Aegerion Pharmaceuticals

Collins Stewart
Independent thinking

US | Biotechnology | AEGR

December 6, 2010

Lomitapide Headed for a HoFH Regulatory Filing; Init. with a Buy

Recommendation	BUY
Price	$10.99
Target	$16.00

Fiscal Year Ends: Dec		2010E	2011E	2012E
Revenue (MM)		$0	$0	$6
GAAP Earnings per Share				
	Q1	-	(0.20)	-
	Q2	-	(0.22)	-
	Q3	(3.61)A	(0.26)	-
	Q4	(0.32)	(0.29)	-
Year		(6.70)	(0.98)	(1.16)
Diluted Shares (MM)		4.2	17.8	18.1

Key Data

52-Week Range	$12.00-$9.00
Shares Outstanding (MM)	17.6
Market Cap (MM)	$193
Float (MM)	5.0
% Held Institutionally	NA
Cash Per Share	$2.80

Stock Performance

	1m	3m	12m
Performance	9.9%		

Source: Bloomberg

■ **Investment Thesis:**
We are initiating coverage of AEGR with a Buy rating and $16 PT. AEGR's value proposition is centered on its lead compound, lomitapide, which is an oral, small-molecule MTP-inhibitor currently in a P3 78-week study (N=29) for the treatment of homozygous familial hypercholesterolemia (HoFH), an orphan genetic disease associated with extremely elevated levels of LDL cholesterol. Given that lomitapide has already met its primary endpoint, demonstrating a significant reduction in LDL-C of 45% (ITT) at week 26 vs. baseline, and an acceptable safety profile to date given the unmet need, we believe U.S. and E.U. approval in mid-2012 and YE12, respectively, is likely. We estimate lomitapide WW sales of $164M+ in 2018, with higher HoFH prevalence, pricing and familial chylomicronemia (FC) representing upside.

■ **U.S. lipid center survey suggests HoFH market of ~1.7K:**
We conducted a survey of 10 of the ~43 U.S.-based lipid centers, which suggests there are ~520 HoFH patients (as defined by AEGR's P3 study enrollment criteria), with an additional ~500+ patients at least under the care of cardiologists. We arrive at a prevalence of ~800 HoFH patients in the U.S. and ~940 in the E.U. Physicians suggest ~25% of these patients are <18 years of age. Given the unmet need and apheresis pricing, we assume annual pricing of $175K (U.S.) and $160K (E.U.). We estimate WW lomitapide sales of $164M+ in 2018 and view a potentially larger addressable HoFH market (AEGR estimates 6K patients) and higher lomitapide pricing (AEGR has guided to $150K-$250K/year) as upside.

■ **Physicians optimistic regarding lomitapide approval/uptake:**
Physicians we spoke with were impressed by the LDL-C and triglyceride reductions exhibited by lomitapide at 26 weeks in the P3 HoFH study, and believe regulatory approval and significant uptake is likely, given the significant unmet need in this population.

■ **Approval in FC represents a free-call option:**
AEGR expects to initiate a P2 lomitapide trial in FC, an orphan disease of severe high triglyceride levels, in 2011. Given the significant triglyceride reductions (45% vs. baseline (ITT) at week 26) in the P3 HoFH study, we believe the drug could demonstrate significant benefit in this indication, representing $100M+ in upside to our lomitapide revenue estimates.

Aegerion is a biopharmaceutical company focused on the development and commercialization of treatments for patients with severe lipid disorders. The company's lead therapeutic is lomitapide, an oral small-molecule inhibitor of MTP, which is in a Phase 3 clinical trial for the treatment of patients with Homozygous Familial Hypercholesterolemia (HoFH). Aegerion was founded in 2005 and is headquartered in Bridgewater, New Jersey.

Please see Page 4 for price target valuation method and risks to achieving that target.

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Investment Thesis

We are initiating coverage of AEGR with a Buy rating and $16 PT. The primary value driver for AEGR is lomitapide, an oral, small-molecule MTP-inhibitor, which is in Phase 3 development for the treatment of patients with homozygous familial hypercholesterolemia (HoFH), an orphan genetic disorder which results in extremely elevated levels of cholesterol. Given that lomitapide has demonstrated dramatic efficacy, with an acceptable safety profile to date, in Phase 3 trials (see below) in HoFH, we believe U.S. and E.U. approval in mid-2012 and YE12, respectively, is likely. We assume HoFH prevalence of 1.7K patients in the U.S. and E.U. (vs. AEGR's 6K patients through market research), based on a survey of U.S. lipid centers, and estimate annual pricing of $175K (U.S.) and $160K (E.U.) (AEGR's suggests a price range of $150K-$250K). Based on our analysis, we estimate WW peak sales of $164M+ in 2018 and recognize upside to our estimates based on higher patient prevalence and pricing. In addition, familial chylomicronemia (FC), an orphan disease characterized by severe high triglyceride levels, which is entering Phase 2 trials in 2011, could represent $100M+ in upside to our lomitapide revenue estimates.

Lomitapide approval likely in 2012. Lomitapide has shown significant low-density lipoprotein cholesterol (LDL-C) lowering abilities in HoFH patients, with the ongoing Phase 3 study having met its primary efficacy endpoint of a significant percent reduction in LDL-C (45%) at Week 26 vs. baseline (ITT). Importantly, lomitapide treatment also resulted in 8 of 29 HoFH patients reducing their LDL-C levels to below 100 mg/dL, the optimal range recommended by the American Heart Association. In addition to dramatic efficacy, the lomitapide safety profile appears acceptable at current levels, with mild-to-moderate gastrointestinal-related side effects representing the most common adverse events, liver enzyme elevations that can be managed, and liver fat elevations that appear to decrease over time. Once Phase 3 data from the 78-week time point, as well as data from several smaller studies (including a thorough QT study and a study in HoFH patients with renal impairment) is in hand (2H11), we expect AEGR to submit U.S. and E.U. regulatory filings for lomitapide in HoFH by YE11. We believe approval is likely, given the dramatic efficacy shown in lomitapide-treated patients to date and a safety profile that, while it warrants monitoring, should be acceptable given the significant unmet need.

HoFH represents a $164M+ market for lomitapide. We performed an survey of U.S. lipid centers, which suggests that there are ~520 HoFH patients as defined by the Phase 3 pivotal study criteria (patients with documented HoFH genetic mutations, or skin fibroblast LDL receptor activity <20% normal, or untreated total cholesterol >500 mg/dL and triglycerides <300 mg/dL and both parents have documented total cholesterol >250 mg/dL, or LDL-C levels >300 mg/dL on maximum doses of currently available therapies) in the U.S. under the care of lipid specialists, with another ~500+ patients currently under the care of cardiologists. We arrive at a prevalence of ~800 HoFH patients in the U.S., which we believe is likely conservative, and ~940 in the E.U. (assuming a similar prevalence) for a total market of 1.7K. Physician feedback suggests ~25% of these patients are under the age of 18. We model for peak penetration of ~60% in the U.S. and ~55% in the E.U. based on physician feedback suggesting that the majority of HoFH patients will be eligible for lomitapide treatment. Given that HoFH is an orphan disease in an area of high unmet medical need, we believe lomitapide will secure premium pricing and model for $175K per patient per year in the U.S. (with 3% increases annually) and $160K per patient per year in the E.U. (no price increases). Based on these

assumptions, we arrive at worldwide sales of lomitapide of $164M in 2018. We view a potentially larger addressable HoFH market (AEGR estimates 6K) and higher lomitapide pricing (AEGR has guided to $150K to $250K/yr) as upside to our numbers.

