COMPANY NOTE

Initiating Coverage

USA | Healthcare | Biotechnology

October 10, 2011

Jefferies

EQUITY RESEARCH AMERICAS

Aegerion Pharmaceuticals (AEGR)

Initiating with Buy: Targeting Niche Orphan Markets

BUY

Price target $20.00

Price $13.14

Key Takeaway

Aegerion has one product, lomitapide, for treatment of phenotypic HoFH. Based on positive Ph3 data, the company plans to file for approval in 1Q12 in the U.S./Europe. While regulatory risks remain (AdComm panel, FDA action), given our 75% probability-adjusted valuation, we think AEGR offers an attractive opportunity for medium/high-risk tolerant investors at current levels. We initiate coverage with a Buy rating and $20 price target.

Aegerion's lomitapide targets a niche market where there is a lack of effective therapies (severe hypercholesterolemia). On positive Ph3 data for lomitapide in phenotypic homozygous familial hypercholesterolemia (HoFH), the company plans to file for approval in 1Q12. Aegerion also plans to expand indications by conducting additional Ph3 studies for pediatric HoFH and severe familial chylomicronemia (FC), with data potentially in ~2013. Our current valuation is solely based on adult phenotypic HoFH (~6,000 patients in the U.S. and five major E.U. countries combined, according to Aegerion).

We conservatively assume commercial launches of lomitapide in the U.S./E.U. in early-2013/2H13 and peak annual sales potential of ~$315M/~$250M, respectively. Our assumptions include a standard 10-month review at FDA, annual Tx cost of ~$150K/patient, marketing exclusivity of lomitapide through ~2020 in the U.S. and ~1H22 in Europe, and Aegerion commercializing the drug using its sales force.

Risks are high heading into regulatory events (AdComm panel, potential FDA action); however, we think AEGR is attractive for medium/high-risk tolerant investors at current valuation. Aegerion's success entirely depends on its one product—lomitapide. We expect safety/tolerability and risk/benefits to be a focus at the FDA's Endocrinologic and Metabolic Drugs Advisory Committee meeting in ~2Q12. While we have not seen detailed Ph3 data yet, based on the reported data to date, in this severe hypercholesterolemia patient population, we think the lomitapide benefits outweigh risks.

Valuation/Risks

Our $20 PT is based on an NPV analysis of Aegerion's lomitapide sales in the U.S. and Europe. Risks include but are not limited to: (1) success depends on the company's one product lomitapide; (2) regulatory delays/failure for lomitapide; (3) commercial failure of lomitapide if approved; and (4) and general risks for the drug industry (e.g., patent infringement, changes in regulatory and/or healthcare policies, pricing/reimbursement).

USD	Prev.	2010A	Prev.	2011E	Prev.	2012E	Prev.	2013E
Rev. (MM)	--	0.0	--	0.0	--	0.0	--	13.9
EV/Rev								14.3x
Consensus	--	--	--	--	--	56.72	--	121.82
EPS								
Mar	--	NA	--	(0.39)A	--	(0.53)	--	--
Jun	--	NA	--	(0.49)A	--	(0.59)	--	--
Sep	--	(3.61)	--	(0.45)	--	(0.53)	--	--
Dec	--	(0.92)	--	(0.47)	--	(0.57)	--	--
FY Dec	--	(5.07)	--	(1.79)	--	(2.21)	--	(2.10)

EPS: AEGR completed its IPO in 3Q10

Financial Summary	
Book Value (MM):	$75.6
Book Value/Share:	$4.27
Net Debt (MM):	$(78.2)
Long-Term Debt (MM):	$9.4
Cash/Share:	$4.94
Cash (MM):	$87.5

Market Data	
52 Week Range:	$25.92 - $9.00
Total Entprs. Value (MM):	$199.1
Market Cap. (MM):	$277.3
Insider Ownership:	32.5%
Institutional Ownership:	41.9%
Shares Out. (MM):	21.1
Float (MM):	14.0
Avg. Daily Vol.:	61,930

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Price Performance

Aegerion Pharmaceuticals, Inc.

Buy: $20 Price Target

THE LONG VIEW

Scenarios

Target Investment Thesis

- Cash at end-2Q11 of ~$87M sufficient to fund operations through 2012
- Our NPV analysis pegs a target price of $20/sh (75% probability adjusted U.S. /EU lomitapide sales of ~$11/sh and ~$9/sh, respectively, discounted at 11%)

Upside Scenario

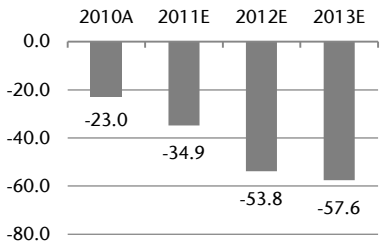
- Timely regulatory approval of lomitapide
- Potential expansion of indications for lomitapide beyond HoFH (e.g., pediatric HoFH, FC)
- If lomitapide achieves regulatory approval in the U.S./EU, we estimate AEGR's fair value per share at $30

Downside Scenario

- Delays/failures in regulatory approval of lomitapide
- Limited market uptake of lomitapide upon approval due to side effects or competition from mipomersen
- Distant profitability
- If lomitapide fails to reach the market, AEGR shares could trade below cash (our estimated cash of ~$3/sh at end-2011)

Long Term Analysis

Net Income/Loss ($ in MM)



Source: Capital IQ, Jefferies estimates

Long Term Financial Model Drivers

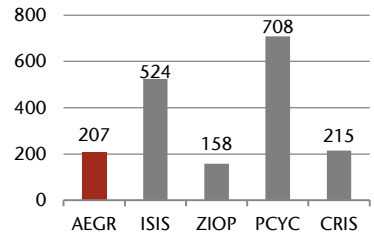
LT Revenue CAGR ('13-'17)	**83%**
Organic Revenue Growth	**83%**
Acquisition Contribution	**0%**
Operating Margin Expansion	**NA**

Other Considerations

Currently AEGR has only one product lomitapide; if it fails to reach the market, there will likely be very little value assigned to the company. We assess lomitapide potential in apheresis-eligible FH (3.7% of FH), not differentiating HoFH (~0.1% of FH) from severe HeFH; and forecast ~$560M in 2020 sales in U.S./EU. If initial approval is restricted to genotypic HoFH, there would be significant downside to our estimates.

Peer Group

Enterprise Value ($ in MM)



Source: Capital IQ, Jefferies estimates

Recommendation / Price Target

Ticker	Rec.	PT
AEGR	**Buy**	**$20**
ISIS	Buy	$12
ALNY	NC	NC
HALO	Buy	$10
IMGN	NC	NC

Catalysts

- 72-week efficacy/safety data from Phase 3 study for lomitapide in late-2011
- U.S./EU regulatory filing for lomitapide in HoFH in 1Q12
- Potential FDA's Endocrinologic and Metabolic Drugs Advisory Committee meeting for lomitapide in ~2Q12
- Potential FDA approval of lomitapide in late-2012/early-2013 (assuming 10-month review)

Company Description

Founded in 2005 and completed its IPO in 2010, Aegerion Pharmaceuticals is an emerging biotechnology company with a focus on developing and commercializing drugs for the treatment of severe lipid disorders. AEGR's lead drug candidate, lomitapide (microsomal tryglyceride transfer protein, or MTP, inhibitor), showed positive Phase 3 data in patients with the rare disease homozygous familial hypercholesterolemia (HoFH).

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Executive Summary

We are initiating coverage of Aegerion Pharmaceuticals with a Buy rating and $20 price target. Aegerion has one product, lomitapide, in Phase 3 development for the treatment of phenotypic homozygous familial hypercholesterolemia (HoFH). Based on positive Phase 3 data, Aegerion plans to file for approval in 1Q12 both in the U.S. and in Europe. We expect an AdComm panel (the FDA's Endocrinologic and Metabolic Drugs Advisory Committee) for lomitapide and potentially competitor ISIS' mipomersen in 2Q12. While regulatory risks remain, on 75% probability-adjusted valuation, we view AEGR offers an attractive risk/reward at the current valuation.

We project lomitapide peak sales opportunity in patients with phenotypic HoFH at~$315M in the U.S. and ~$250M in Europe. According to Aegerion, there are ~6,000 patient candidates for lomitapide in the U.S. and five major EU countries combined. On our estimated annual cost of ~$150K/patient, this represents ~$900M market opportunity. We assume lomitapide launches in early-2013 in the U.S. and in 2H13 in Europe, and Aegerion commercializing the drug using its sales force. We currently assume marketing exclusivity of lomitapide through ~2020 in the U.S. and ~1H22 in Europe.

Lomitapide vs. competitor mipomersen. Given that lomitapide and mipomersen work via a different mechanism of action (MOA), we view both drugs have potential in treating phenotypic HoFH. A clear advantage of lomitapide is its oral dosing vs. weekly subcutaneous (SC) injection for mipomersen. On efficacy, in the absence of a head-to-head study, we view both drugs are comparably efficacious in lowering low-density lipoprotein cholesterol (LDL-C) levels. On safety, on-target effects include liver toxicity (e.g., fatty liver, liver function test abnormality). In addition, lomitapide's gastrointestinal (GI) toxicity and mipomersen's injection site reactions led to therapy discontinuation in ~10% and ~6%, respectively, in their respective HoFH clinical studies.

Risks are high heading into regulatory events; however, we view AEGR is attractive at current valuation for medium/high-risk tolerant investors. Aegerion's success entirely depends on one product, lomitapide. We expect safety/tolerability to be a focus of discussion at the AdComm panel. While we have not seen detailed data yet, based on the data reported to date, in this severe hypercholesterolemia patient population, we think benefits outweigh risks.

Upcoming catalysts for lomitapide include: (1) 72-week efficacy/safety data from Phase 3 study in late-2011; (2) NDA/MAA filing for FDA/EMA approval in 1Q12; (3) Phase 3 initiation of lomitapide in pediatric HoFH in 1Q12; (4) Phase 3 initiation of lomitapide in FC in 1Q12; (5) potential FDA's Endocrinologic and Metabolic Drugs Advisory Committee meeting for lomitapide (& potentially for mipomersen) in 2Q12; and (6) potential FDA approval of lomitapide in late-2012/early-2013 (assuming 10-month review).

