



### **Aegerion Pharmaceuticals**

AEGR: NASDAO: US\$10.84

**Target: US\$16.00** 

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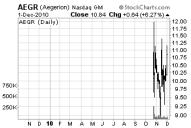
#### **COMPANY STATISTICS:**

Market Cap (M): U\$\$171.9 52-week Range: U\$\$9.00 - 12.00

#### **EARNINGS SUMMARY:**

| FYE Dec  |    | 2009A  | 2010E   | 2011E  |  |  |
|----------|----|--------|---------|--------|--|--|
| Revenue: |    | 0.0    | 0.0     | 0.0    |  |  |
| EPS:     |    | (9.19) | (3.17)  | (1.87) |  |  |
|          |    |        |         |        |  |  |
| Revenue: | Q1 | -      | 0.0A    | -      |  |  |
|          | Q2 | -      | 0.0A    | -      |  |  |
|          | Q3 | -      | 0.0A    | -      |  |  |
|          | Q4 | -      | 0.0E    | -      |  |  |
| Total    |    | 0.0    | 0.0     | 0.0    |  |  |
| EPS:     | Q1 | -      | (1.41)A | -      |  |  |
|          | Q2 | -      | (1.37)A | -      |  |  |
|          | Q3 | -      | (3.60)A | -      |  |  |
|          | Q4 | -      | (0.38)E | -      |  |  |
| Total    |    | (9.19) | (3.17)  | (1.87) |  |  |

#### **SHARE PRICE PERFORMANCE:**



#### **COMPANY DESCRIPTION:**

Aegerion Pharmaceuticals is an emerging biopharmaceutical company focused on novel therapeutics to treat severe but rare genetic lipid disorders. The company's lead drug, lomitapide, is currently in pivotal development for homozygous familial hypercholesterolemia, characterized by very high LDL levels that do not respond well to statin therapy.

All amounts in US\$ unless otherwise noted.

Life Sciences -- Biotechnology

**BUY** 

# SECOND CHANCE WITH AN ORPHAN INDICATION AND POWERFUL DATA

#### **Investment recommendation**

Initiating with BUY, \$16 target on lomitapide's potential as a best-inclass HoFH drug. Lomitapide is Aegerion's Phase 3 oral MTP inhibitor for homozygous familial hypercholesterolemia (HoFH). We think lomitapide may become the best-in-class HoFH drug on strong LDL lowering and good safety and tolerability. We think final pivotal data will be positive and that Aegerion will submit the lomitapide NDA for HoFH in late 2011 and receive FDA approval mid-2012. Our \$16 target is based on a pNPV analysis.

#### **Investment highlights**

Lomitapide has shown best-in-class LDL lowering of ~50% on top of optimized background therapy. Lomitapide's open-label pivotal trial is ongoing but has already yielded primary endpoint data showing 50% LDL reduction from baseline with optimized background therapy. 35% of patients were able to achieve LDL<100 mg/dL despite mean baseline LDL of 354 mg/dL, suggesting a very clinically meaningful benefit.

We think lomitapide has good safety, acceptable tolerability given HoFH's severity and unmet medical need. No patients discontinued due to changes in liver enzymes. We think lomitapide has a mild, transient impact on liver enzymes and drug-associated liver fat elevations are transient and may not be clinically meaningful. We also think GI side effects are manageable, improving with duration of treatment.

We think lomitapide has a high chance of regulatory and commercial success. We think lomitapide will successfully complete the remaining safety portion of a pivotal trial and other small PK safety studies. Given HoFH's unmet medical need, we think lomitapide will receive expedited review and approval in 2012 with a broad label. We think the drug will be appropriate for ~4,000-6,000 patients worldwide and predict worldwide peak sales of over \$400M in HoFH alone.

