

September 9, 2014

HEALTHCARE/SPECIALTY AND GENERIC PHARMACEUTICALS

Stock Rating:

**OUTPERFORM**

12-18 mo. Price Target \$12.00  
MRNS - NASDAQ \$8.92

3-5 Yr. EPS Gr. Rate NM  
52-Wk Range \$10.58-\$7.04  
Shares Outstanding 13.9M  
Float 5.4M  
Market Capitalization \$124.0M  
Avg. Daily Trading Volume NA  
Dividend/Div Yield \$0.00/0.00%  
Book Value \$1.06  
Fiscal Year Ends Dec  
2014E ROE NM  
LT Debt \$2.0M  
Preferred \$0.0M  
Common Equity \$9M  
Convertible Available No  
*Trading range since July 2014 IPO.*

| EPS Diluted | Q1     | Q2     | Q3     | Q4     | Year   | Mult. |
|-------------|--------|--------|--------|--------|--------|-------|
| 2014E       | (0.28) | (0.14) | (0.52) | (0.45) | (1.50) | NM    |
| 2015E       | (0.53) | (0.44) | (0.41) | (0.34) | (1.69) | NM    |
| 2016E       | --     | --     | --     | --     | (1.02) | NM    |
| 2017E       | --     | --     | --     | --     | (0.92) | NM    |

## Marinus Pharmaceuticals

**Novel Antiepileptic in a Crowded But Large Market; Initiate with Outperform**

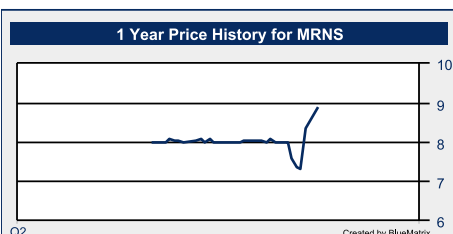
### SUMMARY

We are initiating coverage of Marinus Pharmaceuticals with an Outperform rating and \$12 price target. Marinus IPO'd at the end of July at an offering price of \$8.00 per share. Since then, the shares are up nearly 12% (vs. the S&P 500 +4%). The company's lead product, ganaxolone, is an antiepileptic drug (AED) with a novel mechanism of action (MOA) that mimics an endogenous neurosteroid, allopregnanolone. With ganaxolone, Marinus offers a product that has the same therapeutic benefit as the endogenous product through a natural mechanism with the potential for a lower side effect profile. We believe that ganaxolone, once approved, will penetrate the epilepsy market as adjunctive treatment to existing therapy.

### KEY POINTS

- **Epilepsy Market:** The epilepsy market is large and crowded, with IMS Health data indicating global sales of AEDs at approximately \$14B in 2011. There are more than 15 approved AEDs available in the US and worldwide. Currently available medications, however, create a number of side effects, which is where ganaxolone may be differentiated.
- **Consultant Feedback:** We spoke to a neurologist consultant and an expert familiar with a competitive launch for the drug Keppra. Both indicated that drug-drug interactions/side-effect profile were top-of-mind considerations when considering AEDs, as many patients tend to be on polypharmacy. With ganaxolone mimicking a natural mechanism, its favorable side effect profile/limited interactions should prove advantageous.
- **Model Forecasts:** Epilepsy prescriptions grew 9% year/year in 2013 and 1H14, according to Symphony Health data. We conservatively estimate 5% y/y growth for FY 2014 and 3% growth in the out years. We do not anticipate revenues for Marinus until 2020 and forecast ~\$32M in ganaxolone sales for 2020, moving to ~\$625M by 2024.
- **Valuation:** We believe fair value for MRNS is \$12, which represents ~35% of potential upside from current levels. Our valuation is based on a DCF methodology. Using a discount rate of ~25%, and an exit EBITDA multiple of 4.0x the 2025E terminal year EBITDA of ~\$500 million, we arrive at our valuation.

### Stock Price Performance



### Company Description

Marinus is a clinical stage biopharmaceutical company focused on developing and commercializing neuropsychiatric therapeutics.

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## Investment Thesis

We are initiating coverage of Marinus Pharmaceuticals with an Outperform rating and 12- to 18-month \$12 price target. Marinus is a clinical stage biopharmaceutical company focused on developing and commercializing neuropsychiatric therapeutics. The company's clinical stage product, ganaxolone, is a small molecule, add-on therapy for the treatment of partial onset seizures in adults with epilepsy.

Ganaxolone has a novel mechanism of action that mimics an endogenous neurosteroid, allopregnanolone, produced in the central nervous system. It is a synthetic analog of allopregnanolone designed to have the same effects, but that is longer acting than the naturally occurring compound. With ganaxolone, Marinus offers a product that has the same therapeutic benefit as the endogenous product through a natural mechanism. However, ganaxolone may potentially offer lower side effects compared to other marketed antiepileptic medications.

Our investment thesis for Marinus is threefold: (1) ganaxolone will show positive safety and efficacy results in Phase 2b and potential Phase 3 studies, (2) the company will gain FDA acceptance for ganaxolone in the epilepsy indication some time in the late 2019 or early 2020 time frame, and (3) ganaxolone will penetrate the epilepsy market as adjunctive treatment to existing therapy.

Marinus is also developing ganaxolone as a treatment for behaviors associated with Fragile X Syndrome, or FXS, and for treatment of PCDH19 female pediatric epilepsy. Both are potential orphan indications with approval estimated in the 2017 or 2018 timeframe. The company would likely seek higher pricing for ganaxolone in FXS and PCDH19 than with the epilepsy indication, then bring pricing back down once the epilepsy indication is approved. However, we focus only on the epilepsy indication and assign no value to ganaxolone's other indications, leaving any additional therapeutic treatments or label expansion as upside.

## Valuation

We believe fair value for Marinus' shares is \$12, which represents ~35% of potential upside from current levels. Our valuation is based on a discounted cash flow methodology. Using a discount rate of approximately ~25%, and an exit EBITDA multiple of 4.0x on the 2025 terminal year EBITDA of close to \$500 million, we arrive at a \$12 per share valuation. The 25% discount rate, we believe, adequately reflects the clinical development risk that yet remains with ganaxolone along with the execution risk for commercialization.

**Exhibit 1. Marinus DCF Valuation Matrix**

|               |       | Exit EBITDA Multiple |          |          |          |          |
|---------------|-------|----------------------|----------|----------|----------|----------|
|               |       | 2.0x                 | 3.0x     | 4.0x     | 5.0x     | 6.0x     |
| Discount Rate | 22.0% | \$ 8.27              | \$ 12.28 | \$ 16.29 | \$ 20.30 | \$ 24.30 |
|               | 24.0% | \$ 6.27              | \$ 9.62  | \$ 12.97 | \$ 16.33 | \$ 19.68 |
|               | 26.0% | \$ 4.66              | \$ 7.47  | \$ 10.28 | \$ 13.09 | \$ 15.90 |
|               | 28.0% | \$ 3.35              | \$ 5.72  | \$ 8.08  | \$ 10.45 | \$ 12.81 |
|               | 30.0% | \$ 2.30              | \$ 4.30  | \$ 6.29  | \$ 8.28  | \$ 10.28 |

Source: Company reports, Symphony Health Solutions, and Oppenheimer & Co. estimates

As of December 31, 2013, Marinus had Federal and state net operating loss carryforwards of approximately \$59.0 million that will begin expiring in 2023. As part of our valuation, we estimate that the company will not pay taxes until 2023 with a full normalized rate beginning in 2024. We estimate pricing on ganaxolone at roughly \$250 per prescription at the launch in 2020. Last, with regards to IP, we note that Marinus has formulation patents and synthesis process patents on ganaxolone, the earliest of which will expire in 2026 and 2030, respectively.

Marinus went public on July 31, 2014. The shares closed flat at \$8.00 on the IPO date but are up almost 12% since then (versus the S&P 500 up 4%). We believe that Marinus represents attractive value given the large size of the epilepsy market and ganaxolone's potential favorable side effect profile. We do not anticipate revenues for Marinus until 2020, with the company potentially achieving profitability by 2022. Our CY14 and CY15 EPS estimates are (\$1.50) and (\$1.69), respectively, versus consensus of (\$2.53) and (\$2.12). However, we note that there are only three other contributing firms in consensus.

## Key Risks

Key risks to Marinus' outperformance and price target include the following: clinical development risk and either a failure by ganaxolone to meet clinical trial endpoints or show efficacy, or an inability by Marinus to gain approval from the FDA for ganaxolone; a failure to successfully introduce ganaxolone into the market or a failure to penetrate the highly competitive epilepsy market; an inability to gain formulary acceptance or to receive unfavorable payor reimbursement; a failure to develop a commercial organization capable of manufacturing, selling, marketing and distributing any products that the company intends to sell in the markets in which it chooses to commercialize on its own; the performance of third-party CROs and CMOs; forecasting error in determining Marinus' market opportunity due to the company's lack of having yet established a track record of profitability; limited intellectual property (IP) to protect and control trade secrets and other technological innovation; the ability of competitors to independently develop similar or better proprietary information and techniques and disclose them publicly; the success of competing therapies and products that are or become available; Medicare, Medicaid, and insurer increased efforts to manage drug spend; economic weakness impacting unemployment, commercial insurance coverage, and utilization; a small float which could lead to illiquidity; and liquidity risk as more than 50% of the shares are tied up through insider holdings. Other risks include that the company may require substantial additional funding or may finance future cash needs primarily through public or private offerings, debt financings or strategic collaborations, which may dilute stockholdings in the company.

## Investment Summary

Epilepsy affects approximately 50 million people globally, and, according to 2012 Decision Resources statistics, over 5 million people are under treatment in the United States, Europe and Japan. It is estimated that approximately 3.8% of people will develop epilepsy during their lifetime, with a higher incidence in men than women.

New cases of epilepsy are most common among children, especially during the first year of life. The rate of new cases gradually declines until roughly age 10 and then becomes more stable. Remission is common for children as they age. After age 55 to 60, the rate of new epilepsy cases starts to increase, as the elderly are more likely to develop strokes, brain tumors, or Alzheimer's disease.

IMS Health data indicate that global sales of antiepileptic drugs, or AEDs, were approximately \$14 billion in 2011. Existing AEDs attempt to control seizures through a variety of mechanisms and are effective in reducing seizure frequency in many patients. Specifically, there are more than 15 approved AEDs available in the United States and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Recent market entrants include branded products developed by UCB, GlaxoSmithKline, Eisai, and Sunovion Pharmaceuticals.

Many of these approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors tend to encourage the use of generic products. Currently available medications, however, create a number of side effects. These effects include mood and behavioral changes, increased cardiovascular risks, weight changes, potential reproductive toxicity, liver enzyme induction, kidney stones, sedation and adverse effects on cognitive function.

Newly diagnosed epilepsy patients are treated with daily administration of an AED. Approximately 60% of patients will achieve an adequate level of seizure control with a single AED, and the remainder will resort to using multiple drugs, or polypharmacy. Even with polypharmacy, approximately 30 to 35% of all patients do not reach an acceptable level of seizure control. For this subset of focal onset seizure patients that cannot gain acceptable seizure control through pharmacologic treatment options, they may resort to implantable devices, Vagus nerve stimulation (VNS), or surgery, which may include removal of the part of the brain causing the seizures.

Marinus estimates that the market opportunity for the refractory patient population affected by pharmacologic treatment options, which will be ganaxolone's initial target segment, will exceed \$4 billion in the United States, Europe and Japan. Ganaxolone may be an attractive treatment option for patients that require polypharmacy to control their seizures, due to its novel mechanism of action and its safety and tolerability profile. The company is targeting ganaxolone as a first-in-class therapy with the potential to provide treatment advantages for adults with focal onset seizures who do not achieve adequate seizure control from, have developed tolerance to, or have safety concerns with currently available medications.

The company's future success depends on the successful clinical development, regulatory approval, and commercialization of ganaxolone, which is currently undergoing a clinical trial in epilepsy. The commercial success will of course depend upon attaining significant market acceptance of ganaxolone among physicians, patients, government and private payors, and others in the medical community. Marinus anticipates readout of the Phase 2b ganaxolone trials in epilepsy in late 2015, with a potential Phase 3 study in 2H16, and approval in the late 2019 or early 2020 time frame.