Physician feedback suggests high demand for lomitapide. Our physician feedback suggests a high level of optimism regarding the likelihood of lomitapide approval and uptake, given the dramatic effects on the LDL-C and triglyceride levels in HoFH patients seen to date. Physicians we spoke with intend to use lomitapide in a meaningful proportion of their HoFH patients. These patients are desperately in need of effective therapies, as they are not able to lower their LDL-C levels to within the normal range with available treatments. Although they represent a small percentage of patients, physicians are particularly interested in lomitapide for HoFH patients that are intolerant to currently available medications such as the statin class of drugs and for whom apheresis is the only option. While physicians acknowledge that lomitapide is not without safety risks, they note that ineffectively treating HoFH patients is also associated with significant risks, including death. They are comforted by the fact that lomitapide will be primarily prescribed by lipidologists, who will be informed as to how to dose titrate lomitapide prior to prescribing the drug and who are familiar with monitoring the side effects associated with lomitapide (including liver enzyme and liver fat elevations).

Lomitapide offers several benefits over potential competitor. Once approved, lomitapide could face competition from Isis Pharmaceuticals (ISIS $9.52, Neutral) / Genzyme's (GENZ $70.91, Neutral) mipomersen. Mipomersen is on-track to enter the U.S. and E.U. markets >6 months ahead of lomitapide (1H11 filings vs. lomitapide's YE11 filings). However, our physician feedback suggests that lomitapide may be the more attractive candidate given the following factors: 1) while cross-study comparisons are not valid, lomitapide may offer better efficacy (45% LDL-C reductions from mean baseline levels of 337 mg/dL vs. 25% with mipomersen from mean baseline levels of ~400 mg/dL); 2) lomitapide is easy to dose titrate (dose titrations have not been done with mipomersen, which physicians believe could be problematic when trying to dose through adverse events); and 3) lomitapide is an oral therapy while mipomersen is injectable. Physicians did note that lomitapide and mipomersen could potentially and may likely be used in combination given that their mechanisms of action do not overlap.

Approval in FC represents a free-call option. AEGR expects to initiate a Phase 2 trial investigating lomitapide for the treatment of patients with familial chylomicronemia (FC), an orphan disease characterized by severe high triglyceride levels, in 2011. Given the 45% reductions in triglycerides shown in the ongoing Phase 3 HoFH study (ITT), we believe the drug could demonstrate significant benefit in this patient population. However, given the program's relatively early stage, we currently do not include lomitapide FC revenue in our model, which we believe could represent $100M+ upside to our lomitapide revenue estimates.

Upcoming catalysts: Regarding the HoFH indication, we expect lomitapide Phase 3 interim 56-week data in 2Q11 and full 78-week data in 2H11. Several small studies required for regulatory filings (including a thorough QT and renal impairment study) are also ongoing and will be completed in 2H11. With this data in hand, we expect AEGR to submit U.S. and E.U. regulatory filings for lomitapide in HoFH by YE11. Assuming priority review in the U.S., approval is likely in mid-2012 while E.U. approval is expected by YE12. We expect the initiation of a Phase 3 pediatric HoFH study and a Phase 2 familial chylomicronemia (FC) study in 2011.

Valuation

Our 12-month PT of $16 is based on 27x our fully taxed, fully diluted FY15 GAAP EPS estimate of $1.59 discounted back to YE11 at 28%.

Our U.S. and E.U. revenue assumptions of $89.9M and $74.6M in 2018, respectively, are based on a HoFH population of ~1.7K patients (according to our discussions with a subset of U.S. lipid centers; includes pediatrics and adults) and annual pricing of $175K (with 3% increases annually) and $160K in the U.S. and E.U., respectively. However, we believe our estimates may be somewhat conservative, given AEGR's market diligence estimates that the U.S. and E.U. adult HoFH market is ~6K patients and assuming pricing of $150K-$250K. We view a potentially larger addressable HoFH market and potentially higher annual pricing as upside to our model. See Figure 1 below for a Sensitivity Analysis.

Figure 1: Lomitapide Sensitivity Analysis in HoFH Patients (in $M)

10% Penetration

Number of Patients	Average Annual Price (U.S. and E.U.)										
	$150,000	$160,000	$170,000	$180,000	$190,000	$200,000	$210,000	$220,000	$230,000	$240,000	$250,000
600	$9	$10	$10	$11	$11	$12	$13	$13	$14	$14	$15
1,000	$15	$16	$17	$18	$19	$20	$21	$22	$23	$24	$25
1,500	$23	$24	$26	$27	$29	$30	$32	$33	$35	$36	$38
2,000	$30	$32	$34	$36	$38	$40	$42	$44	$46	$48	$50
2,500	$38	$40	$43	$45	$48	$50	$53	$55	$58	$60	$63
3,000	$45	$48	$51	$54	$57	$60	$63	$66	$69	$72	$75
4,000	$60	$64	$68	$72	$76	$80	$84	$88	$92	$96	$100
5,000	$75	$80	$85	$90	$95	$100	$105	$110	$115	$120	$125
6,000	$90	$96	$102	$108	$114	$120	$126	$132	$138	$144	$150

25% Penetration

Number of Patients	Average Annual Price (U.S. and E.U.)										
	$150,000	$160,000	$170,000	$180,000	$190,000	$200,000	$210,000	$220,000	$230,000	$240,000	$250,000
600	$23	$24	$26	$27	$29	$30	$32	$33	$35	$36	$38
1,000	$38	$40	$43	$45	$48	$50	$53	$55	$58	$60	$63
1,500	$56	$60	$64	$68	$71	$75	$79	$83	$86	$90	$94
2,000	$75	$80	$85	$90	$95	$100	$105	$110	$115	$120	$125
2,500	$94	$100	$106	$113	$119	$125	$131	$138	$144	$150	$156
3,000	$113	$120	$128	$135	$143	$150	$158	$165	$173	$180	$188
4,000	$150	$160	$170	$180	$190	$200	$210	$220	$230	$240	$250
5,000	$188	$200	$213	$225	$238	$250	$263	$275	$288	$300	$313
6,000	$225	$240	$255	$270	$285	$300	$315	$330	$345	$360	$375

50% Penetration

Number of Patients	Average Annual Price (U.S. and E.U.)										
	$150,000	$160,000	$170,000	$180,000	$190,000	$200,000	$210,000	$220,000	$230,000	$240,000	$250,000
600	$45	$48	$51	$54	$57	$60	$63	$66	$69	$72	$75
1,000	$75	$80	$85	$90	$95	$100	$105	$110	$115	$120	$125
1,500	$113	$120	$128	$135	$143	$150	$158	$165	$173	$180	$188
2,000	$150	$160	$170	$180	$190	$200	$210	$220	$230	$240	$250
2,500	$188	$200	$213	$225	$238	$250	$263	$275	$288	$300	$313
3,000	$225	$240	$255	$270	$285	$300	$315	$330	$345	$360	$375
4,000	$300	$320	$340	$360	$380	$400	$420	$440	$460	$480	$500
5,000	$375	$400	$425	$450	$475	$500	$525	$550	$575	$600	$625
6,000	$450	$480	$510	$540	$570	$600	$630	$660	$690	$720	$750

75% Penetration

Number of Patients	Average Annual Price (U.S. and E.U.)										
	$150,000	$160,000	$170,000	$180,000	$190,000	$200,000	$210,000	$220,000	$230,000	$240,000	$250,000
600	$68	$72	$77	$81	$86	$90	$95	$99	$104	$108	$113
1,000	$113	$120	$128	$135	$143	$150	$158	$165	$173	$180	$188
1,500	$169	$180	$191	$203	$214	$225	$236	$248	$259	$270	$281
2,000	$225	$240	$255	$270	$285	$300	$315	$330	$345	$360	$375
2,500	$281	$300	$319	$338	$356	$375	$394	$413	$431	$450	$469
3,000	$338	$360	$383	$405	$428	$450	$473	$495	$518	$540	$563
4,000	$450	$480	$510	$540	$570	$600	$630	$660	$690	$720	$750
5,000	$563	$600	$638	$675	$713	$750	$788	$825	$863	$900	$938
6,000	$675	$720	$765	$810	$855	$900	$945	$990	$1,035	$1,080	$1,125

