

Specialty Pharmaceuticals

Price:	\$11.95
Fair Value Estimate:	\$20.00
52-Week Range:	\$5.49 - \$16.60
Market Cap (MM):	\$170
Shr.O/S-Diluted (mm):	14.2
Average Daily Volume:	82,599
Yield:	0.0%
Cash/Share:	\$(1.40)
FCF Yield:	NA
Debt/Cap:	15%

FYE: Dec	2014A	2015E
EPS:	\$(1.76)A	\$(1.89)E
Prior EPS:		NC
Consensus	NM	-1.96
P/E Ratio:	NA	NA

Quarterly EPS:

Q1	\$(4.79)A	\$(0.50)A
Q2	\$(7.98)A	\$(0.47)E
Q3	\$(0.22)A	\$(0.47)E
Q4	\$(0.33)A	\$(0.46)E



June 24, 2015

Marinus Pharmaceuticals, Inc (MRNS) - BUY

Highlighting MRNS POC Phase 2 Trial in Fragile X on Heels of Alcobra Fail

PORTFOLIO MANAGER BRIEF

As Alcobra (ADHD, not covered) announced this morning the miss on its primary end point on its Fragile X Proof-of-Concept Phase 2, we want to highlight the MRNS POC Phase 2 in Fragile X, in particular as these two trials could not be more different in terms of design, patients, molecule, dosing, and efficacy endpoints. In fact, a younger patient population could benefit MRNS. Fragile X, as an indication has proven difficult, however we believe that the MRNS trial will result in clinically meaningful data, offering upside to our \$20 FV.

ANALYST NOTES

- Quick Review of Fragile X: Fragile X Syndrome (FXS) is a genetic condition that causes a range of intellectual and developmental disabilities as well as various physical characteristics. FXS is the leading known genetic cause of autism. Recent estimates indicate that approximately 50,000 Americans are affected by FXS, which makes the indication orphan and with no currently approved FDA treatments, there is an obvious unmet need.
- ADHD Trial vs. MRNS Trial: With the failed POC Phase 2 announced by ADHD, we decided to revisit the MRNS POC Phase 2. These trials could not be more different with unfounded potential results comparisons. The molecules are different, the trial designs are different, different patient populations, primary and secondary endpoints. The ADHD trial was randomized, double-blind placebo controlled multi-center trial evaluating 62 adolescent to adult patients with FXS. The trial involved 4 weeks of dose optimization followed by two weeks of maintenance. The MRNS trial is a randomized, double-blind placebo controlled cross-over (all 60 patients on drug vs just 30 in ADHD) single center study evaluating ~60 children (6-17 yrs old) with FXS. The trial involves two weeks of titration follow by 4 weeks of treatment. We have included a comparison chart with this report, Exhibit 1.
- Primary and Secondary Endpoints Differ in Measurement: Another key differentiating factor are the endpoints. The ADHD trial endpoint of ADHD rating scales focused more on, well the attention deficit hyperactivity disorder component. The MRNS endpoint of Clinical Global Impression Improvement (CGI-I), though more general in in looking at issues such as anxiety, attention, social behavior and inhibitory control, it measures overall severity of illness and changes over time. MRNS not only includes the KITAP (which ADHD saw stat.sig) but also several other more specific secondary endpoints.

- Scientific Rationale Behind Ganaxolone: FXS is a result of a mutation of the FMR1 gene that codes for the FMRP protein. Preclinical study in FMR1 knock-out models have shown that there is reduced expression in GABAA receptors. This reduced expression heightens sensitivity to sensory stimuli, anxiety and seizures. Ganaxolone, MRNS's lead product candidate has a high-affinity for these GABAA receptors which, in theory should help increase signaling resulting in normalization.
- FXS is All Upside: We are fully aware of the inherent difficulties of CNS trials, and in particular of FXS. Currently, we do not have any value assigned to Fragile X in our \$20/share value. The \$20/share value is based solely on US and EU market potential of Ganaxolone as an adjunctive therapy for focal onset epilepsy. Success in the POC Phase 2 would add value to our fair value target.