Epilepsy prescriptions grew 9% year/year in 2013 and 1H14, according to Symphony Health data. We conservatively estimate 5% y/y growth for full year 2014, 2% y/y growth in 2015, and 3% growth in the out years. We further anticipate ganaxolone acquiring a roughly 0.1% market share in the first full year of launch in 2020, increasing to 1.8% share by 2025.

## Exhibit 2. Oppenheimer Estimates for Epilepsy Prescriptions and Market Share

| Total Prescriptions (000s) - US Epilepsy Market |        |        |        |        |         |        |        |        |        |         |         |         |         |         |         |         |         |         |         |         |
|---|--------|--------|--------|--------|---------|--------|--------|--------|--------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|
| Product   | 1Q14A  | 2Q14A  | 3Q14E  | 4Q14E  | 2014E   | 1Q15A  | 2Q15E  | 3Q15E  | 4Q15E  | 2015E   | 2016E   | 2017E   | 2018E   | 2019E   | 2020E   | 2021E   | 2022E   | 2023E   | 2024E   | 2025E   |
| Ganaxolone (Marinus)                            | 0      | 0      | 0      | 0      | 0       | 0      | 0      | 0      | 0      | 0       | 0       | 0       | 0       | 0       | 128     | 331     | 749     | 1,813   | 2,311   | 2,678   |
| Carbamazepine                                   | 1,150  | 1,164  | 1,130  | 1,132  | 4,576   | 1,159  | 1,161  | 1,164  | 1,165  | 4,660   | 4,790   | 4,833   | 5,081   | 5,234   | 5,391   | 5,553   | 5,719   | 5,751   | 5,923   | 5,952   |
| Phenytoin                                       | 258    | 254    | 215    | 216    | 943     | 221    | 221    | 222    | 222    | 886     | 912     | 940     | 968     | 997     | 1,579   | 1,428   | 1,430   | 1,192   | 1,186   | 1,339   |
| Valproic Acid                                   | 131    | 133    | 135    | 135    | 534     | 138    | 138    | 139    | 139    | 554     | 570     | 587     | 605     | 623     | 642     | 661     | 681     | 701     | 722     | 744     |
| Levetiracetam                                   | 2,024  | 2,092  | 2,019  | 2,021  | 8,155   | 2,070  | 2,074  | 2,079  | 2,081  | 8,304   | 8,553   | 8,810   | 9,074   | 9,346   | 9,588   | 9,876   | 9,941   | 10,239  | 10,402  | 10,714  |
| Lamotrigine                                     | 3,424  | 3,518  | 3,391  | 3,391  | 13,727  | 3,477  | 3,484  | 3,493  | 3,496  | 13,951  | 14,369  | 14,800  | 15,244  | 15,702  | 15,916  | 16,394  | 16,885  | 17,392  | 17,769  | 18,302  |
| Topiramate                                      | 3,202  | 3,357  | 3,230  | 3,233  | 13,022  | 3,312  | 3,318  | 3,327  | 3,330  | 13,287  | 13,685  | 14,096  | 14,519  | 14,954  | 15,403  | 15,865  | 16,341  | 16,831  | 17,336  | 17,707  |
| Oxcarbazepine                                   | 934    | 962    | 915    | 916    | 3,727   | 938    | 940    | 943    | 943    | 3,765   | 3,877   | 3,994   | 4,114   | 4,237   | 4,364   | 4,495   | 4,630   | 4,629   | 4,767   | 4,910   |
| Gabapentin                                      | 11,923 | 12,589 | 12,139 | 12,151 | 48,802  | 12,447 | 12,470 | 12,503 | 12,515 | 49,935  | 50,863  | 52,389  | 53,961  | 54,956  | 56,348  | 58,039  | 59,644  | 61,293  | 63,132  | 65,028  |
| Pregabalin                                      | 2,526  | 2,600  | 2,503  | 2,506  | 10,135  | 2,567  | 2,571  | 2,578  | 2,581  | 10,297  | 10,606  | 10,924  | 11,252  | 11,589  | 11,809  | 12,163  | 12,528  | 12,904  | 13,146  | 13,541  |
| Felbamate                                       | 21     | 21     | 27     | 27     | 97      | 28     | 28     | 28     | 28     | 111     | 114     | 117     | 121     | 125     | 128     | 132     | 136     | 140     | 144     | 149     |
| Zonisamide                                      | 359    | 367    | 350    | 350    | 1,426   | 359    | 359    | 360    | 360    | 1,439   | 1,483   | 1,527   | 1,573   | 1,620   | 1,669   | 1,719   | 1,770   | 1,823   | 1,878   | 1,786   |
| Lacosamide                                      | 190    | 201    | 188    | 189    | 769     | 193    | 194    | 194    | 194    | 775     | 798     | 822     | 847     | 872     | 898     | 925     | 953     | 982     | 1,011   | 1,042   |
| Ezogabine                                       | 3      | 3      | 27     | 27     | 60      | 28     | 28     | 28     | 28     | 111     | 114     | 117     | 121     | 125     | 128     | 132     | 136     | 140     | 144     | 149     |
| Phenobarbital                                   | 647    | 651    | 619    | 620    | 2,537   | 635    | 636    | 638    | 638    | 2,547   | 2,623   | 2,702   | 2,783   | 2,866   | 2,952   | 3,041   | 3,132   | 3,086   | 3,034   | 3,125   |
| Perampanel                                      | 2      | 4      | 27     | 27     | 60      | 28     | 28     | 28     | 28     | 111     | 114     | 117     | 121     | 125     | 128     | 132     | 136     | 140     | 144     | 149     |
| Other   | 0      | 0      | 0      | 0      | 0       | 0      | 0      | 0      | 0      | 0       | 570     | 587     | 605     | 1,246   | 1,284   | 1,322   | 1,362   | 1,403   | 1,445   | 1,488   |
| Total Epilepsy Market                           | 26,794 | 27,916 | 26,915 | 26,942 | 108,567 | 27,598 | 27,650 | 27,722 | 27,750 | 110,721 | 114,043 | 117,464 | 120,988 | 124,618 | 128,356 | 132,207 | 136,173 | 140,258 | 144,466 | 148,800 |
| % Growth Y/Y                                    | 8.9%   | 9.2%   | 2.0%   | 0.5%   | 5.0%    | 3.0%   | -1.0%  | 3.0%   | 3.0%   | 2.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    | 3.0%    |

| Market Share as a Percent of Total Prescriptions - US Epilepsy Market |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |        |
|---|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|
| Product   | 1Q14A  | 2Q14A  | 3Q14E  | 4Q14E  | 2014E  | 1Q15A  | 2Q15E  | 3Q15E  | 4Q15E  | 2015E  | 2016E  | 2017E  | 2018E  | 2019E  | 2020E  | 2021E  | 2022E  | 2023E  | 2024E  | 2025E  |
| Ganaxolone (Marinus)  | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.1%   | 0.3%   | 0.6%   | 1.2%   | 1.6%   | 1.8%   |
| Carbamazepine   | 4.3%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.2%   | 4.1%   | 4.1%   | 4.1%   | 4.0%   |
| Phenytoin   | 1.0%   | 0.9%   | 0.8%   | 0.8%   | 0.9%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 0.8%   | 1.2%   | 1.1%   | 1.1%   | 0.9%   | 0.8%   | 0.9%   |
| Valproic Acid   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   | 0.5%   |
| Levetiracetam   | 7.6%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.5%   | 7.3%   | 7.3%   | 7.2%   | 7.2%   |
| Lamotrigine   | 12.8%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.6%  | 12.4%  | 12.4%  | 12.4%  | 12.4%  | 12.3%  |
| Topiramate  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 12.0%  | 11.9%  |
| Oxcarbazepine   | 3.5%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.4%   | 3.3%   | 3.3%   | 3.3%   | 3.3%   |
| Gabapentin  | 44.5%  | 45.1%  | 45.1%  | 45.1%  | 45.0%  | 45.1%  | 45.1%  | 45.1%  | 45.1%  | 45.1%  | 44.6%  | 44.6%  | 44.1%  | 43.9%  | 43.9%  | 43.9%  | 43.8%  | 43.7%  | 43.7%  | 43.7%  |
| Pregabalin  | 9.4%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.3%   | 9.2%   | 9.2%   | 9.2%   | 9.2%   | 9.1%   | 9.1%   |
| Felbamate   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   |
| Zonisamide  | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.3%   | 1.2%   |
| Lacosamide  | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   | 0.7%   |
| Ezogabine   | 0.0%   | 0.0%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   |
| Phenobarbital   | 2.4%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.3%   | 2.2%   | 2.1%   |
| Perampanel  | 0.0%   | 0.0%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   | 0.1%   |
| Other   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.0%   | 0.5%   | 0.5%   | 0.5%   | 1.0%   | 1.0%   | 1.0%   | 1.0%   | 1.0%   | 1.0%   | 1.0%   |
| Total Epilepsy Market   | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% | 100.0% |

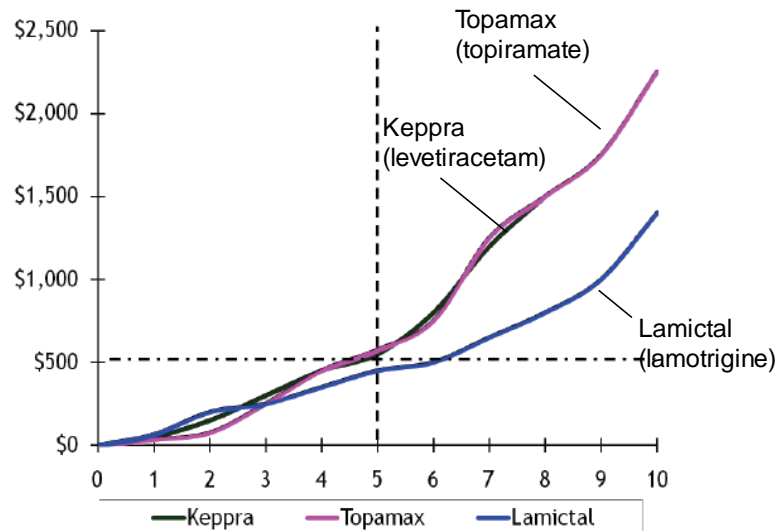
Source: Company reports, Symphony Health Solutions, and Oppenheimer &amp; Co. estimates

## Comparative Launches and the UCB Model

There are more than 15 approved AEDs available in the United States and worldwide. Currently available AEDs control seizures through a variety of mechanisms, including modulation of voltage-activated sodium channels, voltage-activated calcium channels, increasing GABA signaling, and interactions with a 2- d protein or synaptic vesicle protein SV2A.

The top prescribed AEDs include the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Market research firm Decision Resources reports that these AEDs are used to treat a substantial percentage of epilepsy patients. Recent market entrants include Vimpat (UCB), Potiga (GlaxoSmithKline), Fycompa (Eisai), and Aptiom (Sunovion Pharmaceuticals). In addition to ganaxolone, there is one new chemical entity in late stage development called brivaracetam (UCB).

The recent successful introduction of Vimpat (lacosamide) by UCB is an example of market acceptance of a new AED with a novel mechanism. Vimpat was approved in the United States and European Union in 2008, and achieved global sales of approximately \$565 million (€411 million) in 2013. Several other successfully marketed AEDs experienced similar sales levels by their fifth year on the market. UCB has stated that it expects Vimpat to achieve over \$1.6 billion (€1.2 billion) in peak sales globally.

**Exhibit 3. Sales Trajectory for Competitor Launches****Sales of Antiepileptics as Adjunctive Therapy Post-launch**  
(\$ millions, WW sales, years post-launch)**Vimpat® Launch Sales (\$MM)**

- 2009 \$ 62
- 2010 \$168
- 2011 \$287
- 2012 \$437
- 2013 \$565

Source: EvaluatePharma.com, UCB financials

Marinus has stated that efficacy for Vimpat was lower than for Keppra, but that Vimpat benefited from having a novel mechanism of action (sodium channel blocker). In fact, Vimpat was priced at a 50% premium to branded Keppra because of the novel mechanism of action (MOA). We believe that many physicians attempt to add a medication with a new MOA while also trying to minimize side effect burden when selecting a substitute or add-on AED.

