

Biotechnology

Marinus Pharmaceuticals, Inc

(MRNS) - BUY

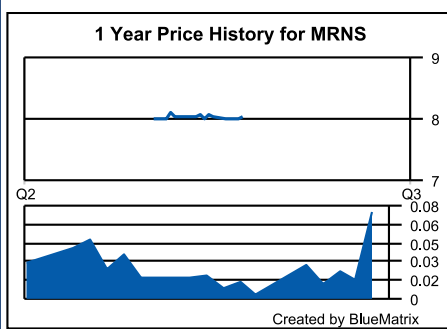
Price:	\$8.04
Fair Value Estimate:	\$13.00
52-Week Range:	\$8.00-\$16
Market Cap (MM):	\$111
Shr.O/S-Diluted (mm):	13.8
Average Daily Volume:	NA

FYE: Dec	2014E	2015E
EPS:	\$(1.68)E	\$(1.95)E
Prior EPS:	NC	NC
P/E:	NA	NA

Quarterly EPS:		
Q1	\$(0.28)A	\$(0.49)E
Q2	\$(0.67)E	\$(0.49)E
Q3	\$(0.39)E	\$(0.48)E
Q4	\$(0.39)E	\$(0.48)E

FYE: Dec	2014E	2015E
Revenue (M):	\$0.0E	\$0.0E

Quarterly Revenue (M):		
Q1	\$0.0A	\$0.0E
Q2	\$0.0E	\$0.0E
Q3	\$0.0E	\$0.0E
Q4	\$0.0E	\$0.0E



Equity Research
Basic Report

MRNS: Ganaxolone – A Differentiated Product; Initiating At Buy; \$13 FV

INVESTMENT CONCLUSION:

We view MRNS' main value driver, ganaxolone, as a first-in-class, novel GABA modulator that should offer safety advantages as an adjunctive treatment of epilepsy over other marketed antiepileptic agents. Its value proposition lies in its unique mechanism of action (MOA) that can confer efficacy in a resistant population and improved tolerability, especially in women of child-bearing age and the elderly. We also consider ganaxolone a pipeline within a product, as promising data could support utility in Fragile X Syndrome, PCDH19 female pediatric epilepsy and other disorders. MRNS' near-term success will be correlated with a successful Phase 2b outcome in refractory epilepsy but other shots on goal can be considered call options, in our view. We see de-risking catalysts such as PCHD19 data in 1H:15, Fragile X data in mid:15, Study 601 data in refractory epilepsy in 2H:15 and a potential collaboration by YE:15 as potentially value-creating events that could drive momentum. We deem MRNS an appealing investment based on a potentially first-in-class product targeting a significant market opportunity, a deep pipeline, near-term catalysts and compelling valuation. As such, we are initiating coverage of MRNS with a BUY rating and \$13 fair value estimate.

KEY POINTS:

- **Ganaxolone - differentiated profile translates into prescribing preference.** What distinguishes ganaxolone from marketed drugs like Keppra or Vimpat is the drug's unique molecular structure and mechanism of action that allows for the same GABA modulation effects as allopregnanolone without the negative steroidal effects, which would allow for chronic use. The drug has demonstrated a statistically significant reduction in seizure frequency as compared to patients on placebo without any cardiovascular (CV) or reproductive signals. Neurologists indicate that they would utilize a drug with a new mechanism and no added significant toxicity to the current regimen and would consider ganaxolone in the refractory population, particularly in the pediatric population, women of child-bearing age and the elderly, given its novel MOA, safety data, lack of psychiatric side effects and lack of cognitive blunting.
- **Targeting a meaningful market opportunity with expansion from other indications.** We estimate peak global sales of approximately \$600M in refractory partial onset seizures, assuming a \$3,300 annual price and 18% peak penetration. Pricing on par with Vimpat suggests upside given ganaxolone's differentiated safety profile. Label expansion from Fragile X Syndrome and PCDH19 could add another \$1B in peak sales, per our estimates.
- **Pipeline provides multiple shots on goal that could result in significant future upside.** Ganaxolone's MOA provides rationale for use in numerous indications. To that extent, ganaxolone is also being tested in a Phase 2 study in patients with PCDH19 female pediatric epilepsy and Fragile X Syndrome, with data expected in 1H15 and mid:15, respectively. Preclinical data so far suggest utility here. We do not include value from these programs yet as we await potentially positive proof-of-concept data that should increase ganaxolone's value proposition.

Research Analyst Certifications and Important Disclosures are on pages 19 - 21 of this report

MRNS: Ganaxolone – A Differentiated Product; Initiating At Buy; \$13 FV

INVESTMENT CONCLUSION:

We view MRNS' main value driver, ganaxolone, as a first-in-class, novel GABA modulator that should offer safety advantages as an adjunctive treatment of epilepsy over other marketed antiepileptic agents. Ganaxolone's value proposition lies in its unique mechanism of action (MOA) that can confer efficacy in a resistant population and improved tolerability especially in women of child-bearing age and the elderly. We also consider ganaxolone a pipeline within a product, as promising data could support utility in Fragile X Syndrome, PCDH19 female pediatric epilepsy and other neuropsychiatric disorders. MRNS' near-term success will be correlated with a successful Phase 2b outcome in refractory epilepsy, expected in 2H15, but other shots on goal can be considered as call options, in our view. We see de-risking clinical and business development catalysts in 2015 such as PCDH19 data in 1H, Fragile X data in mid:15, Study 601 data in refractory epilepsy in 2H:15, and a potential collaboration by YE:15 as potentially value-creating events that could drive momentum. We deem MRNS an appealing investment based on a potentially first-in-class product candidate targeting a significant market opportunity, a deep pipeline, near-term catalysts and compelling valuation. As such, we are initiating coverage of MRNS with a BUY rating and \$13 fair value estimate, which is based on a DCF analysis of cash flows through 2023.

KEY POINTS:









- **Ganaxolone - differentiated profile translates into prescribing preference.** What distinguishes ganaxolone from marketed drugs like Keppra or Vimpat is the drug's unique molecular structure and mechanism of action that allows for the same GABA modulation effects as allopregnanolone without the negative steroidal effects, which would allow for chronic use. The drug has demonstrated a statistically significant reduction in seizure frequency as adjunctive therapy in drug-treated patients as compared to patients on placebo without any cardiovascular (CV) or reproductive signals. Feedback from neurologists has indicated that physicians are seeking to add a drug with a new mechanism without adding significant toxicity to the current regimen and would consider utilizing ganaxolone in the refractory population, particularly in the pediatric population, women of child-bearing age and the elderly, given its novel MOA, safety data, lack of psychiatric side effects and lack of cognitive blunting.
- **Targeting a meaningful global market opportunity with expansion from other indications.** We estimate peak global sales of approximately \$600M in refractory partial onset seizures, assuming a \$3,300 annual price and 18% peak penetration. Pricing on par with Vimpat suggests upside to our assumption given ganaxolone's differentiated safety profile. Label expansion from Fragile X Syndrome and PCDH19 could add another \$1B in peak sales, per our estimates.
- **Pipeline provides multiple shots on goal that could result in significant future upside.** Ganaxolone acts via GABA modulation through activation of GABA_A receptors, an MOA that provides rationale for use in numerous indications. To that extent, ganaxolone is also being tested in a Phase II study in patients with PCDH19 female pediatric epilepsy and Fragile X Syndrome, with data expected in 1H15 and mid:15, respectively. Preclinical data so far suggest utility in these indications but we do not include value from these programs yet as we await potentially positive proof-of-concept data that should increase ganaxolone's value proposition.

Summary and Investment Highlights

Company Description

Marinus Pharmaceuticals (MRNS) is a development stage biopharmaceutical company focused on the development and commercialization of ganaxolone, a novel GABA modulator, for the treatment of proprietary neuropsychiatric therapeutics. The drug has completed a Phase 2a study and is currently in a Phase IIb study in its lead indication as adjunctive therapy for partial or focal onset seizures in adults with epilepsy, with data expected in 2H15. Other clinical programs underway include proof-of-concept studies in PCDH19 female pediatric epilepsy and Fragile X Syndrome and a completed Phase 2 study in Posttraumatic Stress Disorder (PTSD) (Exhibit 1).