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#### **INVESTMENT THESIS**

## Aegerion's MTP-1 inhibitor lomitapide is a promising Phase 3 drug for homozygous familial hypercholesterolemia (HoFH)

Lomitapide is an oral, small molecule MTP inhibitor therapy in Phase 3 development for HoFH. We think that all efficacy and safety data for lomitapide in HoFH at its current dose and treatment regimen are very positive. HoFH, a rare genetic disease that leads to very high cholesterol and LDL levels, represents a significant unmet medical need. Due to the nature of the disease, HoFH patients do not experience the same proportionate amount of cholesterol reduction with standard drugs and treatment as normal high cholesterol patients do. As a result, most HoFH patients have very poor disease control, which in turn leads to very poor disease prognosis. We believe that lomitapide may help a substantial proportion of HoFH patients reach their LDL goals and reduce cardiovascular risk and events.

#### Lomitapide may be the best-in-class late clinical development-stage HoFH drug

There are currently only two late-stage drugs in development for treatment of HoFH. Isis Pharmaceuticals and partner Genzyme are developing a Phase 3 antisense drug called mipomersen, which may also be approved in 2012. However, we believe that of these two development-stage treatments, lomitapide is more effective in lowering LDL and has a superior safety and tolerability profile. We think lomitapide has significant potential to become the gold standard HoFH treatment for use in conjunction with statins and LDL apheresis (a type of blood filtering similar to dialysis).

#### Lomitapide Phase 3 clinical efficacy shows very strong LDL lowering in HoFH

Phase 2 and 3 lomitapide data, both from the current pivotal trial as well as previous Phase 2 and 3 trials, have consistently shown ~50% LDL reduction with drug treatment. This compares to ~25% LDL reduction seen in Phase 3 trials of Isis' mipomersen. 35% of patients were able to achieve LDL<100 mg/dL despite mean baseline LDL of 354 mg/dL, suggesting very clinically meaningful benefit. Additionally, lomitapide has consistently shown 50%-60% reduction in triglyceride (TG) levels, which is another key blood fat level for heart health. We think that the current lower-dose lomitapide treatment regimen (compared to previous trials) will continue to show these levels of LDL and TG reduction in real-world clinical experience. In addition, we think that lomitapide has the potential to induce incremental weight loss in HoFH, which may improve patients' metabolic profiles and cardiac risk.

#### Final Phase 3 safety data will likely resemble current positive safety data

Safety and tolerability have previously been a concern for lomitapide. Its key safety and tolerability issues are hepatic fat, liver enzyme elevations and diarrhea, which occurred at problematic rates in Phase 2 trials. We note these trials were in broader populations and used a higher dose (100mg) than that of the current Phase 3 trial (60mg). In addition, the previous trials did not employ dose titration in the treatment protocol (dose titration is part of the current protocol). Aegerion has already substantially completed the pivotal lomitapide trial, and 22 patients who had hepatic fat measurements taken had modest increases in hepatic fat from a mean of 1.2% to 8.7% at 26 weeks. Fourteen patients had a 56-week mean hepatic fat level of 5.1%. Median change in hepatic fat from baseline in



these patients was 5.48% at 26 weeks of treatment and 2.14% at 56 weeks of treatment. Data consistently suggests that hepatic fat rises initially but stabilizes and then falls around 26 weeks of therapy, likely due to an inherent desensitization mechanism. Furthermore, based on our conversations with lipid experts, we do not think that these elevations in hepatic fat are clinically meaningful. No patients have discontinued thus far due to liver enzyme elevations. So far, four trial patients had ALT elevations (>5x but <11x the upper limit of normal), three of whom had a temporary dose reduction and have since been rechallenged at higher doses. One patient discontinued lomitapide for seven weeks, but was then safely put back on the drug.

### Experts familiar with lomitapide believe that lomitapide is tolerable; patients may manage common treatment side effects with relative ease

Phase 2 lomitapide trial data showed that diarrhea and GI distress were the common side effects of therapy, and very likely a result of the drug's direct mechanism of action. We note that Phase 2 gastrointestinal side effect rates were around 50%, and led to significant drop-outs in the patient population (which we note is significantly more broad with much less severe disease than HoFH). Aegerion has noted that this side effect profile is similar in the current Phase 3 trial, but with lower incident rates and severity. Only three patients (~10%) have dropped out thus far due to GI side effects. Experts who have experience with lomitapide note that patient diarrhea is significantly less severe with dose titration and that there appears to be some degree of desensitization. They have indicated that patients tend to experience significantly fewer and less severe episodes of diarrhea and GI distress with continued lomitapide treatment. They believe that patient compliance will not be a major issue with lomitapide given the degree of clinical benefit widely seen with the drug.