100% Penetration

Number of Patients	Average Annual Price (U.S. and E.U.)										
	$150,000	$160,000	$170,000	$180,000	$190,000	$200,000	$210,000	$220,000	$230,000	$240,000	$250,000
600	$90	$96	$102	$108	$114	$120	$126	$132	$138	$144	$150
1,000	$150	$160	$170	$180	$190	$200	$210	$220	$230	$240	$250
1,500	$225	$240	$255	$270	$285	$300	$315	$330	$345	$360	$375
2,000	$300	$320	$340	$360	$380	$400	$420	$440	$460	$480	$500
2,500	$375	$400	$425	$450	$475	$500	$525	$550	$575	$600	$625
3,000	$450	$480	$510	$540	$570	$600	$630	$660	$690	$720	$750
4,000	$600	$640	$680	$720	$760	$800	$840	$880	$920	$960	$1,000
5,000	$750	$800	$850	$900	$950	$1,000	$1,050	$1,100	$1,150	$1,200	$1,250
6,000	$900	$960	$1,020	$1,080	$1,140	$1,200	$1,260	$1,320	$1,380	$1,440	$1,500

Source: Collins Stewart LLC Research

Investment Risks

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.

Pipeline Summary

AEGR's product pipeline consists of two microsomal triglyceride transfer protein (MTP) inhibitors, lomitapide and implitapide. Lomitapide is the company's lead candidate, which is currently in a Phase 3 pivotal trial for the treatment of patients with homozygous familial hypercholesterolemia (HoFH) who are at least 18 years of age. Top-line 26-week data was released in October 2010, with the 29 patients enrolled experiencing a mean reduction in LDL cholesterol (LDL-C) levels of 45% (the primary efficacy endpoint; intent-to-treat analysis). Additional efficacy and safety data is expected in 2Q11 (top-line interim 56-week data) and 2H11 (full 78-week data). We expect AEGR to submit U.S. and E.U. regulatory filings by YE11 with potential approvals in 2012. Beyond adult HoFH, AEGR intends to initiate a Phase 3 study in pediatric HoFH and a Phase 2 study in patients with familial chylomicronemia (FC) in 2011.

AEGR's second therapeutic, implitapide, was previously in Phase 2 development in patients with hypertriglyceridemia, HoFH and HeFH, but was placed on clinical hold by the FDA in 2007. We do not expect AEGR to conduct additional implitapide trials in the near future.

Figure 2: AEGR Pipeline

Product	Indication	Partner	Target	Status
Lomitapide	HoFH	Proprietary	MTP Inhibitor	Phase 3
Implitapide	HoFH	Proprietary	MTP inhibitor	Phase 2 - On Clinical Hold
	HeFH	Proprietary	MTP inhibitor	Phase 2 - On Clinical Hold
	Hypertriglyceridemia	Proprietary	MTP inhibitor	Phase 2 - On Clinical Hold

Source: Collins Stewart LLC Research and Company Reports

Figure 3: AEGR Upcoming Milestones

Product	Indication	Timing	Milestone
Lomitapide	HoFH	2Q11	Phase 3 56-week data
	HoFH	2H11	Full Phase 3 78-week data
	FC	2011	Initiation of a Phase 2 study
	Pediatric HoFH	2011	Initiation of a Phase 3 study
	HoFH	YE11	Submit U.S. regulatory filing
	HoFH	YE11	Submit E.U. regulatory filing
	HoFH	Mid-2012	Potential FDA approval
	HoFH	YE12	Potential EMEA approval

Source: Collins Stewart LLC Research and Company Reports

Lomitapide: A New Therapy Offering Significant Benefit in HoFH and Beyond – Key Points You Need to Know:

What is lomitapide and why will it help patients with HoFH?

Lomitapide is an oral, small-molecule inhibitor of microsomal triglyceride transfer protein (MTP). MTP is present in the intestines and the liver and plays a critical role in the production and secretion of beta-lipoproteins, including cholesterol and triglycerides. The inhibition of MTP, however, prevents the secretion of these molecules into the body's circulation, and is therefore a logical drug target for patients with severe high cholesterol and triglycerides.

Figure 4: Facts about HoFH

What is HoFH?
Homozygous Familial Hypercholesterolemia is a genetic, inherited disorder in which a patients has extremely high levels of low density lipoprotein (LDL), which can lead to heart disease and heart attacks at an unusually early age. HoFH is caused by loss-of-function mutations in the low-density lipoprotein receptor (LDLR) gene on Chromosome 19, which renders the body incapable of removing LDL from its system.
Who Gets HoFH?
Patients with HoFH have inherited a defective copy of the LDLR gene from each parent. If only one mutated copy is inherited, patients will have heterozygous familial hypercholesterolemia (HeFH).
What are the Symptoms of HoFH?
Patients with HoFH typically present with total cholesterol levels above 300 mg/dL (vs. <200 mg/dL in healthy people), and LDL levels above 200 mg/dL (vs. <100 mg/dL in healthy people). Patients can also present with fatty, cholesterol-rich skin deposits (called xanthomas), cholesterol deposits in the eye lids (call xanthelasmas), chest pain and obesity.
Current Treatments for HoFH
Treatment of HoFH can include: lipid lowering drugs (including statins), LDL apheresis (blood is pumped out of the patient into a column that removes LDL-C and then back into the patient; commonly 1x every 2 weeks), and liver transplant.
What is the Prognosis for HoFH Patients?
Patients with HoFH are at high risk of suffering from coronary atherosclerosis, myocardial infarction or sudden death before the age of 20.

Source: Collins Stewart LLC Research, University of Maryland Medical Center, Kassim, et al. PLoS One; 2010.

A brief history of lomitapide's development…

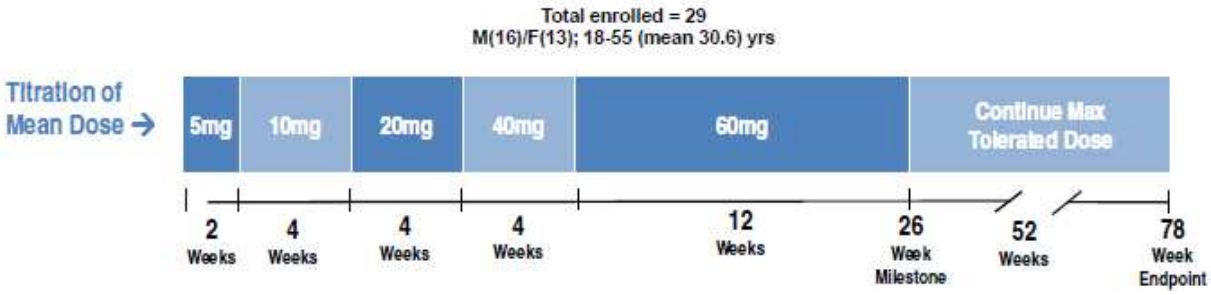
Lomitapide was originally developed by Bristol-Myers Squibb in the 1990s (BMY $25.91, Not Rated) for the treatment of patients with high LDL-C who were at risk of cardiovascular events or intolerant to statins. When dosed at levels as high as 200 mg/day, lomitapide was associated with significant reductions in LDL-C, but also with high discontinuation rates due to gastrointestinal side effects, and elevations in liver fat were observed. Due

to the side effects, BMS turned the rights to the drug over to the University of Pennsylvania in 2003. At UPenn, investigators found that dose titrating lomitapide over time increased the drug's tolerability. In addition, utilizing a lower dosed managed to lower elevations in fatty liver levels. AEGR then acquired the rights to lomitapide from UPenn in 2006. While the drug is still associated with some GI side effects (even with dose titration), GI events are typically now mild-to-moderate in nature and tend to lessen over time. Elevations in liver fat continue to be observed (week 1-26) but start to decrease from peak levels over time (week 26-56).