Exhibit 1: Pipeline

Drug Candidate	Phase 1	Phase 2	Phase 3	Status
Ganaxolone in Epilepsy				
Adjunctive Treatment of Focal Onset Seizures				Phase 2b - Data expected in 2H15
PCDH19 Female Pediatric Epilepsy				Phase 2 POC - Data expected in 1H15
Ganaxolone in Behavioral Symptoms				
Fragile X Syndrome (grant funded)				Phase 2a - Data expected in mid:15
Ganaxolone in Psychiatric Disorders				
Posttraumatic Stress Disorder (grant funded)				Phase 2a - Completed; Data in 2Q14

Source: Company presentation, Janney estimates

We are initiating coverage of Marinus Pharmaceuticals with a Buy rating and \$13 fair value estimate. We view MRNS as a compelling investment for small-cap investors for the following reasons:

- 1) Novel MOA - Ganaxoloneb is a first-in-class, positive allosteric modulator of GABA with a differentiated profile targeting unmet medical needs and significant market opportunities;
- 2) Significant global market opportunity in lead and orphan indications - We estimate peak worldwide sales for ganaxolone could reach \$600M on fairly conservative assumptions for refractory partial onset seizures while sales in PCDH19 and Fragile X Syndrome could add over \$1B in potential revenue;
- 3) Several shots on goal - The company's strategy of developing ganaxolone in multiple indications de-risks its clinical program and provides the company with several potential unmet medical opportunities, in our view;
- 4) Value-creating catalysts – Near-term de-risking milestones could provide share appreciation;
- 5) Compelling valuation – DCF analysis implies approximately 60% upside to current levels, without including value from earlier-stage pipeline candidates

Catalysts:

Upcoming Catalysts	2012				2013				2014				2015			
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4
Partial Onset Epilepsy					Phase 2b											Data
Fragile X Syndrome					Phase 2 POC											Data
PCDH19 Female Pediatric Epilepsy											Open					Data
Post Traumatic Stress Disorder					Phase 2 POC						Data					

Source: Company presentation, Janney estimates

Valuation:

Our 12-month fair value estimate of \$13 is based on a DCF analysis that evaluates cash flow through 2023. Based on a pro-forma fully diluted share count of 13.8M plus the impact of in-the-money options outstanding and assuming the midpoint of the discount rate at 25%, our calculated average intrinsic share value is \$13 (Exhibit 2). MRNS has a cash position of approximately \$40M as of 3Q14, which is sufficient to support operations through 1Q16, per our estimates.

Exhibit 2: Discounted Cash Flow Analysis

		Assuming Discount Rates of:				
		24.0%	24.5%	25.0%	25.5%	26.0%
Present Value of Unlevered Free Cash Flow		\$ (53,228)	\$ (52,686)	\$ (52,148)	\$ (51,616)	\$ (51,088)
Present Value of Terminal Value Assuming	Exit Multiple					
	5.00x	\$ 204,569	\$ 196,071	\$ 187,936	\$ 180,147	\$ 172,689
	5.25	214,797	205,875	197,332	189,154	181,324
	5.50	225,026	215,678	206,729	198,161	189,958
	5.75	235,254	225,482	216,126	207,169	198,592
Annualized Quarterly Revenue (as of 6/30/23)		\$ 333,335				
		6.00	245,483	235,285	225,523	216,176
Enterprise Value		5.00x	\$ 151,341	\$ 143,385	\$ 135,787	\$ 128,531
(PV of Free Cash Flow + PV of Terminal Value)		5.25	161,569	153,189	145,184	137,538
		5.50	171,798	162,992	154,581	146,546
		5.75	182,026	172,796	163,978	155,553
		6.00	192,254	182,599	173,374	164,560
Net Debt (as of 7/1/14)		\$ (40,439)	\$ (40,439)	\$ (40,439)	\$ (40,439)	\$ (40,439)
Options Proceeds		1,109	1,109	1,109	1,109	1,109
Equity Value		5.00x	\$ 192,889	\$ 184,933	\$ 177,335	\$ 170,079
(Enterprise Value - Net Debt + Option Proceeds)		5.25	203,117	194,737	186,732	179,086
		5.50	213,346	204,540	196,129	188,094
		5.75	223,574	214,344	205,526	197,101
		6.00	233,802	224,147	214,922	206,108
Shares Outstanding			13,779	13,779	13,779	13,779
Options Outstanding ("In-the-Money")			1,066	1,066	1,066	1,066
Diluted Shares Outstanding			14,845	14,845	14,845	14,845
Implied Price Per Share		5.00x	\$ 12.99	\$ 12.46	\$ 11.95	\$ 11.46
		5.25	13.68	13.12	12.58	12.06
		5.50	14.37	13.78	13.21	12.67
		5.75	15.06	14.44	13.84	13.28
		6.00	15.75	15.10	14.48	13.88

Source: Janney estimates

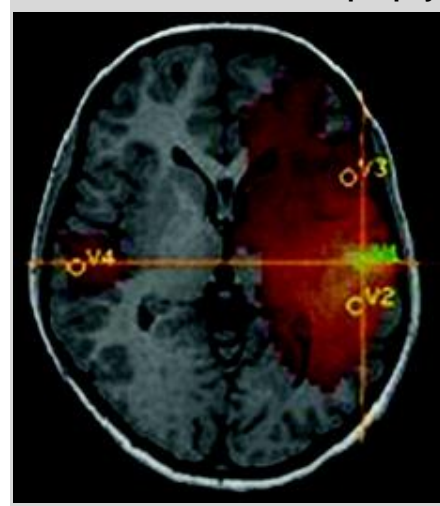
What is Focal Epilepsy and How is it Treated?

Epilepsy is characterized by seizures that are caused by abnormal electrical disturbances within the brain. According to the Institute of Medicine, there were approximately 2.3 to 3 million epilepsy patients in the US in 2012. Decision Resources reported similar numbers with approximately five million people under treatment for epilepsy in the US, Europe and Japan. For the most part, epileptic seizures fall into two categories, primary generalized seizures which affect the whole brain, and focal onset seizures in which the electrical discharge is limited to one area of the brain (Exhibit 3). A diagnosis of epilepsy occurs when a patient experiences at least two seizures that do not have a self-limiting cause.

Treatment for recently diagnosed patients involves chronic administration of an antiepileptic drug (AED). Suppression of seizures is achieved by various different mechanisms of action which come with a variety of side effects. The majority of patients (~60%) achieve adequate control with either first- or second-line monotherapy, which includes generics like carbamazepine, phenytoin or valproic acid. The rest of the patients need additional medications, called polypharmacy, and approximately 35% of patients do not achieve adequate seizure control and remain refractory to therapy. The latter is ganaxolone's initial target population. Physicians balance side effects with the risk of serious reaction, the convenience of administration and potential cost to the patient. For patients who require more than one medication, the side effect of these drugs becomes a very important factor in how physicians proceed with treatment. Unfortunately, the majority of marketed AEDs have many potential side effects associated with chronic use including cardiovascular risk, kidney stones, negative effects on cognition and reproductive risk among women (Exhibit 4).

One of the more common risks for women taking some of these AEDs is potential negative side effects if she becomes pregnant while on drug. Some of the complications associated with these AEDs are risks to the fetus that include birth defects, lowered IQ and low birth weight. Most drugs are categorized by the FDA as Pregnancy Category C which indicates a potential risk to the fetus. Valproate, carbamazepine and phenytoin are classified as Pregnancy Category D, which cautions use as only justifiable if there is a serious condition where the need outweighs the risk to the fetus based on registry data.

Exhibit 3: Scan of focal epilepsy



Source: Neurology.org

Exhibit 4: Current Adjunctive Treatment Comparisons

Generic Drug	Branded Name	Generic	Black Box	Pregnancy Category	Mechanism of Action	Side Effects
levetiracetam	Keppra	Y	N	C	Binds to SV2A protein	Psychotic sx, irritability, aggression, depression
lamotrigine	Lamictal, (XR and ODT)	Y	Y	C	Sodium channel blocker	SJS, TEN, rash, worsening of bipolar disorder and suicidality
topiramate	Topamax	Y	N	D	Glutamate Blocker	Cognitive blunting, depression
oxcarbazepine	Trileptal	Y	N	C	Sodium channel blocker	SJS, hyponatremia, suicidal behavior
pregabalin	Lyrica	N	N	C	Calcium channel blocker	Cognitive blunting
lacosamide	Vimpat	N	N	C	Sodium channel blocker	NA
ezogabine	Potiga	N	Y	C	Neuronal potassium channel opener	Vision loss, confusional state, psychotic sx, hallucinations
rufubamide	Banzel	N	Y	C	Sodium channel regulator	Suicidal ideation
zonisamide	Zonegran	N	N	C	Sodium channel blocker	Kidney stones, metabolic acidosis
vigabatrin	Sabril	N	Y	C	GABA transaminase inhibitor	Acute psychosis, irritability
eslicarbazepine	Aptiom	N	N	C	QD metabolite of oxcarbazepine	Suicidal ideation, memory impairment, amnesia
perampanel	Fycompa	N	Y	C	Glutamate blocker	Neuropsychiatric events, irritability, aggression, etc
ganaxolone	ganaxolone	N	N	B	Postsynaptic GABAA receptor binder	None expected

SJS - Stevens-Johnson Syndrome; TEN - toxic epidermal necrosis

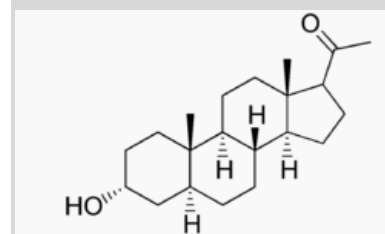
Source: Company reports, Janney estimates

Another common side effect is cognitive issues. Suppression of the seizures can cause brain cells to fire less rapidly and can slow down cognition, typically called cognitive blunting. Mood disorders such as depression, confusion and suicidality have also been associated with taking certain AEDs.