# We see lomitapide's risk/benefit profile as very acceptable given HoFH's unmet medical need and think the drug will receive FDA and EMEA approval in 2012

HoFH is a very severe and poorly managed disease. The average age of a HoFH patient's first cardiovascular adverse event (usually stroke or heart attack) is about 20, and most patients die of a heart attack before reaching the age of 30. Statins have limited efficacy in these patients due to disease mechanism. Regular LDL apheresis sessions can markedly improve disease; however, most patients are not treated at optimal frequencies due to logistical and tolerability difficulties as well as treatment cost. We think that given this very significant need for better HoFH treatments and lomitapide's strong efficacy in lowering LDL, the drug will be approved in a straightforward and accelerated manner. We think the drug will receive Fast Track Status and priority review. We think FDA will convene an advisory committee meeting for lomitapide in H1/12, which we predict will go very favorably. We anticipate lomitapide approval by both the FDA and EMEA in 2012.

#### Lomitapide may become an appropriate treatment for 4,000-6,000 FH patients

We think that lomitapide will see very broad use in almost all patients with genotypically diagnosed HoFH, estimated at about 1,000 patients worldwide. We also think that it will see significant use in patients with "phenotypically diagnosed" FH, where fibroblast cell cultures show malfunctioning LDL receptors, and in patients with familial histories of hypercholesterolemia (despite lack of mutation identification). We estimate this patient population to be an additional 2,000 patients worldwide. Finally, we also think that some number of patients with "functional FH" will also received treatment with lomitapide. These patients have very high total cholesterol and LDL levels that are resistant to statin



treatment, suggesting LDL receptor dysfunction despite no identified FH mutation or well documented family history of high cholesterol. We think that these patients represent another 3,000 total worldwide population, and believe that some meaningful proportion will be treated with lomitapide.

#### We estimate peak annual worldwide Lomitapide sales of ~\$500M with rapid uptake

As Aegerion is developing lomitapide to address an orphan population of HoFH and closely related populations, we think the company will be able to employ the orphan pricing model with little difficulty. We think that lomitapide will be priced at lowest around \$100,000 annually, and at highest around \$250,000. We think that the more restricted lomitapide's final label is, the higher the treatment price, resulting in about the same approximate \$500M annual revenue rate to Aegerion regardless of pricing.

#### **INVESTMENT RISKS**

#### Development risk - Previous clinical trials have shown problematic safety/tolerability

Previous higher dose non-titration lomitapide trials have shown rates of liver fat and liver enzyme elevations that were deemed unacceptable by clinicians for treatment of a broad patient population with moderately elevated LDL levels. Additionally, GI tolerability in these trials was very poor. As a result, lomitapide clinical development was discontinued for some time. Although safety and tolerability data to date is significantly better due to lower dose and titration, some patients still experience significant changes in hepatic fat and liver enzyme levels as well as diarrhea. These side effects could reach problematic levels, but we think the data thus far suggests the drug, at its current dose and treatment schedule, will be a safe and relatively well-tolerated therapy.

# Regulatory risk – Despite unmet need, a single, open-label, uncontrolled Phase 3 trial may not be sufficient to secure FDA or European approval

The FDA normally requires two randomized placebo-controlled pivotal trials for drug approval. Aegerion plans to submit the lomitapide NDA with data from a single uncontrolled open-label Phase 3 trial with a small number of patients. Also, the company does not have a Special Protocol Assessment (SPA) from the FDA, although it has had extensive discussions with the agency as part of the SPA process. We believe the FDA will still approve lomitapide despite its limited Phase 3 data set due to the orphan nature of HoFH as well as the disease's unmet medical need. We think that a placebo-controlled trial would have been unethical, and that a larger trial was not feasible due to the small HoFH patient population. Furthermore, the FDA has previously approved orphan therapies with serious unmet medical needs based on single open-label uncontrolled trials.

