, showed improvement in either the individual lesion or total skin disease as measured by maximal change in either Composite Assessment of Index Lesion Severity or modified Severity Weighted Assessment Tool (mSWAT), respectively.

Subsequent to the presentation at the ASCO Annual Meeting, subjects enrolled in the trial continued to show improvement in their mSWAT scores. As of July 26, seventeen of eighteen subjects treated systemically with MRG-106 showed mSWAT score improvement, and the magnitude of mSWAT improvements generally correlated with amount of time the subject received MRG-106 treatment. Seven of nine subjects receiving more than one month of dosing of MRG-106 showed a 50% or greater improvement in mSWAT scores. Additionally, subjects receiving the treatment via intravenous (IV) infusion appear to have improved at a higher rate than those receiving the treatment via subcutaneous injection. MRG-106 has been generally safe and well-tolerated at all dose levels evaluated to date. The Company has also initiated rapid intravenous dosing in patients after discussing the plan with the U.S. Food and Drug Administration (FDA) during a meeting in June. This route of administration may allow for higher maximal blood concentration and could provide additional convenience to patients.

In May 2017, MRG-106 was granted orphan medicinal product designation for the treatment of CTCL by the European Commission. The FDA granted orphan drug designation to MRG-106 for the treatment of MF in March 2017. Following a discussion with the FDA in June, the Company plans to evaluate MRG-106 in additional oncology indications within the current Phase 1 trial, including adult T-cell leukemia/lymphoma (ATLL), diffuse large B-cell lymphoma (DLBCL) and chronic lymphocytic leukemia (CLL) as in each case the disease process appears to be related to an increase in miR-155 levels. In 2018, the Company intends to initiate a Phase 2 clinical trial with MRG-106 in MF patients.

- **Completed MRG-201 Phase 1 clinical trial, and final clinical trial results planned to be presented in second half of 2017:** The double-blind, placebo-controlled, single and multiple dose-escalation Phase 1 trial evaluating MRG-201 in induced cutaneous fibrosis has been completed, and the Company plans to present final clinical trial results at a scientific conference before the end of this year. In 2018, the Company intends to initiate a Phase 2a trial to evaluate MRG-201 in subjects with a predisposition for keloid formation. Future indications to be studied for miR-29 replacements could include other fibrotic diseases of the eye and the lung.
- **MRG-110 to advance into clinical development in 2018:** miRagen plans to advance MRG-110, a microRNA-based therapy being developed in collaboration with Servier, into clinical development next year. MRG-110, the lead product candidate under the collaboration, inhibits the activity of microRNA-92a, which has been reported in multiple peer reviewed

scientific publications to be a regulator of new blood vessel creation. This may indicate that MRG-110 could be useful in the treatment of cardiovascular disease and certain other diseases where vascular flow is compromised.

- **Announced changes to the Company's Board of Directors:** miRagen appointed Chris Bowden, M.D. and Jeffrey Hatfield to the Company's Board of Directors effective August 10, 2017, following the resignations of John Creecy and Kyle Lefkoff. miRagen believes that the biopharmaceutical expertise of Dr. Bowden and Mr. Hatfield will be invaluable as the Company advances and expands its pipeline of microRNA-targeted therapies. miRagen would like to thank Mr. Creecy and Mr. Lefkoff for their significant and valued contributions to the Company.

Dr. Bowden brings substantial experience in clinical drug development to miRagen's Board of Directors. He currently serves as the Chief Medical Officer of Agios Pharmaceuticals, Inc. Prior to joining Agios, he served as Vice President, Product Development Oncology, Franchise Lead (Signaling Group) at Genentech, Inc., a member of the Roche Group.

Mr. Hatfield brings relevant industry experience and a breadth of expertise to miRagen's Board of Directors. From March 2004 through October 2016, he served as President and Chief Executive Officer of Vitae Pharmaceuticals, Inc., until its acquisition by Allergan in 2016. Prior to working at Vitae, he was with Bristol-Myers Squibb Company serving in numerous executive capacities, including as Senior Vice President of Bristol-Myers's Immunology and Virology divisions.

- **miRagen Therapeutics added to the Russell 2000[®] and 3000[®] Indexes:** The Company has been included in the Russell 2000[®] and Russell 3000[®] Indexes following Russell Investments' reconstitution of its comprehensive set of U.S. and global equity indexes in June 2017. The Russell 2000[®] Index measures the performance of the small-cap segment of the U.S. equity market, and is a subset of the Russell 3000[®] Index.

Anticipated Milestones

- MRG-106 (hematological malignancies)
 - Interim Phase 1 CTCL data presentation at ASH (Q4 2017)
 - Phase 1 trial expansion to include ATLL, DLBCL and CLL (2H 2017)
 - Initiation of a Phase 2 trial in CTCL/Non-Hodgkin's Lymphoma (2H 2018)
- MRG-201 (pathologic fibrosis)
 - Phase 1 results presentation at a scientific conference (2H 2017)
 - Initiation of a Phase 2a trial in keloids (2018)
- MRG-110 (ischemic disease)
 - Completion of Investigational New Drug/Clinical Trial Application enabling studies (Q4 2017)
 - Initiation of a Phase 1 trial (2018)

Upcoming Events

miRagen plans to present at the following upcoming conferences:

- Wedbush PacGrow Healthcare Conference, New York, NY August 15-16, 2017
- European Respiratory Society, Milan, Italy September 9, 2017
- Cantor Fitzgerald Global Healthcare Conference, New York, NY September 25-27, 2017
- DIA Oligonucleotide-Based Therapeutics Conference, Bethesda, MD October 25, 2017

Second Quarter 2017 Financial Results

Cash and cash equivalents at June 30, 2017 were \$46.3 million, compared to \$22.1 million at December 31, 2016. Total net cash used in operating and investing activities for the first half of 2017 was approximately \$14.1 million. The Company expects that its current cash and cash equivalents will be sufficient to fund its operations through the end of 2018.