Valuation

Our $20 PT is based on an NPV analysis of lomitapide U.S./EU sales. We expect $87M in end-2Q11 cash to be sufficient into 2013 and forecast profitability in 2015.

Risks

Risks associated with AEGR shares include, but are not limited to: (1) success depends on its one product lomitapide; (2) potential regulatory delays/failure for lomitapide; (3) commercial failure of lomitapide if approved and potential competition from ISIS' mipomersen; and (4) and general risks for the drug industry (e.g., patent infringement, changes in regulatory and/or healthcare policies, pricing/reimbursement).

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Lomitapide for Rare Lipid Disorders

Designed to address rare genetic lipid disorders, lomitapide is in post-Phase 3 for adult homozygous familial hypercholesterolemia (HoFH) and two additional Phase 3 trials are slated to begin for pediatric HoFH in 1Q12 and for familial chylomicronemia (FC) in 1Q12.

Adult Homozygous Familial Hypercholesterolemia (HoFH) – Near-term Opportunity

Aegerion plans to file for approval of lomitapide in 1Q12 on its positive 56-week Phase 3 data. Assuming a standard 10-month review at the FDA, we expect approval in late-2012/early-2013 in the U.S. and European approval in ~1H13.

Homozygous familial hypercholesterolaemia (HoFH) is a rare genetic disorder in which both LDL-receptor alleles are defective and which affects about one in 1,000,000 people. This results in very high levels of LDL cholesterol in plasma and premature coronary artery disease. Heterozygous familial hypercholesterolemia (HeFH) is a monogenic disorder that affects about one in 500 people.

Low-density lipoprotein cholesterol (LDL-C) is a causative factor for coronary heart disease (CHD) and is a target for lipid-lowering therapy such as statins (HMG-CoA reductase inhibitors). LDL-C reduction has been the gold standard for lipid-lowering drugs approval (the bottom line is lowering LDL-C levels lowers cardiovascular (CV) risks). In patients with CHD, the recommended LDL-C target level is <100 mg/dL. For patients without CHD, the recommended level is <160 mg/dL with fewer than two CHD risk factors (e.g., high blood pressure, cigarette smoking, diabetes, overweight, low HDL cholesterol) and <130 mg/dL with two or more CHD risk factors.

In practice, cardiologists treat hypercholesterolemia patients based on LDL-levels and risk factors, not by genetic testing. Particularly, in HoFH patients, there are several hundred different mutations in other genes causing CV-related morbidities (e.g., mutations in the apolipoprotein B (apoB) gene, PCSK9 gene). Thus, the distinction between HoFH and severe HeFH does not appear to be important to them, although they note that the payers may see this differently and may require more clinically driven outcomes (e.g., death, heart attack, stroke) particularly for costly treatments.

As shown in Exhibit 1, based on our previous survey, we estimate ~23K apheresis-eligible FH patients in the U.S. (with an average LDL-C level ~280 mg/dL); however, ~6,000 of these eligible patients receive apheresis according to the surveyed physicians (Aegerion estimates ~1,000 FH patients on apheresis). Our estimate of 6,000 patients on apheresis seems to be high, likely due to amplification of rough estimates on a small sample size in our survey (50 cardiologists). Apheresis is mechanical filtration of blood (similar to kidney dialysis) used to lower lipid levels. Patient refusal and inaccessible treatment centers (~43 treatment centers in the U.S.) were cited as most common reasons for not receiving apheresis for apheresis-eligible patients. We estimate there are ~600,000 FH patients in the U.S. based on the often cited prevalence estimate of 1/500.

Pediatric HoFH – Potential Market Expansion

Aegerion plans to start a pivotal trial of lomitapide in pediatric HoFH patients in 1Q12. While full trial design and protocol have not as yet been disclosed, we expect the trial to be similar in size to the adult HoFH trial (n=~30; 29 patients enrolled in HoFH Phase 3 study), with a ~12-month accrual period and ~6-month follow-up. Aegerion plans to

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review the proposed pediatric HoFH trial design with the FDA prior to study initiation. We estimate that potential approval in the pediatric indication in the U.S. and EU could occur by 2014; however, we do not currently factor potential sales in pediatric HoFH into our revenue projections.

The company estimates the potential pediatric HoFH market to be ~75%–100% of the adult HoFH market, at roughly ~4,500–6,000 pediatric patients (based on Aegerion's ~6,000 adult phenotypic HoFH patients in the U.S. and five major EU countries combined). If the pediatric indication is granted, it would give lomitapide an additional six months of marketing exclusivity in both the U.S. and EU.

Familial Chylomicronemia (FC) – Potential Market Expansion

Aegerion plans to start a pivotal Phase 3 study in likely genetically-defined FC in 1Q12. While details of the planned Phase 3 study have not been disclosed, we expect the trial to be similar in size to the adult HoFH trial (n=~30), with a ~12-month accrual period and ~6-month follow-up. We estimate that potential approval in FC in the U.S. and EU could occur by 2014; however, we do not currently factor potential sales in FC into our revenue projections.

FC is a rare genetic disorder and a severe form of hypertriglyceridemia (as much as 15X the normal triglyceride levels of <150 mg/dL) often owing to the absence of a key enzyme (lipoprotein lipase) needed to remove triglycerides (TG) from the blood. There is no overlap between the FC and FH populations since FC is diagnosed by elevated triglyceride (TG) levels of typically ~1000 mg/dL – 2,000 mg/dL, whereas FH patients tend to have normal TG levels (<150 mg/dL). Prevalence of FC is approximately one in one million people for homozygous FC, while heterozygous FC is estimated at one in 500 people. Aegerion estimates there are ~1,000 severe FC patients in the U.S. and EU.

In the pivotal HoFH trial, Aegerion demonstrated that patients treated with lomitapide had decreased triglyceride levels at 26- and 56-weeks from baseline. At 26 weeks, patients had a 45% decrease in TG levels to 48 mg/dL from 82 mg/dL at baseline in intent-to-treat (ITT) (n=29) and 54% decrease in completers (n=23) to 43 mg/dL from 97 mg/dL. At 56 weeks, patients had a ~33% decrease in TG levels from baseline (to ~65 mg/dL from ~97 mg/dL). This substantial drop in TGs was the basis for the planned Phase 3 trial in FC patients.

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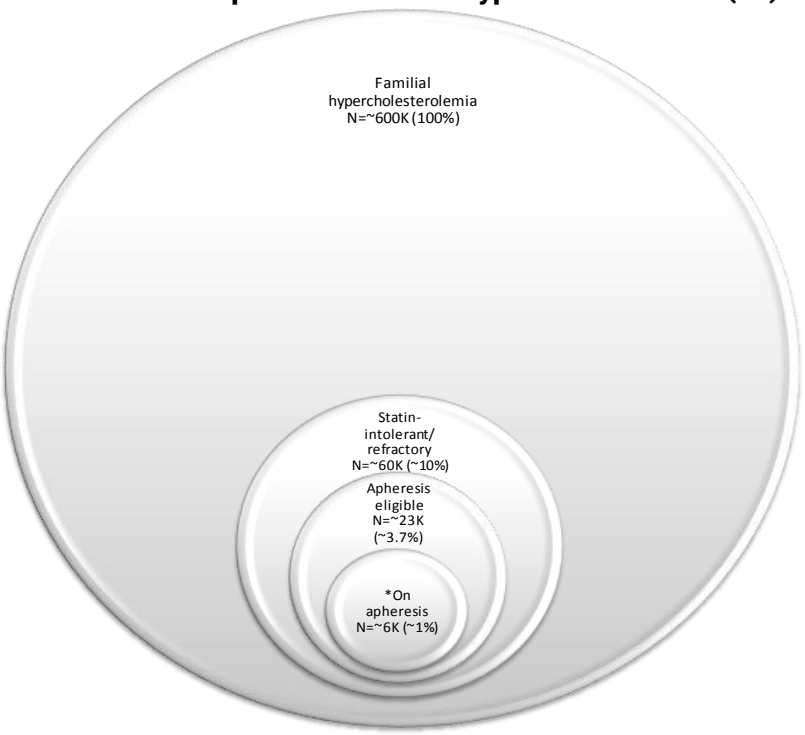
Exhibit 1: Estimated Composition of Familial Hypercholesterolemia (FH) Patients in the U.S.



Source: Company reports and Jefferies & Company, Inc.

Lomitapide

Originally developed by Bristol-Myers Squibb in 1990s. Lomitapide is a microsomal triglyceride transfer protein (MTP) inhibitor, designed to lower LDL cholesterol levels (via daily oral dosing), and prevents very low-density lipoprotein (VLDL) assembly and secretion into the liver. Previously known as BMS-201038, lomitapide was originally developed by Bristol-Myers Squibb (BMY, $32.38, Buy) in the 1990s, donated to the University of Pennsylvania in 2003 and subsequently licensed to Aegerion in 2006. Early Phase 1 and 2 trials were conducted by BMY and the University of Pennsylvania in broader populations (patients with high cholesterol at moderately high risk of a CV event or unable to tolerate statins) with lomitapide monotherapy (doses ranging between 25 mg and 100 mg) as statin replacement therapy. In these trials, investigators saw 'a very high rate' of GI events including diarrhea, nausea and vomiting, elevated liver enzymes in some patients, and a significant increase in hepatic fat accumulation. These adverse side effects led to a high dropout rate in these early trials. Aegerion noted that these early trials did not employ dose titration, and that its subsequent pivotal trial used dose titration as a means to reduce adverse events.

Granted orphan drug status for HoFH in the U.S. and FC in the U.S./EU. In October 2007, the FDA granted lomitapide (AEGR-733) an orphan drug status for the treatment of homozygous familial hypercholesterolemia (HoFH). In Europe, in October 2010, Aegerion withdrew its application to the EMA for orphan drug status of lomitapide in HoFH based on the guidance received from the EMA that lomitapide was a potential treatment for a broader patient population than HoFH. For familial chylomicronemia

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(FC), lomitapide received orphan drug designation from the FDA in March 2011 and from the EMA in October 2010.