Ganaxolone's Value Proposition: Unique Mechanism of Action and Safety, Safety, Safety

Ganaxolone is a small molecule synthetic analog of allopregnanolone, an endogenous neurosteroid that modulates GABA through the activation of GABA_A receptors. As a neurosteroid, ganaxolone interacts with both synaptic and extrasynaptic GABA_A receptors and provides a more constant control of the GABA inhibitory signal that calms overexcited neurons. Unlike some AEDs, ganaxolone activates GABA_A receptors by interacting with the neurosteroid recognition site. Specifically, outside of the synapse, allopregnanolone is absorbed into the cell membrane and slowly diffuses to activate the extrasynaptic GABA_A receptors. As an analog, there is high affinity for extrasynaptic GABA_A receptors with the δ subunit which results in tonal and phasic modulation of GABA-mediated inhibition of neuronal excitation. Structurally, ganaxolone is similar to allopregnanolone with an additional methyl group (Exhibit 5) which prevents the conversion of ganaxolone back to an active steroid. The effect is elimination of unwanted hormonal effects while maintaining the central nervous system activity.

Exhibit 5: Allopregnanolone



Source: Molecular-networks.com

Approximately 1,000 patients have been dosed with ganaxolone, with duration ranging from days to 2 years and on doses as low as 50mg/day to as high as 2,000 mg/day. In clinical trials, the majority of adverse events were not medically serious and resolved upon discontinuation of therapy. As depicted in Exhibits 6 and 7, ganaxolone caused fewer dropouts and adverse events in clinical studies than other marketed adjunctive therapies. Importantly, ganaxolone has a lower risk for reproductive toxicity based on preclinical data in which there were no malformations of the embryo or fetus in rats or mice. This differentiating toxicity profile is key given that all marketed AEDs have demonstrated developmental toxicities in preclinical studies that resulted in a rating of Pregnancy Category C. Marketed therapies such as valproate, carbamazepine, phenytoin and topiramate have been linked to birth defects in humans which have resulted in a Pregnancy Category D for these drugs. As such, we believe ganaxolone's mechanism and data to date may allow for a Pregnancy Category B rating for ganaxolone, which we believe would be an important value proposition for women of childbearing age. Coupled with the lack of psychological and cognitive issues, ganaxolone demonstrates a highly competitive and desirable product profile, in our view, and could present a commercially compelling drug profile that can provide significant safety advantages in the refractory setting.

Successful Clinical Progress to Date:

Exhibit 6: Clinical Drop-Out Rates

Drop-out rates	Levetiracetam (Keppra)	Pregabalin (Lyrica)	Lacosamide (Vimpat)	Ezogabine (Potiga)	Parempanel (Fycompa)	Eslicarbazepine (Aptiom)
On Drug	15%	15%	29%	25%	8%	14%
Placebo	12%	6%	5%	11%	5%	7%

Source: Company reports, Janney estimates

Exhibit 7: Safety Data Comparison

Expected AEs (%)	Ganaxolone (expected)	Levetiracetam (Keppra)	Placebo	Topiramate (Topamax)**	Placebo	Lamotrigine (Lamictal)	Placebo	Pregabalin (Lyrica)	Placebo	Lacosamide (Vimpat)*	Placebo	Ezogabine (Potiga)*	Placebo
Dizziness	8	9	4	25	15	38	13	31	11	53	8	15	9
Somnolence	11	15	8	29	12	14	7	28	11	8	5	15	12
Fatigue	8	n/a	n/a	15	13	n/a	n/a	n/a	n/a	15	6	16	6
Diplopia	1	2	1	10	5	28	7	12	4	16	2	8	2
Blurred Vision	5	n/a	n/a	n/a	n/a	16	5	12	4	16	3	2	2
Vomiting	0	n/a	n/a	n/a	n/a	9	4	n/a	n/a	16	3	n/a	n/a

* 600mg/day

** 200-400mg/day

Source: Company reports, Janney estimates

Study 600 - Phase 2a Study for Adjunctive Treatment of Refractory Focal Onset Seizures: Primary Efficacy Parameter Demonstrated Efficacy. In March 2009, MRNS announced positive data from a 147-patient, Phase 2a double-blind, randomized, placebo-controlled study of an oral suspension of ganaxolone as an adjunctive treatment in refractory focal onset seizures (Exhibit 8). Seventy-five percent of patients

in the study were on two or three AEDs to control the seizures prior to study start. Subjects were treated for ten weeks with placebo or ganaxolone as an adjunctive treatment to their existing therapy. Mean baseline seizure frequency was 6.5 and 9.2 seizures per seven days in the drug-treated and placebo groups, respectively. Dose titration occurred up to 500mg, three times daily for two weeks followed by a maintenance period of 1,500 mg/day for eight weeks. Ninety-five percent of patients reached the target 1,500 mg/day. The primary endpoint was change from baseline in weekly seizure frequency. There were 131 completers and eligible patients entered into the open-label extension study (Study 601).

Exhibit 8: Study 600 - Study Design	
Study 600 - Phase 2a Evaluate Safety, Tolerability, Efficacy of Ganaxolone as Add-On Therapy in Adults with Partial-Onset Seizures	
Purpose	Determine the efficacy and safety of ganaxolone in adults with partial onset seizures taking a maximum of 3 AEDs.
Study Design	Multi-center, randomized, double-blind, placebo controlled - 8 week baseline period, 2 week titration, 10-16 week measurement
Dosing	Ganaxolone - Oral suspension 200-500 mg dosed at 3x per day Placebo Comparator - Oral suspension 200-500 mg dosed at 3x per day
Primary Endpoint	Weekly seizure frequency, analyzed as mean weekly log-transformed seizure frequency
Secondary Endpoints	Change & percent change of mean weekly seizure frequency from baseline Change & percent change of mean weekly seizure frequency from baseline for each week after dosing Responder rate: responders are defined as patients experiencing $\geq 50\%$ of reduction in mean weekly seizure frequency during the maintenance period from the baseline Number of seizure-free days The weekly seizure-free subjects and seizure-free rate Seizure severity questionnaires and quality of life surveys changes Change in rate of seizures in catamenial epilepsy Weekly seizure frequency for each week after dosing.
Patients	N = 147
Safety	Ganaxolone was well tolerated and safe.

Source: Company reports

In the intent-to-treat (ITT) population, ganaxolone-treated patients experienced a 17.6% decrease in mean weekly seizure frequency from baseline and a median decrease of 26% at week 10 compared to a 2% mean increase and a 10.2% decrease in the placebo group. The mean difference of 19.6% was statistically significant ($p=0.014$) while the median difference was 15.7% (Exhibit 9).

Exhibit 9: Phase 2a Study 600 – Percent Seizure Reduction from Baseline			
% Seizure Reduction From Baseline			
	Ganaxolone (n=98)	Placebo (n=49)	Difference
Mean (standard deviation)	-17.6% (48.9)	+2.0 (63.2)	19.60%
Median	-26.00%	-10.20%	15.70%

Source: Company reports

Responders were considered the percentage of subjects with greater than or equal to 50% improvement from baseline and the results were not statistically significant. In the secondary analysis, the percent responders in the ganaxolone group compared to the placebo group in the intent-to-treat population were 23.5% and 14.3% ($p=0.192$) for the titration plus maintenance period and 26.3% and 13.0% ($p=0.057$) for the maintenance period.