Net loss attributable to common stockholders for the second quarter of 2017 was \$7.3 million, or \$0.34 per share (basic and diluted), compared to \$3.5 million or \$5.88 per share for the same period in 2016.

- Revenue decreased to \$0.7 million during the second quarter of 2017, from \$1.1 million during the same period in 2016. The \$0.4 million decrease was due primarily to decreases in license fee and development related revenue under the Company's collaboration agreement with Servier.
- Research and development expenses increased to \$5.5 million during the second quarter of 2017, from \$3.4 million during the same period in 2016. The \$2.1 million increase was due primarily to a \$1.3 million increase in outsourced manufacturing expenses, a \$0.3 million increase in clinical trial expense, and a \$0.8 million increase in personnel costs,

including share based compensation. These increases were partially offset by a \$0.3 million decrease in outsourced preclinical costs.

- General and administrative expenses increased to \$2.6 million during the second quarter of 2017, from \$1.2 million during the same period in 2016. The \$1.4 million increase was due primarily to a \$0.8 million increase in legal and other professional fees and expenses related primarily to increased costs associated with being a public company and increased personnel costs of \$0.4 million, including share based compensation.

Conference Call & Webcast

miRagen's senior management will host a conference call and live audio webcast today at 4:30 p.m. ET to discuss its second quarter 2017 financial results and provide a corporate update. The conference call is being webcast and can be accessed from the miRagen website, www.miragen.com, under Investors & Media. A replay of the webcast will be available for 90 days. The conference call can also be accessed by dialing 877.440.5803 (U.S./Canada) or 719.325.4801 (international) and providing the passcode 3801268.

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's two lead product candidates, MRG-106 and MRG-201, are currently in Phase 1 clinical trials. miRagen's clinical product candidate for the treatment of certain cancers, MRG-106, 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, MRG-201, 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. miRagen also is developing MRG-110, an inhibitor of microRNA-92, under license and collaboration agreement with Servier. MRG-110 is being developed for the treatment of heart failure and other ischemic disease. In addition to these programs, miRagen is developing a pipeline of pre-clinical 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 pre-clinical 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, 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 results of miRagen's Phase 1 clinical trials 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
(in thousands, except share and per share data)
(unaudited)

| Three Months Ended | Six Months Ended |
|---------------------------|-------------------------|
| June 30, | June 30, |

| | <u>2017</u> | <u>2016</u> | <u>2017</u> | <u>2016</u> |
|--|-------------------|-------------------|--------------------|-------------------|
| Revenue: | | | | |
| Collaboration revenue | \$ 488 | \$ 847 | \$ 498 | \$ 1,764 |
| Grant revenue | 230 | 268 | 682 | 268 |
| Total revenue | <u>718</u> | <u>1,115</u> | <u>1,180</u> | <u>2,032</u> |
| Operating expenses: | | | | |
| Research and development | 5,487 | 3,355 | 9,607 | 6,821 |
| General and administrative | 2,581 | 1,210 | 5,862 | 2,202 |
| Total operating expenses | <u>8,068</u> | <u>4,565</u> | <u>15,469</u> | <u>9,023</u> |
| Loss from operations | <u>(7,350)</u> | <u>(3,450)</u> | <u>(14,289)</u> | <u>(6,991)</u> |
| Other income (expense): | | | | |
| Interest and other income | 102 | 9 | 132 | 16 |
| Interest and other related expense | (64) | (83) | (135) | (172) |
| Net loss | <u>(7,312)</u> | <u>(3,524)</u> | <u>(14,292)</u> | <u>(7,147)</u> |
| Accretion of redeemable convertible preferred stock to redemption value | <u>—</u> | <u>(12)</u> | <u>(5)</u> | <u>(24)</u> |
| Net loss available to common stockholders | <u>\$ (7,312)</u> | <u>\$ (3,536)</u> | <u>\$ (14,297)</u> | <u>\$ (7,171)</u> |
| Net loss per share, basic and diluted | <u>\$ (0.34)</u> | <u>\$ (5.88)</u> | <u>\$ (0.87)</u> | <u>\$ (11.92)</u> |
| Weighted-average shares used to compute basic and diluted net loss per share | <u>21,409,708</u> | <u>601,667</u> | <u>16,509,719</u> | <u>601,667</u> |

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

| | <u>June 30,</u> | <u>December 31,</u> |
|---|-----------------|---------------------|
| | <u>2017</u> | <u>2016</u> |
| Cash and cash equivalents | \$ 46,335 | \$ 22,104 |
| Total assets | 50,026 | 24,760 |
| Notes payable, inclusive of current portion | 3,850 | 4,789 |
| Total liabilities | 8,690 | 9,705 |
| Redeemable convertible preferred stock | — | 76,976 |
| Total stockholders' equity (deficit) | 41,336 | (61,921) |

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Source: miRagen Therapeutics, Inc.