

Specialty Pharmaceuticals

Price:	\$9.65
Fair Value Estimate:	\$20.00
52-Week Range:	\$5.49 - \$16.60
Market Cap (MM):	\$137
Shr.O/S-Diluted (mm):	14.2
Average Daily Volume:	26,964
Yield:	0.0%
Cash/Share:	NA
FCF Yield:	NA
Debt/Cap:	0%

FYE: Dec	2014E	2015E
EPS:	\$(1.76)A	\$(2.11)E
Prior EPS:		NC
Consensus	NM	-2.16
P/E Ratio:	NA	NA

Quarterly EPS:

Q1	\$(4.79)A	\$(0.53)E
Q2	\$(7.98)A	\$(0.53)E
Q3	\$(0.22)A	\$(0.53)E
Q4	\$(0.33)A	\$(0.52)E

Quarterly Revenue (M):

Q1	\$0A	\$0E
Q2	\$0E	\$0E
Q3	\$0E	\$0E
Q4	\$0E	\$0E
Year:	\$0E	\$0E



March 24, 2015

## Marinus Pharmaceuticals, Inc (MRNS) - BUY

### Ganaxolone Granted Orphan Designation for PCDH19

#### Flash Takeaways

MRNS announced this afternoon that the FDA has granted orphan status to ganaxolone in the indication of PCDH19, a rare form of epilepsy that effects between 10,000 and 15,000 females in the US. The designation is associated with patient populations of 200,000 or less and allows for a plethora of benefits such as seven year market exclusivity, tax credits, a waiving of the PDUFA filing fee and increased communication with the FDA to help facilitate development. Currently in a Phase 2 Proof of Concept trial, data is expected to read out 2H15.

#### Analysts Notes

- What is Orphan Designation?: Orphan designation is dictated by the affected number of potential patients with orphan being associated with disease states that effect 200,000 patients or less. In order to encourage companies to develop treatments for these small patient populations, orphan designation comes with 7 years of market exclusivity as well other incentives to lighten the financial burden such as tax credits and waiving of the FDA PDUFA fee.
- What is PCDH19?: PCDH19 female pediatric epilepsy is a rare disease effecting between 15,000 to 30,000 females in the US caused by a specific gene mutation. PCDH19 manifests as cluster seizures before the age of 5 with patients experiencing multiple seizure types throughout childhood with developmental delay, intellectual disability, and behavioral problems following into adolescence and adulthood. There are no current approved treatments for PCDH19.
- Proof of Concept Trial Design and Endpoints: The POC trial is small, enrolling ~10 patients between 2 and 10 years old that have confirmed PCDH19 mutation via genetic testing. Baseline seizure will be established and patients will be treated with Ganaxolone for 26 weeks. Primary endpoint is change in seizure frequency per 28 days relative to baseline.
- Maintain Buy rating, \$20 FV: Our \$20 fair value is based on a sum-of-the parts analysis with 2025 US sales of ganaxolone at \$11/share at a 3.5x multiple discounted back 25%, 2025 EU sales of ganaxolone at \$7/share at a 3.5x multiple discounted back 30% with the remaining cash (end 15) and tech value of \$2/share.

## IMPORTANT DISCLOSURES

### Research Analyst Certification

I, Chiara Russo, the Primarily Responsible Analyst for this research report, hereby certify that all of the views expressed in this research report accurately reflect my personal views about any and all of the subject securities or issuers. No part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or views I expressed in this research report.

### Janney Montgomery Scott LLC ("Janney") Equity Research Disclosure Legend

Marinus Pharmaceuticals, Inc currently is, or during the past 12 months was, a Janney Montgomery Scott LLC client. Janney Montgomery Scott LLC, provided investment banking related services.

Janney Montgomery Scott LLC managed or co-managed a public offering of securities for Marinus Pharmaceuticals, Inc in the past 12 months.

Janney Montgomery Scott LLC received compensation for investment banking services from Marinus Pharmaceuticals, Inc in the past 12 months.

Janney Montgomery Scott LLC intends to seek or expects to receive compensation for investment banking services from Marinus Pharmaceuticals, Inc in the next three months.

The research analyst is compensated based on, in part, Janney Montgomery Scott's profitability, which includes its investment banking revenues.

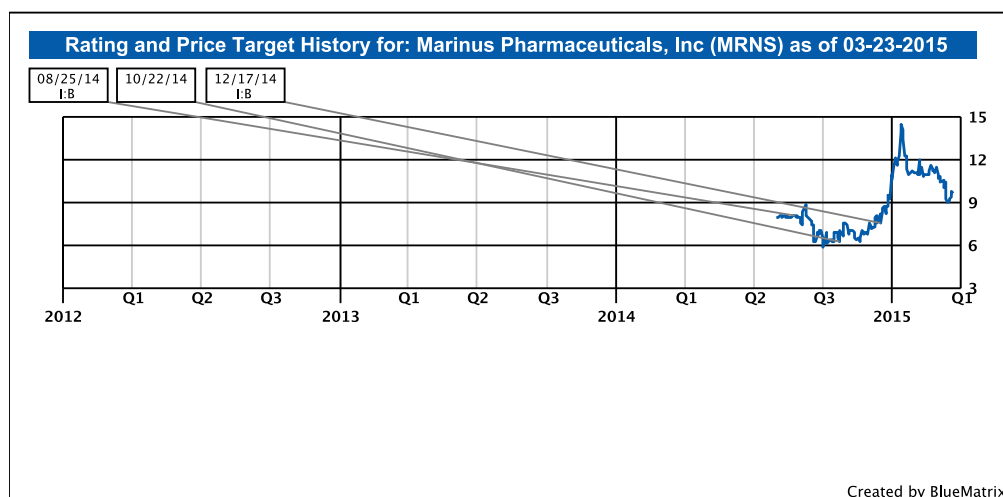
### Definition of Ratings

**BUY:** Janney expects that the subject company will appreciate in value. Additionally, we expect that the subject company will outperform comparable companies within its sector.

**NEUTRAL:** Janney believes that the subject company is fairly valued and will perform in line with comparable companies within its sector. Investors may add to current positions on short-term weakness and sell on strength as the valuations or fundamentals become more or less attractive.

**SELL:** Janney expects that the subject company will likely decline in value and will underperform comparable companies within its sector.

### Price Charts



### Janney Montgomery Scott Ratings Distribution as of 12/31/14

Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
<b>BUY [B]</b>	138	51.30	15	10.87
<b>NEUTRAL [N]</b>	131	48.70	5	3.82
<b>SELL [S]</b>	0	0.00	0	0.00

\*Percentages of each rating category where Janney has performed Investment Banking services over the past 12 months.

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