Ganaxolone was safe and well tolerated. The two treatment groups showed similar discontinuation rates due to adverse events (ganaxolone group 7%, placebo 6%) and serious adverse event rates (ganaxolone 5%, placebo 8%), though most of these were due to the underlying epilepsy. As seen in Exhibit 10, the most common side effects were injury/poisoning, dizziness and fatigue

Exhibit 10: Study 600 Safety Data		
ITT Population	Ganaxolone (n=98)	Placebo (n=49)
	Percent	Percent
Dizziness	16.3	8.2
Fatigue	16.3	8.2
Somnolence	13.3	2.0
Injury, poisoning, procedural complications	17.3	22.4
Headache	8.2	12.2
Coordination abnormal	6.1	6.1
Convulsion	5.1	8.2
Nasopharyngitis	5.1	10.2
Fall	5.1	12.2
Serious Adverse Events	5	8
Discontinuation due to Adverse Event	7	6
At Least One Adverse Event	84	78

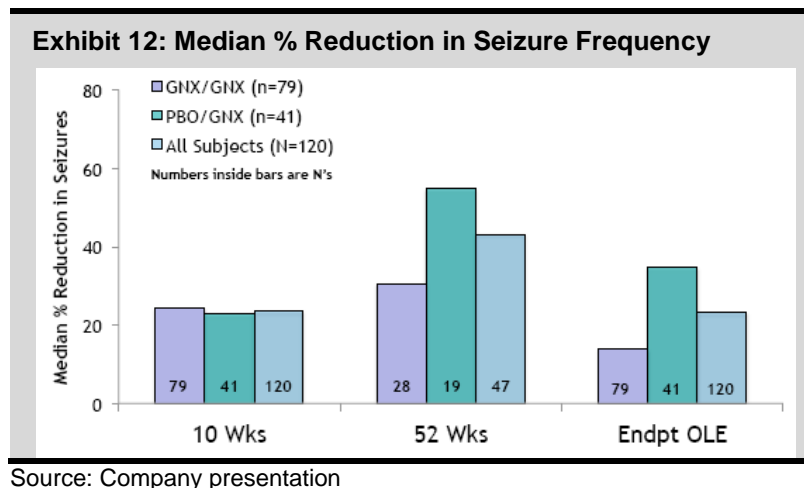
Source: Company presentation

There has been no evidence of cardiovascular, liver or blood adverse events in ganaxolone-treated patients nor any steroidal side effects and weight gain. In preclinical models, there has been no evidence of reproductive toxicity or other toxicities after long-term administration. Maintenance of this favorable safety profile through registration studies would make ganaxolone a compelling product profile for younger women due to the reproductive safety and for the elderly with the lack of cardiovascular and renal effects, in our view.

Study 601 - Phase 2 Open-Label Extension Study: Continued Efficacy Seen. Ninety-five percent of subjects in Study 600 were eligible to continue in the long-term open-label extension. This study examined the long-term safety, tolerability and efficacy of ganaxolone oral suspension at 1,500mg/day (Exhibit 11). The mean duration of treatment was 39 weeks and the primary endpoint was change in seizure frequency versus baseline of the double-blind study as mean and median change. Mean and median percent reductions in weekly seizure frequency were 14.2% and 23.2%, respectively, from baseline to endpoint. Seventy percent of subjects had a reduction in seizure frequency (Exhibit 12). In the secondary analysis, 24% of subjects met the responder criteria, which was a reduction in seizure frequency of 50% or more from baseline.

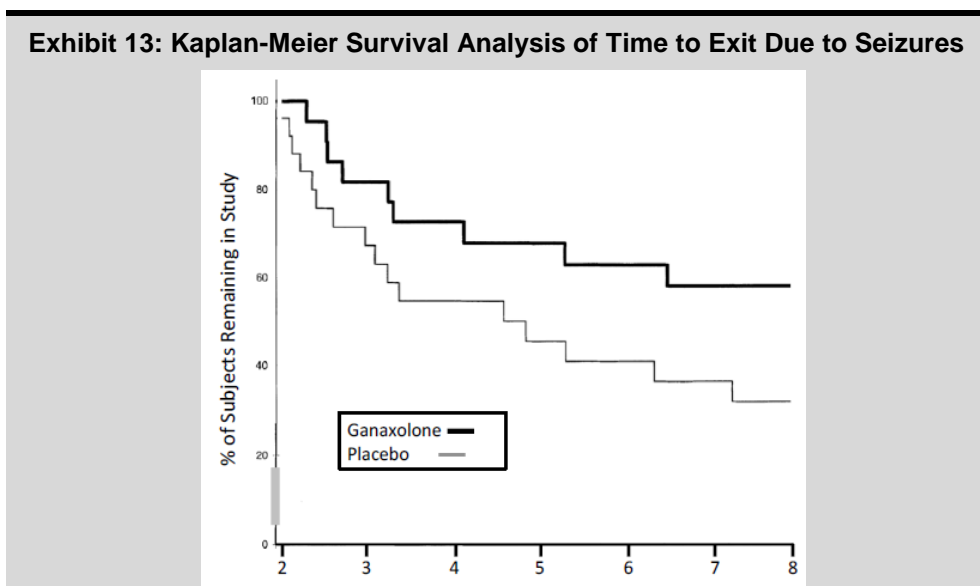
Exhibit 11: Study 600 Safety Data	
Study 601 - Phase 2a Open-Label Extension Trial For Adjunctive Treatment of Drug-Resistant Focal Onset Seizures	
Purpose	Evaluate the safety, tolerability, and efficacy of ganaxolone in adults with partial onset epilepsy with or without secondary generalizations
Study Design	Multi-center open-label extension trial
Dosing	Ganaxolone - Oral suspension 200-500 mg dosed at 3x per day over 39 weeks
Primary Endpoint	Change in weekly seizure frequency at study end compared to baseline at beginning of the double-blind study
Secondary Endpoints	<p>Responder rate. Responder is defined as patients experiencing $\geq 50\%$ of reduction in weekly seizure frequency at study end from the baseline</p> <p>Number of seizure-free subjects and seizure-free rate</p> <p>The weekly seizure frequencies for each seizure subtype: POS with or without secondary generalization, but not non-motor SPS</p> <p>Seizure severity and quality of life surveys</p> <p>Change in rate of seizures in catamenial epilepsy</p>
Patients	N = 125
Safety	Ganaxolone was well tolerated and safe.
Results 09/13	Mean and median % reductions in weekly seizure frequency were 14.2% and 23.2% from baseline to endpoint. 70% of subjects had a reduction in seizure frequency.

Source: Company presentation



Study 104 - Phase 2a Monotherapy Study in Drug Resistant Patients: Drug-Treated Patients More Likely to Remain in Study. The company conducted a 52-patient, Phase 2 monotherapy study with ganaxolone as a monotherapy. Patients stopped their AEDs prior to evaluation for surgical treatment of their seizures and were given 625mg three times daily for eight days. The primary endpoint was duration of treatment prior to emergence of seizures as measured starting at Day 2.

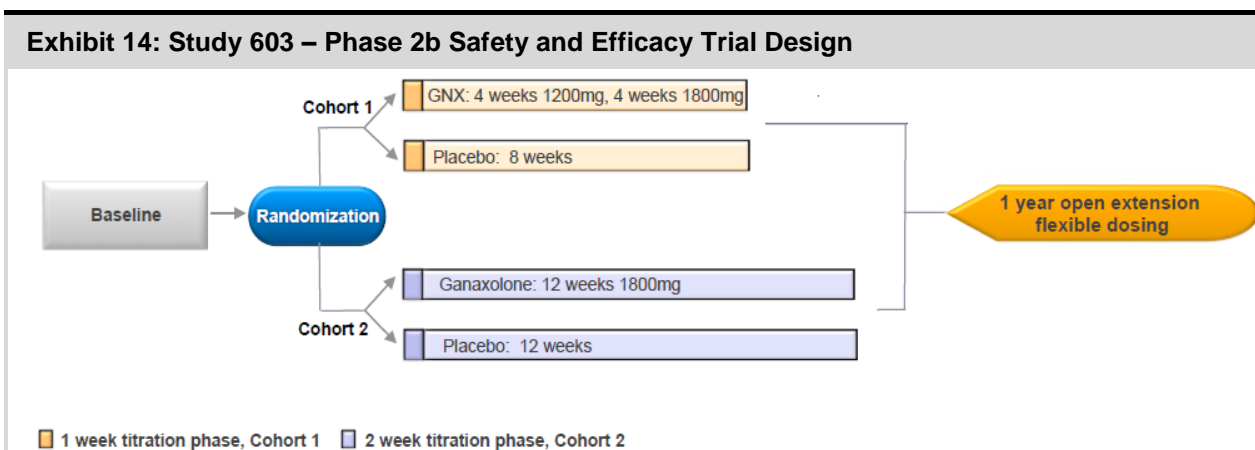
Data demonstrated that placebo patients were more likely to discontinue treatment due to emergent seizures than ganaxolone-treated patients (38% vs 61%; $p=0.08$) by day 8 (Exhibit 13). More ganaxolone subjects completed the study than placebo patients (50% vs. 25%; ($p=0.04$). There was one serious adverse event in each treatment group but there were no major differences in adverse events between groups and importantly, there were no EKG signals.



Current Clinical Program:

Study 603 – Ongoing Phase 2b Study of High Dose Ganaxolone: In October 2013, MRNS initiated an international, randomized, placebo-controlled Phase 2b trial in adults as adjunctive treatment of focal onset seizures. The purpose of the study is to examine responder rate, seizure-free status, changes in seizure subtypes, safety and adverse events. The trial consists of two cohorts with Cohort 1 randomizing 50 patients to placebo or ganaxolone capsules in a step titration of 1,200mg/day for four weeks followed

by 1,800mg/day for four weeks (Exhibit 14). In Cohort 2, an additional 150 participants will be randomized to receive either placebo or 1,800mg/day for 12 weeks. The company intends to expand Cohort 2 to approximately 300 patients to properly power the study as a well-controlled registration trial as part of a US/EU filing. The company anticipates top-line data in 2H15.