Lomitapide advances into a Phase 3 trial in HoFH…

The ongoing Phase 3 trial is an open-label, single-arm study investigating lomitapide as a treatment for 29 patients (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. The primary endpoint of the study is percent change in LDL-C at week 26 vs. baseline, while secondary endpoints include changes in total cholesterol, LDL cholesterol, HDL cholesterol and triglycerides at week 78 (vs. baseline), percent change in hepatic liver fat (assessed by MRI/NMRS), and safety (*See Figure 5 below*). Full data from the Week 78 time point is expected in 2H11.

Figure 5: Design of Lomitapide Phase 3 Study in Patients with HoFH



Source: AEGR 3Q10 Earnings Presentation

Heading towards U.S. and E.U. regulatory filings…

While AEGR previously announced that the Phase 3 study met its primary endpoint at 26 weeks, full 78-week efficacy and safety analyses, expected in 2H11, are needed for the regulatory submissions. In addition, AEGR must also conduct several small studies (See Figure 6 below), most of which are now underway. While these studies will be conducted in human subjects (with the exception of the biomarker study), they will be short in duration, and are expected to be complete prior to the submission of the regulatory filings.

Figure 6: Additional Studies Required for Lomitapide HoFH Regulatory Filings

	Study:	Purpose:
☐	Thorough QT study	Investigate lomitapide treatment in healthy volunteers to assess the effect, if any, of the drug on the heart's QT interval
☐	Renal impairment study	Investigate lomitapide efficacy and safety in patients with renal impairment
☐	Drug-drug interaction study	Investigate if any drug-drug interactions occur with lomitapide, including warfarin and Zocor
☐	Metabolite isolation and identification study	Investigate the metabolites of lomitapide as it is broken down by the body (animals and healthy volunteers)
☐	Biomarker study	Investigate six biomarkers of hepatic inflammation and fibrosis in samples from patients treated with lomitapide

Source: Collins Stewart LLC Research

Once all studies, including the Phase 3 pivotal trial, are complete, AEGR intends to submit U.S. and E.U. lomitapide regulatory filings by YE11. If granted priority review (HoFH is an orphan disease), lomitapide could receive FDA approval in mid-2012. Approval in the E.U. is possible by YE12 (assuming standard ~10 month review period).

Plans for lomitapide beyond adult HoFH…

AEGR expects to initiate a Phase 3 pediatric HoFH study for lomitapide in 2011. Given that ~25% of HoFH patients are under the age of 18, according to our physician feedback, lomitapide approval in this population is important for the AEGR value proposition. In 2011, AEGR also expects to initiate a Phase 2 trial in patients with familial chylomicronemia (FC), an orphan disease characterized by severely high triglyceride levels.

Lomitapide Shows Dramatic Cholesterol Reductions in Patients with HoFH... Here's What We Think:

We believe lomitapide should secure regulatory approval in HoFH…

Lomitapide has demonstrated impressive efficacy in the ongoing Phase 3 HoFH study. While safety and longer-term efficacy analyses are still ongoing (78 weeks in duration), the study already met its primary endpoint of a significant percent change in LDL-C at 26 weeks vs. baseline (statistical analysis cannot be performed until the full 78-week duration of study is complete). At Week 26, lomitapide-treated patients experienced a 45% reduction in LDL-C (intent-to-treat (ITT) analysis). The most common adverse events were gastrointestinal in nature, which appear to improve with time. Four patients have experienced liver ALT elevation >5x ULN and increases in hepatic fat has been seen, but enzyme elevations appear manageable with dose titration and fat levels appear to decrease with time. While the safety of the drug warrants monitoring, we believe the incidence

and severity of these adverse events at current rates should be acceptable, given that HoFH is a life-threatening disease with high unmet need. With a lack of efficacious drugs available for patients with HoFH, we believe regulatory approval of lomitapide is likely in both the U.S. and the E.U.

What do we expect at the 56- and 78-week analyses?

At the 56- and 78-week time points (expected in 2Q11 and 2H11, respectively), we expect to see patients maintain their reduced LDL-C and triglyceride levels. We note that, following the week 26 primary efficacy analysis, patients are permitted to change their background medications, which may result in LDL-C and triglyceride level fluctuations as background therapies are added or removed. We look for continued safety, specifically for successful dose-titrations around any new ALT elevations and for liver fat signals to continue their downward trend.

We note that, since the conclusion of the 26-week phase of the study, at least one patient has become apheresis independent.

Most patients entering the OLE following the 78-week time point…

We are encouraged that, according to management, 10 of the 12 patients that have completed the 78-week study chose to enroll in the open-label extension study, likely reflecting physician and patient satisfaction with lomitapide. Of the two patients that did not enter the OLE, 1 patient moved away from his/her center and 1 had compliance issues.

We believe the HoFH represents a $164M+ opportunity for lomitapide by 2018…

We performed a survey of U.S. lipid centers (see below for details), which suggests that there are ~520 HoFH patients as defined by the Phase 3 pivotal study criteria (patients with documented HoFH genetic mutations, or skin fibroblast LDL receptor activity <20% normal, or untreated total cholesterol >500 mg/dL and triglycerides <300 mg/dL and both parents have documented total cholesterol >250 mg/dL, or LDL-C levels >300 mg/dL on maximum doses of currently available therapies) in the U.S. under the care of lipid specialists with another ~500 patients (or potentially more) HoFH patients currently under the care of cardiologists. We arrive at a prevalence of ~800 HoFH patients in the U.S., and ~940 in the E.U. (assuming a similar prevalence) for a total market of 1.7K. Physician feedback suggests ~25% of these patients are under the age of 18. We model for peak penetration of ~60% in the U.S. and ~55% in the E.U. based on physician feedback suggesting that the majority of HoFH patients will be eligible for lomitapide treatment. Given that HoFH is an orphan disease in an area of high unmet medical need, we believe AEGR will secure premium pricing for lomitapide and model for $175K per patient per year in the U.S. (with 3% increases annually) and $160K per patient per year in the E.U. (no price increases). Based on these assumptions, we arrive at worldwide sales of lomitapide of $164M in 2018. We view a potentially larger addressable HoFH market (AEGR estimates 6K patients) and higher lomitapide pricing (AEGR has guided to $150K to $250K/yr) as upside to our numbers.

Feedback from U.S. lipid centers: ~520 HoFH patients currently treated in lipid centers with many more under the care of cardiologists

We performed a survey of 10 of the ~43 U.S.-based lipid treatment centers. Feedback suggests that there are ~520 HoFH patients (defined as patients with documented HoFH genetic mutations, or skin fibroblast LDL receptor activity < 20% normal, or untreated total cholesterol >500 mg/dL and triglycerides <300 mg/dL and both parents have documented total cholesterol >250 mg/dL, or LDL-C levels >300 mg/dL on maximum doses of available therapies) currently receiving treatment in these centers (*See Figure 7 below*). The individual lipid centers treat a wide range of HoFH patients, with one only treating 1 patient while one is currently treating 50 patients, and others ranging in between. We would note that methods of diagnosing HoFH vary between centers and many HoFH are currently treated by cardiologists (see below), which represents upside to our estimates.

Many HoFH patients likely under the care of cardiologists…

While HoFH is a rare lipid disorder, physicians we spoke with practicing in these centers believe there are a significant number (could be 100% or more vs. those in lipid centers) of HoFH patients (in addition to the ~520 currently in the centers) are currently being treated by cardiologists rather than lipidologists. Diligence on the part of lipid centers has revealed that many cardiologists are not aware of HoFH and not aware that LDL apheresis is an effective treatment option for these patients. In fact, one center we spoke with recently started an outreach program to cardiologists in its area to raise awareness and ensure these patients get the proper attention and best care.