Provided ganaxolone has a similar efficacy profile as other approved AEDs, with perhaps an improved side effect profile, we believe that Vimpat and Keppra may serve as a model for the ganaxolone launch, once approved. We forecast roughly \$32 million in ganaxolone sales for the first full year of availability in 2020, moving to approximately \$625 million in the fifth year of sales, by 2024.

**Consultant Feedback**

We spoke to a consultant familiar with the Keppra launch. The consultant noted a few similarities between UCB's launch and what we anticipate would be Marinus' launch in 2020. Keppra was both efficacious and well tolerated, as ganaxolone's trial data indicate that it would be if approved and launched. Additionally, UCB at the time of Keppra's launch in 2000 was a new company challenged to gain a foothold in a crowded market.

The consultant noted that Keppra was eventually well accepted because it was well tolerated. Ganaxolone is a small molecule that is a synthetic analog of allopregnanolone,



an endogenous neurosteroid. It is meant to deliver its therapeutic benefit through a natural mechanism that may offer safety and efficacy advantages compared to other marketed antiepileptic medications. Thus, ganaxolone's potential favorable side effect profile may be what leads to its eventual adoption in the market.

The consultant further commented that UCB targeted epilepsy specialists (epileptologists) and neurologists with the launch, focusing at the outset on key opinion leaders. Because AEDs are often used in drug cocktails and as add-on therapy, physicians are especially concerned with drug-drug interactions. Marinus has completed preclinical safety pharmacology and toxicology testing, including reproductive toxicology. Animal pharmacokinetic and *in vitro* studies show that ganaxolone is primarily metabolized by the CYP3A family of liver enzymes, a common route of drug metabolism. All *in vitro* studies have shown ganaxolone has low potential for interaction with other drugs at several multiples of observed human ganaxolone levels. Furthermore, neither ganaxolone nor its metabolites have a ketone ring at the 3-position, a requirement for hormonal activity. In binding studies, ganaxolone has no appreciable affinity for estrogen or progesterone receptors.

We also spoke to a neurologist consultant who had a private practice and separately was affiliated with an academic institution. Combined, he was writing roughly 300 epilepsy prescriptions per month between the inpatient and outpatient settings. Out of the 15 or so approved AEDs, the consultant indicated that he probably prescribed 11 out of the 15 within the last month, but that he most consistently used levetiracetam (Keppra), lacosamide (Vimpat), phenytoin (Dilantin), valproic acid (Depakote), and perhaps lamotrigine (Lamictal), but the latter only in an outpatient setting.

The consultant did note that there was not a big void with current AEDs, and that patients were fairly adequately maintained on current therapies. He did indicate that he would like to see different routes of administration for new therapies, including intravenous and liquid formulations. He also indicated in his commentary that there was room for new epileptic therapies in the prescriber universe.

When asked what the consultant was most concerned about when it comes to AED therapies, while efficacy is always important, he indicated that the side effect profile and the drug-drug interactions were top-of-mind considerations. Many of his epilepsy patients have co-morbid states, so they tend to be on polypharmacy. The consultant indicated that more than 50% of his epileptic patients were using three or more drugs when considering all disease states, and close to 30% were on polypharmacy when just considering epilepsy drugs.

The commentary reinforced that of the consultant's affiliated with the Keppra launch on drug-drug interactions. With ganaxolone mimicking endogenous allopregnanolone and its natural mechanism, provided the results of the Phase 2 trials hold in further studies, ganaxolone's favorable side effect profile should prove advantageous. Details of the Phase 2 results are outlined below in the *Company Overview*, *Trial Data* portion of the report. We note that the most frequent treatment-emergent adverse events associated with ganaxolone differing from the placebo arm appear to be dizziness, fatigue, and somnolence.

With respect to ganaxolone, the consultant commented that the trial data sounded reasonable enough for him to try it in his refractory patients at the outset, were ganaxolone to get approved (17.6% mean weekly seizure reduction from baseline at week 10). Roughly 8-10% of his patients are refractory to current treatment. From there, he would see how those patients respond before increasing ganaxolone's use and perhaps using it off-label in a monotherapy setting. The consultant also noted that he does

appreciate therapies with new mechanisms-of-action like ganaxolone, as responses to therapies are very patient specific.

Last for adoption, the consultant commented that AEDs are key opinion leader driven. Ganaxolone would likely have to be discussed at the three significant neurological and epilepsy meetings, the American Epilepsy Society (AES), the American Academy of Neurology (AAN), and the American Neurological Association (ANA). Additionally, the consultant might want to see positive data independent from industry, perhaps NIH funded or from an experience in a big center setting.

## Initial Public Offering

On July 31, 2014, Marinus closed its underwritten public offering of 5.625 million shares of common stock and almost 843,750 shares subject to the underwriter's over allotment option. After giving effect to the over allotment option, the company sold an aggregate of almost 5.8 million shares of common stock at an offering price of \$8 per share.

The net proceeds were roughly \$41 million after deducting underwriter discounts, commissions, and other estimated offering costs. As a result of the offering, including prior cash on the hand, the company now has approximately \$50 million in cash to drive future growth.

Marinus lists the use of funds from the offering to advance the development of ganaxolone and for general corporate purposes. The expenses will include approximately \$16.0 million to increase the size of the company's Phase 2b clinical trial for subjects with focal onset seizures, approximately \$10.0 million to complete manufacturing scale-up, conduct related Phase 1 pharmacokinetic studies, complete development of the intravenous ganaxolone formulation, and other critical path activities for ganaxolone in patients with focal onset seizures, approximately \$7.0 million to complete other preclinical studies, including an animal carcinogenicity study, and approximately \$2.5 million to add investigator sites to accelerate enrollment in the company's Phase 2 clinical trial for Fragile X Syndrome and to initiate a proof-of-concept trial for PCDH19 female pediatric epilepsy.

Marinus believes that the net proceeds from the offering together with existing cash will fund operating expenses and capital expenditure requirements for at least the next 24 months. We conservatively estimate another capital raise in the late 2015 or early 2016 time frame. Overall, we believe the proceeds from the offering will allow Marinus to continue its development efforts and clinical trial studies for ganaxolone.

## Company Overview

Marinus Pharmaceuticals, located in New Haven, CT, is a clinical stage biopharmaceutical company focused on developing and commercializing neuropsychiatric therapeutics. The company's clinical stage product, ganaxolone, is a small molecule that is a synthetic analog of allopregnanolone, an endogenous neurosteroid produced in the central nervous system. Allopregnanolone modulates the brain neurotransmitter gamma-aminobutyric acid, or GABA, and ganaxolone mimics that activity.

The company's lead indication for ganaxolone is as an adjunctive, or add-on, therapy for the treatment of partial, also known as focal, onset seizures in adults with epilepsy. By targeting the same spectrum of GABA<sub>A</sub> receptors as endogenous allopregnanolone, ganaxolone delivers its therapeutic benefit through a natural mechanism that may offer safety and efficacy advantages compared to other marketed antiepileptic medications.



Epilepsy is characterized by seizures that arise from abnormal electrical discharge in the brain, resulting in alterations of consciousness, involuntary movement, or altered sensations. Seizures in epilepsy may be related to a brain injury or heredity, but often the cause is unknown. Epileptic seizures are generally described in two major groups, primary generalized seizures and focal onset seizures.

Primary generalized seizures begin with a widespread electrical discharge that involves both sides of the brain at once. Focal onset seizures begin with an electrical discharge in one limited area of the brain. Generally, a person is diagnosed as having epilepsy when they have had at least two seizures that do not have a self-limiting cause (such as a high fever).

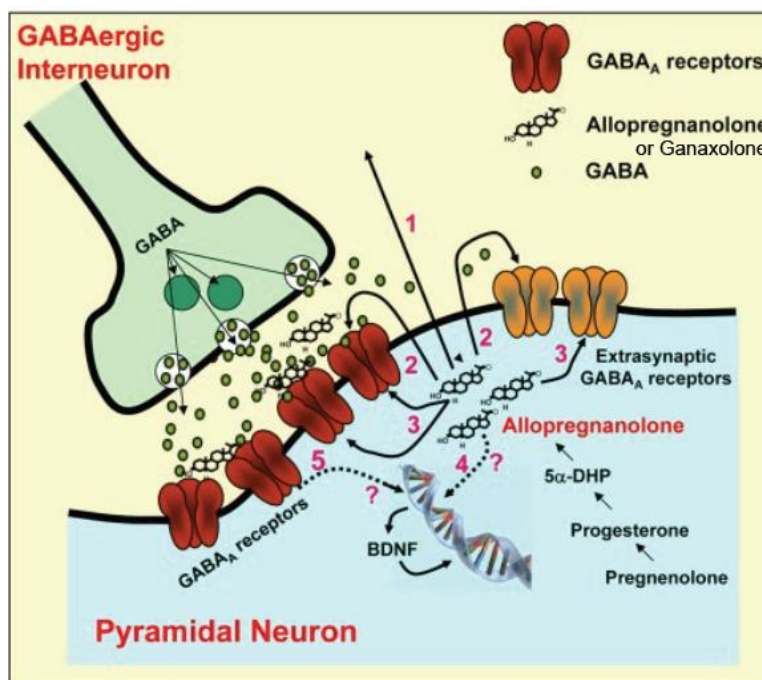
## Product Details

Allopregnanolone has been studied for over two decades, and according to Marinus, its role in controlling seizures and improving anxiety, mood and sleep through positive modulation of GABA<sub>A</sub> type receptors has been well documented. However, because of potential undesired steroidal effects, allopregnanolone may not be suitable for chronic use. The compound can break down after a short while to progesterone, a steroid hormone that can be fairly reactive.

Ganaxolone was designed to have the same GABA modulation effects as allopregnanolone without steroidal effects. Essentially, the design allows for a longer acting product in an oral capsule or tablet. Thus, ganaxolone may potentially offer the same therapeutic benefit as allopregnanolone through a natural mechanism with low side effects.

Ganaxolone and allopregnanolone act on a receptor called GABA, and that modulatory activity of GABA is what's responsible for the anticonvulsive activity in epileptic seizures. They differ from other GABA agents by interacting with unique binding sites on the GABA<sub>A</sub> receptor that are located both within, or synaptic, and outside, or extrasynaptic, the GABA synapse. Ganaxolone's interaction with neurosteroid recognition sites to activate GABA<sub>A</sub> receptors and its activation of the extrasynaptic receptor is a unique mechanism that provides stabilizing effects that may differentiate it from other drugs that increase GABA signaling.

## Exhibit 4. Mechanism for Ganaxolone and Allopregnanolone

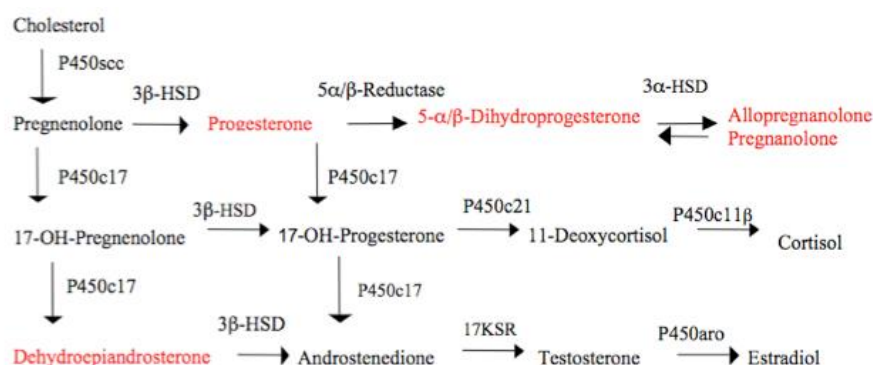


Source: adapted from Pinna et al (2008) *Neurochem Res.* 33(10): 1990-2007. doi 10.1007/s11064-008-9718-5

Allopregnanolone is a positive allosteric modulator of GABA<sub>A</sub> type receptors with extensive binding capability. This capability allows for interaction with both synaptic and extrasynaptic receptors. The synaptic GABA<sub>A</sub> receptors, near to the GABAergic interneuron that releases the GABA neurotransmitter, are able to mediate signal transmission. By interacting with synaptic GABA<sub>A</sub> receptors at unique neurosteroid binding sites, allopregnanolone modulates phasic GABA signaling (Phasic Modulation), smoothing out the signal that the post-synaptic neuron receives.