Source: Company presentation

Potential Phase 3 Study – MRNS intends to initiate a Phase 3 confirmatory, double-blind, randomized fixed-dose trial after the completion of Study 603. The purpose of this study is to examine the change in seizure frequency compared to baseline in adult patients with drug-resistant focal onset seizures who require add-on therapy. There will be three fixed dose arms of drug versus placebo for 12 weeks of maintenance therapy. Patients completing the study will be eligible to enroll in a one-year open-label extension study.

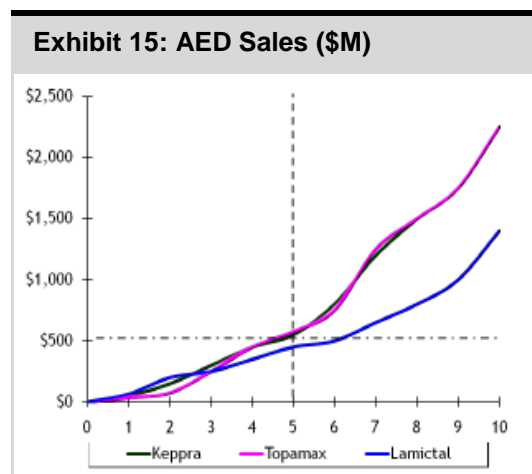
Other Planned Studies – The company plans to advance the tablet formulation of ganaxolone into pivotal studies. To date, ganaxolone has been tested in Studies 600 and 601 as an oral suspension and in Study 603 as a capsule formulation. Next steps include initiation of two pharmacokinetic studies to demonstrate bioequivalence between the capsule and tablet and to establish potency of the oral suspension to the tablet.

Adjunctive Therapy in Partial Onset Seizures is a Proven Market:

Though there are some unwanted side effects for many of these adjunctive therapies, there is still a strong market opportunity. The recent launch of Vimpat (lacosamide) in 2008 proves this out. Vimpat is an adjunctive treatment for refractory patients that had a novel mechanism of action, sodium channel blocker. It is priced at a 50% premium to branded Keppra and has had a very successful launch, reaching over \$500 million in sales five years post launch. Over 150,000 patients have been prescribed the medication and peak sales are estimated to top \$1.5B.

Vimpat Launch Sales (\$M)	
2009	\$62
2010	\$168
2011	\$287
2012	\$437
2013	\$565

Source: company presentation



Source: Company presentation

Competitive Landscape: Few Differentiated Treatments as Adjunctive Therapy in Development

Currently, there are several approved AEDs available to treat partial onset seizures (Exhibit 16). The more recent entrants include Vimpat, Potiga, Fycompa and Aptiom. We highlight Vimpat as one of the newer entrants with a novel MOA to treat refractory patients and has enjoyed a successful launch.

Exhibit 16: Marketed Therapies

Generic Drug	Branded Name	Generic	Black Box	Pregnancy Category	Mechanism of Action	Side Effects	2013 Sales (\$M)
levetiracetam	Keppra	Y	N	C	Binds to SV2A protein	Psychotic sx, irritability, aggression, depression	\$946
lamotrigine	Lamictal, (XR and ODT)	Y	Y	C	Sodium channel blocker	SJS, TEN, rash, worsening of bipolar disorder and suicidality	\$925
topiramate	Topamax	Y	N	D	Glutamate Blocker	Cognitive blunting, depression	NA
oxcarbazepine	Trileptal	Y	N	C	Sodium channel blocker	SJS, hyponatremia, suicidal behavior	NA
pregabalin	Lyrica	N	N	C	Calcium channel blocker	Cognitive blunting	NA
lacosamide	Vimpat	N	N	C	Sodium channel blocker	NA	\$565
ezogabine	Potiga	N	Y	C	Neuronal potassium channel opener	Vision loss, confusional state, psychotic sx, hallucinations	NA
rufubamide	Banzel	N	Y	C	Sodium channel regulator	Suicidal ideation	NA
zonisamide	Zonegran	N	N	C	Sodium channel blocker	Kidney stones, metabolic acidosis	NA
vigabatrin	Sabril	N	Y	C	GABA transaminase inhibitor	Acute psychosis, irritability	Approved in Oct. 2013
eslicarbazepine	Aptiom	N	N	C	QD metabolite of oxcarbazepine	Suicidal ideation, memory impairment, amnesia	Approved in Nov. 2013
perampanel	Fycompa	N	Y	C	Glutamate blocker	Neuropsychiatric events, irritability, aggression, etc	NA
ganaxolone	ganaxolone	N	N	B	Postsynaptic GABAA receptor binder	None expected	Not approved yet

SJS - Stevens-Johnson Syndrome; TEN - toxic epidermal necrosis

Source: Janney estimates

We view UCB's brivaracetam as MRNS' closest competitor currently in development. Brivaracetam represents a new MOA being a high-affinity synaptic vesicle protein 2A ligand. In July, UCB announced positive top-line data from its latest 768-patient Phase 3 study, which compared the efficacy and safety of 100mg and 200mg of brivaracetam to placebo over 12 weeks as adjunctive therapy in adult refractory focal epilepsy patients. The drug demonstrated statistical significance on the 50% responder rate endpoint and the percent reduction in seizures over a 28-day duration. The most common adverse events were somnolence, dizziness, fatigue and headache. We expect the company to file for US/EU approval in early 2015 with potential approvals in early 2016.

Sage Therapeutics is developing Sage-547, an allosteric modulator of GABA, for the treatment of status epilepticus. The drug is an IV formulation, which we believe would not be used for the treatment of partial onset seizures and would need to be re-formulated. Sage-547 is currently in an open-label Phase 1/2a trial of 10-15 patients with data expected by year-end. There are several key differences between Sage-547 and ganaxolone. First and foremost, ganaxolone is an analog of allopregnanolone which converts to a steroid, thus side-stepping some unwanted side effects where as Sage-547 is not an analog. Sage-547 is being formulated as an IV delivery as it is looking to target acute care whereas ganaxolone is formulated in capsules for chronic, long-term therapy. Ganaxolone has been tested in over 1000 patients will exposure duration as long as 2 years whereas Sage-547 has been dosed in less than 10 patients with expose duration of only several days. In the final analysis these compounds, though similar in structure, the two drugs target different spectrums of epilepsy and have different risk profiles (Exhibit 17).

Exhibit 17: Comparison of Ganaxolone to Sage-547

	Ganaxolone	Sage-547
Structure	Analog of allopregnanolone that does not convert to a steroid	Allopregnanolone
Formulation	Capsules and liquid	Interavenous (IV)
Patent Status	Multiple issued in US and worldwide	Application only, none issued
Development Stage	Phase 2b/3	Phase 1/2
Lead Indication	Adult and pediatric partial onset seizures	Super Refractory Status Epilepticus (RSE)
Treatment Setting	Hospital and community	Hospital ICU
Therapy Duration	Chronic, long-term therapy	Acute
Efficacy	Phase 2 - Placebo-controlled, double-blind study with statistically significant data	Open label study of 7 patients
Safety/Tolerability	> 1000 subjects exposed	7 subjects
Exposure Duration	2 years (adults)	Days
Patient Population	5 million (w/line extensions)	Approximately 25,000
Orphan Studies	Fragile X Syndrome, PCDH19	SRSE

Source: Company presentation

Other Potential Indications: PCDH19

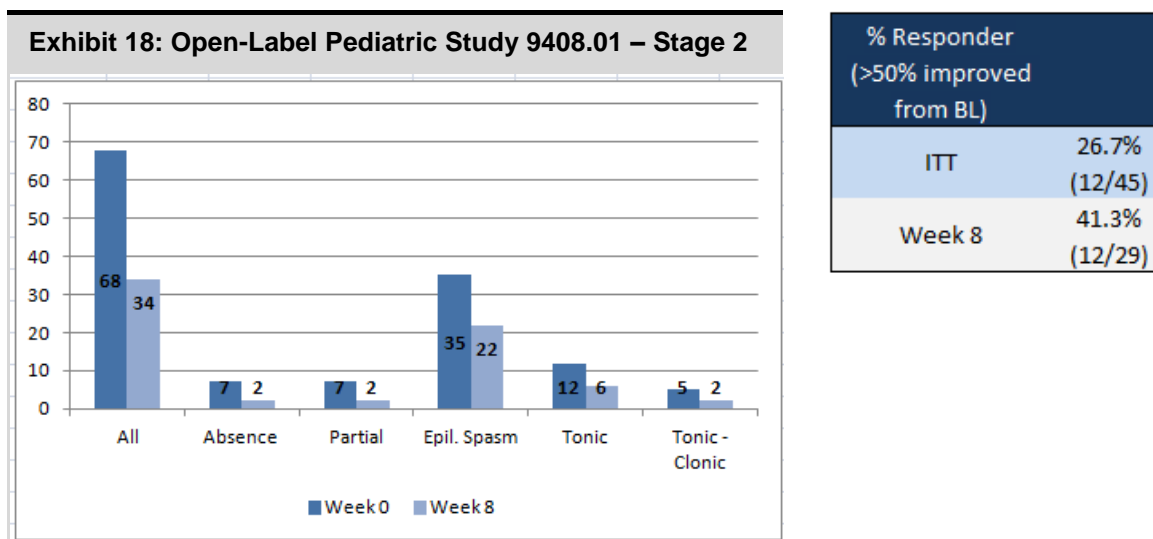
Female Pediatric Epilepsy

PCDH19 female pediatric epilepsy is a disorder caused by mutation of the PCDH19 gene on the X chromosome that results in a wide spectrum of severity in seizures, cognitive delays and other symptoms. PCDH19 can arise as a single case in a family, just through an accident in cell replication, or it

can be inherited. The inheritance pattern is very unusual in that men who carry the gene mutation on their only X-chromosome are typically unaffected, whereas approximately 90% of women who have the mutation on one of their two X-chromosomes, exhibit symptoms.

