

# Marinus Pharmaceuticals, Inc. (MRNS)

3Q14 Earnings Update: Clinical Progress on Track

## MARKET DATA

Price	\$6.52
52-Week Range:	\$4.00 - \$10.58
Shares Out. (M):	13.8
Market Cap (\$M):	\$90.0
Average Daily Vol. (000):	13.0
Cash (M):	\$47
Cash/Share:	\$3.36
Enterprise Value (M):	\$44
LT Debt (M):	\$0

Source: Thomson Reuters and JMP Securities LLC

**MARKET OUTPERFORM** | Price: \$6.52 | Target Price: \$14.00

## INVESTMENT HIGHLIGHTS

**Ganaxolone development on track with multiple key clinical catalysts anticipated throughout 2015; reiterating our Market Outperform rating and \$14 price target on Marinus Pharmaceuticals.** Marinus reported 3Q14 earnings results ahead of our and consensus estimates due to lower than expected operating expenses. The company ended the quarter with \$47M in cash and cash equivalents and guided that it expects this to be sufficient to fund operations into 2016. Marinus is continuing to advance development of ganaxolone in multiple indications including the Phase 3 trial in adults with focal onset epileptic seizures (results in 2H15) and Phase 2 trials in PCDH19 epilepsy patients and Fragile X Syndrome (results from both still on track to read out by mid-2015). Our \$14 price target is derived through an NPV analysis of U.S. ganaxolone sales in the adult partial onset seizure indication.

**Phase 3 trial in focal onset epileptic seizures progressing in line with expectations.** Marinus is enrolling patients in its Phase 3 trial evaluating ganaxolone in adults with treatment-resistant focal onset epileptic seizures. The trial is designed with a target enrollment of 300 patients and currently the company is expanding the number of clinical sites internationally, including in the U.S., Australia, Bulgaria, Germany, Poland and Russia. Results are anticipated in 2H15 and we continue to view the probability of success as favorable based on positive and statistically significant results from the prior Phase 2 trial.

**Phase 2 data in PCDH19 epilepsy on track for 1H15.** In the near term, the company plans to initiate a Phase 2 proof-of-concept, open-label clinical trial of ganaxolone in ~10 female pediatric epilepsy patients with the PCDH19 mutation. Results are expected in 1H15. We note that this indication represents possible upside to our estimates and valuation.

**Phase 2 data in FXS expected in mid-2015.** The Phase 2 proof-of-concept trial evaluating ganaxolone for the treatment of patients with Fragile X Syndrome (FXS) is continuing to enroll patients. Results from the trial are expected by mid-2015 and this indication also represents possible upside to our estimates and valuation.

FY DEC		2013A	2014E	2015E
Revenue (\$M)	1Q	--	\$0.0A	--
	2Q	--	\$0.0A	--
	3Q	--	\$0.0A	--
	4Q	--	\$0.0	--
	<b>FY</b>	<b>\$0.0</b>	<b>\$0.0</b>	<b>\$0.0</b>
EPS	1Q	--	(\$7.40)A	--
	2Q	--	(\$7.98)A	--
	3Q	--	(\$0.22)A	--
	4Q	--	(\$0.36)	--
	<b>FY</b>	<b>(\$19.60)</b>	<b>(\$2.41)</b>	<b>(\$2.06)</b>
Previous FY		NC	(\$2.39)	(\$1.99)

Source: Company reports and JMP Securities LLC

## STOCK PRICE PERFORMANCE



### 3Q14 FINANCIAL SUMMARY

Marinus reported a 3Q14 net income loss of (\$2.0M), above our estimate of (\$5.5M) and consensus of (\$5.6M), due to lower than expected operating expenses. The company did not report revenue, as expected. Total operating expenses were \$2.4M, below our estimate of \$5.5M, driven by lower than expected R&D and SG&A costs. R&D expenses were \$1.6M vs. our estimate of \$3.6M, and SG&A expenses were \$0.9M compared to our estimate of \$1.8M. The company had cash and cash equivalents of \$47.1M as of September 30, 2014.

We have revised our model to include 3Q14 financial results and updated guidance, as summarized in Figure 1.