Simultaneously, allopregnanolone can interact with extrasynaptic GABA<sub>A</sub> receptors to tonally or constantly modulate GABA<sub>A</sub> receptor signaling (Tonal Modulation). Specifically, it activates the extrasynaptic GABA<sub>A</sub> receptors, providing constant modulation of the GABA inhibitory signal that calms overexcited neurons. By interacting with both the synaptic and extrasynaptic receptors, allopregnanolone safeguards against quick transitions in the neurons between excited and resting states (increase in GABA<sub>A</sub> receptor transmission and inhibition of neuronal excitation).

However, allopregnanolone is a byproduct of progesterone metabolism and can convert back to a 5-α-DHP. This compound can be reactive, binding to nuclear progesterone receptors. Ganaxolone is a synthetic analog of allopregnanolone designed to have the same GABA modulation effects. It has the same chemical structure as allopregnanolone with the addition of a methyl group, designed to prevent conversion back to an active steroid. The design potentially eliminates the opportunity for unwanted hormonal effects, while preserving ganaxolone's GABA<sub>A</sub> receptor and central nervous system, or CNS, activity.

**Exhibit 5. Allopregnanolone Synthesis Pathway**

Source: Company reports

**Intellectual Property**—Marinus has global rights to develop and commercialize ganaxolone, excluding Russia and certain other eastern European nations. While ganaxolone itself is off patent, the company has technical knowhow in the solid and liquid nanoparticulate formulation of ganaxolone and expertise with regards to methods employed to manufacture ganaxolone. The company has seven US patents and corresponding foreign patents and patent applications directed to solid and liquid ganaxolone formulations and methods for the making and use thereof, the earliest of which will expire in 2026. Marinus also has a US patent and corresponding foreign patents and patent applications covering ganaxolone's synthesis process, the earliest of which will expire in 2030.

**Trial Data**

Marinus has successfully completed a double-blind, randomized, placebo-controlled, Phase 2 clinical trial of ganaxolone (Study 600) for adjunctive treatment in 147 patients with refractory focal onset seizures. The study was completed under an Investigational New Drug application (IND) originally filed by a third party with the FDA on November 29, 1993. Ganaxolone satisfied the primary efficacy endpoint of the study, a reduction in seizure frequency, and was considered to be generally safe and well tolerated.

The subjects in the trial were adult outpatients from the United States who had been diagnosed with epilepsy on average 25 years prior, and 75% were taking two or three AEDs to control seizures before they entered the study. Subjects were treated for ten weeks with placebo or ganaxolone as adjunctive treatment to existing therapy and recorded their seizures daily in a diary. Mean baseline seizure frequency was 6.5 and 9.2 seizures per seven days in the ganaxolone and placebo groups, respectively.

Subjects gradually increased their daily dose up to 1,500 milligrams per day (mg/day) over the first two weeks, known as the titration period, followed by maintenance dosing at 1,500 mg/day for eight weeks, known as the maintenance period. The primary efficacy endpoint was change from baseline in weekly seizure frequency.

**Exhibit 6. Phase 2 Results: Percent Seizure Reduction from Baseline (Study 600)**

|                  | <b>Ganaxolone (n=98)</b> | <b>Placebo (n=49)</b> | <b>Difference</b> |
|------------------|--------------------------|-----------------------|-------------------|
| Mean (std. dev.) | -17.6% (48.9)            | +2.0% (63.2)          | 19.6% (p=0.014)   |
| Median           | -26.0%                   | -10.2%                | 15.7%             |

Source: Company reports

In the intention-to-treat, or ITT, population, which included all study subjects who took at least one dose of study medication, there was a statistically significant reduction in the percent change in mean weekly seizure frequency in the ganaxolone group, which decreased 17.6% from baseline at week 10. In the placebo group, mean weekly seizure frequency increased by 2% compared to baseline at week 10, a difference of 19.6% ( $p=0.014$ ). Essentially, ganaxolone demonstrated a 20% mean seizure reduction from baseline compared to placebo in a difficult-to-treat refractory patient population.

Additionally, in the ganaxolone group, median seizure frequency decreased by 26.0%, whereas in the placebo group, median seizure frequency decreased by 10.2%. The difference of between the two groups was 15.7%. Secondary analyses included the analysis of the percentage of subjects with greater than or equal to 50% improvement from baseline, or responder analysis, mean and percent change in seizure frequency from baseline, number of seizure-free days and seizure-free subjects, change in seizure frequency by week, and change from baseline in types of seizures. In general, the results of secondary efficacy analyses supported the primary outcome that subjects treated with ganaxolone showed improved seizure control compared to those treated with placebo.

Ganaxolone was considered to be generally safe and well tolerated in the Phase 2 adjunctive treatment trials. The majority of adverse events associated with ganaxolone treatment related to known CNS effects of GABA, were not medically serious, and resolved upon discontinuation of therapy. The most frequent treatment-emergent adverse events, or TEAEs, observed in Study 600 are presented in Exhibit 7.

#### Exhibit 7. Most Frequently Reported TEAEs ( $\geq 5\%$ of Subjects, Study 600)

| <u>Preferred Term</u>                          | <u>Ganaxolone<br/>(n=98)<br/>%</u> | <u>Placebo<br/>(n=49)<br/>%</u> |
|--|------------------------------------|---------------------------------|
| Dizziness                                      | 16.3                               | 8.2                             |
| Fatigue  | 16.3                               | 8.2                             |
| Somnolence                                     | 13.3                               | 2.0                             |
| Injury, poisoning and 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                            |

Source: Company reports

The two treatment groups had similar rates of discontinuation due to adverse events (7% ganaxolone, 6% placebo) and similar rates of serious adverse events, or SAEs (5% ganaxolone, 8% placebo), mostly related to the underlying epilepsy. The majority of TEAEs resolved with continued treatment or dose reduction. In contrast to some marketed AEDs, the incidence of behavioral TEAEs (reported as depression, insomnia, affective disorder, confusional state, affect lability, aggression, and anxiety) was similar in the ganaxolone and placebo treatment groups.

In the open label extension to the study (Study 601), 120 subjects received ganaxolone for a mean duration of 39 weeks. The objective was to evaluate the long-term safety, tolerability and efficacy of ganaxolone at a target dose of 1,500 mg/day. The primary endpoint was change in seizure frequency at endpoint compared to baseline of the double-blind study, presented as mean and median change. The mean and median percent reductions in weekly seizure frequency were 14.2% and 23.2% from baseline to endpoint, respectively.

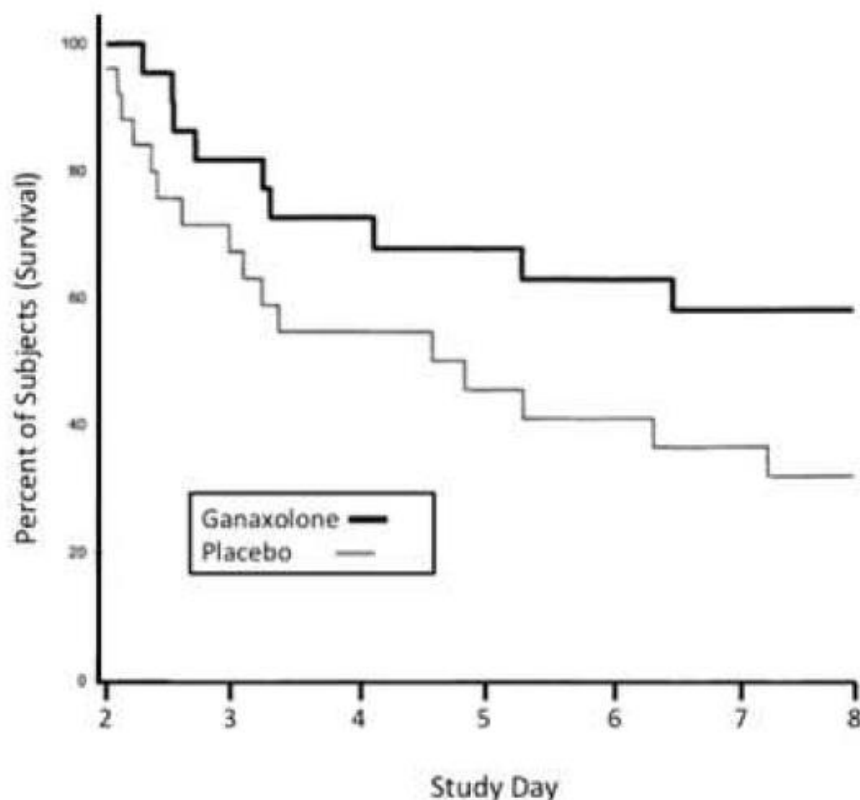
In total, 70% of subjects had a reduction in seizure frequency during the study. Importantly, subjects previously randomized to placebo in the double-blind study (Study 600) that were switched to ganaxolone in the open-label study showed mean and median reduction in seizure frequency comparable to patients randomized to ganaxolone in the double-blind study.

The most common adverse events considered related to ganaxolone treatment were fatigue (14%), dizziness (9%) and somnolence, also known as sleepiness (7%). Eleven percent of the subjects discontinued due to one or more adverse events. One SAE out of 17 reported was considered related to ganaxolone treatment, a 59 year old female on 900 mg/day whose liver enzymes were elevated after 57 days of treatment. The enzyme levels returned to normal with a reduction in dose to 600 mg/day. In this long-term open-label study of ganaxolone for adjunctive treatment of focal onset seizures, no new safety concerns were identified during extended treatment with doses up to 1,500 mg/day.

Ganaxolone has also been studied in a Phase 2 proof-of-concept clinical trial (Study 104) as the sole treatment, or monotherapy, for adults with treatment resistant focal onset seizures. The primary endpoint was duration of treatment prior to withdrawal from the trial due to seizures, as measured from day 2 of the study. The double-blind, randomized, placebo-controlled, clinical trial involved 52 subjects who were withdrawn from their antiepileptic medications prior to evaluation for surgical treatment of their seizures (presurgical patients). The subjects were treated with ganaxolone monotherapy 625mg three times per day for eight days.

In the trial, 61% of subjects in the placebo group left the study due to emergence of seizures by day 8, compared with 38% of ganaxolone subjects. While the results, we believe, indicate an important trend, they were not statistically significant ( $p=0.08$ ). There was though a statistically significant difference between completion rates in the two groups, with 50% of ganaxolone subjects completing the study compared to 25% of placebo subjects ( $p=0.04$ ).

**Exhibit 8. Phase 2 POC Results: Kaplan-Meier Survival Function of Subjects Remaining in the Study (Study 104)**



Source: Company reports

Again, ganaxolone was generally well-tolerated, and the profile of adverse events between the two groups was similar. Dizziness, which was reported in four ganaxolone and three placebo subjects, was the most frequent adverse event. One SAE was reported in each group, with the ganaxolone subject experiencing severe agitation and depression and the placebo subject experiencing postictal psychosis. As in the other studies, no clinically meaningful differences between treatment groups were noted in exam results.

Combined, in Phase 1 and 2 clinical trials, ganaxolone has been administered in approximately 1,000 subjects at therapeutically relevant dose levels and treatment regimens for up to two years. No drug-related deaths occurred in any of these clinical trials, and ganaxolone was generally well tolerated with no adverse effects on cardiovascular, liver, blood or other systems. Additionally in animal studies, there was no evidence of reproductive toxicity or other toxicities after long-term administration of ganaxolone.

Ganaxolone was engineered to be a synthetic analog of a natural molecule, allopregnanolone. This engineering may, in part, help explain ganaxolone's improved safety and tolerability profile. In addition to the lack of toxicity to the major body systems, ganaxolone also had limited side effects common to other AEDs. This safety profile may make ganaxolone a treatment of choice in antiepileptic polypharmacy regimens.