We assume marketing exclusivity through ~2020 in the U.S. and ~1H of 2022 in Europe. In our assumptions, we do not include potential extended protection from the recently issued "Titration" patent for lomitapide (August 19, 2027, expiry in the U.S.; in EU, patent was allowed but not yet issued and would expire in 2025). In the U.S., the "Composition of Matter" patent for lomitapide expires on April 14, 2015; however, Aegerion expects an additional 5-year Hatch-Waxman extension from the patent expiry date plus 6-month pediatric exclusivity, providing marketing exclusivity until ~October 2020. Note that lomitapide also has 7-year orphan drug exclusivity from launch.

In Europe, Aegerion expects 10-year data exclusivity as a new chemical entity from the date of regulatory filing (protection through ~1Q22) and additional 6-month pediatric exclusivity, providing protection until ~mid-2022. In addition, the composition of matter patent for lomitapide in EU expires on February 16, 2016, but the Supplementary Protection Certificate (SPC) for lomitapide would provide up to an additional five years of protection from patent expiry in the EU. The SPC is applied for only after Aegerion receives EU approval and is both applied for and granted on a country-by-country basis.

Regulatory Timeline for Lomitapide

Regulatory filing for approval in the U.S/EU in 1Q12. Aegerion's NDA submission will seek approval of the 5-, 10-, and 20-mg doses of lomitapide in functional HoFH. On June 15, 2011, Aegerion had a pre-NDA meeting with the FDA and continues to expect the FDA would accept the single-arm, open-label Phase 3 trial as a pivotal study for full approval. In addition, according to Aegerion, the FDA would expect the initial patient profile for lomitapide to include one of the following qualifiers: (1) historical total cholesterol levels of >500 mg/dL and parents with history of total cholesterol levels of >250 mg/dL; (2) less than 20% fibroblast LDL receptor function; or (3) genotypically characterized HoFH. Aegerion noted that these patients would have LDL-C levels of >400 mg/dL if not on statins. With statins, their LDL-C levels would be ~25% lower. Thus, with one of the inclusion criteria in its Phase 3 study (maximally treated LDL-C of >300 mg/dL), Aegerion noted that the FDA's patient profile definition is consistent with the inclusion criteria for Aegerion's Phase 3 study. Aegerion's EMA submission package also seeks approval in functional HoFH.

Aegerion's original request for Fast Track designation was denied as a Fast Track review requires clinical endpoints, not a surrogate endpoint (i.e., percentage change in LDL-C levels, the primary endpoint for the lomitapide Phase 3 trial). While the company anticipates that there is still the potential for a 6-month priority review, we conservatively assume a 10-month standard review.

With a standard 10-month review at the FDA, we expect potential FDA approval in late-2012/early-2013 and EU approval in 1H13. As a new chemical entity (NCE), we expect an FDA Endocrinologic and Metabolic Drugs Advisory Committee (AdComm) meeting for lomitapide and ISIS' (ISIS, $7.26, Buy) /SNY's (SAN FP, €49.15, Hold) mipomersen in 2Q12. Assuming no delay, we anticipate FDA approval of lomitapide in late-2012/early-2013 and assume a U.S. launch in early-2013. Aegerion noted that a rapporteur and co-rapporteur (country-specific regulatory authority in the EU) have been selected to provide independent assessments to the CHMP. In the EU, assuming a regulatory filing in 1Q12, we anticipate approval/launch in 1H13 (no orphan status in EU). Note that SNY submitted an MAA to EMA for mipomersen (Kynamro) to treat homozygous and severe heterozygous FH on July 28, 2011.

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Potential Regulatory Risks for Lomitapide

Single-arm, open-label study with a small number of patients. Our regulatory consultant noted a potential risk that the FDA may view a single-arm, open-label, 29-patient study as insufficient for lomitapide approval in HoFH. However, we note that Carbaglu for NAGS deficiency was approved by the FDA last year (Exhibit 2) based on a single-arm, open-label study (n=7), which was a very small study of seven patients. In addition, the recent approval of Adcetris in relapsed/refractory Hodgkin's lymphoma and systemic anaplastic large cell lymphoma (ALCL) shows that a non-randomized, single-arm, open-label trial would be sufficient to gain FDA approval (although it's accelerated approval; Exhibit 2). Aegerion underscores that the FDA confirmed the protocol, the endpoint, and the design of the trial as appropriate for NDA filing at the June 15th pre-NDA meeting. In addition, the company noted that there is enough clinical exposure to feel comfortable with the safety profile (~700 subjects exposed to date). Oftentimes at the FDA, "acceptable for filing" does not necessarily translate into approval; and the FDA can change its mind, according to our regulatory consultant. Given the clear efficacy of lomitapide and unmet therapeutic needs in an ultra-small patient population, we view its benefits outweigh limited clinical data and potential safety issues. However, unpredictability around the FDA's final decision cannot be ruled out.

Competitor ISIS' placebo-controlled Phase 3 trials for mipomersen. In ISIS' HoFH study, 51 genetically-defined HoFH patients were randomized 2:1 to either mipomersen or placebo (compared to n=29 functionally-defined HoFH for lomitapide single-arm trial). The FDA might view ISIS' placebo-controlled studies as setting a higher bar for lomitapide approval (see Exhibit 5 for details of lomitapide and mipomersen trials). Aegerion's rationale for having run an open-label, single-arm study was based on the FDA's input that a placebo-controlled trial in the HoFH population would be unethical. However, the fact that ISIS has successfully run a randomized, controlled study in a very similar patient population may give the agency reason to reassess its position when reviewing the two applications. Our consultant proposed that even in the setting of orphan indications, the FDA may want to see data from approximately 10% of the target population, which would correlate to a 200- to 300-patient trial (according to ~3,000 adult phenotypic HoFH patients in the US cited by Aegerion). Particularly, lomitapide is the first in a new class of drugs (MTP inhibitors), and this is the first time that Aegerion is seeking approval of a drug at the FDA.

Per the FDA guidance documents for oncology drugs in 2007, the FDA notes that single-arm studies may be allowed where there are no available therapies and as evidence for accelerated approval (Subpart H, accelerated approval, which may require post-marketing studies). In the past 12 months, there have been ~13 drugs approved for orphan indications. Of these 13 drugs, five were for cancer and the remaining eight were for various indications ranging in severity from ocular inflammation to infantile spasms. Exhibit 2 illustrates selected drugs for orphan indications granted FDA approval on the basis of a single, small clinical trial. These drugs include Shire's (SHPGY, $93.61, Hold) Elaprase and Biomarin's (BMRN, $33.34, Buy) Naglazyme, which were granted full approval for their respective indications of Hunter syndrome and Mucopolysaccharidosis type VI (MPS-VI) on the basis of a single, small, randomized study. Recently, Seattle Genetics' (SGEN, $21.52, Buy) Adcetris was granted accelerated approval in relapsed or refractory Hodgkin's lymphoma and systemic ALCL on the basis of non-randomized, single-arm, open-label trials.

Safety concerns over liver toxicity. Our consultant noted that the Advisory Committee will likely scrutinize the liver toxicity profile of lomitapide (and mipomersen), especially in the HoFH patient population, who already have a higher risk of

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cardiovascular disease. Panel discussions are likely to focus around the mechanism of liver toxicity and how toxicity can be managed or prevented. Aegerion mentioned on its June 16 conference call that it has proactively submitted a Risk Evaluation and Mitigation Strategy (REMS) plan to the FDA, but no additional details were disclosed around what the REMS program would entail and if/how such a program would limit the patient population for lomitapide.

Exhibit 2: Selected FDA Orphan Drug Applications Submitted on a Single Pivotal Trial[1]

Drug	Company	FDA Response	Indication	Pivotal Trial Design
Adcetris (brentuximab vedotin)	Seattle Genetics	Accelerated approval: 8/19/11	Relapsed or refractory Hodgkin's Lymphoma	-non-randomized, single-arm, open-label trial (n=102) -1.8 mg/kg IV Adcetris/3 weeks, up to 16 cycles
			Relapsed or refractory systemic ALCL	-non-randomized, single-arm, open-label trial (n=58) -1.8 mg/kg IV Adcetris/3 weeks, up to 16 cycles
Folotyn (pralatrexate)	Allos	Accelerated approval: 9/24/2009	Relapsed or refractory PTCL	-non-randomized, single-arm, open-label trial (n=109) -30 mg/m2 Folotyn for 6/7 weeks
Uplyso (taliglucerase alpha)	Protalix	PDUFA date: 2/1/12; CRL: 2/25/11; requested additional data from switchover trial (n=29; Cerezyme to Uplyso), and long-term 24 month extension of original Ph3 study; CMC information also requested	Gaucher's disease	-randomized, double-blind, parallel group, dose-ranging trial (n=32) -30 U/kg vs. 60 U/kg Uplyso
Carbaglu (carglumic acid)	Orphan Europe	Approved: 3/18/2010	NAGS deficiency	-non-randomized, single-arm, open-label trial (n=7) ongoing -100 mg/kg/day Carbaglu -additional retrospective data review (n=23) submitted
Elaprase (idursulfase)	Shire	Approved: 7/24/2006	Hunter syndrome (Mucopolysaccharidosis type II, MPS-II)	-randomized, double-blind, placebo-controlled trial (n=96) -0.5 mg/week vs. 0.5mg/2 weeks Elaprase vs. placebo
Aldurazyme (laronidase)	BioMarin	Approved: 4/30/2003	Mucopolysaccharidosis type I (MPS-I)	-randomized, double-blind, placebo-controlled trial (n=45) -0.58 mg/kg/week Aldurazyme vs. placebo
Fabrazyme (agalsidase beta)	Genzyme	Accelerated approval: 4/24/2003	Fabry's Disease	-randomized, double-blind, placebo-controlled trial (n=58) -1.0 mg/kg/2 weeks Fabrazyme vs. placebo -also submitted data from extension study
Cerezyme (imiglucerase)	Genzyme	Approved: 5/23/1994	Gaucher's disease	-randomized, double-blind, parallel group trial (n=30) -60 U/kg/2 weeks Cerezyme vs. Ceredase
Naglazyme (galsulfase)	BioMarin	Approved: 5/31/2005	Mucopolysaccharidosis type VI (MPS-VI)	-randomized, double-blind, placebo-controlled trial (n=38) -1 mg/kg/week Naglazyme vs. placebo
Soliris (eculizumab)	Alexion	Approved: 3/16/2007	Paroxysmal Noctural Hemoglobinuria (PNH)	2 pivotal trials: -1 randomized, double-blind, placebo-controlled trial (n=87) -1 non-randomized, single-arm, open-label trial (n=97) -both studies: 600 mg/week Soliris titrated up to 900 mg

(1) With the exception of Soliris in PNH, these drugs were FDA approved in the listed indications based on a single trial
CMC: chemistry, manufacturing, and controls
CRL: complete response letter
PTCL: peripheral T-cell lymphoma
ALCL: anaplastic large cell lymphoma
NAGS: N -acetylglutamate synthetase (essential enzyme in the urea cycle)

Source: Company reports, FDA.gov, and Jefferies & Company, Inc.