# Commercial risk – Lomitapide may not have as large a market as estimated, since current market assumptions are relatively new and as yet unproven

While there is little dispute on the number of HoFH patients with definitive genotypic diagnosis (600-1,000 patients worldwide), there is controversy over the additional number of HoFH patients whose exact genetic mutations have not yet been identified. Lomitapide may not be approved or reimbursed for patients with LDL levels characteristic of HoFH but without genotypic, cell culture or familial history diagnosis. Furthermore, Aegerion may face pricing pressure on lomitapide's orphan pricing. As such, the exact potential patient population and market size for lomitapide is uncertain.



Competitive risk – Lomitapide may compete with Isis' mipomersen, which is partnered with Genzyme, a large-cap biotechnology with an established orphan business unit.

We believe that lomitapide may be approved for HoFH around the same time as Isis Pharmaceuticals' mipomersen, which is partnered with Genzyme. We note that Genzyme has pioneered the orphan disease business model and has considerable experience at launching and commercializing orphan drugs. However, while Genzyme would represent significant commercial competitive entity, we think that lomitapide still has a very good chance at becoming the gold standard HoFH treatment based on its superior efficacy, safety and ease of use, as well as its (at worst) comparable tolerability.

#### **VALUATION**

We have built our valuation of Aegerion using a model that we believe is the appropriate method of capturing the value of Aegerion's potential pipeline.

| Figure 1: Aegerion pNPV analysis) |                              |         |        |         |                  |              |               |         |
|-----------------------------------|------------------------------|---------|--------|---------|------------------|--------------|---------------|---------|
| Drug name                         | Indication                   | Status  | Launch | Success | Peak sales (\$M) | Royalty Rate | Profitability | NPV     |
| Lomitapide                        | Genotypically diagnosed HoFH | Phase 3 | 2012   | 65%     | \$89             | 90%          | 75%           | \$3.93  |
| Lomitapide                        | Expanded (Phase 3) HoFH      | Phase 3 | 2012   | 65%     | \$187            | 90%          | 75%           | \$8.26  |
| Lomitapide                        | Functional HoFH              | Phase 3 | 2012   | 40%     | \$140.5          | 90%          | 75%           | \$3.81  |
| TOTAL                             |                              |         |        |         |                  |              |               | \$16.00 |

Source: Canaccord Genuity estimates

#### Potential upside to valuation

We see the following as potential drivers of upside to our model:

- Stronger-than-expected clinical safety data from lomitapide. Lomitapide's side effects are very much related to the drug's mechanism of action. Side effect rates are currently well within expected and relatively safe parameters. Should side effect rates or severity fall toward trial completion, it could mean significant upside to our chances of approval, market estimates (especially in the functional HoFH population) and valuation.
- Delays in the clinical or commercial development of lomitapide's major competitor. We expect that Isis/Genzyme's HoFH drug mipomersen will be approved around 2012. However, carcinogenicity tests are still ongoing and side effects have raised questions on the drug's commercial viability. Genzyme is currently the subject of a hostile takeover attempt by Sanofi Aventis, and change of control could jeopardize the mipomersen commercial strategy. Any delay to mipomersen would mean upside to our market model for lomitapide.
- Acquisition at a significant premium to current market value. We think that Aegerion
  represents a compelling acquisition candidate, as lomitapide is a very promising drug
  candidate and Aegerion as a company represents a very therapeutically focused and
  discrete acquisition candidate. We note that many large pharma companies are
  currently launching new orphan drug business units and looking to fill pipelines with
  late-stage drug candidates. Should Aegerion be acquired, the purchase price may
  represent upside to our target price.



#### Potential downside to valuation

As with all companies in preclinical or clinical development, there always exists the risk of failed or inconclusive clinical trials, which would lead to downward pressure on the stock.

#### RECOMMENDATION

We think that Aegerion's lomitapide is a very promising new therapy for patients who are homozygous for familial hypercholesterolemia (HoFH) or have the HoFH cholesterol profile phenotype without the formally diagnosed genotypic mutation.