### Current Epilepsy Study

In October 2013, Marinus initiated a Phase 2b clinical trial in epilepsy patients with focal onset seizures to evaluate ganaxolone compared to placebo as adjunctive treatment for

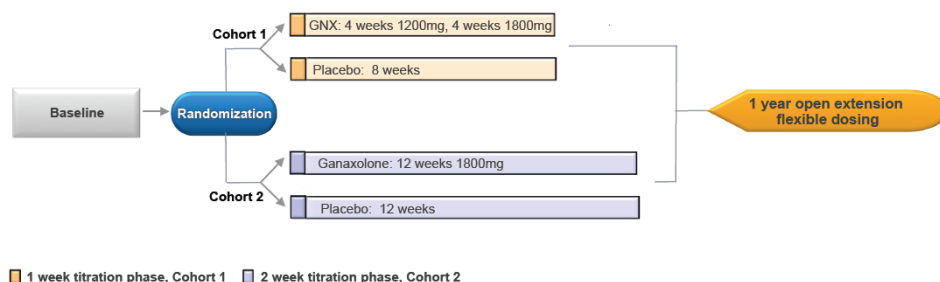


12 weeks (Study 603). This international, randomized, placebo-controlled trial was designed to enroll approximately 150 adult subjects with focal onset seizures. With the proceeds from IPO offering, however, the company intends to increase the size of this study to approximately 300 subjects in order to meet statistical power requirements so that it may be considered as an adequate and well-controlled trial in an FDA or EMA filing package for registration. Marinus expect to complete this clinical trial in 2H15.

In Cohort 1, 50 subjects were randomized to receive either placebo or ganaxolone capsules in a step titration of 1,200 mg/day for four weeks followed by 1,800 mg/day for four weeks. Blood levels of ganaxolone were assessed at steady state for each dose level. Cohort 1 is complete with the open label study still ongoing.

In Cohort 2, an additional roughly 300 subjects will be randomized to receive either placebo or 1,800 mg/day of ganaxolone for 12 weeks. With the proceeds from the IPO offering, Marinus plans to increase the size of Cohort 2 from approximately 150 to 300 subjects in order to meet the statistical power requirements for the FDA and other regulatory bodies to consider this trial as one of the company's adequate and well-controlled studies as part of an FDA or EMA filing package for registration. The primary endpoint of this trial will be change in seizure frequency per month compared to baseline. Marinus will also capture adverse events and other measures of safety as well as responder rate, seizure-free status, and changes in seizure subtypes. The trial design is shown below in Exhibit 9.

#### Exhibit 9. Phase 2b Study Trial Design (Study 603)



Source: Company reports

Ganaxolone is currently formulated as an oral suspension and as a capsule. Study 600 and 601 were conducted with an oral suspension formulation and Study 603 is being conducted with a capsule formulation. Marinus was planning to use a tablet formulation in future studies, as tablets typically can accommodate higher dosing better than capsules. The company has developed a prototype for a tablet and intravenous formulation.

The tablet formulation may be more convenient for patient compliance and ease of administration as compared to the current capsule and oral suspension formulations. In order to support clinical trials with a tablet formulation, Marinus was planning two pharmacokinetic studies, one to demonstrate bioequivalence between the capsule and the tablet, and one to establish relative potency of the oral suspension to the tablet.

While future trials with a tablet formulation may still be a possibility, with IPO proceeds coming in at the lower end of the range, management has indicated that the tablet formulation is now less of a priority. Cohort 1 of the 603 study was conducted with 2-3 300mg capsules (depending on the dose) with BID dosing. Cohort 2 will be BID dosing as well. Though Marinus was planning on formulating 450mg tablets, it could go ahead with Phase 3 studies and commercialization with the 300mg capsules.

## Pipeline

Marinus has a Phase 2 proof-of-concept pediatric clinical trial in progress for ganaxolone as a treatment for behaviors associated with Fragile X Syndrome, or FXS, in children. The company also is initiating an expanded access protocol under its epilepsy IND for the use of ganaxolone in the treatment of PCDH19 female pediatric epilepsy. Both disorders have been related to mutations affecting neurosteroid signaling at extrasynaptic GABA<sub>A</sub> receptors, and Marinus believes both are potential orphan indications.

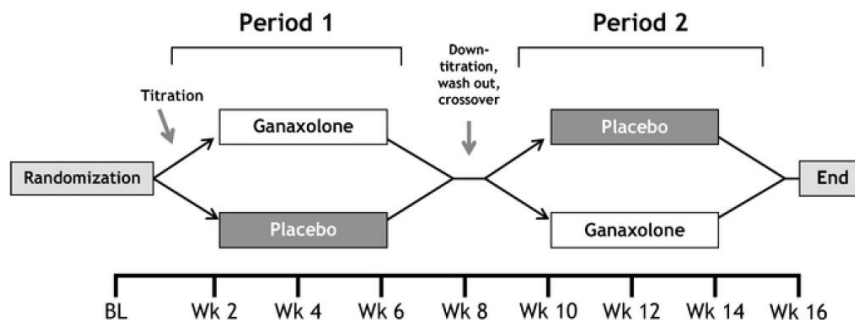
FXS is a genetic condition that causes intellectual disability, behavioral and learning challenges and various physical characteristics. It arises from a mutation of a gene known as the *fmr1* gene in the coding for the Fragile X mental retardation protein. In mouse models of this gene mutation, certain brain regions show lower levels of the extrasynaptic GABA<sub>A</sub> receptors and reduction of proteins and enzymes responsible for GABA function.

People with FXS are affected throughout their lives. The impairment can range from learning disabilities to more severe cognitive or intellectual disabilities. Patients with FXS exhibit autism-like symptoms including cognitive impairment, anxiety and mood swings, attention deficit, and heightened stimuli. Approximately one million individuals in the United States have, or are at risk for developing, a Fragile X-associated disorder, with approximately 100,000 people having FXS.

According to the Centers for Disease Control and Prevention, FXS affects 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females of all races and ethnic groups. Approximately 1 in 151 women carry the Fragile X gene and could pass it to their children. Approximately 1 in 468 men carry the Fragile X gene and their daughters will also be carriers. Approximately 7% of women and 18% of men with FXS have seizures.

Marinus is currently conducting a Phase 2 proof-of-concept randomized, placebo-controlled, clinical trial in approximately 60 FXS patients (IND filed by Marinus on July 2, 2012). The trial is being conducted in collaboration with the MIND Institute at the University of California, Davis. The MIND Institute has been awarded a medical research grant from the US Department of Defense (DoD) to study ganaxolone for treatment of behaviors in FXS in children and adolescents.

In the trial, subjects are titrated up to a maximum dose of 1,800 mg/day of ganaxolone or placebo over a two week period 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 outcome measure of the study is Clinical Global Impression Rating Scale for Improvement. Secondary outcome measures include the Aberrant Behaviors Checklist and ratings scales for specific behaviors associated with childhood FXS. Marinus expects the trial (Study 800) to be completed during the middle of 2015.

**Exhibit 10. Phase 2 POC Study Trial Design, Ganaxolone in FXS (Study 800)**

Source: Company reports

Fragile X is an orphan indication in which GABA activity has been implicated in the expression of the disorder. Ganaxolone and other agents that have been shown to improve GABA function have also been shown to improve FXS symptoms in mouse models. Ganaxolone, with its high-affinity for extrasynaptic GABA<sub>A</sub> receptors, may increase signaling at existing receptors to normalize GABA function thereby reducing anxiety, hyperactivity and other disabilities associated with this inherited disorder.

Currently, there are no known cures for FXS. There are also no drugs approved for the treatment of behavioral and mental health conditions associated with FXS, although various classes of medications are used off-label. Special education and symptomatic treatments are employed to lessen the burden of illness. Treatment approaches focus primarily on supportive care and medications addressing development delays, learning disabilities, and social and behavioral problems caused by the disease.

Some patients with FXS benefit from medications that treat attention deficient disorders. Other patients who experience general anxiety, social anxiety and other chronic conditions may benefit from different types of anti-anxiety medications and other neuropsychiatric treatments. Additionally, there are several drugs in development for the treatment of behavioral and mental health conditions associated with FXS, including compounds being developed by Roche, Sunovion Pharmaceuticals, Afraxis and Neuren Pharmaceuticals.

Marinus is also developing ganaxolone as a treatment for PCDH19 female pediatric epilepsy, a disorder in which a mutation of the PCDH19 gene causes deficits or impairment in GABAergic signaling both at the agonist and receptor levels. The mutation is X chromosome-linked, and the seizures occur in females, often beginning before their first birthday. Although formal epidemiologic data is not available, there is a genetic test available to determine whether or not a child has the PCDH19 mutation.

Results from diagnostic screenings indicate that approximately 10% of girls who have seizure onset before five years of age have PCDH19 mutations. Marinus estimates the PCDH19 population to be approximately 15,000 to 30,000 patients in the United States. Patients typically experience multiple types of seizures, including focal and generalized, as well as clusters of seizures that can last from one day to weeks. Many of these patients will also experience developmental delay, intellectual disability, and behavioral problems. Seizures often improve in adolescence, although developmental complications persist.

Marinus believes that ganaxolone may be useful in treating patients with PCDH19 female pediatric epilepsy because it is a positive allosteric modulator of GABA<sub>A</sub> receptors used to increase GABAergic signaling. Additionally, ganaxolone's safety profile and its availability

in a liquid suspension and oral capsule form may also be beneficial. The company also believes that data from its open label trial of ganaxolone in pediatric patients with multiple seizure types has demonstrated ganaxolone's ability to treat multiple seizure types. This broad treatment range would be important in PCDH19 female pediatric patients, because the seizures they experience can be of varying types.

The company is initiating an expanded access protocol under its epilepsy IND for a trial that will provide proof-of-concept data in approximately 10 patients with PCDH19 female pediatric epilepsy. Marinus is currently working with physician advisors to finalize the design of the trial. It expects to be able to initiate this trial in 2H14 and have preliminary data in 1H15.

Again, both the FXS and PCDH19 female pediatric epilepsy indications are potential orphan indications with approval estimated in the 2017 or 2018 time frame. Marinus would likely seek higher pricing for ganaxolone in FXS and PCDH19 than with the epilepsy indication, then bring pricing back down once the epilepsy indication is approved. Management has indicated roughly \$50,000 for annual therapy with the orphan indications, an approximate 10x increase from the epilepsy indication. However, we focus only on the epilepsy indication and assign no value to ganaxolone's other indications, leaving any additional therapeutic treatments or label expansion as upside.

Due to its mechanism of action, Marinus believes ganaxolone may have potential in a broad range of neuropsychiatric disorders beyond the company's current clinical focus. Potential additional ganaxolone indications include generalized anxiety disorder; posttraumatic stress disorder and depression, or PTSD; multiple sclerosis; addictive behaviors such as alcoholism and smoking; perinatal depression; attention deficit hyperactivity disorder, or ADHD; and other neurodegenerative disorders.

Additionally, ganaxolone might be useful in several rare genetic disorders related to impaired GABA function such as the orphan indications Super Refractory Status Epilepticus, or SRSE; Neimann Pick Disease, Type C; and certain autism subtypes. We note that the company would need to undertake further clinical studies in order to obtain any additional labeled indications. However, Marinus has stated that it plans to pursue other potential indications related to ganaxolone's mechanism when non-dilutive opportunities arise.

As an example, preclinical studies and clinical trials to evaluate ganaxolone in patients suffering from PTSD have been primarily conducted by the US Department of Veterans Affairs. The Injury and Traumatic Stress Consortium, or INTRuST, a group of PTSD treatment centers in the United States, has received funding from the DoD to evaluate new treatments for PTSD. In collaboration with Marinus, INTRuST conducted a proof-of-concept study of ganaxolone versus placebo in adults with PTSD. The Phase 2 clinical trial was conducted at seven Veterans Administration centers in the United States (under an IND filed by Marinus on January 4, 2010). The study enrolled 114 adults with PTSD who were treated for six weeks with ganaxolone or placebo in ascending doses followed by six weeks on open label ganaxolone.