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FDA's Endocrinologic and Metabolic Committee. While we cannot predict the panel composition for lomitapide (and mipomersen), as a baseline view into the Committee's opinions, we looked at previous committee decisions in the past three years, as shown in Exhibit 3. It is evident that the focus of the committee's discussions has been safety issues. Based on lomitapide profiles, we expect the Endocrinologic and Metabolic Committee discussion on the drug to be focused on safety (potential liver toxicity) and risk-benefit. Our regulatory consultant reiterates that safety is a key focus at the FDA, particularly for a new class of drugs such as lomitapide.

Exhibit 3: Outcomes of Endocrinologic and Metabolic Advisory Committee Meetings

Drug(s)	Company	Date	Decision	Panel vote/ FDA approval	Indication	Issues
dapagliflozin	Bristol-Myers Squibb	Jul-11	9-6 against dapagliflozin approval	No/PDUFA date: 10-28-11	Type 2 diabetes	- Safety a key concern - Hepatic safety (5 patients with Hy's law or > 2x ULN bilirubin and >3x ULN ALT or AST) levels - Bladder and breast cancers observed
Trilipix (fenofibrate)	Abbott Labs	May-11	Majority (10/13) in favor of revising Trilipix label or withdrawing from market	-	Hypertriglyceridemia, hyperlipidemia	-Concerns on efficacy as add-on therapy to statins -Concerns over subgroup findings -Concerned over use of surrogate endpoints
Contrave (naltrexone/ bupropion)	Orexigen	Dec-10	13-7 against Contrave approval	No/No	Obesity	-Efficacy did not outweight risks (CV disease)
Meridia (sibutramine)	Abbott Labs	Sep-10	Majority (14/16) in favor of revising Meridia's label or withdrawing from market	-	Obesity	-Concerns over higher risk of CV events
Lorgress (lorcaserin)	Arena	Sep-10	9-5 against Lorgress approval	No/No	Obesity	-Benefits (small magnitude of weight loss) did not outweigh risks (cancer and diabetes)
Avandia (rosiglitazone)	GlaxoSmithKline	Jul-10	Majority (22/32) voted to revise Avandia's label or withdraw drug from the market	-	Type 2 diabetes	-CV safety of Avandia
Egrifta (tesamorelin)	Theratechnologies	May-10	16-0 in favor of Egrifta approval	Yes/Yes	Lipodystrophy in HIV patients	-Concerns over potential CV outcomes: postmarketing study recommended
Zavesca (miglustat)	Actelion	Jan-10	10-3 in favor of Zavesca approval	Yes/No	Niemann-Pick Disease	-Concerns that the study was not well-controlled, and did not show substantial evidence
Carbaglu (carglumic acid)	Orphan Europe	Jan-10	12-0 in favor of Carbaglu approval	Yes/Yes	NAGS deficiency	-Concerns over off-label use of Carbaglu
Crestor (rosuvastatin)	AstraZeneca	Dec-09	12-4 in favor of Crestor approval	Yes/Yes	Primary prevention of CV disease	-Caution around defining population for extended use
Onglyza (saxagliptin)	Bristol-Myers Squibb	Apr-09	10-2 in favor of Onglyza approval	Yes/Yes	Type 2 diabetes	-Concerns over safety: postmarketing study recommended
Victoza (liraglutide)	Novo Nordisk	Apr-09	12-0 in favor of Victoza approval	Yes/Yes	Type 2 diabetes	-Concerns over risk of thyroid cancer
Myozyme 2000L (alglucosidase alpha)	Genzyme	Oct-08	14-3 in favor of accelerated approval of Myozyme 2000L (vs. full approval)	Yes/No	Pompe Disease	-Concerns over similarity of 2000L product to 160L product; recommendation for head-to-head trial

MACE: major adverse cardiac event
NAGS: N -acetylglutamate synthetase

Source: Company reports and Jefferies & Company, Inc.

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Clinical Data for Lomitapide

Aegerion conducted a single-arm Phase 3 study in HoFH, and the University of Pennsylvania sponsored one Phase 2 study in patients with HoFH phenotypes (published in *NEJM 2007*), as shown in Exhibit 4. Aegerion also conducted four Phase 2 studies in the broader patient population of hypercholesterolemia. In the 1990s, BMY conducted Phase 1 and 2 studies of lomitapide at doses ranging 25-100 mg in a broader population (patients with high cholesterol at moderately high risk of a CV event or unable to tolerate statins) although development was halted due to the high discontinuation rate stemming from gastrointestinal adverse events as well as a high rate of hepatic steatosis (accumulation of fat in the liver).

Positive Phase 3 efficacy data at 26 weeks: In a single-arm, dose-titration Phase 3 trial of lomitapide in patients with HoFH, 29 patients were enrolled according to demonstrated functional homozygous FH by at least one of the following criteria: (1) untreated total cholesterol (TC) levels of greater than 500 mg/dL, triglyceride (TG) levels of less than 300 mg/dL, and parents with historical cholesterol levels greater than 250 mg/dL; (2) skin fibroblast LDL receptor activity <20% normal; or (3) documented functional mutation in both LDL receptor alleles or alleles known to affect LDL receptor function. While detailed patient stratification has not been disclosed, approximately one-half of the 23 patients were on background apheresis, and all patients were on a low-fat diet. Aegerion noted that patients with HoFH are typically maintained on a low-fat diet since diagnosis, and the low-fat diet had no contribution to further LDL-C reduction.

Patients had a 6-week run-in period on current lipid-lowering therapy to determine baseline measurements. The mean baseline LDL-C levels in the 29 patients were 336 mg/dL (vs. normal levels of LDL-C for adults of 115 mg/dL). According to the American Heart Association, optimal fasting LDL-C levels that correspond to decreased risk of CV disease are <100 mg/dL (with recommendations of <70 mg/dL for patients with very high risk of heart disease). The trial was designed with a 26-week dose-titration phase to reach a maximum tolerated dose of lomitapide (not to exceed 60 mg/day), with an additional 52-week safety phase for a total of 78 weeks. Patients were permitted to continue on background lipid-lowering therapy that may include apheresis. Following 78 weeks, patients have the option to continue on lomitapide in an open-label extension trial for an additional 48 weeks, with continuation until regulatory approval is granted.

Interim Phase 3 data after six months was presented in a poster (#1077, Cuchel *et al.*) at the 2009 American Heart Association (AHA) meeting. The poster included results from 14 patients at 26 weeks and seven patients at 56 weeks. At 26 weeks, the median dose was 40 mg, and the mean reduction in LDL-C levels from baseline was 49% (from 351 mg/dL to 163 mg/dL, p<0.001). Among the subset of seven patients who received lomitapide for 56 weeks, the median dose was 40 mg and the mean reduction in LDL-C levels from baseline was 35% at 56 weeks (specific data and p-value not disclosed).

Subsequently, full 26-week results showed an average decrease of 40.1%/50.2% in LDL-C levels in the intent-to-treat (ITT) population (n=29)/completer population (n=23), respectively. This translates into mean LDL-C level reductions of 200 mg/dL/175 mg/dL for ITT population/completers. Safety data demonstrated that at 26 weeks, hepatic fat levels were elevated to 9.0% (n=22) from baseline levels of 1.0%.

Phase 3 efficacy maintained at 56 weeks: Aegerion reported top-line 56-week results in May 2011. At 56 weeks, the 23 completers experienced a mean reduction in LDL-C levels of 44%, from mean levels of 352 mg/dL to 197 mg/dL. Approximately 35% of patients (8/23) were able to achieve LDL-C levels of <100 mg/dL. At 56 weeks, mean

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hepatic fat levels were 7.3% (n=21) from baseline levels of 1.2%. Between weeks 26 and 56, 13 of the 23 patients (56.5%) were able to have their background lipid-lowering therapies reduced. Aegerion noted that "several" patients were able to come off apheresis between 26 and 56 weeks (no specific number provided). The most commonly reported adverse event was mild-to-moderate GI events (predominantly diarrhea of an undisclosed grade), and most of these events occurred upon titrating up to a higher dose (maximum dose achieved of 60 mg/day; mean dose of ~40 mg/day). Aegerion noted that three patients (3/29, 10%) dropped out due to GI adverse events, and an additional three patients (3/29, 10%) withdrew consent due to undisclosed reasons.

Aegerion noted that no patients dropped out due to liver enzyme elevations. Four patients (4/23, 17%) experienced consecutive alanine transaminase (ALT) or aspartate transaminase (AST) elevations between 5x and 11x the upper normal limit (normal ALT of 7-35 U/L for women and 10-40 U/L for men; normal AST of 13-35 U/L for women and 15-40 U/L for men). We note that while ISIS's Phase 3 HoFH trial for mipomersen included a stopping limit of >5x ULN ALT or AST elevation, Aegerion's Phase 3 protocol mandated that patients with elevated aminotransferase levels have their doses back-titrated; thus, no patients were taken off the drug. Three out of the four patients were later titrated back to the levels above the dose at which they experienced elevated aminotransferase levels.

Phase 3 data at 78 weeks in late-2011: The 78-week data could be presented at a medical conference in late-2011. We note that per Aegerion management, the FDA is not requiring the 78-week data to be included in the NDA filing.