**FIGURE 1. 3Q14 Earnings Summary and Changes to Our Model**

MRNS (\$MM)	3Q14			2014 est			2015 est		
	JMP old	Cons	Actual	JMP old	Cons	JMP new	JMP old	Cons	JMP new
Revenue	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total operating expense	5.5		2.4	17.2		13.4	32.4		29.6
R&D	3.6		1.6	13.0		10.0	26.0		22.4
SG&A	1.8		0.9	4.3		3.4	6.4		7.2
Net income (loss)	(5.5)	(5.6)	(2.0)	(19.1)	(17.8)	(14.8)	(32.4)	(28.4)	(29.6)
Shares outstanding	13.8		9.4	7.2		6.1	14.3		14.4
EPS (diluted)	(\$0.40)	(\$0.45)	(\$0.22)	(\$2.64)	(\$2.47)	(\$2.41)	(\$2.26)	(\$2.03)	(\$2.06)

Source: JMP Securities LLC and Company Reports

## Company Description

Marinus Pharmaceuticals is a clinical-stage biopharmaceutical company focused on developing and commercializing innovative neuropsychiatric therapeutics. The company's lead drug candidate is ganaxolone, an oral, small molecule synthetic analog of the neurosteroid allopregnanolone. The lead development indication for ganaxolone is an adjunctive therapy for the treatment of partial, (focal) onset seizures in adults with epilepsy. The company has completed a Phase 2 trial in the refractory treatment setting, and is currently conducting a Phase 2b trial. We believe positive Phase 2b results, together with a confirmatory Phase 3 trial, could support regulatory approvals in the U.S. and Europe. Marinus is also developing ganaxolone for additional indications, including an orphan pediatric epilepsy population and Fragile X syndrome.

## Investment Risks

**Clinical risk.** We note that results from early trials cannot always be replicated, and the drug may fail to produce positive data in later trials. There may be dosing, efficacy, or safety issues related to product candidates undergoing clinical trials that could preclude continued development. In addition, there may be manufacturing issues, including challenges with the scale-up to commercial quantities. Any of these issues could pose a risk to clinical development success.

**Regulatory risk.** The company's potential regulatory filing for its NDA may not receive approval from the FDA or ex-U.S. agencies. The FDA may request further studies, in which case the approval pathway will likely take longer, and cost significantly more.

**Market risk.** Market estimates of patients, or patients eligible for ganaxolone treatment, may be overestimated. Furthermore, new drugs entering the market could provide greater competition for the product candidate. This would impact the ability to reach revenue and profitability projections. In addition, the company must retain its intellectual property rights. Other companies may file patent applications or may receive patents that claim the same methods or formulations. Generic competition would affect operations and potential business prospects.

**Financial risk.** Marinus has incurred losses each year since inception due to research and development expenses for the ganaxolone pre-clinical and clinical programs. These expenses and losses are expected to continue to incur in the near future. It has not generated revenue to date from sales, and if there are any issues preventing the successful commercialization of products, the company may not reach profitability. We believe the company's cash runway will last ~24 months, into mid-2016. We anticipate that Marinus will likely need to raise additional funds to continue future operations. Raising additional funds may cause dilution to Marinus shares or require that it give up rights to product candidates. Any of the aforementioned scenarios may jeopardize the business. Additionally, as usual, the share price is subject to market volatility risk.

## JMP FACTS AND DISCLOSURES

### Analyst Certification:

The research analyst(s) who prepared this report does/do hereby certify that the views presented in this report are in accordance with my/our personal views on the securities and issuers discussed in this report. As mandated by SEC Regulation AC no part of my/our compensation was, is or will be directly or indirectly related to the specific views or recommendations expressed herein. This certification is made under the obligations set forth in SEC Regulation AC. Any other person or entity may not use it for any other purpose. This certification is made based on my/our analysis on the date of this report's publication. I/We assume no obligation to update this certification to reflect any facts, circumstances or events that may subsequently come to my/our attention. Signed Jason N. Butler

### JMP Securities Disclosures:

JMP Securities currently makes a market in the security of Marinus Pharmaceuticals, Inc.

JMP Securities was manager or co-manager of a public offering of securities for Marinus Pharmaceuticals, Inc. (MRNS) in the past 12 months, and received compensation for doing so.

JMP Securities expects to receive OR intends to seek compensation for investment banking services from Marinus Pharmaceuticals, Inc. in the next 3 months.

### JMP Securities Investment Opinion Definitions:

Market Outperform (MO): JMP Securities expects the stock price to outperform relevant market indices over the next 12 months.