**Exhibit 11. Pipeline and Near-Term Marinus Catalysts**

|  | Phase 1 | Phase 2 | Phase 3 | Results  |
|--|---------|---------|---------|----------|
| Ganaxolone in Epilepsy                             |         |         |         |          |
| Adjunctive Treatment of Focal Onset Seizures       |         |         |         | 2H 2015  |
| Ganaxolone in Developmental Disorders              |         |         |         |          |
| Fragile X Syndrome ( <i>grant funded, orphan</i> ) |         |         |         | Mid 2015 |
| Ganaxolone in Pediatric Epilepsy                   |         |         |         |          |
| PCDH19 Female Pediatric Epilepsy ( <i>orphan</i> ) |         |         |         | 1H 2015  |

Source: Company reports

**New Competitive Products**

In addition to ganaxolone, UCB has a new chemical entity in late stage development called brivaracetam. In July, UCB announced positive top-line results from a Phase 3 study (n=768) that achieved a statistically significant reduction in partial-onset seizure frequency. In the randomized, double-blind, placebo-controlled study, UCB evaluated the efficacy and safety of brivaracetam for a 12-week period as an adjunctive treatment in patients suffering from focal epilepsy with partial-onset seizures.

The drug demonstrated statistical significance on the 50% responder rate endpoint and the percent reduction in seizures over a 28-day duration. The study was the last one scheduled under the Phase 3 program on brivaracetam. UCB intends to file for approval of brivaracetam in the US and the EU in early 2015 with a potential approval in 2016.

Sage Therapeutics, as well, is developing Sage-547 for Super Refractory Status Epilepticus (SRSE). The company's Phase 1/2a trial of 10-15 patients is under way with data expected by year-end. We note several important differences, however, between Sage's lead product and ganaxolone.

In addition to the different indications, the compounds themselves are slightly different. While ganaxolone is a synthetic analog of allopregnanolone with a methyl substitution, Sage's product is allopregnanolone in an IV formulation. Thus, some of the drawbacks from allopregnanolone regarding reactivity may be an issue for Sage. Additionally, Sage is developing Sage-547 for an acute setting, whereas ganaxolone is being developed for a hospital and outpatient setting.

## Management

### Exhibit 12. Marinus Management Team

| Name                   | Position  |
|------------------------|---|
| Christopher M. Cashman | President and Chief Executive Officer             |
| Edward F. Smith        | Chief Financial Officer                           |
| Gail M. Farfel, Ph. D. | Chief Clinical Development and Regulatory Officer |
| Julia Tsai, Ph. D.     | Senior Director of Clinical Development           |

Source: Company reports

Christopher M. Cashman has served as chairman of Marinus' board of directors since September 2011 and as the company's president and chief executive officer since October 2012. From August 2010 to May 2011, Mr. Cashman provided consulting services to Quaker Partners, a private venture capital fund. From 2003 to June 2010, Mr. Cashman served as president and chief executive officer of Protez Pharmaceuticals, Inc., a company specializing in the development of antibiotics. Prior to his time with Protez, Mr. Cashman served as president and CEO of Message Pharmaceuticals Inc., and as vice president for both Pfizer Inc. and SmithKline Beecham plc. Mr. Cashman began his pharmaceutical career at SmithKline Corporation. He currently serves on the board of directors of Rapid Micro Biosystems, Inc., Noble Biomaterials, Inc. and MBF Therapeutics Inc. Mr. Cashman holds an M.S. in economics from Purdue University and B.S. in business management from the University of Minnesota.

Edward F. Smith has served as Marinus' vice president, chief financial officer, Secretary and treasurer since November 2013. From July 2013 to November 2013, Mr. Smith served as a financial advisor in a consulting capacity for TetraLogic Pharmaceuticals Corporation. From January 2006 to April 2013, he served as CFO of PolyMedix, Inc., a company engaged in the development of smallmolecule drugs for the treatment of serious acute care conditions, which voluntarily filed for Chapter 7 bankruptcy on April 1, 2013. From September 2000 to December 2005, Mr. Smith was executive director of finance at InKine Pharmaceutical Company, Inc., a biopharmaceutical company focused on the diagnosis and treatment of gastrointestinal disorders. Earlier in his career, Mr. Smith held various positions of increasing responsibility 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 and holds a B.S. in business administration from the University of Hartford.

Gail M. Farfel, Ph.D. has served as Marinus' chief clinical development and regulatory officer since December 2012. From May 2008 until December 2012, she served as president of G. Meredith Consulting LLC, a firm providing strategic consulting and support to biopharmaceutical and software development companies, of which Marinus was a client. From March 2006 through April 2008, Dr. Farfel was employed at Novartis Pharmaceuticals Corp., a US subsidiary of Novartis AG, as vice president, therapeutic area and head for neuroscience clinical development and medical affairs. From November 1996 to February 2006, Dr. Farfel held a variety of leadership positions in Clinical Development and Global Medical Affairs at Pfizer, Inc. where she directed programs through all stages of clinical development and regulatory submissions including development of the first product approved globally to treat PTSD. Dr. Farfel currently serves on the board of directors of PrintedArt. She is the author of over 50 scientific articles in the areas of neuropsychopharmacology and drug treatment. Dr. Farfel 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. She has a Bachelor's degree in Biochemistry from the University of Virginia.

Julia Tsai, Ph.D. has served as Marinus' senior director of clinical development and project management since November 2012. From September 2006 to November 2012, Dr. Tsai served as the company's director, drug development. and from 2004 to 2006, she served as Marinus' manager, drug development. Dr. Tsai is responsible for all facets of the company's drug development operations. 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.

## Financial Overview

Epilepsy affects approximately 50 million people globally, with IMS Health data indicating global sales of antiepileptic drugs, or AEDs, at approximately \$14 billion in 2011. According to 2012 Decision Resources statistics, over 5 million people are under treatment in the United States, Europe and Japan. Accordingly, with the large market size, the epilepsy space represents a somewhat crowded market with therapies.

Specifically, there are more than 15 approved AEDs available in the United States and worldwide. Newly diagnosed epilepsy patients are treated with daily administration of an AED. Approximately 60% of patients will achieve an adequate level of seizure control with a single AED, and the remainder will resort to using multiple drugs, or polypharmacy. Even with polypharmacy, approximately 30 to 35% of all patients do not reach an acceptable level of seizure control.

Marinus estimates that the market opportunity for this refractory patient population, which will be ganaxolone's initial target segment, will exceed \$4 billion in the United States, Europe and Japan. Ganaxolone may be an attractive treatment option for patients that require polypharmacy to control their seizures, due to its novel mechanism of action and its safety and tolerability profile. The company is targeting ganaxolone as a first-in-class therapy with the potential to provide treatment advantages for adults with focal onset seizures who do not achieve adequate seizure control from, have developed tolerance to, or have safety concerns with currently available medications.

Ganaxolone is currently undergoing a clinical trial in epilepsy. Marinus' commercial success will of course depend upon gaining approval for ganaxolone and attaining significant market acceptance among physicians, patients, government and private payors, and others in the medical community. Marinus anticipates readout of the Phase 2b ganaxolone trials in epilepsy in late 2015, with a potential Phase 3 study in 2H16, and approval in the late 2019 or early 2020 time frame.

The recent successful introduction of Vimpat (lacosamide) by UCB is an example of market acceptance of a new AED with a novel mechanism. Vimpat was approved in the United States and European Union in 2008, and achieved global sales of approximately \$565 million (€411 million) in 2013. Several other successfully marketed AEDs experienced similar sales levels by their fifth year on the market. UCB has stated that it expects Vimpat to achieve over \$1.6 billion (€1.2 billion) in peak sales globally.

Marinus has stated that efficacy for Vimpat was lower than that of another UCB product, Keppra, which also had a successful launch. Vimpat, however, benefited from having a novel mechanism of action (sodium channel blocker). In fact, Vimpat was priced at a 50% premium to branded Keppra because of the novel mechanism of action. We believe that many physicians attempt to add a medication with a new MOA while also trying to minimize side effect burden when selecting a substitute or add-on AED.

Management has indicated an estimate of approximately \$5,000 for annual therapy with the epilepsy indication at the time of the launch. We do not anticipate revenues for Marinus until 2020 and estimate pricing on ganaxolone at roughly \$250 per prescription at the launch (\$3,000 for annual therapy). We forecast roughly \$32 million in ganaxolone sales for the first full year of availability in 2020, moving to approximately \$625 million in the fifth year of sales, by 2024. We further anticipate ganaxolone acquiring a roughly 0.1% market share in the first full year of launch in 2020 increasing to 1.8% share by 2025.

## Penheimer Estimates for Epilepsy Sales, Prescriptions, and Market Share

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Marinus is also developing ganaxolone as a treatment for behaviors associated with FXS and for treatment of PCDH19 female pediatric epilepsy. Both are potential orphan indications with approval estimated in the 2017 or 2018 time frame. Marinus would likely seek higher pricing for ganaxolone in FXS and PCDH19 than with the epilepsy indication, then bring pricing back down once the epilepsy indication is approved. Management has indicated roughly \$50,000 for annual therapy with the orphan indications, an approximate 10x increase from the company's estimated pricing for the epilepsy indication. However, we focus only on the epilepsy indication and assign no value to ganaxolone's other indications, leaving any additional therapeutic treatments or label expansion as upside.

In 2004, Marinus entered into a license agreement with Purdue Neuroscience Company granting the company exclusive rights to certain know-how and technology relating to ganaxolone. As a result, Marinus must pay a single digit royalty to Purdue based on net sales (the "know-how" royalty). The obligation to pay royalties expires, on a country-by-country basis, ten years from the first commercial sale of ganaxolone in each country. We embed this royalty in our COGS estimate and our 90% gross margin forecast.

As of December 31, 2013, Marinus had Federal and state net operating loss carryforwards of approximately \$59.0 million that will begin expiring in 2023. As part of our valuation, we estimate that the company will not pay taxes until 2023 with a full normalized rate beginning in 2024.

With regards to cash, on July 31, 2014, Marinus closed its underwritten public offering of 5.625 million shares of common stock and almost 843,750 shares subject to the underwriter's over allotment option. After giving effect to the over allotment option, the company sold an aggregate of almost 5.8 million shares of common stock at an offering price of \$8 per share. The net proceeds of the offering were roughly \$41 million after deducting underwriter discounts, commissions, and other estimated offering costs.

As a result of the offering, including prior cash on the hand, the company now has approximately \$50 million in cash to drive future growth. Marinus believes that the net proceeds from the offering together with existing cash will fund the company's operating expenses and capital expenditure requirements for at least the next 24 months. We conservatively estimate another capital raise in the late 2015 or early 2016 timeframe. Overall, we believe the proceeds from the offering will allow Marinus to continue its development efforts and clinical trial studies for ganaxolone. Lastly, with regards to IP, we note that Marinus has formulation patents and synthesis process patents on ganaxolone, the earliest of which will expire in 2026 and 2030, respectively.