The lomitapide Phase 3 pivotal study has already yielded very positive efficacy data, with primary endpoint of LDL reduction of ~50%. Secondary endpoint triglyceride reduction has also been very positive. Safety data has shown hepatic fat and liver enzyme level changes that we think are transient and not clinically significant, and we think the tolerability profile thus far will be manageable and acceptable to patients. We think that final safety and efficacy data (due H1/11) will be very similar to data released thus far.

We think that Aegerion will successfully complete additional preclinical requirements before the end of 2011 and will submit the lomitapide NDA to the FDA shortly thereafter. We expect that the FDA will schedule an advisory committee for lomitapide in H1/12 that will be a key event in the drug's approval pathway. Given the unmet medical need in HoFH, we expect this advisory committee meeting to go positively, with the panel recommending approval.

We see lomitapide's chances of approval as high (65%+). We expect that the drug will be approved by the FDA around mid-year 2012 with a label for treatment of genotypically and phenotypically diagnosed HoFH patients. We think the drug has a good chance for approval (45%) for use in "functional" HoFH patients as well.

We believe that lomitapide will see very rapid market uptake given the unmet medical need and orphan disease treatment dynamics of HoFH. We think that Aegerion will commercialize the drug itself using a very small, efficient sales force targeting the small number of doctors and tertiary care centers that provide treatment for HoFH patients. We think that Aegerion will also set up very effective clinician and patient outreach programs and develop strong relationships with FH patient advocacy groups. We believe the company will also set up effective patient assistance and coverage assistance programs to help with securing insurance coverage of lomitapide, especially for patients with functional HoFH. Overall, we expect peak worldwide lomitapide sales to reach \$400-\$500M.

