



Madrigal Pharmaceuticals Reports 2017 Third Quarter Financial Results

- Top-line results from MGL-3196 Phase 2 clinical trials in NASH and HeFH expected in late 2017 and early 2018, respectively –

Conshohocken, PA – November 9, 2017 – Madrigal Pharmaceuticals, Inc. (NASDAQ: MDGL) today announced its third quarter 2017 financial results and several clinical accomplishments that occurred during the third quarter including:

- the completion of patient enrollment in two Phase 2 clinical studies of MGL-3196, a liver-directed thyroid hormone receptor (THR) β -selective agonist, in patients with non-alcoholic steatohepatitis (NASH) and heterozygous familial hypercholesterolemia (HeFH), respectively;
- a second recommendation from the Drug Safety Monitoring Board (DSMB) that included a review of the data from both the NASH and HeFH Phase 2 clinical studies; and,
- the presentation of preclinical results at The Liver Meeting® 2017, American Association for the Study of Liver Diseases (AASLD) demonstrating that MGL-3196 provides metabolic, anti-inflammatory and anti-fibrotic benefits in a long-term, high fat diet, mouse NASH model.

“With patient enrollment completed for the Phase 2 NASH and HeFH clinical studies we are on track to report top-line results for NASH late this year and HeFH early in 2018,” said Paul Friedman, M.D., Chief Executive Officer of Madrigal. “Both indications have serious unmet patient needs and, based on both the preclinical and Phase 1 data that we have developed for MGL-3196, we believe our compound has the potential to be the first-and best-in-class THR β -selective agonist to safely and effectively meet these needs.”

Rebecca Taub, M.D., Chief Medical Officer and Executive VP, Research & Development of Madrigal added, “Because safety is such an important requirement for patients with these diseases, we are encouraged by the latest recommendation from our DSMB to continue both the NASH and HeFH clinical trials with no modifications to either protocol. We look forward to the upcoming data readouts which, if positive, will enable us to initiate Phase 3 trials in 2018.”

Clinical Program Summaries for MGL-3196

NASH

Non-alcoholic Steatohepatitis (NASH) is a common liver disease in the United States and worldwide, unrelated to alcohol use, that is characterized by a build-up of fat in the liver, inflammation, damage (ballooning) of hepatocytes and increasing fibrosis. Although people with NASH may feel well and often do not know they have the disease, NASH can lead to permanent damage, including cirrhosis and impaired liver function in a high percentage of NASH patients.

In October 2016, the first patient was treated in Madrigal’s Phase 2 trial of MGL-3196 for the treatment of NASH. The randomized, double-blind, placebo-controlled, multi-center study enrolled 125 patients 18

years of age and older with biopsy-confirmed NASH and more than 10% liver fat as confirmed by a magnetic resonance imaging-proton density fat fraction (MRI-PDFF).

In this trial, patients were randomized 2:1 to receive either MGL-3196 or placebo. The primary endpoint of the trial is the reduction of liver fat, assessed by MRI-PDFF at 12 weeks. Recent published data show a high correlation of reduction of liver fat measured by MRI-PDFF to NASH scoring on liver biopsy.

Efficacy will be confirmed at the end of the trial (36 weeks) by repeat MRI-PDFF and conventional liver biopsy to examine histological evidence for the resolution of NASH. Additional secondary endpoints include changes in clinically relevant biomarkers at 12 and 36 weeks, improvement in fibrosis by at least one stage, improvement of NASH, and safety and tolerability.

HeFH

Heterozygous familial hypercholesterolemia (HeFH), and a much rarer form called homozygous familial hypercholesterolemia (HoFH), are severe genetic dyslipidemias typically caused by inactivating mutations in the LDL receptor. Both forms of FH lead to early onset cardiovascular disease. HeFH, the most common dominantly inherited disease, is present in up to 1 in 200 people; the disease is found in higher frequencies in certain more genetically homogenous populations. Treatments exist for both HeFH and HoFH but many patients (as many as 40 percent of HeFH patients) are not able to reach their cholesterol (LDL-C) reduction goals on these therapies, reflecting the lifetime burden of cholesterol buildup in their bodies. Based on evidence of impressive LDL cholesterol lowering in Phase 1, and data suggesting that MGL-3196 has a mechanism of action that is different from and complementary to statins, Madrigal initiated a Phase 2 proof-of-concept trial in HeFH in February 2017. Patient recruitment for the study was completed in September 2017 and included 116 patients.

The 12-week, randomized, double-blind, placebo-controlled, multi-center study was expected to enroll up to 105 patients with HeFH randomized in a 2:1 ratio to receive either MGL-3196 or placebo, in addition to their current drug regimen (including high dose statins and ezetimibe). The primary endpoint of the study is reduction of LDL cholesterol, with secondary endpoints including reductions in triglycerides, Lp(a), and ApoB, as well as safety. Lp(a) is a severely atherogenic lipid particle, commonly elevated in familial hypercholesterolemia patients and poorly controlled by existing lipid lowering therapies. THR- β agonism is one of the few therapeutic approaches that can substantially lower Lp(a).