Phase 2 dose-escalation study published in NEJM (Cuchel et al, 2007; 356:148-56). This was a small open-label Phase 2 study of six patients with genetically confirmed HoFH (mutations in the LDL receptor). Lomitapide was administered at four escalating doses – 0.03, 0.1, 0.3, and 1.0 mg/kg for four weeks each (a total of 16 weeks), translating into mean doses of 2.0, 6.7, 20.1, and 67 mg/day. Four weeks prior to the baseline measurement and during the study, lipid-lowering treatments including apheresis were suspended, and patients were advised to maintain a low-fat diet.

At baseline, the mean LDL-C level was 614 mg/dL. Following the treatment, the mean LDL-C levels in the two highest dose groups were as follows: 465 mg/dL on the 0.3 mg/kg dose (at 12 weeks, $p<0.001$) and 303 mg/dL on the 1.0 mg/kg dose (at 16 weeks, $p<0.001$). A significant reduction from baseline in triglycerides and apolipoprotein B levels were also seen with the 1.0 mg/kg dose of lomitapide at 16 weeks ($p<0.001$; mean TGs reduced to 88 mg/dL from 283 mg/dL; mean apoB reduced to 136 mg/dL from 310 mg/dL).

In this small study, while there were no changes in total bilirubin or alkaline phosphatase in any of the subjects, there was wide variation in liver transaminase enzyme levels, including increases in ALT and AST. Two of the six subjects had normal transaminase levels throughout the entire study. However, patient #3 and #6 experienced an increase in liver enzymes one week after titrating up to the 0.3 mg/kg dose. At the 9th visit, at which patients had been on the 0.3 mg/kg dose for seven days, patient #3 had ALT levels increase to 710 from 38 U/L; AST levels to 415 from 41 U/L. Patient #6 had ALT levels increase to 144 from 14 U/L; AST levels to 126 from 23 U/L. At the 1.0 mg/kg dose, four subjects had increased ALT levels to 107–488 U/L from baseline of 14-38 U/L, as measured after 14 days of treatment (visit 13).

Hepatic fat increased substantially from baseline in four of the six patients treated with lomitapide in this study (baseline numbers not given). At the maximal 1.0 mg/kg dose,

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two patients had increases in hepatic fat to 18-24% and two had increases to more than 30%, while two had increases to less than 10% throughout the study.

Exhibit 4: Lomitapide (AEGR-733) Clinical Studies in HoFH

Study	Indication	Starting Date	Completion Date	# Patients	Study Design/ Efficacy Data
Phase 3 "733-005" (pivotal, single-arm, open-label)	HoFH (phenotypic)	Dec-07	26- and 56-week top-line data reported; 78-week data in late-2011; potential NDA/MAA filings in 1Q12	23 (29 enrolled)	**To evaluate AEGR-733 as add-on therapy to concurrent lipid lowering medications/apheresis:** - AEGR-733 titrated up to 60 mg/day MTD - Primary endpoint of LDL-C at 26 wks - Hepatic fat and lipid levels measured up to 1.5 years - 40%/50% reduction in LDL-C levels to 200 mg/dL in ITT (n=29)/completers (n=23) from 336 mg/dL/352 mg/dL, respectively
					- Primary endpoint of LDL-C at 56 wks - 44% reduction in LDL-C levels to 197 mg/dL from 352 mg/dL (n=23)
					- Follow-on study: patients eligible to continue on previous MTD - Long-term safety data collected at 78 wks
Phase 2 (single-arm, open-label)	HoFH (genotypic)	NA	NEJM, Cuchel et al, 2007;356:148-56	6	**To evaluate safety and efficacy of AEGR-733 (dose escalation):** - AEGR-733 (0.03, 0.1, 0.3, 1.0 mg/kg) daily for 4 wks at each dose - 25% reduction in LDL-C levels to 465 mg/dL from 614 mg/dL ($p<0.001$) with 0.3 mg/kg dose - 51% reduction in LDL-C levels to 303 mg/dL ($p<0.001$) with 1.0 mg/kg dose
Phase 2 (randomized, double-blind)	Healthy subjects: hepatic fat and GI adverse event study	Oct-07	Nov-08	~260	**To evaluate hepatic fat accumulation (by MRI) with AEGR-733:** - AEGR-733 (dose-ranging 2.5 mg to 10 mg) vs. placebo and in combination with atorvastatin, ezetimibe, and fenofibrate at 12 wks
Phase 2 (open-label)	Healthy subjects: PK study	Mar-06	Nov-07	125	**To evaluate PK profile of AEGR-733 in combination with lipid-lowering agents:** - AEGR-733 (10 mg) in combination with atorvastin (20 mg), ezetimibe (10 mg), simvastatin (20 mg), rosuvastatin (20 mg), or micronized fenofibrate (145 mg); AEGR-733 (60 mg) with dextromethorphan (30 mg) or rosuvastatin (20 mg)

HoFH: Homozygous familial hypercholesterolemia
NEJM: New England Journal of Medicine

Source: Company reports, clinicaltrials.gov, and Jefferies & Company, Inc.

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Exhibit 5: 26-Week Phase 3 Data for Lomitapide and Mipomersen in HoFH

Study	Lomitapide HoFH (phenotypic)	Mipomersen HoFH (genotypic)
Number of patients	29 patients (single-arm, open-label)	51 patients (randomized 2:1, double-blind, placebo-controlled)
Design	Lomitapide dose-titrated to MTD (<60 mg/dL) -1x daily -oral	Mipomersen 200 mg vs. placebo -1x weekly -subcutaneous injection
Baseline LDL (average)	336 mg/dL (352 mg/dL for completers); all were on lipid lowering therapy & ~12 patients on apheresis (~50% of 23 patients)	439mg/dL on mipomersen and 400mg/dL on placebo (a minimum of >400 mg/dL; 50/51 on lipid lowering therapy, 12 statin alone, 38 statin + another lipid lowering agent, 37 statin + ezetimibe)
Efficacy at 26 weeks	-40% LDL-C reduction on lomitapide -35% achieved levels <100mg/dL (p value not disclosed)	-25% LDL-C reduction on mipomersen vs. 3% on placebo (p=0.0003)
Total Drop-out Rate Due to AEs	~10% (n=3/29) lomitapide (GI adverse events)	~12% (n=4/34) mipomersen (2 injection site rxn, 1 elevated ALT, 1 rash) vs. 0% placebo
Injection Site Reactions	N/A	76% (n=26/34) mipomersen vs. 24% (n=4/17) placebo
Hepatic Fat Content (MRI)	9.0% (n=22) mean hepatic fat levels compared to 1.0% baseline	NA; measured only in patients with ALT >3x ULN; 1 patient had an increase to 24.8% from 9.6% baseline
Elevated Liver Enzymes	14% (n=4/29) had consecutive AST or ALT elevation >5x-11x ULN; per protocol, patients with elevated levels were back-titrated instead of taken off drug	12% on mipomersen (n=4/34) had ALT >3x ULN, of which 2 patients had one >3x ULN (6%); 1 had persistent ALT >3x ULN (~3%); 1 had ALT >5x ULN at baseline and 17 wks (~3%)
Drop-out Due to Elevated Liver Enzymes	None	1 drop-out (stopping rule = 5x ULN)

Lomitapide (AEGR-733 oral): small molecule microsomal triglyceride transfer protein (MTP-1) inhibitor
Mipomersen (ISIS301012 subcutaneous): second-generation antisense inhibitor of Apolipoprotein B-100 mRNA
HoFH: Homozygous familial hypercholesterolemia

Source: Company reports and Jefferies & Company, Inc.

Competitive Landscape in FH

Mipomersen (Kynamro): EU filing on July 28, 2011; U.S. filing in 4Q11

ISIS has completed four Phase 3 trials for mipomersen (ISIS-301012), including one in HoFH (Exhibit 5), one in HeFH with coronary artery disease, one in severe HeFH/functional FH and one in hypercholesterolemia at high risk of developing coronary heart disease (CHD). As we expect ISIS/partner SNY to file for approval for HoFH in the U.S. in 4Q11, it is likely that both lomitapide and mipomersen will undergo regulatory reviews at the same time. In EU, SNY submitted an MAA for HoFH and severe HeFH on July 28, 2011. Mipomersen is an antisense drug that targets and binds to the apolipoprotein B-100 mRNA, predominantly expressed in the liver. As shown in Exhibit 5, we compare/contrast Phase 3 data in HoFH between lomitapide and mipomersen.

Anti-PCSK-9 product candidates in Phase 2

PCSK9 (proprotein convertase subtilisin/kexin type 9) is a serine protease expressed in the liver and intestine, which functions as an LDL receptor inhibitor. PCSK9 binds to the LDL

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receptor on the cell surface, causing LDL receptor to be internalized and targeted for lysosomal degradation. "Gain-of-function" mutations in PCSK9 have been correlated with FH, while "loss-of-function" mutations have been correlated with decreased LDL-C levels. It is thought that the dominant version of the PCSK9 protein causes down-regulation of the LDL receptor on the cell surface, therefore increasing plasma LDL-C levels.

As shown in Exhibit 6, Amgen (AMGN, $56.27, Buy) and Regeneron (REGN, $63.02, Hold) are both developing fully humanized antibodies targeting the PCSK9 protein. AMG-145 is currently enrolling a ~150 HeFH patient in a randomized, placebo-controlled Phase 2 trial. Regeneron's compound, REGN727, partnered with Sanofi-Aventis, is currently in Phase 2 trials for hypercholesterolemia. In addition, two antisense drug candidates targeting PCSK9 mRNA are currently in Phase 1, including Alnylam's (ALNY, $6.43, NC) ALN-PCS01 and ISIS' BMS-PCSK9Rx in partnership with Bristol-Myers Squibb.

SLx-4090: Intestine-specific MTP inhibitor in Phase 2

Kadmon Pharma holds the worldwide licensing rights to SLx-4090, an oral MTP inhibitor originally developed by Surface Logix (acquired by Nano Terra in April 2011). SLx-4090 was designed to target only enterocytes in the small intestine, in order to potentially avoid systemic toxicity. Originally developed for the treatment of dyslipidemia and FH, it has the potential to avoid the adverse events, including hepatic and GI toxicity, seen with systemic MTP inhibitors. In March 2009, Surface Logix announced the start of a U.S.-based randomized, double-blind, placebo-controlled Phase 2b trial in 133 patients with hyperlipidemia (>100 mg/dL LDL-C levels on concurrent statin therapy); however, to date, there has been no update on SLx-4090's clinical trials.