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| Sales forecasts                                           | 2010E | 2011E | 2012E        | 2013E        | 2014E        | 2015E         |
|-----------------------------------------------------------|-------|-------|--------------|--------------|--------------|---------------|
| oules forecasts                                           | 20102 | 20112 | ZUIZL        | ZUTUL        | 20142        | 20101         |
| US Genotype Diagnosed HoFH                                |       |       |              |              |              |               |
| # US pts                                                  | 300   | 308   | 315          | 323          | 331          | 339           |
| growth rate (increasing diagnosis/survival)               | 2.5%  | 2.5%  | 2.5%         | 2.5%         | 2.5%         | 2.5%          |
| Estimated lopitamide market penetration                   | 0.0%  | 0.0%  | 75.0%        | 90.0%        | 95.0%        | 95.0%         |
| # of US HoFH patients on lopitamide                       | 0     | 0     | 236          | 291          | 315          | 322           |
| US lopitamide revenue per patient (3% yoy \$ increase)    | \$0.0 | \$0   | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| US genotype HoFH lopitamide revenues to Aegerion          | \$0   | \$0   | \$35,458,594 | \$44,922,492 | \$50,061,750 | \$52,852,693  |
| EU/SA Genotype Diagnosed HoFH                             |       |       |              |              |              |               |
| # EU pts                                                  | 300   | 305   | 309          | 314          | 318          | 323           |
| growth rate (increasing diagnosis/survival)               | 1.5%  | 1.5%  | 1.5%         | 1.5%         | 1.5%         | 1.5%          |
| Estimated market penetraion                               | 0.0%  | 0.0%  | 75.0%        | 90.0%        | 95.0%        | 95.0%         |
| # of EU HoFH patients on lopitamide                       | 0     | 0     | 232          | 282          | 302          | 307           |
| EU lopitamide revenue per patient (3 % yoy \$ increase)   |       |       | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| EU/SA genotype HoFH lopitamide revenues to Aegerion       |       |       | \$34,770,094 | \$43,620,473 | \$48,136,525 | \$50,324,330  |
|                                                           |       |       |              |              |              |               |
| US Inheritance Pattern/Fibroblast Assay FH                |       |       |              |              |              |               |
| #US pts                                                   | 1,000 | 1,025 | 1,051        | 1,077        | 1,104        | 1,131         |
| growth rate (increasing diagnosis/survival)               | 2.5%  | 2.5%  | 2.5%         | 2.5%         | 2.5%         | 2.5%          |
| Estimated lopitamide market penetration                   | 0.0%  | 0.0%  | 25.0%        | 45.0%        | 55.0%        | 60.0%         |
| # of US HoFH patients on lopitamide                       | 0     | 0     | 263          | 485          | 607          | 679           |
| US lopita mide revenue per patient (3 % yoy \$ increase)  | \$0.0 | \$0   | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| US Lopitamide IP/FA Revenues                              | \$0   | \$0   | \$39,398,438 | \$74,870,821 | \$96,610,395 | \$111,268,827 |
| EU/SA Inheritance Pattern/Fibroblast Assay FH             |       |       |              |              |              |               |
| # EU/SA pts                                               | 1,000 | 1,015 | 1,030        | 1,046        | 1,061        | 1,077         |
| growth rate (increasing diagnosis/survival)               | 1.5%  | 1.5%  | 1.5%         | 1.5%         | 1.5%         | 1.5%          |
| Estimated lopitamide market penetration                   | 0.0%  | 0.0%  | 25.0%        | 45.0%        | 55.0%        | 60.0%         |
| # of EU/SA HoFH patients on lopitamide                    | 0     | 0     | 258          | 471          | 584          | 646           |
| EU/SA lopitamide revenue per patient (3% yoy \$ increase) |       |       | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| EU/SA lopitamide IP/FA Revenues                           |       |       | \$38,633,438 | \$72,700,789 | \$92,895,049 | \$105,945,959 |
|                                                           |       |       |              |              |              |               |
| US Functional FH                                          |       |       |              |              |              |               |
| # US pts                                                  | 1,500 | 1,538 | 1,576        | 1,615        | 1,656        | 1,697         |
| growth rate (increasing diagnosis/survival)               | 2.5%  | 2.5%  | 2.5%         | 2.5%         | 2.5%         | 2.5%          |
| Estimated lopitamide market penetration                   | 0.0%  | 0.0%  | 10.0%        | 20.0%        | 25.0%        | 30.0%         |
| # of US HoFH patients on lopitamide                       | 0     | 0     | 158          | 323          | 414          | 509           |
| US lopitamide revenue per patient (3% yoy \$ increase)    | \$0.0 | \$0   | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| US lopitamide Functional Revenues                         | \$0   | \$0   | \$23,639,063 | \$49,913,880 | \$65,870,724 | \$83,451,620  |
| EU/SA Functional FH                                       |       |       |              |              |              |               |
| # EU/SA pts                                               | 1,500 | 1,523 | 1,545        | 1,569        | 1,592        | 1,616         |
| growth rate (increasing diagnosis/survival)               | 1.5%  | 1.5%  | 1.5%         | 1.5%         | 1.5%         | 1.5%          |
| Estimated lopitamide market penetration                   | 0.0%  | 0.0%  | 10.0%        | 20.0%        | 25.0%        | 30.0%         |
| # of EU/SA HoFH patients on lopitamide                    | 0     | 0     | 155          | 314          | 398          | 485           |
| EU/SA lopitamide revenue per patient (3% yoy \$ increase) |       |       | \$150,000    | \$154,500    | \$159,135    | \$163,909     |
| EU/SA lopitamide IP/FA Revenues                           |       |       | \$23,180,063 | \$48,467,193 | \$63,337,533 | \$79,459,469  |

Source: Canaccord Genuity



#### **FINANCIALS**

Our forecast financial model is built on the assumption that lomitapide will be approved around early 2012 with a broad label encompassing HoFH patients who have been genotypically and phenotypically diagnosed. Our revenue model does not assume that lomitapide's label will include the group of patients we have referred to as "functional HoFH"; we do assume that the most severe of these patients will likely receive lomitapide treatment off-label.