Alcobra vs. Marinus
Fragile X Syndrome – Proof of Concept Phase 2

Alcobra

Marinus

<p>Purpose: This study is a multisite, randomized, double-blind, placebo-controlled, phase 2 study of MG01CI (low dose and high dose once daily) for 6 weeks compared with placebo in a 1:1 ratio of 60 adolescent and adult subjects with Fragile X Syndrome (FXS).</p>	<p>This Phase 2 proof-of-concept study is a double-blind, randomized, placebo-controlled, crossover study to investigate ganaxolone treatment in children with fragile x syndrome (FXS). Up to 60 subjects (ages 6-17 yrs) will be randomized to receive either ganaxolone or placebo for 6 weeks and then cross over to the opposite treatment for another 6 weeks.</p>
<p>Demographic: Age- 15-55 years of age Gender-Both Accepts Healthy Volunteers-No</p> <ul style="list-style-type: none"> • Subject has fragile X syndrome with a molecular genetics confirmation of the full FXS (FMR1) mutation • Man or non-pregnant woman aged 15-55 y/o, inclusive • Has a score of 12 or greater on the ADHS RS-IV scale • Current treatment with no more than 3 prescribed psychotropic medications • Epileptic medication are accepted for the disorder, but for any other treatment purposes = not accepted • Behavioral treatments must be stable for 4 weeks prior to screening • Males and Females must use effective forms of birth control if they're sexually active & screen negatively on urine samples • Attend a clinic regularly and reliably • Able to swallow tablets and capsules • Subject/guardian must be able to understand, read, and write English • Be able to sign consent to participate 	<p>Demographic: Age-6-17 years of age, inclusive Gender-Both Accepts Healthy Volunteers- No</p> <ul style="list-style-type: none"> • Molecular documentation of FMR1 Full Mutation • Sexually active participants must use a form of birth control
<p>Primary Outcome Measures: Evaluation of efficacy of MG01CI by Attention Deficit Hyperactivity Disorder rating scale (ADHD RS-IV) (6 week duration) *then compared against placebo recipients</p> <p>Primary Endpoints: Objective - To show a reduction in the symptom score on the ADHD RS-IV inattentive subscale.</p> <ol style="list-style-type: none"> 1. The difference between the MDX treated group and placebo was not statistically 	<p>Primary Outcome Measures: Clinician's Global Impression-Movement (CGI-I) Time Frame – Weeks 3,6,8,11,14</p>