Exhibit 6: Drugs in Development for Familial Hypercholesterolemia (FH)

Drug	Delivery	Company	Phase	Type of Compound	Target
Lomitapide	Oral	Aegerion	Filing for US/EU approval in 1Q12	Small molecule	Microsomal triglyceride transfer protein (MTP) inhibitor
Mipomersen	SC	Isis	Filed in EU 7/28/11; US filing in 1Q12	Antisense (ssRNA)	Apolipoprotein B-100 mRNA inhibitor
Eprotirome	Oral	Karo Bio	Phase 3; Potential EU MAA filing in HeFH in 2014	Small molecule	Liver-specific thyroid hormone receptor agonist
SLx-4090	Oral	Kadmon/Nano Terra	Phase 2	Small molecule	Enterocyte-specific microsomal triglyceride transfer protein (MTP) inhibitor
REGN727 (SAR236553)	SC	Regeneron	Phase 2	Fully humanized mAb	Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor
AMG-145	SC	Amgen	Phase 1/2	Fully humanized mAb	Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor
ALN-PCS01	SC	Alnylam	Phase 1	Antisense (ssRNA)	Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor
BMS-PCSK9Rx	SC	Isis	Phase 1	Antisense (ssRNA)	Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor
TKM-ApoB (ApoB SNALP)	SC	Tekmira	Phase 1	siRNA (dsRNA)	Apolipoprotein B-100 mRNA inhibitor

SC: subcutaneous; mAb: monoclonal antibody; ssRNA: single-stranded RNA; dsRNA: double-stranded RNA
Source: Company reports, clinicaltrials.gov, and Jefferies & Company

Source: Company reports, clinicaltrials.gov, and Jefferies & Company, Inc.

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Market Opportunities for Lomitapide in HoFH

Aegerion to build ~35-person commercial team

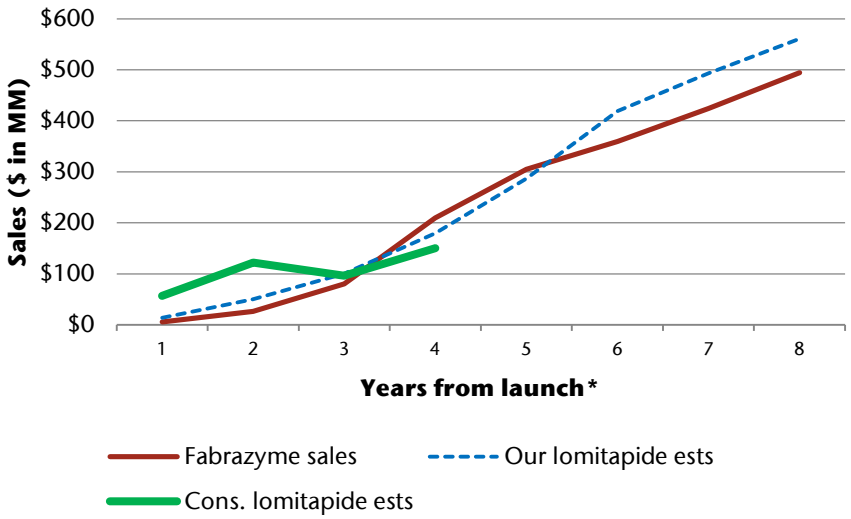
Upon approval/launch, Aegerion plans to target 43 and 75 LDL apheresis centers in the U.S. and in five EU countries, respectively; and 400 and 500 prescribing lipidologists in the U.S. and in five EU countries, respectively. Aegerion is building a focused, small sales/marketing group, including one global VP of sales, one head of U.S. sales, 15 specialized orphan drug clinical specialists in the U.S., and 18 clinical specialists in five EU countries. Aegerion plans to hire clinical specialists prior to lomitapide regulatory approval, based on the rationale that they will lose 6-9 months of sales revenue if they wait until approval to hire a sales force. Aegerion sees minimal risk of FDA approval; therefore, management views hiring of a sales force pre-approval as not risky.

At annual treatment cost of $150K-$200K, adult functional HoFH market size could be $900M-$1,200M in the U.S. and five EU countries combined

Aegerion estimates ~3,000 adult patients with functional HoFH in the U.S. and ~3,000 in five major EU countries based on one of the following patient profile definition: (1) maximally treated LDL-C levels >300 mg/dL; (2) a history of TC >500 mg/dL and parents with history of TC >250mg/dL; (3) <20% fibroblast LDL receptor function; or (4) genotypically characterized HoFH.

Based on our previous survey, we estimate a target population for lomitapide from apheresis-eligible FH patients. In the U.S., out of an estimated ~600,000 FH patients, we estimate approximately 3.7% of those are eligible for apheresis, translating into ~23K apheresis-eligible FH patients (~27K in EU). In 2020, we assume a penetration rate of ~13% in the U.S. and ~9% in the EU. However, we also assume a discontinuation rate of ~35% (potentially due to GI toxicity, abnormal liver function tests, non-compliance, competition, etc.). Thus, we forecast ~2,100 HoFH patients in the U.S. and 1,750 in the EU to be on lomitapide in 2020 (a total of 3,850). Assuming annual treatment cost of ~150K per patient, this translates into ~$315M in the U.S. and ~$245M in the EU, with total sales of ~$560M (see Exhibit 7 for our lomitapide sales estimates vs. consensus).

Exhibit 7: Sales Since Launch: Fabrazyme Historical vs. Lomitapide Estimates



*For lomitapide, we estimate launch in early-2013; consensus sales estimates starting in 2012.
Fabrazyme launched in August 2001 in EU and April 2003 in US

Source: Company reports and Jefferies & Company, Inc.

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Temporary Authorizations for Use (ATU) in France
The ATU procedure is a pre-marketing approval measure in France that allows drugs for a serious unmet need to be accessed by patients prior to regulatory approval. Available since 1994, this has previously allowed drugs for cancer and AIDS to be available to patients months in advance of approval. ATU pricing is key to how a drug will be priced in the EU once it is approved. Aegerion has submitted an application for ATU approval for lomitapide and expects approval before YE11. To date, ~75 eligible patients have been identified in France. The company estimates ATU pricing to be between $200K and $400K per patient per year, although price negotiations in France will still occur upon approval.

Management Team

Aegerion's board of directors is comprised of five outsiders and one insider, including David Scheer, President of Scheer & Company and Chairman of Aegerion Pharmaceuticals, Achillion, Tengion, Optherion, and Axerion Therapeutics; Marc Beer, CEO of Aegerion; Sol Barer, Ph.D., Chairman of Celgene; Paul Thomas, CEO of Roka Bioscience; Antonio Gotto, M.D., D.Phil, Dean of Weill Medical College of Cornell University and Provost for Medical Affairs; and Alison Kiley, director of Alta Partners.

Marc Beer – Chief Executive Officer, Board of Directors
Mr. Beer became the CEO of Aegerion Pharmaceuticals in August 2010. With more than 20 years of experience in the biotechnology industry, he was most recently the founding CEO of Viacell (2000–2007), which was acquired by PerkinElmer in 2007. Previously, Mr. Beer was VP of Global Marketing at Genzyme where he was responsible for the commercial launch of several global products for orphan diseases (1996–2000). Mr. Beer has also held senior management positions at Biostar, Inc. and Abbott Laboratories. He is Chairman of the board of Good Start Genetics, and serves on the boards of the Biotechnology Industry Organization (BIO) Emerging Companies Section Governing Board, Seaside Therapeutics, and ERYtech Pharma.

Mark Fitzpatrick – Chief Financial Officer
Mr. Fitzpatrick has served as Aegerion's CFO since April 2011. Mr. Fitzpatrick has more than 17 years of biotechnology industry experience, and he was most recently CFO at Proteon Therapeutics (2007–2011) and CFO at RenaMed Biologics (2005–2007). He has also served as CFO of Dynogen Pharmaceuticals, WorldStreet Corporation, and Diacrin.

Paul Merrigan – Vice President of Global Marketing
Joining Aegerion in July 2011, Mr. Merrigan was most recently VP and general manager of Neuromuscular Diseases at Genzyme Corporation, where he was responsible for the global launch and commercialization of Myozyme/Lumizyme and the commercialization in products in development for neuromuscular diseases. During his tenure at Genzyme (1995–2011), Mr. Merrigan held several global marketing positions for rare diseases. Prior to Genzyme, Mr. Merrigan held positions at Genentech in various capacities within sales, marketing, and health outcomes in the cardiovascular and cystic fibrosis disease areas.

Martha Carter – Chief Regulatory Officer
Martha Carter joined Aegerion in February 2011, bringing more than 30 years of regulatory affairs experience in the life sciences industry. Before joining Aegerion, she was SVP and Chief Regulatory Officer at Proteon Therapeutics (2006–2011). Previously, she was SVP of Regulatory Affairs at Trine Pharmaceuticals and VP of Regulatory Affairs at

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GelTex Pharmaceuticals, where she led the regulatory functions for commercial and investigational drug products.

Diane Tribble, Ph.D. – Chief Scientific Officer
Joining Aegerion in February 2011, she was most recently VP of clinical development at Isis Pharmaceuticals, where she led the development of the cholesterol-reducing agent mipomersen. Prior to ISIS, she was Director of Clinical Research at Merck, where she worked on the development of several cholesterol-reducing drug candidates, including the CETP inhibitor anacetrapib and Vytorin. She was also a member of the Cholesterol Task Force of the Merck/Schering-Pough joint venture.

Mark Sumeray, MD – Chief Medical Officer
Mark Sumeray joined Aegerion in July 2011 from Bristol-Myers Squibb, where he was most recently VP of Cardiovascular/Metabolics. At Bristol-Myers Squibb, he was responsible for U.S. medical strategy for the CV and metabolics teams (2009–2011). Previously, he was VP of medical business development at The Medicines Company (2004–2009) and VP of clinical development at Johnson & Johnson (2000–2004).

Christine Pellizzari – Executive VP, General Counsel, and Secretary
Christine Pellizzari joined Aegerion in August 2007. Most recently, Ms. Pellizzari was SVP and General Counsel of Dendrite International (1998–2007), a provider of promotional and compliance solutions until the sale of the company to Cegedim, and she specialized in technology and healthcare transactions at the law firm Wilentz, Goldman & Spitzer (1995–1998).