We assume that lomitapide has standard small-molecule, single-digit percentage cost of goods and relatively simple manufacturing synthesis parameters. We also assume that Aegerion will choose to launch and market the drug itself in the US, Europe and possibly South Africa. We believe that the company will likely strike commercialization partnerships for other rest of world territories, but have not modeled any possible royalties as we think they would likely be immaterial to overall revenues.

We expect AMR101's launch to be successful and relatively rapid given HoFH's unmet medical need. We expect rapid sales growth in the 2012 and 2013 time frame. We think that lomitapide will generally be regarded as the best-in-class drug for HoFH treatment, and do not think that our revenue estimates will be greatly impacted by potential approval and launch of Isis/Genzyme's mipomersen. We think patients and clinicians will overwhelmingly prefer lomitapide due to its ease of use, stronger efficacy, and potentially better tolerability profile. We assume 95% peak market penetration of the genotypic HoFH market, 60% peak penetration of the phenotypic HoFH market and 30% peak penetration of the functional HoFH market

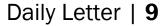
At the end of June 30, 2010, Amarin had \$5 million in cash, but raised close to \$45M in its October IPO. We think total cash on hand represents at least 18 months of operation, and we think it is likely that the current cash balance will last through first lomitapide revenues in H1/12.

#### **MANAGEMENT**

Figure 3: Management team

| Name                       | Title                                              | Experience prior to Aegerion                                                                                   | At Aegerion since: |
|----------------------------|----------------------------------------------------|----------------------------------------------------------------------------------------------------------------|--------------------|
| Marc D. Beer               | CEO                                                | Founding CEO of ViaCell;<br>VP Global Marketing, Genzyme<br>Abbott Labs                                        | August 2010        |
| Will H. Lewis              | President                                          | Director, BayStar Capital;<br>Managing Director, Head of Capital<br>Markets Investment Banking, Wells<br>Fargo | 2005               |
| Christine A.<br>Pellizzari | Executive Vice<br>President and<br>General Counsel | Pfizer Global Research and<br>Development                                                                      | 2007               |

Source: Company reports





We believe that the Aegerion senior management has extensive experience in the biotechnology and pharmaceutical industry. Notably, CEO Marc Beer founded and led ViaCell through commercial development, its IPO process and ultimate acquisition by Perkins Elmer. Mr. Beer has also had extensive experience with the commercialization and marketing of orphan drugs from his experience at Genzyme, which is considered the flagship orphan disease-focused biotechnology firm. President Will Lewis has had extensive experience in the field of biotechnology finance and capital markets.