PCDH19 is characterized by seizures with an onset in infancy with cognitive impairment. The seizures often occur in clusters and are often provoked by fever or illness. The affected girls experience multiple seizure types (focal, generalized and absence) and developmental delay, intellectual disability and behavioral problems that persist after the seizures resolve in adolescence. Estimates suggest that the US incidence is between 40k-50k cases.

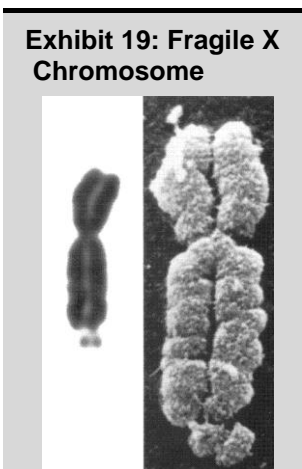
Ganaxolone's efficacy in children with multiple seizure types has been demonstrated in the open label pediatric study 9408.01/2 (Exhibit 18). In addition, the company believes that ganaxolone being a synthetic analog of allopregnanolone can be used to increase GABAergic signaling in these patients. The company plans to initiate an expanded access protocol to provide proof-of-concept in approximately 10 patients with PCDH19 female pediatric epilepsy. MRNS is finalizing the study design and expects to initiate the study in 2H14 followed by top-line data in 1H15.



Source: Company presentation

Other Potential Indications: Fragile X Syndrome

Fragile X Syndrome, or FXS, is a genetic condition that causes a range of intellectual and developmental disabilities as well as various physical characteristics. The studies conducted by the Centers for Disease Control and Prevention, show that FXS affects 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females and does not discriminate based on race or ethnicity. In the US, approximately 100,000 people are affected with the disease and one million individuals are at risk for developing a Fragile X associated disorder.



Source: Medicineworld.org

The Fragile X gene can be passed down through each generation with daughters born to male carriers inheriting their fathers' affected X chromosome to become carriers. Sons born to male carriers will not inherit their fathers' X chromosome and will not be affected. Approximately 1 in 151 women and 1 in 468 men carry the Fragile X gene. Patients with this specific genetic condition exhibit autism-like symptoms such as cognitive impairment, anxiety and mood swings, attention deficit and heightened stimuli. Seizures are also associated with the disease with approximately 7% of women and 18% men affected.

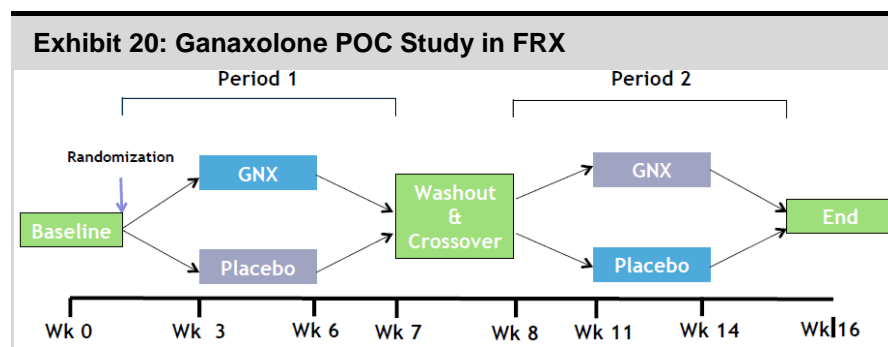
People with FXS are affected throughout their entire lives and currently, there are no known cures or approved therapies for FXS. Treatment approaches focus primarily on supportive care and medications addressing development delays, learning disabilities, and social and behavioral problems caused by the disease. A variety of medications are available to treat behavioral and mental health conditions associated with FXS. Depending upon the patients' symptoms, some have benefited from attention deficient disorder medications while others have been helped by various types of anti-anxiety medications. Available treatments that reduce the burden of the illness include special education and symptomatic treatments.

Why Ganaxolone?

Fragile X Syndrome (FXS) is a result of a mutation of the *fmr1* gene that codes for the FMRP protein. Ganaxolone has a high-affinity for GABA-A/ δ receptors which should increase signaling at existing receptors to normalize GABA-mediated inhibition. Studies in *fmr1* knock-out mouse model confirm that there is a deficit in the production of GABA_A receptors with a δ subunit, a deficit in GABA_A receptor-mediated inhibition in the neocortex and a down-regulation of GABA-ergic enzymes and protein in cerebellum and neocortex. The consequences of reduced GABA_A expression in Fragile X Syndrome are heightened sensitivity to sensory stimuli, anxiety, and seizures in up to 30% of patients. Through clinical trials, ganaxolone has been successfully assessed in changes in anxiety, attention and social withdrawal. The novel mechanism is working via established GABA_A pathways for anticonvulsant efficacy.

Clinical Progress:

Study 800 - Phase 2a Proof-of-Concept Study: The MIND Institute at the University of California, Davis has been awarded a medical research grant from the Department of Defense (DoD) to study ganaxolone for treatment of behaviors in FXS in children and adolescents. Together with Marinus, the MIND Institute at the University of California, Davis is conducting a randomized, placebo-controlled, 60-patient Phase 2 proof-of-concept clinical study (Exhibit 20). The subjects are titrated up to a maximum dose of 1,500mg/day of ganaxolone or placebo over two weeks followed by four weeks of treatment. At the end of the first treatment period and following a washout period, subjects are crossed over to the other



treatment for a similar two-week titration period followed by four weeks of treatment. The primary endpoint of the study is the Clinical Global Impression Rating Scale for Improvement. Secondary endpoints include the Aberrant Behaviors Checklist and ratings scales for specific behaviors associated with childhood FXS. The company anticipates completion of this study in 2H15.

Other Potential Indications: Post-Traumatic Stress Syndrome (PTSD): Posttraumatic Stress Disorder, or PTSD, is a high profile public health issue and poised to become a national crisis with

Exhibit 21: Current Treatment Options for PTSD		
Drug Name	Drug Specification	% of 2013 Sales
Sertraline	Antidepressant	11%
Escitalopram	Antidepressant	6%
Bupropion	Antidepressant	6%
Citalopram	Antidepressant	5%
Fluoxetine	Antidepressant	5%
Duloxetine	Antidepressant	4%
Mirtazapine	Antidepressant	3%
Trazodone	Antidepressant & Sleep Aid	5%
Quetiapine	Antipsychotic	7%
Clonazepam	Benzodiazepine	6%
Alprazolam	Benzodiazepine	4%
Prazosin	Sleep Aid	3%
All Others		35%

Source: Company presentation

approximately 3.6% of the US population affected and nearly 10-20% of veterans suffer from PTSD. Current medications (Exhibit 21) manage symptoms but few medications successfully induce remission. New and improved agents are needed to address the \$2B genericized US market.

Clinical Progress:

MRNS, in collaboration with the Inquiry and Traumatic Stress Consortium, or INTRuST, conducted a Phase 2 proof-of-concept, dose finding clinical trial of ganaxolone versus a placebo in 114 adults with PTSD. Eighty-six subjects completed the study. The subjects began the trial with preliminary screening and randomization phases, and then over the course of a 13-week study, were given a range of dosages of either ganaxolone or the placebo for the first six weeks. Beginning in the seventh week, all subjects received different dosages of ganaxolone. The group of subjects who received ganaxolone from Week 0, received the drug in intervals of 400mg/day every two weeks. The maximum dosage of drug for both groups was 1200mg/day. Once the subject received the maximum dosage, they were observed for a downward titration. In the placebo group, all subjects received placebo for the first six weeks of the trial. Beginning in the seventh week, the placebo group was treated with ganaxolone in intervals of 400mg/day every two weeks. By the twelfth week, they received the maximum of 1200mg/day and were observed for a downward titration. The last subject visit was on January 9, 2014 and the database was locked in March 2014. Analysis remains ongoing.