Financial Projections and Analysis

As of June 30, 2011, Aegerion had approximately ~$87M in cash, including proceeds from the recent $50M secondary offering on 6/23/11. We estimate Aegerion's cash to be sufficient to fund operations through 2012, and we currently assume an equity raise in 2H12 following our assumption of lomitapide approval in HoFH in late-2012/early-2013.

Revenues

In 2010, Aegerion did not generate any meaningful revenue, and we do not forecast any revenue until a commercial launch of lomitapide. We assume a U.S. launch by early-2013 and an EU launch in ~2H13. For lomitapide, we project total sales of ~$14M, $51M, $101M, and $180M for 2013-2016, reaching ~$500M in 2019 on ~1,930 and ~1,450 patients on lomitapide in the U.S. and Europe, respectively. We expect Aegerion to reach profitability in ~2015.

Expenses

With anticipated increases in R&D activities from initiation of two Phase 3 clinical trials for lomitapide in pediatric HoFH and FC in 1Q12, and regulatory expenses, we estimate R&D spending to be ~$19M in 2011 (+155% y/y), ~$29M (+47% y/y) in 2012, and ~$34M in 2013 (+20% y/y). In preparation of commercial launches of lomitapide in the U.S. and the EU, we expect SG&A spending to grow substantially from our estimated ~$15M in 2011 to $25M in 2012 (+67% y/y), and $35M in 2013 (+40% y/y). We estimate gross margin for lomitapide to be ~92%.

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Valuation

Our $20 price target per share is based on a sum-of-parts NPV analysis of Aegerion. Our analysis assigns a value of ~$11/share for lomitapide U.S. sales and ~$9/share for lomitapide EU sales, discounting at an annual rate of 11%. We assume a 75% probability on U.S./EU sales of ~$315M and ~$250M, respectively, in 2020.

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Exhibit 8: Sales Projections for Lomitapide

	2010	2011E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
In the U.S.											
U.S. Population ('000)	308,000	310,772	313,569	316,391	319,239	322,112	325,011	327,936	330,887	333,865	336,870
Prevalence of FH patients (1/500)	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*
Apheresis-eligible FH patients from our doc survey	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*
FH Apheresis-eligible Patients											
# of FH patients	616,000	621,544	627,138	632,782	638,477	644,223	650,021	655,872	661,775	667,730	673,740
# of apheresis-eligible FH patients	22,792	22,997	23,204	23,413	23,624	23,836	24,051	24,267	24,486	24,706	24,928
Penetration into apheresis-eligible FH patients in U.S.			0.0%	0.4%	1.4%	2.8%	4.8%	7.5%	11.0%	12.0%	13.0%
# of apheresis-eligible FH patients on lomitapide (add-on/switching)			-	94	331	655	1,142	1,820	2,693	2,965	3,241
Discontinuation rate			20%	25%	30%	35%	35%	35%	35%	35%	35%
Net FH apheresis-eligible patients on lomitapide			-	70	232	426	743	1,183	1,751	1,927	2,106
Annual Tx cost per patient			150,000	150,000	150,000	150,000	150,000	150,000	150,000	150,000	150,000
U.S. Sales for lomitapide in apheresis-eligible FH patients ($ in thousands)			**-**	**10,536**	**34,727**	**63,911**	**111,385**	**177,454**	**262,609**	**289,061**	**315,967**
y/y growth					*229.6%*	*84.0%*	*74.3%*	*59.3%*	*48.0%*	*10.1%*	*9.3%*

In EU	2010	2011E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
E.U. Population ('000)	369,600	372,926	376,283	379,669	383,086	386,534	390,013	393,523	397,065	400,638	404,244
Prevalence of FH patients (1/500)	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*	*0.2%*
Apheresis-eligible FH patients from our doc survey	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*	*3.7%*
FH Apheresis-eligible Patients											
# of FH patients	739,200	745,853	752,565	759,339	766,173	773,068	780,026	787,046	794,129	801,277	808,488
# of apheresis-eligible FH patients	27,350	27,597	27,845	28,096	28,348	28,604	28,861	29,121	29,383	29,647	29,914
Penetration into apheresis-eligible FH patients in EU			0.0%	0.1%	0.5%	1.3%	2.5%	4.0%	5.8%	7.5%	9.0%
# of FH apheresis-eligible patients on lomitapide (add-on/switching)			-	28	142	358	722	1,165	1,690	2,224	2,692
Discontinuation rate				20%	25%	30%	35%	35%	35%	35%	35%
Net FH apheresis-eligible patients on lomitapide			-	22	106	250	469	757	1,098	1,445	1,750
Annual Tx cost per patient			150,000	150,000	148,500	147,015	145,545	144,089	142,649	141,222	139,810
EU Sales for lomitapide in apheresis-eligible FH patients ($ in thousands)			**-**	**3,371**	**15,787**	**36,795**	**68,259**	**109,096**	**156,654**	**204,109**	**244,663**
y/y growth					*368.2%*	*133.1%*	*85.5%*	*59.8%*	*43.6%*	*30.3%*	*19.9%*
Total # of patients treated			-	93	338	676	1,212	1,940	2,849	3,372	3,856
Total lomitapide US/EU sales			**-**	***13,907***	***50,513***	***100,706***	***179,644***	***286,550***	***419,263***	***493,169***	***560,631***
y/y growth					*263.2%*	*99.4%*	*78.4%*	*59.5%*	*46.3%*	*17.6%*	*13.7%*

Source: Company reports and Jefferies & Company, Inc.

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Exhibit 9: Aegerion's Income Statement

($ in thousands except per share)

	2010	1Q11	2Q11	3Q11E	4Q11E	2011E	1Q12E	2Q12E	3Q12E	4Q12E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Revenues																			
Lomitapide total sales											-	*13,907*	*50,513*	*100,706*	*179,644*	*286,550*	*419,263*	*493,169*	*560,631*
Growth y/y													*263%*	*99%*	*78%*	*60%*	*46%*	*18%*	*14%*
U.S. Sales											-	10,536	34,727	63,911	111,385	177,454	262,609	289,061	315,967
Ex-U.S. Sales											-	3,371	15,787	36,795	68,259	109,096	156,654	204,109	244,663
Research Contract Revenues																			
Total Revenues	-	-	-	-	-	-	-	-	-	-	-	**13,907**	**50,513**	**100,706**	**179,644**	**286,550**	**419,263**	**493,169**	**560,631**
% growth y/y													*263.2%*	*99.4%*	*78.4%*	*59.5%*	*46.3%*	*17.6%*	*13.7%*
Expenses																			
Cost of Goods Sold	-	-	-	-	-	-	-	-	-	-	-	2,107	3,473	5,752	8,911	14,196	21,009	23,125	25,277
% gross margin												*80.0%*	*90.0%*	*91.0%*	*92.0%*	*92.0%*	*92.0%*	*92.0%*	*92.0%*
R&D	7,629	3,297	5,138	5,475	5,544	19,454	6,500	7,000	7,410	7,687	28,597	34,317	25,738	20,590	20,590	22,237	24,016	25,938	28,013
% growth y/y	*8.4%*					*155.0%*					*47.0%*	*20.0%*	*-25.0%*	*-20.0%*	*0.0%*	*8.0%*	*8.0%*	*8.0%*	*8.0%*
% of total revenues													*51.0%*	*20.4%*	*11.5%*	*7.8%*	*5.7%*	*5.3%*	*5.0%*
SG&A	5,922	3,491	3,205	3,900	4,209	14,805	4,800	5,750	6,600	7,574	24,724	34,614	41,191	46,546	51,200	56,320	61,952	68,147	74,962
% growth y/y	*92.6%*					*150.0%*					*67.0%*	*40.0%*	*19.0%*	*13.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*	*10.0%*
% of total revenues													*81.5%*	*46.2%*	*28.5%*	*19.7%*	*14.8%*	*13.8%*	*13.4%*
Total Expenses	13,551	6,787	8,343	9,375	9,753	34,259	11,300	12,750	14,010	15,262	53,322	71,038	70,401	72,888	80,701	92,754	106,977	117,210	128,252
Income (loss) from Operations (EBIT)	**(13,551)**	**(6,787)**	**(8,343)**	**(9,375)**	**(9,753)**	**(34,259)**	**(11,300)**	**(12,750)**	**(14,010)**	**(15,262)**	**(53,322)**	**(57,131)**	**(19,888)**	**27,818**	**98,943**	**193,796**	**312,286**	**375,959**	**432,379**
% growth y/y															*255.7%*	*95.9%*	*61.1%*	*20.4%*	*15.0%*
Operating margin														*27.6%*	*55.1%*	*67.6%*	*74.5%*	*76.2%*	*77.1%*
Other Income, Net (Int. Income/Expense)	(11,247)	(45)	(262)	(150)	(143)	(600)	(125)	(125)	(125)	(125)	(500)	(500)	(500)	(500)	500	1,000	2,000	3,000	4,000
Earnings (Loss) Before Taxes	(24,798)	(6,832)	(8,605)	(9,525)	(9,897)	(34,859)	(11,425)	(12,875)	(14,135)	(15,387)	(53,822)	(57,631)	(20,388)	27,318	99,443	194,796	314,286	378,959	436,379
Provision for Taxes	1,793													1,366	9,944	29,219	62,857	94,740	130,914
Tax Rate														*5.0%*	*10.0%*	*15.0%*	*20.0%*	*25.0%*	*30.0%*
Net Income (Loss) - GAAP	**(23,005)**	**(6,832)**	**(8,605)**	**(9,525)**	**(9,897)**	**(34,859)**	**(11,425)**	**(12,875)**	**(14,135)**	**(15,387)**	**(53,822)**	**(57,631)**	**(20,388)**	**25,952**	**89,499**	**165,577**	**251,428**	**284,220**	**305,465**
% growth y/y															*244.9%*	*85.0%*	*51.9%*	*13.0%*	*7.5%*
Profit margin														*25.8%*	*49.8%*	*57.8%*	*60.0%*	*57.6%*	*54.5%*
EPS (LPS) - Basic	(5.07)	**(0.39)**	**(0.49)**	**(0.45)**	**(0.47)**	**(1.79)**	**(0.53)**	**(0.59)**	**(0.53)**	**(0.57)**	**(2.21)**	**(2.10)**	**(0.74)**	0.93	3.16	5.80	8.72	9.75	10.38
EPS (LPS) - Diluted	**(5.07)**	(0.39)	(0.49)	(0.45)	(0.47)	(1.79)	(0.53)	(0.59)	(0.53)	(0.57)	(2.21)	(2.10)	(0.74)	**0.74**	**2.54**	**4.66**	**7.01**	**7.87**	**8.39**
% growth y/y (diluted)																			
Shares - Basic	4,537	17,642	17,729	21,157	21,262	19,447	21,475	21,690	26,907	27,176	24,312	27,447	27,722	27,999	28,279	28,562	28,847	29,136	29,427
Shares - Diluted	4,537	17,642	17,729	21,157	21,262	19,447	21,475	21,690	26,907	27,176	24,312	27,447	27,722	34,999	35,279	35,562	35,847	36,136	36,427
Cash, Cash Equivalents & Investments	44,101	46,288	87,540	78,015	68,119	68,119	56,694	43,819	123,684	108,297	108,297	50,666	30,278	56,231	145,730	311,307	562,735	846,955	1,152,420

Source: Company reports and Jefferies & Company, Inc.

