

30 September 2011

Today's Changes	Annual EPS	Annual Revenue	Rating/Target
	2011E \$(2.09) from \$(1.92)	No change	No change
	2012E \$6.97 from \$5.90		

## Aegerion Pharmaceuticals

AEGR : NASDAQ : US\$12.72

**BUY****Target: US\$24.00**

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### COMPANY STATISTICS:

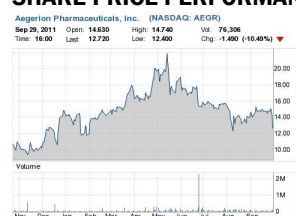
Shares Out (M): 21.06  
 Market Cap (M): US\$267.93  
 52-week Range: US\$9.00 - 25.92

### EARNINGS SUMMARY:

FYE Dec	2010A	2011E	2012E
Revenue:	0.0	0.0	195.1
EPS:	(5.07)	(2.09)	6.97

Revenue:	Q1	0.0A	-
	Q2	0.0A	0.0A
	Q3	0.0A	0.0
	Q4	0.0A	0.0
Total	0.0	0.0	195.1
EPS:	Q1	(0.39)A	-
	Q2	(2.77)A	(0.49)A
	Q3	(3.61)A	(0.63)
	Q4	(0.92)A	(0.59)
Total	(5.07)	(2.09)	6.97

### SHARE PRICE PERFORMANCE:



Source: Interactive Data Corporation

### 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

## POSITIVE EMA MEETINGS: Q1/12 FILING OF LOMITAPIDE NDA AND MAA NOW PLANNED

### Investment recommendation

**Reiterate BUY, \$24 target on lomitapide potential as best-in-class HoFH drug.** Lomitapide is AEGR's Phase 3 drug for HoFH, a rare genetic disease that causes very high LDLs. We think lomitapide is the best-in-class drug vs. SNY's Phase 3 mipomersen. AEGR plans to submit an NDA and MAA in Q1/12 for H2/12 approvals. Our \$24 target is based on a pNPV analysis.

### Investment highlights

- **Positive feedback meetings on completeness of application dataset from three recent EU regulatory meetings.** Meetings with the EMA and AEGR's two chosen rapporteur countries indicated regulators consider lomitapide's open-label 29-patient Phase 3 study, as well as lomitapide's anticipated non-clinical and clinical data package, as adequate for MAA filing. The EU requested standard 12-month stability from three drug batches.
- **We have no concerns on the slight pushback of lomitapide HoFH NDA filing to Q1/12: AEGR maximizing chance of first-pass success.** The FDA requested shorter stability data from fewer drug batches, but AEGR plans to take incrementally more time to carefully compile the lomitapide NDA. Original guidance was for NDA submission by end of 2011.

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30 September 2011

**Figure 1: AEGR upcoming catalysts**

Expected date	Drug/Program	Item	Impact
Q4/11	Lomitapide in HoFH	78-week safety data	+
Q1/12	Lomitapide in HoFH	NDA filing	+
H1/12	Lomitapide in HoFH	Advisory committee meeting	++

Source: Company reports, Canaccord Genuity estimates

**Figure 2: AEGR pNPV**

Drug name	Indication	Status	Launch	Success	Sales (US\$m)	Royalty	Profitability	NPV (US\$)
lomitapide	HoFH - genotype diagnosis	Phase 3	2012	70%	89.0	90%	75%	6.72
lomitapide	HoFH - phenotype diagnosis	Phase 3	2012	60%	187.3	90%	75%	12.12
lomitapide	HoFH - functional diagnosis	Phase 3	2012	33%	140.5	90%	75%	5.00
<b>Total</b>								<b>23.85</b>

Source: Canaccord Genuity estimates

30 September 2011

Figure 3: AEGR P&amp;L

	2010A	Q1/11A	Q2/11A	Q3/11E	Q4/11E	2011E	2012E	2013E
Lomitapide - US	-	-	-	-	-	-	98.5	169.7
Lomitapide - EU/SA	-	-	-	-	-	-	96.6	164.8
<b>Total product revenues</b>	-	-	-	-	-	-	<b>195.1</b>	<b>334.5</b>
Revenue from royalties and royalty rights	-	-	-	-	-	-	-	3.0
Revenues from license agreements	-	-	-	-	-	-	-	0.5
<b>Total revenues</b>	-	-	-	-	-	-	<b>195.1</b>	<b>338.0</b>
Cost of goods sold	-	-	-	-	-	-	19.5	33.4
<b>Gross Profit</b>	-	-	-	-	-	-	<b>175.6</b>	<b>304.5</b>
R&D expense	7.6	3.3	5.1	5.5	7.0	20.9	20.0	20.0
SG&A expense	5.9	3.5	3.2	3.5	4.5	14.7	20.0	20.0
Other operating expense	-	-	-	-	-	-	-	-
<b>Total operating expense</b>	<b>13.6</b>	<b>6.8</b>	<b>8.3</b>	<b>9.0</b>	<b>11.5</b>	<b>35.6</b>	<b>40.0</b>	<b>40.0</b>
<b>Operating income</b>	<b>(13.6)</b>	<b>(6.8)</b>	<b>(8.3)</b>	<b>(9.0)</b>	<b>(11.5)</b>	<b>(35.6)</b>	<b>135.6</b>	<b>264.5</b>
(interest expense)	(2.4)	(0.1)	(0.3)	(0.4)	(0.6)	(1.4)	(1.6)	(1.6)
Interest income	0.1	0.1	0.1	0.0	0.1	0.2	0.2	0.2
Change in fair value warrant liability	(0.4)	-	-	(1.8)	1.1	(0.8)	-	-
Other non-operating income (expense)	0.2	-	-	0.0	0.2	0.3	-	-
<b>Pre-tax income</b>	<b>(16.0)</b>	<b>(6.8)</b>	<b>(8.6)</b>	<b>(11.2)</b>	<b>(10.7)</b>	<b>(37.4)</b>	<b>134.2</b>	<b>263.1</b>
Income tax expense (benefit)	(1.8)	-	-	-	-	-	1.8	6.3
Accretion of Dividends	8.8	-	-	-	-	-	-	-
<b>Net income</b>	<b>(23.0)</b>	<b>(6.8)</b>	<b>(8.6)</b>	<b>(11.2)</b>	<b>(10.7)</b>	<b>(37.4)</b>	<b>132.4</b>	<b>256.8</b>
<b>Basic EPS</b>	<b>(5.07)</b>	<b>(0.39)</b>	<b>(0.49)</b>	<b>(0.63)</b>	<b>(0.59)</b>	<b>(2.09)</b>	<b>6.97</b>	<b>12.88</b>
<b>Diluted EPS</b>	<b>(5.07)</b>	<b>(0.39)</b>	<b>(0.49)</b>	<b>(0.63)</b>	<b>(0.59)</b>	<b>(2.09)</b>	<b>6.97</b>	<b>12.88</b>
Basic shares outstanding	4.5	17.6	17.7	17.9	18.1	17.8	19.0	19.9
Diluted shares outstanding	4.5	17.6	17.7	17.9	18.1	17.8	19.0	19.9

Source: Company reports and Canaccord Genuity estimates

**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. Although safety and tolerability data to date is significantly better due to lower dose and titration, some patients still experience side effects that could reach problematic levels, albeit 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.

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, 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, 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.

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30 September 2011

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Global Stock Ratings  
(as of 7 September 2011)

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	#	%			%
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Speculative Buy	83	10.7%			62.7%
Hold	193	24.8%			19.2%
Sell	13	1.7%			23.1%
	777	100%			

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