**Stock prices of other companies mentioned in this report (as of 9/8/2014):**

Eisai (4523.TSE, ¥4,253.5, Not Covered)  
GlaxoSmithKline (GSK-NYSE, \$46.78, Not Covered)  
Neuren Pharmaceuticals (NEU-ASX, \$0.10, Not Covered)  
Novartis AG (NVS-NYSE, \$93.28, Not Covered)  
Pfizer Inc. (PFE-NYSE, \$29.40, Not Covered)  
PolyMedix, Inc. (PYMXQ-OTC, \$0.02, Not Covered)  
Roche Holding AG (RHHBY-OTC, \$36.13, Not Covered)  
SAGE Therapeutics, Inc. (SAGE-Nasdaq, \$27.39, Not Covered)  
TetraLogic Pharmaceuticals (TLOG-Nasdaq, \$4.70, Not Covered)  
UCB Pharma (UCBJF-Euronext Brussels, \$95.35, Not Covered)

| Marinus Pharmaceuticals               |         |         | 2014E   |         |         |         |         |         | 2015E   |         |         |         |         |         |         |         |         |  |
|---------------------------------------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|---------|--|
| Income Statement (\$MM)               |         | 2013    | 1Q-14   | Q2-14E  | Q3-14E  | Q4-14E  | 2014E   | Q1-15E  | Q2-15E  | Q3-15E  | Q4-15E  | 2015E   | 2016E   | 2017E   | 2018E   | 2019E   | 2020E   |  |
|                                       |         | Mar-14  | Jun-14  | Sep-14  | Dec-14  |         |         | Mar-15  | Jun-15  | Sep-15  | Dec-15  |         |         |         |         |         |         |  |
| Revenue                               |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |  |
| Products                              | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 32.1    |  |
| Total Revenue                         | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 32.1    |  |
| Cost of Services                      |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |  |
| Total Cost of Services                | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 3.2     |  |
| Gross Profit                          | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 28.9    |  |
| Gross margin                          | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | 90.0%   |  |
| SG&A                                  | 1.2     | 0.5     | 0.3     | 0.7     | 0.7     | 2.2     | 0.9     | 0.9     | 0.9     | 0.9     | 3.4     | 3.8     | 4.2     | 8.5     | 23.5    | 75.4    |         |  |
| % of revenue                          | NM      | NM      | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 235.0%  |  |
| Research & development                | 4.2     | 2.1     | 1.3     | 6.0     | 6.0     | 15.4    | 7.0     | 7.0     | 7.0     | 7.0     | 28.0    | 23.0    | 21.0    | 17.0    | 14.0    | 12.8    |         |  |
| % of revenue                          | NM      | NM      | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 40.0%   |  |
| Contingent consideration              | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| % of revenue                          | NM      | NM      | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    |  |
| Amortization of intangible assets     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| % of revenue                          | NM      | NM      | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | NM      | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    |  |
| Depreciation and amortization         | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Gain on sale/Merger expenses          | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Total operating expenses              | 5.4     | 2.7     | 1.6     | 6.7     | 6.7     | 17.7    | 7.9     | 7.9     | 7.9     | 7.9     | 31.4    | 26.8    | 25.2    | 25.5    | 37.5    | 88.2    |         |  |
| Operating Income                      | -5.4    | -2.7    | -1.6    | -6.7    | -6.7    | -17.7   | -7.9    | -7.9    | -7.9    | -7.9    | -31.4   | -26.8   | -25.2   | -25.5   | -37.5   | -59.4   |         |  |
| Operating margin                      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | -185.0% |  |
| Adjusted Operating Income             | -5.1    | -2.6    | -1.6    | -6.7    | -6.7    | -17.6   | -7.8    | -7.8    | -7.8    | -7.8    | -31.3   | -26.7   | -25.1   | -25.3   | -37.3   | -59.2   |         |  |
| Adj. Operating margin                 | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | -184.4% |  |
| EBITDA                                | -5.4    | -2.7    | -1.6    | -6.7    | -6.7    | -17.7   | -7.9    | -7.9    | -7.9    | -7.9    | -31.4   | -26.8   | -25.2   | -25.5   | -37.5   | -59.4   |         |  |
| Stock option compensation             | 0.2     | 0.0     | 0.0     | 0.0     | 0.0     | 0.1     | 0.0     | 0.0     | 0.0     | 0.0     | 0.1     | 0.1     | 0.1     | 0.2     | 0.2     | 0.2     |         |  |
| Other adjustments                     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Adjusted EBITDA                       | -5.1    | -2.6    | -1.6    | -6.7    | -6.7    | -17.6   | -7.8    | -7.8    | -7.8    | -7.8    | -31.3   | -26.7   | -25.1   | -25.3   | -37.3   | -59.2   |         |  |
| Adj EBITDA margin                     | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | -184.4% |  |
| Interest income                       | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Interest expense                      | -0.1    | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Fair value change of derivatives      | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Gain on sale of assets                | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Realized gain (loss) on investments   | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Unrealized gain (loss) on investments | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Litigation settlement                 | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Other                                 | 0.2     | 0.4     | 0.4     | 0.4     | 0.4     | 1.7     | 0.4     | 1.7     | 0.4     | 1.7     | 4.3     | 4.3     | 4.3     | 4.3     | 4.3     | 4.3     |         |  |
| Pretax Income                         | -5.3    | -2.2    | -1.2    | -6.3    | -6.3    | -15.9   | -7.4    | -6.1    | -7.4    | -6.1    | -27.1   | -22.5   | -20.9   | -21.2   | -33.2   | -55.1   |         |  |
| Taxes                                 | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Tax rate                              | 0.5%    | -0.1%   | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    | 0.0%    |         |  |
| Net Income                            | -5.3    | -2.2    | -1.2    | -6.3    | -6.3    | -15.9   | -7.4    | -6.1    | -7.4    | -6.1    | -27.1   | -22.5   | -20.9   | -21.2   | -33.2   | -55.1   |         |  |
| Non-Controlling Interest              | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Net Income                            | -5.3    | -2.2    | -1.2    | -6.3    | -6.3    | -15.9   | -7.4    | -6.1    | -7.4    | -6.1    | -27.1   | -22.5   | -20.9   | -21.2   | -33.2   | -55.1   |         |  |
| Recurring Net Income ex SFAS          | -5.0    | -2.2    | -1.1    | -6.2    | -6.2    | -15.8   | -7.4    | -6.1    | -7.4    | -6.1    | -27.0   | -22.4   | -20.8   | -21.0   | -33.0   | -54.9   |         |  |
| Preferred stock dividends             | -3.8    | -1.1    | -0.5    | 0.0     | 0.0     | -1.6    | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     | 0.0     |         |  |
| Net Income less dividends             | -9.1    | -3.3    | -1.7    | -6.3    | -6.3    | -17.5   | -7.4    | -6.1    | -7.4    | -6.1    | -27.1   | -22.5   | -20.9   | -21.2   | -33.2   | -55.1   |         |  |
| Basic GAAP EPS                        | -\$0.71 | -\$0.28 | -\$0.14 | -\$0.52 | -\$0.45 | -\$1.50 | -\$0.53 | -\$0.44 | -\$0.41 | -\$0.34 | -\$1.69 | -\$1.02 | -\$0.92 | -\$0.92 | -\$1.22 | -\$1.99 |         |  |
| Diluted GAAP EPS                      | -\$0.71 | -\$0.28 | -\$0.14 | -\$0.52 | -\$0.45 | -\$1.50 | -\$0.53 | -\$0.44 | -\$0.41 | -\$0.34 | -\$1.69 | -\$1.02 | -\$0.92 | -\$0.92 | -\$1.22 | -\$1.99 |         |  |
| Adjusted EPS (ex SFAS)                | -\$0.68 | -\$0.27 | -\$0.14 | -\$0.52 | -\$0.45 | -\$1.49 | -\$0.53 | -\$0.43 | -\$0.41 | -\$0.34 | -\$1.68 | -\$1.01 | -\$0.92 | -\$0.91 | -\$1.22 | -\$1.98 |         |  |
| EPS ex dividends                      | -\$1.22 | -\$0.41 | -\$0.20 | -\$0.52 | -\$0.45 | -\$1.65 | -\$0.53 | -\$0.44 | -\$0.41 | -\$0.34 | -\$1.69 | -\$1.02 | -\$0.92 | -\$0.92 | -\$1.22 | -\$1.99 |         |  |
| Basic Shares                          | 7.4     | 8.1     | 8.4     | 12.1    | 13.9    | 10.6    | 14.0    | 14.1    | 18.1    | 18.2    | 16.1    | 22.2    | 22.7    | 23.2    | 27.2    | 27.7    |         |  |
| Fully Diluted shares                  | 7.4     | 8.1     | 8.4     | 12.1    | 13.9    | 10.6    | 14.0    | 14.1    | 18.1    | 18.2    | 16.1    | 22.2    | 22.7    | 23.2    | 27.2    | 27.7    |         |  |
| Margins                               |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |         |  |
| Total Cost of Services                | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | NM      | 10.0%   |  |

Source: Company Reports and Oppenheimer &amp; Co. estimates

**Marinus Pharmaceuticals**

| Marinus Pharmaceuticals                                 |       | 2014E |        |        |        |       | 2015E  |        |        |        |        |        |        |        |        |        |
|---|-------|-------|--------|--------|--------|-------|--------|--------|--------|--------|--------|--------|--------|--------|--------|--------|
| Balance Sheet (\$MM)                                    | F2013 | 1Q-14 | 2Q-14E | 3Q-14E | 4Q-14E | 2014E | 1Q-15E | 2Q-15E | 3Q-15E | 4Q-15E | 2015E  | 2016E  | 2017E  | 2018E  | 2019E  | 2020E  |
| Cash  | 10.0  | 8.8   | 7.7    | 46.5   | 40.3   | 40.3  | 32.9   | 26.8   | 64.4   | 58.4   | 58.4   | 101.1  | 80.5   | 59.6   | 66.7   | 4.7    |
| Marketable Securities                                   | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Restricted Cash   | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Accounts receivable                                     | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 3.5    |
| Inventories, net  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.8    |
| Income taxes receivable/prepaid                         | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Prepaid expenses  | 1.8   | 1.2   | 1.2    | 1.2    | 1.2    | 1.2   | 1.2    | 1.2    | 1.2    | 1.2    | 1.2    | 1.2    | 1.2    | 1.2    | 1.2    | 3.2    |
| Deferred income tax assets                              | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Other current assets                                    | 0.0   | 0.5   | 0.5    | 0.5    | 0.5    | 0.5   | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    | 0.5    |
| Total Current Assets                                    | 11.8  | 10.5  | 9.4    | 48.2   | 42.0   | 42.0  | 34.6   | 28.5   | 66.1   | 60.1   | 60.1   | 102.8  | 82.2   | 61.3   | 68.4   | 12.7   |
| PP&E  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.1    | 0.1    | 0.1    | 0.1    | 0.1    | 0.1    | 0.2    | 0.2    | 0.2    |
| Intangible assets                                       | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| In-process research and development                     | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Goodwill  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Investments, net  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Other assets, net                                       | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 1.3    |
| Total Assets  | 11.8  | 10.6  | 9.4    | 48.2   | 42.0   | 42.0  | 34.6   | 28.6   | 66.2   | 60.1   | 60.1   | 102.9  | 82.3   | 61.4   | 68.5   | 14.1   |
| Accounts payable  | 0.1   | 1.8   | 1.8    | 1.8    | 1.8    | 1.8   | 1.8    | 1.8    | 1.8    | 1.8    | 1.8    | 1.8    | 1.8    | 1.8    | 1.8    | 1.3    |
| Convertible notes payable                               | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Accrued expenses  | 1.1   | 0.3   | 0.3    | 0.3    | 0.3    | 0.3   | 0.3    | 0.3    | 0.3    | 0.3    | 0.3    | 0.3    | 0.3    | 0.3    | 0.3    | 1.0    |
| Returned goods reserve                                  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Deferred revenues                                       | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Current portion of lines of credit and notes payable    | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Accrued compensation                                    | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Income taxes payable                                    | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Other current liabilities                               | 1.2   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Total Current Liabilities                               | 2.4   | 2.0   | 2.0    | 2.0    | 2.0    | 2.0   | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.3    |
| Long-term debt  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Convert Preferred Stock Series A                        | 30.6  | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Convert Preferred Stock Series B                        | 17.9  | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Convert Preferred Stock Series C                        | 21.3  | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Convert Preferred Stock Series D                        | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Contingent long-term payables                           | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Deferred rent   | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Income tax receivable                                   | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Deferred tax liabilities                                | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Contingent consideration                                | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Other LT Liabilities                                    | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Total Liabilities                                       | 72.2  | 2.0   | 2.0    | 2.0    | 2.0    | 2.0   | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.0    | 2.3    |
| Preferred stock   | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Common stock  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Treasury stock  | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Additional paid-in capital                              | 1.1   | 72.3  | 72.3   | 117.3  | 117.3  | 117.3 | 117.4  | 117.4  | 162.4  | 162.5  | 162.5  | 227.6  | 227.7  | 227.9  | 268.1  | 268.3  |
| Accumulated other comprehensive loss                    | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Retained earnings                                       | -61.5 | -63.7 | -64.9  | -71.1  | -77.4  | -77.4 | -84.8  | -90.9  | -98.3  | -104.4 | -104.4 | -126.7 | -147.5 | -168.5 | -201.6 | -256.4 |
| Marinus Total Equity                                    | -60.4 | 8.5   | 7.4    | 46.2   | 40.0   | 40.0  | 32.6   | 26.5   | 64.2   | 58.1   | 58.1   | 100.9  | 80.3   | 59.4   | 66.5   | 11.8   |
| Noncontrolling interest                                 | 0.0   | 0.0   | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    | 0.0    |
| Total Equity  | -60.4 | 8.5   | 7.4    | 46.2   | 40.0   | 40.0  | 32.6   | 26.5   | 64.2   | 58.1   | 58.1   | 100.9  | 80.3   | 59.4   | 66.5   | 11.8   |
| Total Liabilities and Equity                            | 11.8  | 10.6  | 9.4    | 48.2   | 42.0   | 42.0  | 34.6   | 28.6   | 66.2   | 60.1   | 60.1   | 102.9  | 82.3   | 61.4   | 68.5   | 14.1   |
| Source: Company Reports and Oppenheimer & Co. estimates |       |       |        |        |        |       |        |        |        |        |        |        |        |        |        |        |