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Exhibit 10: Aegerion Balance Sheet and Cash Flow Statement

Aegerion Pharmaceuticals (AEGR)

Balance Sheet

($ in thousands, except per share)

	2009	2010	2011E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Assets												
Cash & cash equivalents	1,429	44,101	54,983	45,547	28,345	18,432	44,911	84,993	71,216	73,361	58,373	114,714
Marketable securities		0	13,136	62,750	22,322	11,847	11,320	60,737	240,090	489,374	788,582	1,037,705
Prepaid expenses and other current assets	536	505	796	835	877	921	967	1,015	1,066	1,119	1,175	1,234
Total current assets	**1,965**	**44,606**	**68,914**	**109,132**	**51,543**	**31,199**	**57,198**	**146,745**	**312,373**	**563,855**	**848,130**	**1,153,654**
Property and equipment, net	15	16	88	93	98	102	108	113	119	124	131	137
Other non-current assets	670	1,125	1,238	1,361	1,497	1,647	1,812	1,993	2,192	2,412	2,653	2,918
Total assets	**2,650**	**45,747**	**70,240**	**110,586**	**53,138**	**32,949**	**59,117**	**148,851**	**314,684**	**566,391**	**850,913**	**1,156,709**
Liabilities and Stockholders' Equity (Deficit)												
Accounts payable & accrued expenses	747	3,569	2,869	3,156	3,471	3,818	4,200	4,620	5,082	5,591	6,150	6,765
Other	22,089	43	979	989	10,998	1,108	1,119	1,131	1,142	1,153	1,165	1,177
Total current liabilities	**22,836**	**3,612**	**3,848**	**4,144**	**14,470**	**4,927**	**5,320**	**5,751**	**6,224**	**6,744**	**7,315**	**7,941**
Deferred revenue/rent	0	44	-	-	-	-	-	-	-	-	-	-
Long term debt			10,000	10,000	-	-	-	-	-	-	-	-
Other/convertible/preferred stock	49,940	27,311	733	733	733	733	733	733	733	733	733	733
Total liabilities	**72,776**	**30,967**	**14,581**	**14,877**	**15,203**	**5,660**	**6,053**	**6,484**	**6,957**	**7,477**	**8,048**	**8,674**
Stockholders' equity (deficit)	**(70,126)**	**14,780**	**55,659**	**95,709**	**37,935**	**27,289**	**53,064**	**142,367**	**307,726**	**558,913**	**842,866**	**1,148,035**
Total Liabilities & Stockholders' Equity	**2,650**	**45,747**	**70,240**	**110,586**	**53,138**	**32,949**	**59,117**	**148,851**	**314,684**	**566,391**	**850,913**	**1,156,709**
Cash/share	$0.86	$9.72	$3.50	$4.45	$1.85	$1.09	$1.61	$4.13	$8.75	$15.70	$23.44	$31.64
Book value/share	($42.33)	$3.26	$2.86	$3.94	$1.38	$0.98	$1.52	$4.04	$8.65	$15.59	$23.32	$31.52
Long-term debt-to-capitalization ratio	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA
Return on equity	NA	NA	NA	NA	NA	NA	64.6%	91.6%	73.6%	58.0%	40.6%	30.7%

Cash Flow Statement

($ in thousands except per share)

	2009	2010	2011E	2012E	2013E	2014E	2015E	2016E	2017E	2018E	2019E	2020E
Operating Activities												
Net income/(loss)	(12,196)	(14,254)	(34,859)	(53,822)	(57,631)	(20,388)	25,952	89,499	165,577	251,428	284,220	305,465
Depreciation & amortization	28	10	10	11	11	11	12	12	12	13	13	13
Change in working capital	(813)	1,541	(1,063)	243	269	298	331	366	406	449	497	550
Other	2,448	4,203	(1,107)	243	269	298	331	366	406	449	497	550
Net cash from operations	**(10,532)**	**(8,499)**	**(37,019)**	**(53,326)**	**(57,081)**	**(19,780)**	**26,625**	**90,244**	**166,400**	**252,339**	**285,226**	**306,578**
Investing Activities												
Capital expenditures	0	(11)	(100)	(110)	(121)	(133)	(146)	(161)	(177)	(195)	(214)	(236)
Securities transactions	-			(50,000)	40,000	10,000		(50,000)	(180,000)	(250,000)	(300,000)	(250,000)
Other												
Net cash from investments	**0**	**(11)**	**(100)**	**(50,110)**	**39,879**	**9,867**	**(146)**	**(50,161)**	**(180,177)**	**(250,195)**	**(300,214)**	**(250,236)**
Financing Activities												
Issuance of common/preferred stock	-	49,689	48,000	94,000								
Repayments/proceeds of debt, net	2,956	1,493										
Other	-	-										
Net cash from financing	**2,956**	**51,183**	**48,000**	**94,000**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**
Net Change in Cash and Cash Equivalents	**(7,576)**	**42,672**	**10,881**	**(9,436)**	**(17,202)**	**(9,913)**	**26,479**	**40,083**	**(13,777)**	**2,144**	**(14,988)**	**56,342**
Foreign exchange translations												
Cash and cash equivalents at beginning of period	9,005	1,429	44,101	54,983	45,547	28,345	18,432	44,911	84,993	71,216	73,361	58,373
Cash and cash equivalents at end of period	1,429	44,101	54,983	45,547	28,345	18,432	44,911	84,993	71,216	73,361	58,373	114,714

Source: Company reports and Jefferies & Company, Inc.

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Company Description

Aegerion Pharmaceuticals, Inc., a biopharmaceutical company, engages in the development and commercialization of life-altering therapeutics for rare and often fatal genetic lipid disorders. Its lead product in Phase III clinical development, lomitapide, is a small molecule, oral therapy to treat severe lipid disorders for an orphan patient population. Lomitapide has demonstrated the lowering of both bad cholesterol (LDL-C) and triglyceride (TG) levels in patients with a rare form of hypercholesterolemia. The company plans to initiate a clinical program to treat patients with a severe genetic form of hypertriglyceridemia called familial chylomicronemia (FC). Aegerion Pharmaceuticals, Inc. was founded in 2005 and is headquartered in Bridgewater, New Jersey.

Analyst Certification

I, Eun K. Yang, Ph.D., certify that all of the views expressed in this research report accurately reflect my personal views about the subject security(ies) and subject company(ies). I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or views expressed in this research report.

I, Eileen Flowers, Ph.D., certify that all of the views expressed in this research report accurately reflect my personal views about the subject security(ies) and subject company(ies). I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or views expressed in this research report.

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In June 2011 Jefferies acted as Joint Book-Running Manager in connection with an Equity offering for Aegerion Pharmaceuticals Inc.

James Dodwell owns shares of Bristol-Myers Squibb common stock.

In February 2011, Jefferies acted as a Bookrunner in a Follow-On Offering of equity for Seattle Genetics Inc.

For Important Disclosure information on companies recommended in this report, please visit our website at https://javatar.bluematrix.com/sellside/Disclosures.action or call 212.284.2300.

Meanings of Jefferies Ratings

Buy - Describes stocks that we expect to provide a total return (price appreciation plus yield) of 15% or more within a 12-month period.

Hold - Describes stocks that we expect to provide a total return (price appreciation plus yield) of plus 15% or minus 10% within a 12-month period.

Underperform - Describes stocks that we expect to provide a total negative return (price appreciation plus yield) of 10% or more within a 12-month period.

The expected total return (price appreciation plus yield) for Buy rated stocks with an average stock price consistently below $10 is 20% or more within a 12-month period as these companies are typically more volatile than the overall stock market. For Hold rated stocks with an average stock price consistently below $10, the expected total return (price appreciation plus yield) is plus or minus 20% within a 12-month period. For Underperform rated stocks with an average stock price consistently below $10, the expected total return (price appreciation plus yield) is minus 20% within a 12-month period.

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Valuation Methodology

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Risk which may impede the achievement of our Price Target

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Other Companies Mentioned in This Report

- Amgen, Inc. (AMGN: $56.27, BUY)
- BioMarin Pharmaceutical Inc. (BMRN: $33.34, BUY)
- Bristol-Myers Squibb (BMY: $32.38, BUY)
- ISIS Pharmaceuticals, Inc. (ISIS: $7.26, BUY)
- Regeneron Pharmaceuticals, Inc. (REGN: $63.02, HOLD)
- Sanofi (SAN FP: €49.15, HOLD)
- Seattle Genetics (SGEN: $21.52, BUY)
- Shire (SHPGY: $93.61, HOLD)

Distribution of Ratings

Rating	Count	Percent	IB Serv./Past 12 Mos. Count	IB Serv./Past 12 Mos. Percent
BUY	733	54.10%	35	4.77%
HOLD	550	40.60%	33	6.00%
UNDERPERFORM	73	5.40%	2	2.74%

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