Intellectual Property:

Currently, MRNS holds seven issued US patents that cover the use of ganaxolone nanoparticles in oral

Exhibit 22: Intellectual Property			
Patent Number	Content	Expires	Product
8,618,087	Solid ganaxolone formulations and methods for the making and use thereof	2026	Ganaxolone
8,455,002	Stable corticosteroid nanoparticulate formulations and methods for the making and use thereof	2026	Corticosteroid Nanoparticulate
8,367,651	Solid ganaxolone formulations and methods for the making and use thereof	2026	Ganaxolone
8,362,286	Method for making 3.alpha.-hydroxy, 3.beta.- substituted-5.alpha.-pregnan-20-ones	2030	Ganaxolone
8,318,714	Liquid ganaxolone formulations and methods for the making and use thereof	2026	Ganaxolone
8,022,054	Liquid ganaxolone formulations and methods for the making and use thereof	2026	Ganaxolone
7,858,609	Solid ganaxolone formulations and methods for the making and use thereof	2026	Ganaxolone

Source: US Patent Office, Janney estimates

solid and liquid dose formulations, the last of which expires in 2030 (Exhibit 22). All the intellectual property behind the MRNS current product line is wholly-owned by MRNS.

Collaborations:

NovaMedica

In December 2012, MRNS entered into a Technology Transfer Agreement, or the Transfer Agreement, and the company was transferred to NovaMedica, LLC. or NovaMedica. To ensure the assignment of DRI's rights under the Transfer Agreement to NovaMedica, they agreed to take all the actions necessary that are required to record the patent transfers to DRI in each country in the Covered Territory. DRI and Rusnano Medinvest LLC, or Rusnano Medinvest jointly own NovaMedica. A wholly-owned subsidiary of Rusnano Medinvest, RMI Investments, is a significant stockholder.

Under the Transfer Agreement, NovaMedica is permitted to manufacture, purchase supplies and conduct clinical trials of a Covered Product, in the Covered Territory as long as it does not interfere with development and commercialization outside the Covered Territory. The purchases are made on a cost-plus basis and must enter into the Supply Agreement in the Covered Territory within 60 days from NovaMedica's request.

Under the Clinical Development and Collaboration Agreement, or the Collaboration Agreement, that was entered by MRNS with NovaMedica, on June 25, 2013, both parties agreed that NovaMedica would assist in the development and commercialization of Covered Products in the Covered Territory in the Field. The agreement is monitored by a committee of representatives from both parties to oversee the development and commercialization, where all decisions are made unanimously. This committee will have complete responsibility for creating a development plan for ganaxolone in clinical trials, giving NovaMedica the right to conduct its own clinical studies in the Covered Territory. NovaMedica also has the right to file applications for approval of Covered Products in the Covered Territory and has the capability to request development support, data and regulatory files and consultation from MRNS that are necessary to conduct its clinical trials.

NovaMedica is required to reimburse MRNS for any out-of-pocket expenses, except for expenses incurred in MRNS' participation on the joint committee. Proceeding the Transfer and Collaboration Agreements, NovaMedica has agreed to use commercially reasonable efforts to include sites in the Russian Federation in clinical trial programs. Under the Transfer Agreement, at least 36 months prior to the first commercial sale of a product candidate in the Covered Territory, both parties have agreed to negotiate on a supply agreement after a third party contract manufacturer has authorized them to manufacture and supply the Covered Products in the Covered Territory. The purchases are made on a cost-plus basis and if the parties disagree on an acceptable supply agreement then an unbiased consulting firm will perform an analysis to determine the final pricing. The Collaboration Agreement expires three years after the first commercial sale of a product candidate in the Covered Territory and ends upon the termination of the Transfer Agreement. NovaMedica is able to terminate the Collaboration Agreement at its convenience, with 90 days prior notice.

Purdue

MRNS entered into a licensed agreement with Purdue in September 2004, which was restated in May 2008, giving MRNS exclusive rights to certain information and technology relating to ganaxolone. The agreement gives MRNS the right to sublicense Purdue and to date have paid an aggregate of \$200,000 in license fees under this agreement. This payment has permitted MRNS to issue 1,189,812 shares of Series A convertible preferred stock and 630,318 shares of common stock, as well as, pay Purdue certain royalties. The obligation to pay these royalties expires 10 years after the first commercial sale of a licensed product. Since the underlying patents are not applicable to ganaxolone or have expired, MRNS is not expecting to pay royalties. MRNS is also expected to pay Purdue a percentage of mid-single digits of the non-royalty consideration received, 20-29% of milestone payments for indications not associated with

mood disorders. Under the agreement, they are committed to use commercially reasonable efforts to market at least one license product. If either party becomes incompetent, the other party has the right to terminate their side of the agreement. Termination on behalf of either party will not affect accrued rights, but other rights and licenses will be terminated. Although, MRNS has the right to terminate the agreement upon 180 days written notice to Purdue, and all rights and information will revert to Purdue at no cost or obligations.

Risks to Fair Value Estimate:

Development risk – Preclinical and even early clinical data are not necessarily predictive of success in pivotal studies.

Regulatory risk – The company is dependent on getting ganaxolone to market and thus, its success is dependent upon regulatory approval. There is a risk that the drug does not get approved.

Manufacturing risk – Currently, the company is dependent on a single source manufacturer and raw material supplier. Their commercial success is dependent on the successful scale-up and validation of the new equipment and FDA approval.

Commercial risk – A large portion of the current market is made of generics. Generic entrants may make marketing difficult, requiring increased marketing expenses. In addition, the drug may not gain market acceptance from physicians.

Competitive risk – There are numerous approved AEDs, including generic products that are favored by insurers and third-party payors. Recent market entrants include branded products which have become widely accepted and established therapies, making it potentially challenging for MRNS to gain market share. Additionally, there are several drugs in development for the treatment of Fragile X Syndrome that could compete with ganaxolone.

Management:

Christopher M. Cashman - Chairman, President & CEO: Mr. Cashman is President and Chief Executive Officer of Marinus Pharmaceuticals, Inc. since 2012 and a member of the Company's Board of Directors since 2011. Prior to joining Marinus Pharmaceuticals, Inc., Mr. Cashman provided consulting services to Quaker Partners, a private venture capital fund from 2010-2011. Prior to his time with Quaker Partners, starting in 2003, Mr. Cashman served as President and Chief Executive Officer of Protez Pharmaceuticals, Inc., also a private company. Prior to his time with Protez, Mr. Cashman served as President and Chief Executive Officer of Message Pharmaceuticals Inc., and as Vice President for both Pfizer, Inc. (NASDAQ: PFE) and SmithKline Beecham PLC. Mr. Cashman began his pharmaceutical career at SmithKline Corp. Currently he serves on the board of directors of Rapid Micro Biosystems, Inc., Noble Biomaterials, Inc. and MBF Therapeutics Inc. He holds an M.S. in Economics from Purdue University and B.S. in Business Management from the University of Minnesota.

Edward F. Smith - Vice President & Chief Financial Officer: Mr. Smith has served as the Company's Vice President, Chief Financial Officer, Secretary and Treasurer since 2013. Prior to joining Marinus Pharmaceuticals Inc., Mr. Smith served as an advisor to Tetralogic Pharmaceuticals Corp. (NASDAQ: TLOG) in its initial public offering in 2013. From January 2006 to April 2013, Mr. Smith served as Chief Financial Advisor of PolyMedix, Inc. (NASDAQ: PYMXQ), after previously serving as an Executive Director of Finance at InKine Pharmaceutical Company, Inc. Mr. Smith began his career holding various positions in public accounting, most recently as a manager in the audit practice at Deloitte & Touche LLP. Mr. Smith is licensed as a Certified Public Accountant in Pennsylvania, holding a B.S. in Business Administration from the University of Hartford.

Gail M. Farfel Ph.D. – Chief Clinical and Regulatory Officer: Dr. Farfel has served as the Company's Chief Clinical Development and Regulatory office since January 2010. Prior to joining Marinus Pharmaceuticals Inc. she founded and served as President of G. Meredith Consulting in 2008. From March 2006 to April 2008, Dr. Farfel served as the U.S. Vice President of Therapeutic Area and Head for Neuroscience Clinical Development and Medical Affairs at Novartis Pharmaceuticals Corp. (NASDAQ: NVS). From November 1996 to February 2006, Dr. Farfel held several leadership positions in Clinical Development and Global Medical Affairs at Pfizer, Inc. (NASDAQ: PFE). At Pfizer, Inc. she directed programs that focused on all stages of clinical development and regulatory submissions. This includes the development of the first product that was approved globally to treat PTSD. Dr. Farfel currently serves on the Board of Directors of PrintedArt and is the author of over 50 scientific articles in the areas of neuropsychopharmacology and drug treatment. She holds a Ph.D. in Neuropsychopharmacology from the University of Chicago, where she trained as a behavioral scientist and neurotoxicologist, and received the Ginsburg Prize for Dissertation Excellence. Dr. Farfel has a Bachelor's degree in Biochemistry from the University of Virginia.