Market Perform (MP): JMP Securities expects the stock price to perform in line with relevant market indices over the next 12 months.

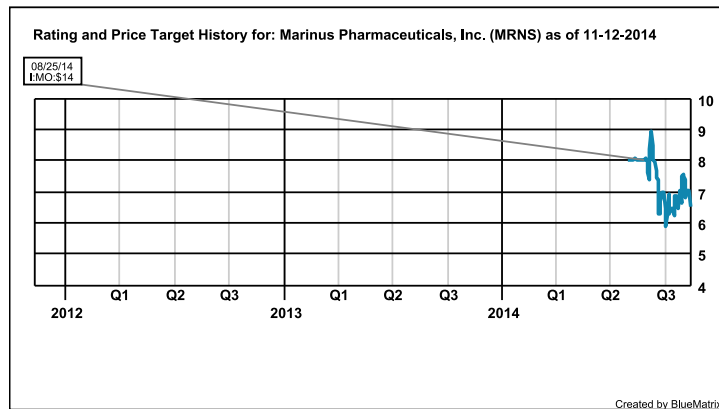
Market Underperform (MU): JMP Securities expects the stock price to underperform relevant market indices over the next 12 months.

### JMP Securities Research Ratings and Investment Banking Services: (as of November 13, 2014)

JMP Rating	Regulatory Equivalent	# Co's Under Coverage	% of Total	# Co's Receiving IB Services in Past 12 Months				
				Regulatory Equivalent	# Co's Under Coverage	% of Total	Services in Past 12 Months	% of Co's With This Rating
MARKET OUTPERFORM	Buy	285	61.03%	Buy	285	61.03%	104	36.49%
MARKET PERFORM	Hold	141	30.19%	Hold	141	30.19%	15	10.64%
MARKET UNDERPERFORM	Sell	2	0.43%	Sell	2	0.43%	0	0%
COVERAGE IN TRANSITION		36	7.71%		36	7.71%	0	0%
TOTAL:		467	100%		467	100%	121	25.91%

### Stock Price Chart of Rating and Target Price Changes:

Note: First annotation denotes initiation of coverage or 3 years, whichever is shorter. If no target price is listed, then the target price is N/A. In accordance with NASD Rule 2711, the chart(s) below reflect(s) price range and any changes to the rating or price target as of the end of the most recent calendar quarter. The action reflected in this note is not annotated in the stock price chart. Source: JMP Securities.



#### JMP Disclaimer:

JMP Securities LLC (the "Firm") compensates research analysts, like other Firm employees, based on the Firm's profitability, which includes revenues from the Firm's institutional sales, trading, and investment banking departments as well as on the quality of the services and activities performed that are intended to benefit the Firm's institutional clients. These data have been prepared by JMP Securities LLC for informational purposes only and are based on information available to the public from sources that we believe to be reliable, but we do not guarantee their accuracy or completeness. Any opinions and projections expressed herein reflect our judgment at this date and are subject to change without notice. These data are neither intended nor should be considered as an offer to sell or a solicitation or a basis for any contract for the purchase of any security or other financial product. JMP Securities LLC, its affiliates, JMP Group LLC, Harvest Capital Strategies LLC, and their respective partners, directors, officers, and associates may have a long or short position in, may act as a market maker for, or may purchase or sell a position in the securities mentioned herein. JMP Securities LLC or its affiliates may be performing, have performed, or seek to perform investment banking, advisory, or other services and may have acted as manager or co-manager for a public offering of securities for any company mentioned herein. The reader should assume that JMP Securities LLC will solicit business from the company covered in this report. Members of our Sales and Trading Department provide oral and/or written market opinions and trading strategies to our clients that reflect their personal opinions about stocks that are the subject of the firm's research reports. Our research analysts discuss trading strategies with clients that sometimes reflect short-term expectations for the price of the securities that are the subject of research reports. These trading strategies are distinct from the analysts' fundamental rating for the stock, which is based upon the analysts' view compared to other stocks under coverage for the relevant time period. © Copyright 2014. All rights reserved by JMP Securities LLC. JMP Securities LLC is a member of FINRA, NASDAQ, and SIPC.