Source: Company Reports and Oppenheimer & Co. estimates



| Marinus Pharmaceuticals                   |      | 2014E  |        |        |        |       |        | 2015E  |        |        |       |       |       |       |       |       |  |
|---|------|--------|--------|--------|--------|-------|--------|--------|--------|--------|-------|-------|-------|-------|-------|-------|--|
| Statement of cash flow (\$MM)             | 2013 | 1Q-14E | 2Q-14E | 3Q-14E | 4Q-14E | 2014E | 1Q-15E | 2Q-15E | 3Q-15E | 4Q-15E | 2015E | 2016E | 2017E | 2018E | 2019E | 2020E |  |
| Operating Activities                      |      |        |        |        |        |       |        |        |        |        |       |       |       |       |       |       |  |
| Net Income                                | -5.3 | -2.2   | -1.2   | -6.3   | -6.3   | -15.9 | -7.4   | -6.1   | -7.4   | -6.1   | -27.1 | -22.5 | -20.9 | -21.2 | -33.2 | -55.1 |  |
| Income from discount. ops, net of tax     | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Depreciation and amortization             | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Non-cash interest on converts             | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Amortization of deferred financing        | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Losses from investments in investees      | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Stock-based compensation expense          | 0.2  | 0.0    | 0.0    | 0.0    | 0.0    | 0.1   | 0.0    | 0.0    | 0.0    | 0.0    | 0.1   | 0.1   | 0.1   | 0.2   | 0.2   | 0.2   |  |
| Provision for bad debts                   | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Provision for inventory obsolescence      | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Revenue from receipt of equity            | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Realized Loss on investments              | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Loss on conversion of converts            | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Loss (Gain) on disposal of assets         | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Change in fair value of derivatives       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Change in fair value of cont. consid.     | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Unrealized (gain) loss on investments     | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Gain on litigation settlement             | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Proceeds from litigation settlement       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Noncash interest on convertible notes p   | 0.1  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Cumulative earnings less than (in excess  | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Change in allowance for doubtful debts    | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Deferred income tax                       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Other                                     | -0.2 | -0.4   | 0.0    | 0.0    | 0.0    | -0.4  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Change in operating assets & liabilities  |      |        |        |        |        |       |        |        |        |        |       |       |       |       |       |       |  |
| Accounts receivable                       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | -3.5  |  |
| Inventories                               | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | -0.8  |  |
| Prepaid expenses                          | -1.7 | 0.6    | 0.0    | 0.0    | 0.0    | 0.6   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | -2.0  |  |
| Other assets                              | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | -1.3  |  |
| Income taxes receivable/payable           | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Accounts payable and other                | 0.2  | 0.9    | 0.0    | 0.0    | 0.0    | 0.9   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | -0.5  |  |
| Foreign currency measurement              | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Accrued expenses                          | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.7   |  |
| Rebates, chargebacks and returns payab    | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Other current assets and liabilities      | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Deferred revenues                         | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Cash flow from operations                 | -6.6 | -1.1   | -1.1   | -6.2   | -6.2   | -14.7 | -7.4   | -6.1   | -7.4   | -6.1   | -27.0 | -22.4 | -20.7 | -21.0 | -33.0 | -62.1 |  |
| Cash flow from discount. operations       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Net cash flow from operations             | -6.6 | -1.1   | -1.1   | -6.2   | -6.2   | -14.7 | -7.4   | -6.1   | -7.4   | -6.1   | -27.0 | -22.4 | -20.7 | -21.0 | -33.0 | -62.1 |  |
| Investing Activities                      |      |        |        |        |        |       |        |        |        |        |       |       |       |       |       |       |  |
| Investments in investees                  | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Proceeds from sale of equity securities   | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Acquisition of businesses, net of cash ac | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Purchase of marketable securities         | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Maturities of ST marketable securities    | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Proceeds from sale of assets              | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Purchase of PP&E                          | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | -0.1  | -0.1  |  |
| Deferred payments on acquisitions         | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Acquisition of licensing technology       | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Investment in capitalized software        | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Cash flow from investing                  | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | -0.1  | -0.1  |  |
| Financing Activities                      |      |        |        |        |        |       |        |        |        |        |       |       |       |       |       |       |  |
| Change in debt                            | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Payment of dividends                      | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Proceeds from stock options               | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.1   | 0.0    | 0.0    | 0.0    | 0.0    | 0.1   | 0.1   | 0.1   | 0.2   | 0.2   | 0.2   |  |
| Payments of tax withholdings/stock opti   | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Noncontrolling interest distribution      | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Excess tax benefit from stock options     | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Capital contributions                     | 8.0  | -0.1   | 0.0    | 45.0   | 0.0    | 44.9  | 0.0    | 0.0    | 45.0   | 0.0    | 45.0  | 65.0  | 0.0   | 0.0   | 40.0  | 0.0   |  |
| Other                                     | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Cash flow from financing                  | 8.0  | -0.1   | 0.0    | 45.0   | 0.0    | 45.0  | 0.0    | 0.0    | 45.0   | 0.0    | 45.1  | 65.1  | 0.1   | 0.2   | 40.2  | 0.2   |  |
| Effect of exchange rate changes           | 0.0  | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0    | 0.0    | 0.0    | 0.0    | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   | 0.0   |  |
| Beginning cash                            | 8.6  | 10.0   | 8.8    | 7.7    | 46.5   | 10.0  | 40.3   | 32.9   | 26.8   | 64.4   | 40.3  | 58.4  | 101.1 | 80.5  | 59.6  | 66.7  |  |
| Net change in cash                        | 1.4  | -1.2   | -1.1   | 38.8   | -6.2   | 30.2  | -7.4   | -6.1   | 37.6   | -6.1   | 18.1  | 42.7  | -20.6 | -20.9 | 7.1   | -62.0 |  |
| Cash Balance Adj for divestiture          |      |        |        |        |        |       |        |        |        |        |       |       |       |       |       |       |  |
| Ending cash                               | 10.0 | 8.8    | 7.7    | 46.5   | 40.3   | 40.3  | 32.9   | 26.8   | 64.4   | 58.4   | 58.4  | 101.1 | 80.5  | 59.6  | 66.7  | 4.7   |  |

Source: Company Reports and Oppenheimer &amp; Co. estimates

## Investment Thesis

We are initiating coverage of Marinus Pharmaceuticals with an Outperform rating and 12- to 18-month \$12 price target. Marinus is a clinical stage biopharmaceutical company focused on developing and commercializing neuropsychiatric therapeutics. The company's clinical stage product, ganaxolone, is a small molecule, add-on therapy for the treatment of partial onset seizures in adults with epilepsy. Our investment thesis for Marinus is threefold: (1) ganaxolone will show positive safety and efficacy results in phase 2b and potential phase 3 studies, (2) the company will gain FDA acceptance for ganaxolone in the epilepsy indication some time in the late 2019 or early 2020 time frame, and (3) ganaxolone will penetrate the epilepsy market as adjunctive treatment to existing therapy.

## Price Target Calculation

We believe fair value for Marinus' shares is \$12, which represents ~35% of potential upside from current levels. Our valuation is based on a discounted cash flow methodology. Using a discount rate of approximately ~25%, and an exit EBITDA multiple of 4.0x on the estimated 2025 terminal year EBITDA of close to \$500 million, we arrive at a \$12 per share valuation. The 25% discount rate, we believe, adequately reflects the clinical development risk that yet remains with ganaxolone along with the execution risk for commercialization.

## Key Risks to Price Target

Key risks to Marinus' outperformance and price target include include the following: clinical development risk and either a failure by ganaxolone to meet clinical trial endpoints or show efficacy, or an inability by Marinus to gain approval from the FDA for ganaxolone; a failure to successfully introduce ganaxolone into the market or a failure to penetrate the highly competitive epilepsy market; an inability to gain formulary acceptance or to receive unfavorable payor reimbursement; a failure to develop a commercial organization capable of manufacturing, selling, marketing and distributing any products that the company intends to sell in the markets in which it chooses to commercialize on its own; the performance of third-party CROs and CMOs; forecasting error in determining Marinus' market opportunity due to the company's lack of having yet established a track record of profitability; limited intellectual property (IP) to protect and control trade secrets and other technological innovation; the ability of competitors to independently develop similar or better proprietary information and techniques and disclose them publicly; the success of competing therapies and products that are or become available; Medicare, Medicaid, and insurer increased efforts to manage drug spend; economic weakness impacting unemployment, commercial insurance coverage, and utilization; a small float which could lead to illiquidity; and liquidity risk as more than 50% of the shares are tied up through insider holdings. Other risks include that the company may require substantial additional funding or may finance future cash needs primarily through public or private offerings, debt financings or strategic collaborations, which may dilute stockholdings in the company.

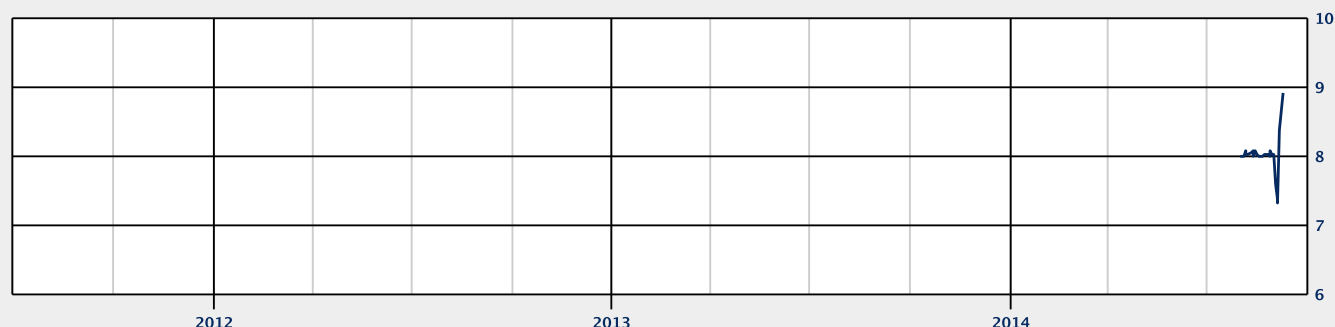
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## Rating and Price Target History for: Marinus Pharmaceuticals (MRNS) as of 09-08-2014



Created by BlueMatrix

All price targets displayed in the chart above are for a 12- to 18-month period. Prior to March 30, 2004, Oppenheimer & Co. Inc. used 6-, 12-, 12- to 18-, and 12- to 24-month price targets and ranges. For more information about target price histories, please write to Oppenheimer & Co. Inc., 85 Broad Street, New York, NY 10004, Attention: Equity Research Department, Business Manager.

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## Distribution of Ratings/IB Services Firmwide

| Rating           | IB Serv/Past 12 Mos. |         |       |         |
|------------------|----------------------|---------|-------|---------|
|                  | Count                | Percent | Count | Percent |
| OUTPERFORM [O]   | 317                  | 52.14   | 145   | 45.74   |
| PERFORM [P]      | 281                  | 46.22   | 97    | 34.52   |
| UNDERPERFORM [U] | 10                   | 1.64    | 3     | 30.00   |

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