Feedback suggests pediatrics would greatly benefit from a new oral therapy…

Physicians we spoke with estimate that ~25% of HoFH patients are under the age of 18. Feedback suggests that the majority of young patients are treated with statins and other lipid lowering drugs, rather than apheresis, as physicians generally reserve this treatment until patients are older due to the stress and inconvenience associated with apheresis. As such, lipidologists we spoke with are extremely excited about a new, more effective oral drug treatment for these young patients, given their limited options.

Lipidologists typically infer HoFH diagnosis…

Of note, the physicians we spoke with all make HoFH diagnoses based on patients' cholesterol levels (>500 mg/dL) and the cholesterol levels on their parents. Only one physician we spoke with used genetic testing, which he employed merely to confirm his diagnosis. No center we spoke with used skin fibroblast tests. We believe, however, that inferred diagnoses based on phenotype and family history is consistent with AEGR's clinical trial enrollment criteria, and therefore, do not expect that the diagnosis of HoFH will present significant challenges to lomitapide's uptake.

Figure 7: U.S. Lipid Center Survey of Functional HoFH Patients

	Number of HoFH Patients
Site 1	1
Site 2	3
Site 3	8
Site 4	10
Site 5	9
Site 6	15
Site 7	5
Site 8	14
Site 9	5
Site 10	50
Total	**120**

Source: Collins Stewart LLC Research.

Physicians Optimistic Regarding Lomitapide Approval and Uptake

HoFH patients are in need of new, more effective treatments…

We performed additional diligence with HoFH-treating physicians outside of lipid centers. Our physician feedback suggests a high level of optimism regarding the likelihood of lomitapide approval and uptake, given the dramatic effects on the LDL-C and triglyceride levels in HoFH patients seen to date. Physicians we spoke with intend to use lomitapide in a meaningful proportion of their HoFH patients. These patients are desperately in need of effective therapies, as they are not able to lower their LDL-C levels to within the normal range with available treatments. Although they represent a small percentage of patients, physicians are particularly interested in lomitapide for HoFH patients that are intolerant to currently available medications, such as statins, and for whom apheresis is the only option. While physicians acknowledge that lomitapide is not without safety risks, they note that ineffectively treating HoFH patients is also associated with significant risks, including death.

Physicians believe side effects are manageable…

Physicians we spoke with are not overly concerned with increases in liver fat seen in the Phase 3 trial to date. They noted that the implications of high liver fat are unclear given that some healthy individuals have unusually high liver fat (>8%), but do not appear to suffer any adverse consequences. In addition, feedback suggests that the liver ALT elevations seen in some lomitapide-treated patients are also not significantly concerning, as they appear to be related to the drug's efficacy, such that patients with more significant LDL-C reductions have a higher likelihood of liver enzyme

elevations. Physicians noted that patients with liver ALT elevations have not experienced simultaneous increases in bilirubin or cases of Hy's law, suggesting that the likelihood of serious liver damage is low. Thus, while liver fat and enzyme elevations warrant monitoring, physicians noted lipidologists, who will be the primary prescribers of lomitapide, will be informed as to how to properly dose titrate the drug and are familiar with the monitoring of these side effects. Further, they are willing to take some safety risk because of the complete lack of efficacious alternatives.

Lomitapide is particularly appealing for pediatric patients…

Physicians we spoke with also noted that an oral drug for HoFH is particularly attractive for pediatric patients. While these young patients are eligible for LDL apheresis, physicians typically put off this procedure for as long as possible, instead trying to control LDL levels with statins and other lipid lowering drugs.

Only concern noted by physicians related to reimbursement…

Some physicians we spoke with anticipate that a subset of HoFH patients may need to continue LDL apheresis treatments, even with lomitapide. While apheresis is burdensome, the combination of the two could help those patients with severely high cholesterol get their LDL-C levels below 100 mg/dL. As such, the one concern physicians did have was related to reimbursement. Given that lomitapide will likely be priced in the range of $150K-$250K annually (according to management), physicians worry about the difficulty to get adequate reimbursement if the drug is used in combination with apheresis (costs >$100K annually). However, given the unmet need in HoFH, physicians hope to see little push back from payors.

Lomitapide's benefit over the competition…

Physicians noted several reasons that lomitapide may be the more attractive candidate, including: 1) while cross-study comparisons are not valid, lomitapide could potentially have superior efficacy (45% LDL-C reductions vs. 25% with mipomersen with mean baseline LDL-C levels of 337 mg/dL and ~400 mg/dL, respectively (ITT analysis)); 2) lomitapide is easy to dose titrate (dose titrations have not been done with mipomersen but physicians noted that this could be problematic when trying to dose through adverse events); and 3) lomitapide is oral while mipomersen is injectable. Physicians did note that lomitapide and mipomersen could potentially and will likely be used in combination given that their mechanisms of action do not overlap.

Data Download: Phase 3 Lomitapide Study in HoFH Meets Primary Endpoint

AEGR is conducting a Phase 3 open-label, single-arm study investigating lomitapide as a treatment for HoFH patients. The primary endpoint of the study is percent change in LDL-C at Week 26 vs. baseline, while secondary endpoints include changes in total cholesterol, LDL cholesterol, HDL

cholesterol and triglycerides at Week 78 (vs. baseline), percent change in hepatic liver fat (assessed by MRI/NMRS), and safety. An additional interim analysis at 56 weeks is expected in 2Q11, with full Week 78 data likely in 2H11.

This Phase 3 study enrolled 29 patients 18 years or older with functional 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.

Once enrolled in the study, patients underwent a 6-week run-in period on their current therapies (including apheresis and statins) followed by 26 weeks on ascending doses of lomitapide starting on 5 mg/day and increasing to 60 mg/day. After week 26 (the primary endpoint of the study), patients were maintained at their highest tolerated dose of lomitapide for an additional 52 weeks (safety extension). Of note, through week 26, patients received fixed doses of background medication, but were then permitted to alter background therapies from week 27 through week 78.

As of September 30th, 23 HoFH patients (of the 29 total patients enrolled) had completed the study through week 26, with 12 of these patients having also completed the full 78 weeks of the study. A total of 6 patients discontinued the study, 3 due to GI-related side effects and 3 withdrew consent.

Phase 3 26 Week Intent-to-Treat Analysis

At week 26, the mean reduction in LDL-C vs. baseline was 45% (mean baseline LDL-C of 337 mg/dL vs. 176 mg/dL at Week 26), while the median reduction in triglycerides at Week 26 was also 45% vs. baseline, (median TG levels of 82 mg/dL at baseline vs. 42 mg/dL at Week 26). At week 26, the mean daily lomitapide dose was 37 mg (N=29). The intent-to-treat analysis includes results from all 29 patients enrolled in the study, including the 6 patients that discontinued (analysis used last observation carried forward (LOCF)). *See Figure 8 below.*

Phase 3 26 Week Completer Analysis

Of the patients that completed the 26 week efficacy portion of the trial, (n=23), the mean reduction in LDL-C was 50% vs. baseline (mean baseline LDL-C of 354 mg/dL vs. 167 mg/dL at Week 26). The median reduction in TG level at Week 26 was 54% vs. baseline (median TG levels of 97 mg/dL at baseline vs. 43 mg/dL at Week 26).

Of note, using a completer analysis, 65% of patients (15/23) were able to reduce their LDL-C to below 175 mg/dL and 35% of patients (8/23) were able to reduce their LDL-C to below 100 mg/dL. At Week 26, the mean daily dose of lomitapide was 43 mg in the 23 patients that completed the trial. *See Figure 8 below.*

Figure 8: Lomitapide 26-Week Phase 3 HoFH Data

AEGR Lomitapide Phase 3 in HoFH:	ITT Analysis	Completer Analysis
Number of patients	N=29	n=23
Mean daily dose at Week 26	37 mg	43 mg
Mean reduction in LDL-C vs. baseline	45%	50%
Mean LDL-C at baseline	337 mg/dL	354 mg/dL
Mean LDL-C at Week 26	176 mg/dL	167 mg/dL
Number of patients achieving LDL-C <100 mg/dL		8
Number of patients achieving LDL-C <175 mg/dL		15
Median reduction in triglycerides vs. baseline	45%	54%
Median triglycerides at baseline	82 mg/dL	97 mg/dL
Median triglycerides at Week 26	42 mg/dL	43 mg/dL

Source: Collins Stewart LLC Research, Company Reports

Phase 3 26 Week Safety Analysis

Regarding safety, the most commonly reported adverse events in the first 26 weeks were gastrointestinal-related. Consistent with Phase 2 studies in HoFH patients, increases in liver ALTs and liver fat have also been observed.