significant (p=.21)			
2. Total ADHD RS score and CGI were similarly non significant			
<p>Secondary Outcome Measures:</p> <p>Evaluation of efficacy of MG01C as measured by total score on the ADHD-RS-IV (6 week duration)</p> <p>*compared against placebo recipients</p> <p>Secondary Endpoints:</p> <p>Two domains of the Vineland Adaptive Behavior Scale (VABS), the Aberrant Behavior Checklist (ABC), the Test of Attention Performance for Children (KiTAP), and the Repeatable Battery for the Assessment of Neuropsychological Status List Learning test (RBANS-LL) were used as measures</p> <ol style="list-style-type: none">MDX showed a statistically significant benefit over placebo, in the ITT population, on the VABS Daily Living Skills Domain (p=0.044) and on the Computerized KiTAP distractibility test (p=0.017).Findings on both the VABS and KiTAP showed meaningful clinical effect sizes of 0.56 and 0.63.The remaining secondary measures did not yield significant findings in the ITT population.		<p>Secondary Outcome Measures:</p> <p>Pediatric Anxiety Rating Scale (PARS) (Week 3,6,8,11,14)</p> <p>Visual Analog Scale (Week 6,8,14)</p> <p>Anxiety, Depression, and the Mood Scale (ADAMS) (Week 6,8,14)</p> <p>Aberrant Behavior Checklist (Weeks 6,8,14)</p> <p>Swanson, Nolan, and Pelham-IV Questionnaire (SNAP-IV) (Week 6,8,14)</p> <p>Other Outcomes:</p> <p>KiTAP – Test of Attentional Performance for Children (Baseline, Weeks 6, 8, 14)</p> <p>Prepulse Inhibition (PPI) (Baseline, Weeks 6, 8, 14)</p> <p>Social Gaze (eye tracking) (Baseline, Weeks 6, 8, 14)</p> <p>Event-related brain potentials (ERP) (Baseline, Weeks 6, 8, 14)</p>	
<p>Enrollement:</p> <p>60</p>		<p>Estimated Enrollment:</p> <p>60</p>	
<p>Groups</p>	<p>Assigned Interventions</p>	<p>Groups</p>	<p>Assigned Interventions</p>
<p>Experimental:</p> <p>Metadoxine</p> <p>Immediate/Slow-release</p> <p>low dose or high dose administered orally once daily</p>	<p>Drug: MG01CI</p> <p>extended-release tablet</p> <p>Other Name:</p> <p>Metadoxine (pyridoxol</p> <p>L-2-pyrrolidone-5-carboxylate)</p>	<p>Experimental: Ganaxolone</p> <p>3 mg/kg up to 12 mg/kg, with maximum of 1500 mg/day</p>	<p>Drug: Ganaxolone</p> <p>oral suspension, given in 3 divided doses</p> <p>Other Names:</p> <p>GNX</p> <p>GNX OS</p>
<p>Placebo Comparator:</p> <p>Placebo</p> <p>Placebo tablet identical</p>		<p>Placebo Comparator:</p> <p>Placebo</p> <p>non active</p>	<p>Drug: Placebo</p> <p>oral suspension, given in 3 divided doses</p> <p>Other Name: PBO</p>

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.

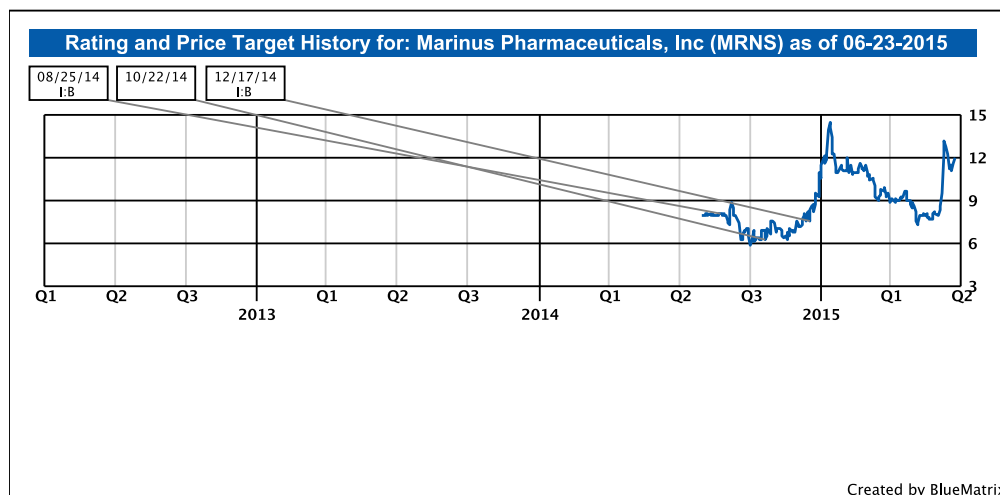
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BUY: Janney expects that the subject company will appreciate in value. Additionally, we expect that the subject company will outperform comparable companies within its sector.

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.

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Price Charts



Janney Montgomery Scott Ratings Distribution as of 3/31/15

Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [B]	140	50.36	21	15.00
NEUTRAL [N]	137	49.28	14	10.22
SELL [S]	1	0.36	0	0.00

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

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