Aegerion Pharmaceuticals

Collins Stewart
Independent thinking

US | Biotechnology | AEGR

June 16, 2011

Lomitapide Headed Towards 4Q11 U.S. and E.U. Filings

Recommendation	**BUY**
Price	**$16.91**
Target	**$23.00**

■ **AEGR reaffirms that the lomitapide filings are on-track for 4Q11:**
Following a pre-NDA meeting with the FDA, AEGR reiterated its expectation to submit the lomitapide regulatory filing in HoFH in 4Q11. While the FDA did not grant lomitapide a fast-track designation due to a technicality associated with the use of LDL lowering as a primary endpoint (a surrogate endpoint vs. a clinical endpoint), resulting in a 10-month review period, this in-line with our model which had conservatively assumed 4Q12 U.S. and E.U. approvals. Importantly, however, lomitapide is still eligible for a 6-month review if granted priority review by the FDA. We are encouraged that the FDA is on-board with the open-label nature of P3 lomitapide study (which will form the foundation of the NDA) and that AEGR has received preliminary buy-in from the FDA regarding the criteria used to define HoFH in the P3 clinical trial. With the recently announced positive P3 56-week data in hand, we continue to believe regulatory approval of lomitapide in HoFH is likely and conservatively estimate lomitapide WW sales of $164M+ in 2018, with higher-than-expected HoFH prevalence and pricing as well as approval in FC representing upside to numbers.

■ **Feedback suggests the FDA is comfortable with AEGR's definition of HoFH:**
Following the pre-NDA meeting with the FDA, AEGR believes the FDA is comfortable with the criteria used to define HoFH in the ongoing Phase 3 trial. Should the potential label be consistent with enrollment criteria, lomitapide would be indicated for use in adults (18 years and older) with HoFH defined as patients with: 1) documented HoFH genetic mutations; or 2) skin fibroblast LDL receptor activity <20% normal; or 3) untreated total cholesterol >500 mg/dL and triglycerides <300 mg/dL and both parents have documented total cholesterol >250 mg/dL,; or 4) LDL-C levels >300 mg/dL on maximum doses of currently available therapies.

■ **AEGR continues to take a proactive stance on the potential for a FDA AdCom panel and REMS:**
Consistent with prior comments, AEGR continues to expect the lomitapide regulatory submission to come under the review of a FDA advisory committee panel, given that the drug is a new chemical entity. In addition, the company intends to submit a REMS program to the FDA to restrict lomitapide use to the appropriate prescribing base.

■ **Checking off the boxes for 4Q11 NDA/MAA submissions:**
The remaining pre-clinical studies required for regulatory submissions (including the tQT, renal impairment and biomarker study) are underway and will be completed in time for 4Q11 submissions. Full lomitapide P3 78-week data is expected by YE11 and will be submitted separately from the NDA/MAA.

See Page 2 for Valuation & Risks

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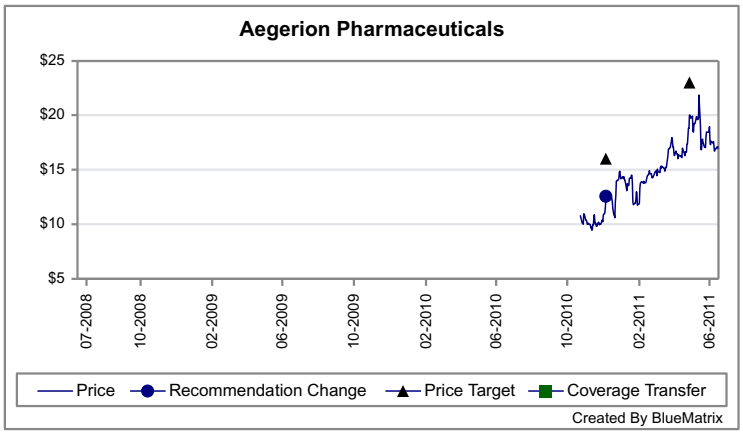
Valuation & Risks

Our 12-month PT of $23 is based on 35x our fully taxed, fully diluted FY15 GAAP EPS estimate of $1.52 discounted back to mid-2012 at 28%. The primary investment risks for AEGR include: 1) lomitapide clinical development risk (particularly regarding potential new safety signals in the ongoing Phase 3 trial); 2) lomitapide regulatory risk; 3) commercial risk, including the possibility that lomitapide does not achieve peak commercial revenue estimates in our model (due to market size, penetration rates, and/or pricing); 4) potential product competition; and 5) financing risk.

Important Disclosure / Disclaimer Information

Other Public Companies Mentioned in this Report

Company	Ticker	Price	Recommendation
Aegerion Pharmaceuticals	AEGR	$16.91	BUY



Ticker	Date	Action	Prior Rating	Current Rating	Price	Target Price
AEGR	2011-04-28	Raising Target Price	BUY	BUY	$20.00	$23.00
AEGR	2010-12-6	Initiation of Coverage	NA	BUY	$12.57	$16.00

Collins Stewart LLC Ratings

Valuation and Risks

The recommendation and price target contained within this report are based on a time horizon of 12 months but there is no guarantee the objective will be achieved within the specified time horizon. Price targets are determined by a subjective review of fundamental and/or quantitative factors of the issuer, its industry, and the security type. A variety of methods may be used to determine the value of a security including, but not limited to, discounted cash flow, earnings multiples, enterprise value, book value, peer group comparisons, and the sum of the parts. Overall market risk, interest rate risk, and general economic risks impact all securities. Specific information regarding the price target and recommendation is provided in the text herein or in our most recent full research report on the subject company.

BUY: Improving fundamentals and/or identifiable catalysts in place expected to cause stock to outperform its industry

NEUTRAL: Company's fundamental backdrop suggest stock should perform in line with industry

SELL: Deteriorating fundamentals and/or identifiable catalysts in place expected to cause stock to underperform its industry

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	% of CSTI Universe with this rating	% of rating tier for which CSTI provided IB services
Buy	54%	0%
Hold	43%	0%
Sell	2%	0%

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