Regarding liver fat, 22 patients had their hepatic fat measured through Week 26. The mean hepatic fat increased from 1.2% at baseline to 8.7% at Week 26. Of the 14 patients who had hepatic fat measurements at Week 56, mean fat levels were reduced to 5.1%. Thus, the median change from baseline was 7.5% at Week 26 and 3.9% at Week 56. We note that, while increases in hepatic fat warrant monitoring, the implications of these increases are unclear, as some otherwise healthy patients have high hepatic fat (>8%) without complication.

Regarding liver ALT elevations, as of the September 30th analysis, 4 patients had ALT elevations >5X ULN (upper limit of normal), however, these elevations resolved with dose-reduction (3 patients) or dose–suspension (1 patient) and all patients successfully resumed treatment. Of note, no lomitapide patient that experienced ALT elevations had concomitant increases in bilirubin, suggesting that liver damage is unlikely.

Previous Phase 2 Study in HoFH Demonstrated Preliminary Signs of Efficacy

Prior to the initiation of the Phase 3 trial, lomitapide had been investigated in several Phase 2 trials, including a single-arm, open-label study in HoFH patients conducted by the University of Pennsylvania. This study enrolled a total of 6 patients that were treated with ascending doses of lomitapide at 4-week intervals (doses were 0.03 mg/kg, 0.1 mg/kg, 0.3 mg/kg, and 1.0 mg/kg), for a total of 16 weeks. The mean weight-adjusted dose of lomitapide at the 1.0 mg/kg level was 67.0 mg/day.

At week 16 (with the 4 most recent weeks at the 1.0 mg/kg dose), patients experienced a 50.9% mean reduction in total cholesterol from baseline ($p<0.001$; mean total cholesterol was 851 mg/dL at baseline vs. 349 mg/dL at Week 16). The mean LDL-C reduction at week 16 was 50.9% ($p<0.001$; mean LDL-C was 614 mg/dL at baseline vs. 303 mg/dL at week 16) and the mean triglyceride reduction was 65.2% after 16 weeks of lomitapide treatment ($p<0.001$; mean triglyceride level was 283 mg/dL at baseline vs. 88 mg/dL at Week 16).

All 6 patients completed the 16-week study. The most common adverse events were gastrointestinal in nature. Elevated liver aminotransferase levels were seen in 4 of the 6 patients, however, levels returned towards normal upon dose reduction. Four of the 6 patients also had hepatic fat increases, which returned to baseline levels in 3 patients upon completion of the study (the fourth patient consumed substantial amounts of alcohol).

Lomitapide Offers Benefit Over HoFH Competition

Currently available treatments for HoFH include common lipid lowering treatments, such as statins (Lipitor) and cholesterol absorption inhibitors (Zetia), as well as LDL apheresis. However, many HoFH patients do not experience sufficient benefit from available drugs and apheresis, while effective at lipid lowering, is laborious (can be 2-3 hours per session, 1x per 2 weeks) and expensive (>$100K/year). Thus, we expect lomitapide to be used in conjunction with or to replace these therapies.

We expect lomitapide to face competition from ISIS/GENZ's mipomersen, for which we anticipate U.S. and E.U. regulatory filings to be submitted in 1H11. While we are hesitant to compare efficacy across trials, as the patient populations enrolled in each trial are different, we believe lomitapide does have the advantage of being a once-a-day oral tablet compared to mipomersen's once-weekly injection. Physician feedback suggests that, given the differing mechanisms of action, lomitapide and mipomersen may eventually be used in combination in HoFH patients.

Lomitapide Market Assumptions and Pricing

Market Size

We performed a survey of 10 of the ~43 U.S.-based lipid treatment centers. Feedback suggests that there are ~520 HoFH patients (defined as patients with documented HoFH genetic mutations, or skin fibroblast LDL receptor activity <20% normal, or untreated total cholesterol >500 mg/dL and triglycerides <300 mg/dL and both parents have documented total cholesterol >250 mg/dL, or LDL-C levels >300 mg/dL on maximum doses of currently available therapies) currently receiving treatment in these centers. However, physicians we spoke believe there are a significant number (could be 100% or more of those in lipid centers) of HoFH patients currently being treated by cardiologists rather than lipidologists. Based on this feedback, we estimate that there are ~800 HoFH patients in the U.S., 25% of whom are under the age of 18. Assuming a similar prevalence in the E.U., we arrive at ~940 patients for a U.S. and E.U. total market size of 1.7K HoFH patients eligible for lomitapide treatment.

We note that this is a conservative assumption, as the number of patients that fall into this HoFH definition that are sitting in cardiology offices may be larger. In addition, according to AEGR's market diligence, there are ~3K HoFH patients in the U.S. and another ~3K in the E.U. Given that it is difficult to confirm these estimates, we view any additional patients (above our 1.7K estimate) as upside to our numbers.

Literature suggests that the prevalence of HoFH in the E.U. is similar to that of the U.S. We, therefore, arrive at an E.U. prevalence of ~940 patients, with ~25% of patients under the age of 18.

Physician feedback suggests that the majority of HoFH patients will be eligible for lomitapide treatment. We, therefore, model for peak penetration of ~60% in the U.S. (in both the adult and pediatric populations) and ~55% in the E.U. (in both adults and peds).

Lomitapide Pricing

Given that HoFH is an orphan disease in an area of high unmet medical need, we believe AEGR should likely secure premium pricing for lomitapide. We model for pricing of $175K per patient per year (the company has guided to $150K to $250K/yr), with 3% price increases annually. In the E.U., we assume a slightly lower annual price of $160K, with no price increases.

Taken together, we estimate worldwide sales of lomitapide of $164M in 2018.

We note that lomitapide received an orphan drug designation by the FDA for HoFH, giving the drug 7 years of market exclusivity. The drug did not receive this designation in the E.U., as the E.U. does not distinguish HoFH as a separate disease from HeFH, viewing both as subgroups of "familial hypercholesterolemia". AEGR owes royalties of up to 10% on all lomitapide sales to the University of Pennsylvania.

AEGR Plans Solo Lomitapide Sales and Marketing Strategy

AEGR intends to commercialize lomitapide on its own in both the U.S. and the E.U. Upon approval, the company intends to hire 15 sales representatives to target 400 lipidologists and 43 lipid treatment centers in the U.S. In the E.U. AEGR expects to hire 18 sales representatives to target 500 lipidologists at 75 lipid treatment centers.

Beyond HoFH – FC Opportunity Could Add Meaningful Patient Numbers to Lomitapide's Addressable Market

In 2011, AEGR expects to initiate a Phase 2 study of lomitapide in patients with familial chylomicronemia (FC), a rare disease characterized by extremely high levels of triglycerides. Patients with FC have genetic deficiencies in the lipoprotein lipase (LPL) gene or in apolipoprotein C-II, which prevent their bodies from breaking down fat. As a result, these patients present at very young ages with fasting triglyceride levels above 1,000 mg/dL and as high as 10,000 mg/dL vs. the <200 mg/dL recommended by the American Heart Association. Complications include xanthomas (lipid deposits under the skin), and acute pancreatitis (associated with abdominal pain and risk of death).