Figure 4: Aegerion P&L

|                                           | 2009A  | Q1/10A     | Q2/10A     | Q3/10A        | Q4/10E        | 2010E  | 2011E  | 2012E | 2013E  |
|-------------------------------------------|--------|------------|------------|---------------|---------------|--------|--------|-------|--------|
| Lomitapide - US                           |        |            |            |               |               |        |        | 98.5  | 169.7  |
| Lomitapide - EU/SA                        |        |            |            | -             |               | -      | -      | 96.6  | 164.8  |
| Total product revenues                    | -      | -          | -          | -             | -             | -      | -      | 195.1 | 334.5  |
| Revenue from royalties and royalty rights | -      | -          | -          | -             | -             | -      | -      |       | 3.0    |
| Revenues from license agreements          | -      | -          | -          |               | -             | -      | -      | 40= 4 | 0.5    |
| Total revenues                            | -      | -          | -          | -             | -             | -      | -      | 195.1 | 338.0  |
| Cost of goods sold                        | _      | _          | _          | _             | _             | _      | _      | 9.7   | 16.5   |
| Gross Profit                              | _      | _          | -          | _             | _             | _      | _      | 185.4 | 321.5  |
| 01033 1 10Ht                              | _      |            |            |               | _             | _      | _      | 100.4 | 32 1.3 |
| R&D expense                               | 7.0    | 1.1        | 1.1        | 1.3           | 1.5           | 5.1    | 20.0   | 20.0  | 20.0   |
| SG&A expense                              | 3.1    | 0.8        | 0.8        | 1.5           | 3.0           | 6.2    | 10.0   | 20.0  | 15.0   |
| Other operating expense                   | -      | _          | -          | -             | _             | -      | -      | -     | -      |
| Total operating expense                   | 10.1   | 2.0        | 2.0        | 2.9           | 4.5           | 11.3   | 30.0   | 40.0  | 35.0   |
| Operating income                          | (10.1) | (2.0)      | (2.0)      | (2.9)         | (4.5)         | (11.3) | (30.0) | 145.4 | 286.5  |
| operating meeting                         | (1011) | (=.0)      | (=,        | (=,           | ( )           | (1110) | (00.0) |       | 200.0  |
|                                           |        |            |            |               |               |        |        |       |        |
| (interest expense)                        | (2.1)  | (0.6)      | (0.6)      | (0.6)         | (0.6)         | (2.4)  | 0.2    | 0.3   | 0.5    |
| Interest income                           | 0.2    | 0.0        | 0.0        | 0.0           | 0.0           | 0.1    | (0.2)  | (0.2) | (0.2)  |
| Change in fair value warrant liability    | (0.2)  | 0.2        | 0.2        | (1.8)         | (0.5)         | (2.0)  | (3.0)  | -     |        |
| Other non-operating income (expense)      | -      | 0.0        | 0.0        | 0.0           | 0.0           | 0.1    | -      | -     | -      |
|                                           |        |            |            |               |               |        |        |       |        |
| <b>.</b>                                  | (40.0) | (O.1)      | (0. A)     | ( <b>=</b> 0) | ( <b>-</b> 0) | (4= 0) | (22.2) |       | 200.0  |
| Pre-tax income                            | (12.0) | (2.4)      | (2.4)      | (5.3)         | (5.6)         | (15.6) | (33.0) | 145.5 | 286.8  |
| Income tax expense (benefit)              |        | 0.9        | 0.9        | _             |               | 1.8    | _      | 1.5   | 6.0    |
| Accretion of Dividends                    | (3.3)  | (0.9)      | (0.9)      | (0.9)         | (0.9)         | (3.5)  | -      | 1.5   | 0.0    |
| Accretion of Dividends                    | (3.3)  | (0.9)      | (0.9)      | (0.3)         | (0.9)         | (3.3)  |        |       |        |
| Net income                                | (15.3) | (2.3)      | (2.3)      | (6.2)         | (6.5)         | (17.4) | (33.0) | 144.0 | 280.8  |
|                                           |        |            |            |               |               |        |        |       |        |
| Basic EPS                                 | (9.19) |            | (1.37)     | (3.60)        | (0.38)        | (3.17) | (1.87) | 7.40  | 13.12  |
| Diluted EPS                               | (9.19) | (1.41)     | (1.37)     | (3.60)        | (0.38)        | (3.17) | (1.87) | 7.40  | 13.12  |
| Desir charge system diese                 | 4 -    | 4 7        | 4 7        | 4 7           | 10.0          |        | 477    | 40.5  | 04.4   |
| Basic shares outstanding                  | 1.7    | 1.7<br>1.7 | 1.7<br>1.7 | 1.7           | 16.9          | 5.5    | 17.7   | 19.5  | 21.4   |
| Diluted shares outstanding                | 1.7    | 1.7        | 1.7        | 1.7           | 16.9          | 5.5    | 17.7   | 19.5  | 21.4   |

Source: Canaccord Genuity estimates, company reports



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Global Stock Ratings (as of 1 December 2010)

|                 | Coverage U | Coverage Universe |       |  |
|-----------------|------------|-------------------|-------|--|
| Rating          | #          | %                 | %     |  |
| Buy             | 428        | 58.9%             | 35.3% |  |
| Speculative Buy | 75         | 10.3%             | 56.0% |  |
| Hold            | 204        | 28.1%             | 20.6% |  |
| Sell            | 20         | 2.8%              | 5.0%  |  |
|                 | 727        | 100.0%            |       |  |

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| _ |                          |                |
|---|--------------------------|----------------|
|   | Company                  | Disclosure     |
|   | Aegerion Pharmaceuticals | 1A, 2, 3, 5, 7 |

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