Financial Results for the Three Months and Nine Months Ended September 30, 2017

As of September 30, 2017, Madrigal had cash, cash equivalents and marketable securities of \$62.1 million, compared to \$40.5 million at December 31, 2016. The increase in cash and marketable securities resulted primarily from the net proceeds of \$34.9 million from Madrigal's private placement of equity in June 2017, and \$3.4 million of net proceeds from issuances of common stock in the first quarter of 2017 pursuant to Madrigal's at-the-market sales agreement, partially offset by cash used in operations of \$16.4 million.

Operating expenses were \$8.6 million and \$23.2 million for the three month and nine month periods ended September 30, 2017, compared to \$14.1 million and \$17.5 million in the comparable prior year periods.

Research and development expenses for the three month and nine month periods ended September 30, 2017 were approximately \$6.7 million and \$17.9 million, respectively, as compared to \$7.8 million and \$10.4 million in the comparable prior year periods. The decrease for the comparable three month periods was due primarily to lower non-cash stock based compensation in 2017, partially offset by increased expenses for our Phase 2 clinical development programs for MGL-3196 in NASH and HeFH. The increase for the comparable nine month periods is primarily attributable to increased expenses for our Phase 2 clinical development programs for MGL-3196 in NASH and HeFH, partially offset by lower non-cash stock based compensation.

General and administrative expenses for the three month and nine month periods ended September 30, 2017 decreased to approximately \$2.0 million and \$5.3 million, respectively, as compared to \$6.3 million and \$7.1 million in the comparable prior year periods. These decreases are primarily attributable to one-time, merger related costs in the third quarter of 2016.

Interest income (expense), net, for the three month and nine month periods ended September 30, 2017 was \$174 thousand and \$342 thousand, respectively, as compared to \$42 thousand and \$(1.2) million for the comparable prior year periods. The decreases in interest expense in 2017 were due to the conversion of convertible debt to shares of common stock in connection with Madrigal's merger, which closed on July 22, 2016.

About Madrigal Pharmaceuticals

Madrigal Pharmaceuticals, Inc. (Nasdaq: MGDJ) is a clinical-stage biopharmaceutical company pursuing novel therapeutics that target a specific thyroid hormone receptor pathway in the liver, which is a key regulatory mechanism common to a spectrum of cardio-metabolic and fatty liver diseases with high unmet medical need. Madrigal's lead candidate, MGL-3196, is a first-in-class, orally administered, small-molecule, liver-directed, thyroid hormone receptor (THR) β -selective agonist that is currently in Phase 2 development for [NASH](#) and [HeFH](#). For more information, visit www.madrigalpharma.com.

Forward-Looking Statements

This communication contains "forward-looking statements" made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Such statements contain words such as "expect," "could," "may," "will," "believe," "estimate," "continue," "future," or the negative thereof or comparable terminology and the use of future dates. Forward-looking statements reflect management's current knowledge, assumptions, judgment and expectations regarding future performance or events. Although management believes that the expectations reflected in such statements are reasonable, they give no assurance that such expectations will prove to be correct and you should be aware that actual results could differ materially from those contained in the forward-looking statements. Forward-looking statements are subject to a number of risks and uncertainties including, but not limited to, the company's clinical development of MGL-3196, the timing and outcomes of clinical studies of MGL-3196, and the uncertainties inherent in clinical testing. Undue reliance should not be placed on forward-looking statements, which

speaking only as of the date they are made. Madrigal undertakes no obligation to update any forward-looking statements to reflect new information, events or circumstances after the date they are made, or to reflect the occurrence of unanticipated events. Please refer to Madrigal's filings with the U.S. Securities and Exchange Commission for more detailed information regarding these risks and uncertainties and other factors that may cause actual results to differ materially from those expressed or implied.

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(Tables Follow)

Madrigal Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2017	2016	2017	2016
Revenues:				
Total revenues	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	6,682	7,806	17,878	10,410
General and administrative	1,955	6,285	5,273	7,058
Total operating expenses	8,637	14,091	23,151	17,468
Loss from operations	(8,637)	(14,091)	(23,151)	(17,468)
Interest income (expense), net	174	42	342	(1,171)
Other income	100	-	100	-
Net loss	\$ (8,363)	\$ (14,049)	\$ (22,709)	\$ (18,639)
Basic and diluted net loss per common share	\$ (0.68)	\$ (1.59)	\$ (1.87)	\$ (6.04)
Basic and diluted weighted average number of common shares outstanding	12,378,622	8,847,155	12,126,008	3,087,588

Madrigal Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	September 30,	December 31,
	2017	2016
Assets		
Cash, cash equivalents and marketable securities	\$ 62,137	\$ 40,500
Other current assets	583	707
Other non-current assets	111	3
Total assets	\$ 62,831	\$ 41,210
Liabilities and Equity		
Current liabilities	\$ 8,523	\$ 4,800
Long-term liabilities	-	-
Stockholders' equity	54,308	36,410
Total liabilities and stockholders' equity	\$ 62,831	\$ 41,210