Given lomitapide's demonstrated triglyceride-lowering activity in the Phase 3 HoFH study (median reduction of 45% in ITT analysis at Week 26 vs. baseline), we believe the drug could demonstrate significant benefit in this patient population. We note that two patients with severely high triglycerides are already being treated with lomitapide under the FDA's compassionate use program, and we expect the initiation of a formal Phase 2 clinical trial next year. AEGR expects to receive orphan drug designation for lomitapide from both the FDA and EMEA for FC. The EMEA has already informed the company of its intention, while AEGR expects to file for orphan drug status in the U.S. by YE10.

The incidence of FC is estimated to be 1 per 1 million people, suggesting that ~300 patients in the U.S. and ~400 in the E.U. have this disease. Therefore, in entering the FC market, AEGR could significantly increase lomitapide addressable market. Based on lomitapide annual pricing of $175K in the U.S. (with 3% price increases annually) and $160K in the E.U., and assuming ~55% peak penetration, we believe approval in FC could add $100M+ to AEGR's topline. However, we do not currently include lomitapide FC revenue in our model and we would view approval in this indication as a free-call option.

We note that AEGR will gain significant leverage from its HoFH sales force, as the majority of FC patients are also seen by lipidologists.

Lomitapide Intellectual Property

Aegerion currently holds several issued patents for lomitapide in the U.S. including composition of matter (expires 2015; possible extension to 2020) as well as methods of use and methods of treating diseases such as hyperlipidemia and hypercholesterolemia (expire 2013-2019). The ex-U.S. lomitapide composition of matter patents expire in 2016. The company has filed for additional patents that cover use of lomitapide in combination with statins, method of dose titration and triple drug combinations (expire in mid-2020).

Lomitapide was granted an orphan drug designation by the FDA for HoFH in 2007, giving the drug 7 years of market exclusivity (HoFH is not eligible for an orphan drug designation in the E.U.).

Management Team

Marc D. Beer joined AEGR as Chief Executive Officer of AEGR in August, 2010. Prior to this, Mr. Beer served as the President and Chief Executive Officer of ViaCell. Before this, he served as Vice President of Global Marketing at Genzyme, and prior to this, was Vice President, Sales and Marketing at Biostar. Before joining Biostar, Mr. Beer held several sales and marketing positions at Abbott Labs. He currently serves as the Chairman of the board of directors of TxCell, the Vice-Chairman of PCT Therapeutics, and Chairman of Good Start Genetics and Seaside Therapeutics. Mr. Beer received a B.S. from Miami University of Ohio.

William H. Lewis is President of AEGR, and is also a co-founder of the company. Prior to his promotion to President in 2009, Mr. Lewis served as Chief Financial Officer. During his time at AEGR, he also served as Secretary, Treasurer, and Vice President of Administration as well as Senior Vice President of Finance and Administration. Prior to joining AEGR, Mr. Lewis was a Managing Director at a hedge fund, and, prior to this, was a Managing Director, Head of Capital Markets Investment Banking at Wells Fargo Securities. He has also served as a Principal at Robertson Stephens & Company and a Vice President at J.P. Morgan. Mr. Lewis received a B.A. with Honors from Oberlin College and a M.B.A./J.D with Honors from Case Western Reserve University.

Christine A. Pellizzari is Executive Vice President, General Counsel and Secretary of AEGR. Prior to this, Ms. Pellizzari was Vice President, General Counsel and Secretary. Prior to her time at AEGR, she held several positions of increasing responsibility at Dendrite International, Inc., most recently as Senior Vice President, General Counsel and Secretary. Ms. Pellizzari received a B.A. from the University of Massachusetts at Amherst and a J.D. from the University of Colorado School of Law at Boulder.

John T. Cavan is Chief Accounting Officer and Vice President of AEGR. Prior to this, Mr. Cavan was AEGR's Corporate Controller. Before his time at AEGR, he served as Controller of AlgoRx Pharmaceuticals. Prior to this, Mr. Cavan held several financial and operational positions with a variety of companies including Sony, American Express and Nestle. Mr. Cavan received a B.B.A. in Accountancy from Iona College and an M.B.A. in Finance from Seton Hall University.

Financials

As of September 30th 2010, AEGR had $0.4M in cash and cash equivalents. This does not include ~$48.8M in net proceeds AEGR received from its October 27, 2010 IPO. At the end of 3Q10, AEGR had $3.1M in notes payable due 2011, $21.9M in convertible notes (8% interest) due December 31, 2010, and a warrant liability of $2.1M. However, notes payable were repaid following the IPO and all convertible notes automatically converted into common stock (at 80% of the IPO price of $9.50 per share). The warrant liability (387, 238 shares of series A redeemable preferred stock) was converted to 107,779 common shares with an exercise price of $6.68. The company has 1.8M options outstanding. In 3Q10, AEGR reported a net loss of $6.2M, or $(3.61) per share. We believe that AEGR's cash and equivalents are sufficient to support operations through the commercialization of lomitapide.

Revenue Builds – Lomitapide

Homozygous Familial Hypercholesterolemia (HoFH) Market

U.S.

	FY 2010E	FY 2011E	FY 2012E	FY 2013E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E
Total U.S. Population ('000)	310,110	312,600	316,359	319,206	322,079	324,978	327,902	330,854	333,831
Prevalence of homozygous FH patients (1/1,000,000)	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*
Total number of homozygous FH patients	806	813	823	830	837	845	853	860	868
Proportion of homozygous adult FH patients	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*
Total number of adult homozygous FH patients	605	610	617	622	628	634	639	645	651
Market penetration into adult homozygous FH	*0.0%*	*0.0%*	*4.5%*	*14.9%*	*25.1%*	*35.2%*	*42.8%*	*48.2%*	*52.1%*
Total number of adult HoFH patients on lomitapide	0	0	28	93	158	223	274	311	339
Proprotion of homozygous pediatric FH patients	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*
Total number of pediatric homozygous FH patients	202	203	206	207	209	211	213	215	217
Market penetration into pediatric homozygous FH	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*4.1%*	*14.4%*	*24.7%*	*34.9%*	*41.9%*
Total number of pediatric HoFH patients on lomitapide	-	-	-	-	9	30	53	75	91
Total number of HoFH patients on lomitapide	-	-	28	93	166	253	326	386	430
Annual cost per patient	-	-	$ 175,000	$ 180,250	$ 185,658	$ 191,227	$ 196,964	$ 202,873	$ 208,959
Total U.S. revenue from lomitapide ($'000)	**$ -**	**$ -**	**$ 4,858**	**$ 16,717**	**$ 30,861**	**$ 48,473**	**$ 64,272**	**$ 78,314**	**$ 89,868**

E.U.

	FY 2010E	FY 2011E	FY 2012E	FY 2013E	FY 2014E	FY 2015E	FY 2016E	FY 2017E	FY 2018E
Total E.U. Population ('000)	362,527	365,436	369,091	372,781	376,509	380,274	384,077	387,918	391,797
Prevalence of homozygous FH patients (1/1,000,000)	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*	*0.0003%*
Total number of homozygous FH patients	943	950	960	969	979	989	999	1,009	1,019
Proportion of homozygous adult FH patients	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*	*75%*
Total number of adult homozygous FH patients	707	713	720	727	734	742	749	756	764
Market penetration into adult homozygous FH	*0.0%*	*0.0%*	*0.8%*	*9.9%*	*19.0%*	*28.1%*	*36.8%*	*43.4%*	*48.2%*
Total number of adult HoFH patients on lomitapide	0	0	6	72	139	208	276	328	368
Proprotion of homozygous pediatric FH patients	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*	*25%*
Total number of pediatric homozygous FH patients	236	238	240	242	245	247	250	252	255
Market penetration into pediatric homozygous FH	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*2.3%*	*12.1%*	*21.8%*	*31.4%*	*38.4%*
Total number of pediatric HoFH patients on lomitapide	-	-	-	-	6	30	54	79	98
Total number of HoFH patients on lomitapide	-	-	6	72	145	238	330	407	466
Annual cost per patient	-	-	$ 160,000	$ 160,000	$ 160,000	$ 160,000	$ 160,000	$ 160,000	$ 160,000
Total E.U. revenue from lomitapide ($'000)	**$ -**	**$ -**	**$ 921**	**$ 11,514**	**$ 23,220**	**$ 38,125**	**$ 52,806**	**$ 65,195**	**$ 74,567**