**Jeffrey H. Spurr**  
**Director of Research**  
 (415) 835-3903

## RESEARCH PROFESSIONALS

### FINANCIAL SERVICES

#### Alternative Asset Managers

Devin Ryan (212) 906-3578  
 Brian McKenna (212) 906-3545

#### Commercial & Specialty Finance

Christopher York (415) 835-8965  
 Hannah Kim, CFA (415) 835-8962

#### Consumer Finance

David M. Scharf (415) 835-8942  
 Douglas Greiner (212) 906-3525

#### Financial Processing & Outsourcing

David M. Scharf (415) 835-8942  
 Douglas Greiner (212) 906-3525

#### Insurance

Matthew J. Carletti (312) 768-1784  
 Christine Worley (312) 768-1786

#### Investment Banks & Brokers

Devin Ryan (212) 906-3578  
 Brian McKenna (212) 906-3545

#### Mortgage Operating Companies

##### REITs: Agency, Hybrid, & Commercial Mortgage

Steven C. DeLaney (404) 848-7773  
 Trevor Cranston, CFA (415) 869-4431  
 Charter Robinson (757) 613-8955  
 Benjamin Zucker (212) 906-3529

### HEALTHCARE

#### Biotechnology

Liisa A. Bayko (312) 768-1785  
 Masha Chapman (415) 835-8944  
 Andrew Prigodich, PhD (312) 768-1788  
 Bhumika Sharma, PhD (312) 768-1795  
 Jason N. Butler, PhD (212) 906-3505  
 Caroline Palomeque (212) 906-3509  
 Michael G. King, Jr. (212) 906-3520  
 Bryan Czyzewski, PhD (212) 906-3577

#### Healthcare Services & Facilities

Peter L. Martin, CFA (415) 835-8904  
 Aaron Hecht (415) 835-3963  
 Arthur Kwok (415) 835-8908

#### Life Science Tools & Diagnostics

J. T. Haresco, III, PhD (415) 869-4477  
 Marie T. Casey, PhD (415) 835-3955

#### Medical Devices

J. T. Haresco, III, PhD (415) 869-4477  
 Marie T. Casey, PhD (415) 835-3955

#### Medical Devices & Supplies

David Turkaly (212) 906-3563  
 John Gillings (212) 906-3564

#### Specialty Pharmaceuticals

Oren G. Livnat, CFA (212) 906-3566  
 Nazibur Rahman (212) 906-3519

### REAL ESTATE

#### Housing & Land Development

Peter L. Martin, CFA (415) 835-8904  
 Aaron Hecht (415) 835-3963  
 Bharathwajan Iyengar (415) 835-3902

#### Lodging & Leisure

Robert A. LaFleur (212) 906-3510  
 Whitney Stevenson (212) 906-3538

#### Property Services

Mitch Germain (212) 906-3546  
 Peter Lunenburg (212) 906-3537

#### REITs: Healthcare, Residential, & Specialty

Peter L. Martin, CFA (415) 835-8904  
 Aaron Hecht (415) 835-3963  
 Arthur Kwok (415) 835-8908

#### REITs: Office, Industrial, & Diversified

Mitch Germain (212) 906-3546  
 Peter Lunenburg (212) 906-3537

#### Residential Services

Peter L. Martin, CFA (415) 835-8904  
 Aaron Hecht (415) 835-3963  
 Bharathwajan Iyengar (415) 835-3902

### TECHNOLOGY

#### Communications Infrastructure & Internet Security

Erik Suppiger (415) 835-3918  
 John Lucia (415) 835-3920

#### Internet & Digital Media

Ronald V. Josey III (212) 906-3528  
 Andrew Boone, CFA (415) 835-3957  
 Ignatius Njoku (415) 835-8960  
 Michael Wu (415) 835-8996

#### Software

Patrick Walravens (415) 835-8943  
 Peter Lowry (415) 869-4418  
 Mathew Spencer (415) 835-8930  
 Greg McDowell (415) 835-3934  
 Rishi Jaluria (415) 835-3961

#### Wireless & Cloud Computing Technologies

Alex Gauna (415) 835-8998

## ADDITIONAL CONTACTS

**Thomas R. Wright**  
**Director of Equities**  
 (212) 906-3599

**Dan Wychulis**  
**Director of Institutional Sales**  
 (617) 235-8530

**600 Montgomery Street, Suite 1100**  
 San Francisco, CA 94111  
[www.jmpsecurities.com](http://www.jmpsecurities.com)