Virinder Nohira, M.D., Ph.D. – Chief Medical Advisor: Dr. Nohira serves as the Company's Chief Medical Advisor and is also an experienced biotechnology entrepreneur and drug developer with great success in the pharmaceutical and biotechnology sector. Alongside his work with Marinus Pharmaceuticals Inc., Dr. Nohira co-founded and serves as the President of Vidara Therapeutics, a private biopharmaceutical company. Prior to joining Marinus, Dr. Nohira was Chief Medical Officer, Chief Compliance Officer and Executive Vice President of Alaven Pharmaceutical and Alaven Consumer Health LLC. In the United States, Dr. Nohira worked for Eli Lilly on Zyprexa and at UCB, serving as the clinical lead for submission and commercialization of Keppra in the States. From 2003-2005, he was Vice President and Chief Medical Officer of Xcel Pharmaceuticals where he led development of retigabine (ezogabine) for the treatment of epilepsy. Dr. Nohira is on the Board of Directors of Promentis Pharmaceuticals Inc., and is a board-certified neurologist with special qualification in child neurology. He conducted his medical training in England at the University of Cambridge and completed his postgraduate degree at Duke University. He also holds a Ph.D. in Neuropharmacology and has authored many publications and book chapters.

Julia Tsai, Ph.D.- Senior Director of Clinical Development & Project Management: Dr. Tsai has served as the Company's Senior Director of Clinical Development & Project Management since November 2012. Before her current position she served as the Company's Director of Drug Development from September 2006 to November 2012 and served as the Company's Manager of Drug Development from 2004 to 2006. Dr. Tsai's direct responsibilities at Marinus Pharmaceuticals Inc. include all facets of the drug development operations. She is also an author of several scientific articles and abstracts in the area of neuroscience and neurology. Dr. Tsai holds a Ph.D. in Neuroscience and Physiology from the Sackler Institute of Graduate Biomedical Sciences, New York University School of Medicine and a B.A. from Cornell University.

Marinus Pharmaceuticals, Inc. (NASDAQ: MRNS)
Income Statement
(In thousands, except per share data)

	2012 A	For the Quarter Ending				2013 A	For the Quarter Ending				2014 E	2015 E
		Q1:13 A	Q2:13 A	Q3:13 A	Q4:13 A		Q1:14 A	Q2:14 E	Q3:14 E	Q4:14 E		
Revenue:												
Collaboration / Milestones	100	-	-	-	-	-	-	-	-	-	-	-
Product Revenue	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue	100	-	-	-	-	-	-	-	-	-	-	-
Cost of Product Sales	-	-	-	-	-	-	-	-	-	-	-	-
Gross Profit	100	-	-	-	-	-	-	-	-	-	-	-
Cost and Expenses:												
Research and Development	846	747	-	-	-	4,150	2,149	4,696	4,696	4,696	16,237	24,661
Sales & Marketing	-	-	-	-	-	-	-	-	-	-	-	-
General and Administrative	685	180	-	-	-	1,229	517	734	734	734	2,718	3,249
Total Costs and Expenses	1,531	927	-	-	-	5,379	2,665	5,430	5,430	5,430	18,956	27,910
Operating Income (Loss)	(1,431)	(927)	-	-	-	(5,379)	(2,665)	(5,430)	(5,430)	(5,430)	(18,956)	(27,910)
Other Income (Expense):												
Interest Income	(318)	(42)	-	-	-	(72)	4	5	16	26	51	59
Change in Fair Value of Warrants	336	-	-	-	-	153	428	-	-	-	428	-
Other	4	0	-	-	-	28	(2)	-	-	-	(2)	-
Loss Before Income Taxes	(1,409)	(970)	-	-	-	(5,270)	(2,235)	(5,425)	(5,415)	(5,404)	(18,480)	(27,851)
Income Taxes	-	-	-	-	-	-	-	-	-	-	-	-
Net Income	\$ (1,409)	\$ (970)	\$ -	\$ -	\$ -	\$ (5,270)	\$ (2,235)	\$ (5,425)	\$ (5,415)	\$ (5,404)	\$ (18,480)	\$ (27,851)
Basic Earnings Per Share	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	\$ (0.71)	\$ (0.28)	\$ (0.67)	\$ (0.39)	\$ (0.39)	\$ (1.68)	\$ (1.95)
Diluted Earnings Per Share	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	#DIV/0!	\$ (0.71)	\$ (0.28)	\$ (0.67)	\$ (0.39)	\$ (0.39)	\$ (1.68)	\$ (1.95)
Basic Shares Outstanding	-	-	-	-	-	7,409	8,063	8,154	13,779	13,929	10,981	14,304
Diluted Shares Outstanding	-	-	-	-	-	7,409	8,063	8,154	13,779	13,929	10,981	14,304
Effective Tax Rate	0.0%	0.0%	#DIV/0!	#DIV/0!	#DIV/0!	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%	0.0%
EBITDA Calculation:												
Loss Before Income Taxes	\$ (1,409)	\$ (970)	\$ -	\$ -	\$ -	\$ (5,270)	\$ (2,235)	\$ (5,425)	\$ (5,415)	\$ (5,404)	\$ (18,480)	\$ (27,851)
Less: Interest Income	318	42	-	-	-	72	(4)	(5)	(16)	(26)	(51)	(59)
Plus: Depreciation	-	-	-	-	-	-	-	2	2	2	7	65
EBITDA	\$ (1,090)	\$ (927)	\$ -	\$ -	\$ -	\$ (5,198)	\$ (2,240)	\$ (5,428)	\$ (5,428)	\$ (5,428)	\$ (18,523)	\$ (27,845)
Margins:												
Gross	100.0%	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M
Operating	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M
Net Income (Loss)	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M
EBITDA	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M	N/M
Growth Rates:												
Total Revenue						-100.0%	N/M	N/M	N/M	N/M	N/M	N/M
Operating Income						N/M	N/M	N/M	N/M	N/M	N/M	N/M
Net Income (Loss)						N/M	N/M	N/M	N/M	N/M	N/M	N/M
Research and Development Expense						390.8%	187.5%	N/M	N/M	N/M	291.3%	51.9%
Selling, General and Administrative Expense						79.3%	186.8%	N/M	N/M	N/M	121.2%	19.5%

Source - Company reports and Janney Montgomery Scott LLC estimates

IMPORTANT DISCLOSURES

Research Analyst Certification

I, Kimberly Lee, 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.

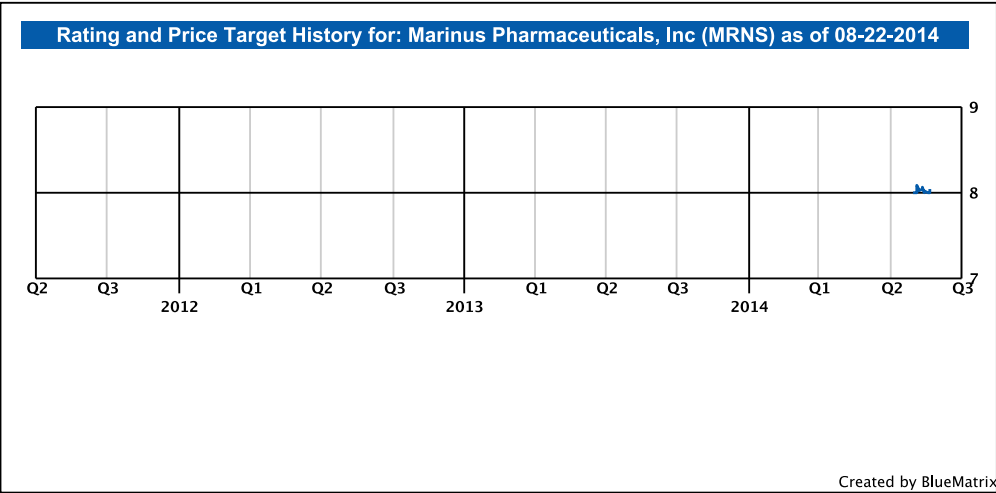
Definition of Ratings

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 6/30/14

Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [B]	207	53.80	53	25.60
NEUTRAL [N]	176	45.70	28	15.90
SELL [S]	2	0.50	0	0.00

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

Other Disclosures

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Investment opinions are based on each stock's 6-12 month return potential. Our ratings are not based on formal price targets, however, our analysts will discuss fair value and/or target price ranges in research reports. Decisions to buy or sell a stock should be based on the investor's investment objectives and risk tolerance and should not rely solely on the rating. Investors should read carefully the entire research report, which provides a more complete discussion of the analyst's views. Supporting information related to the recommendation, if any, made in the research report is available upon request.

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