TOTAL LOMITAPIDE SALES - WW ($'000)	**$ -**	**$ -**	**$ 5,779**	**$ 28,232**	**$ 54,081**	**$ 86,598**	**$ 117,078**	**$ 143,509**	**$ 164,435**

Source: Collins Stewart LLC Research and Company Reports

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Consolidated Income Statement
($thousands, except per share data)

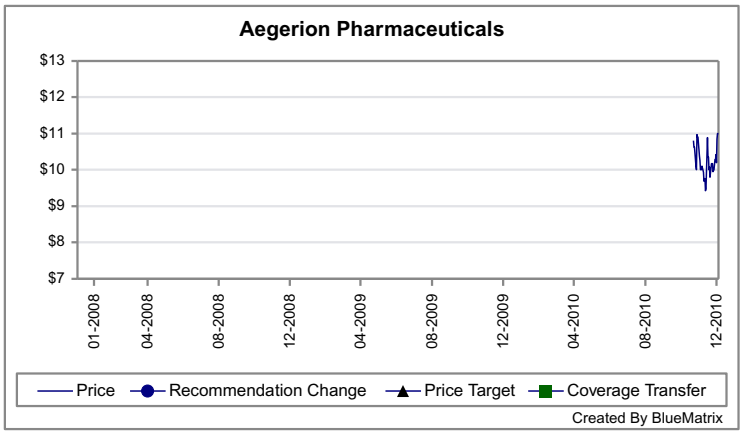
	FY 2008A	FY 2009A	1H10A	Sep 3Q10A	Dec 4Q10E	FY 2010E	Mar 1Q11E	Jun 2Q11E	Sep 3Q11E	Dec 4Q11E	FY 2011E	FY 2012E	FY 2013E	FY 2014E	FY 2015E
Revenue															
Lomitapide Total Revenue	**-**	**-**	-	-	-	**-**	-	-	-	-	**-**	**$ 5,779**	**$ 28,232**	**$ 54,081**	**$ 86,598**
Lomitapide HoFH - U.S.	**-**	**-**	-	-	-	**-**	-	-	-	-	**-**	4,858	16,717	30,861	48,473
Lomitapide HoFH - E.U.	**-**	**-**	-	-	-	**-**	-	-	-	-	**-**	921	11,514	23,220	38,125
Total Revenue	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ -**	**$ 5,779**	**$ 28,232**	**$ 54,081**	**$ 86,598**
COGS	-	-	-	-	-	-	-	-	-	-	-	462	3,670	7,031	10,392
Gross profit	-	-	-	-	-	-	-	-	-	-	-	5,317	24,562	47,050	76,206
Operating expense															
R&D	17,712	7,041	2,293	1,333	1,453	5,079	1,946	2,201	2,743	3,211	10,101	11,535	13,003	14,523	16,491
SG&A	5,185	3,075	1,670	1,531	1,878	5,079	1,701	1,823	2,014	2,156	7,694	15,126	20,047	23,547	27,046
Total operating expense	22,897	10,116	3,963	2,864	3,331	10,158	3,647	4,024	4,757	5,367	17,795	26,661	33,050	38,070	43,537
Operating income (loss)	**(22,897)**	**(10,116)**	**(3,963)**	**(2,864)**	**(3,331)**	**(10,158)**	**(3,647)**	**(4,024)**	**(4,757)**	**(5,367)**	**(17,795)**	**(21,344)**	**(8,488)**	**8,980**	**32,669**
Interest Expense	(1,127)	(2,083)	(1,183)	(607)	(202)	(1,992)	-	-	-	-	-	-	-	-	-
Interest Income	533	177	39	15	23	77	44	40	36	31	151	17	9	8	33
Change in fair value of warrant liability	91	(174)	349	(1,835)	72	(1,414)	52	56	61	64	233	241	266	-	-
Other income, net	(1,635)	-	(30)	-	-	(30)	-	-	-	-	-	-	-	-	-
Total Other Income	(2,138)	(2,080)	(825)	(2,427)	(107)	(3,359)	96	96	97	95	384	258	275	8	33
Income Before Income Taxes	(25,035)	(12,196)	(4,788)	(5,291)	(3,438)	(13,517)	(3,551)	(3,928)	(4,660)	(5,272)	(17,411)	(21,086)	(8,213)	8,988	32,702
Income Tax Provision	-	-	1,793	-	-	1,793	-	-	-	-	-	-	-	-	-
Accretion of preferred stock dividends	(6,242)	(3,287)	(1,734)	(881)	(294)	(2,908)	-	-	-	-	-	-	-	-	-
Net income (GAAP)	**$ (31,277)**	**$ (15,483)**	**$ (4,728)**	**$ (6,172)**	**$ (3,732)**	**$ (14,632)**	**$ (3,551)**	**$ (3,928)**	**$ (4,660)**	**$ (5,272)**	**$ (17,411)**	**$ (21,086)**	**$ (8,213)**	**$ 8,988**	**$ 32,702**
GAAP EPS (diluted)	**$ (20.92)**	**$ (9.35)**	**$ (2.77)**	**$ (3.61)**	**$ (0.32)**	**$ (6.70)**	**$ (0.20)**	**$ (0.22)**	**$ (0.26)**	**$ (0.29)**	**$ (0.98)**	**$ (1.16)**	**$ (0.45)**	**$ 0.44**	**$ 1.59**
Weighted shares outstanding diluted - GAAP	1,495	1,657	1,705	1,708	11,741	4,215	17,691	17,780	17,869	17,958	17,824	18,138	18,319	20,364	20,568
Margin Analysis:															
Cost of product sales	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	8%	13%	13%	12%
Product gross margin	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	92%	87%	87%	88%
R&D (non-GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	200%	46%	27%	19%
SG&A (non-GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	262%	71%	44%	31%
Stock-based compensation expense	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	26%	9%	6%	5%
Total operating expense	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	461%	117%	70%	50%
Operating margin	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	-369%	-30%	17%	38%
Income tax provision	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	0%	0%	0%	0%
Net margin (GAAP)	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	17%	38%
Y/Y change:															
Total revenue	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	nm	388%	92%	60%
R&D	31%	-60%	nm	nm	nm	-28%	nm	nm	nm	nm	99%	14%	13%	12%	14%
SG&A	-15%	-41%	nm	nm	nm	65%	nm	nm	nm	nm	51%	97%	33%	17%	15%
Stock-based compensation expense	-30%	-25%	nm	nm	nm	-36%	nm	nm	nm	nm	56%	62%	70%	36%	27%
Total operating expense	17%	-56%	nm	nm	nm	0%	nm	nm	nm	nm	75%	50%	24%	15%	14%
Operating income	17%	-56%	nm	nm	nm	0%	nm	nm	nm	nm	75%	20%	-60%	-206%	264%
Net income (GAAP)	31%	-50%	nm	nm	nm	-5%	nm	nm	nm	nm	19%	21%	-61%	-209%	264%
GAAP EPS (diluted)	9%	-55%	nm	nm	nm	-28%	nm	nm	nm	nm	-85%	19%	-61%	-198%	260%
Shares outstanding - GAAP	20%	11%	nm	nm	nm	154%	nm	nm	nm	nm	323%	2%	1%	11%	1%

Source: Collins Stewart LLC Research and Company Reports

Important Disclosure / Disclaimer Information

Other Public Companies Mentioned in this Report

Company	Ticker	Price	Recommendation
Aegerion Pharmaceuticals	AEGR	$10.99	



Ticker	Date	Action	Prior Rating	Current Rating	Price	Target Price

Collins Stewart LLC Ratings

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