# Aegerion Pharmaceuticals Collins Stewart

Independent thinking

US | Biotechnology | AEGR

# Lomitapide 4Q11 Filings On-Track

| Fiscal Year Ends: Dec   |     | 2010A  | 2011E   | 2012E  |  |
|-------------------------|-----|--------|---------|--------|--|
| Revenue (MM)            |     | \$0    | \$0     | \$8    |  |
| Previous Revenue (MM)   |     |        |         | \$6    |  |
| GAAP Earnings per Share | 0.4 |        | (0.00)  |        |  |
|                         | Q1  | -      | (0.39)A | -      |  |
|                         | Q2  | -      | (0.49)A | -      |  |
|                         | Q3  | (3.61) | (0.48)  | -      |  |
|                         | Q4  | (0.92) | (0.61)  | Ξ      |  |
| Year                    |     | (5.07) | (1.96)  | (1.90) |  |
| Previous GAAP EPS       |     |        | (1.69)  | (1.51) |  |
| P/E GAAP                |     | NM     | NM      | NM     |  |
| Diluted Shares (MM)     |     | 4.5    | 19.5    | 21.6   |  |

■ Investment Thesis - Increasing PT to \$27 on higher pricing:

This morning, AEGR reported 2Q11 EPS of \$(0.49), vs our estimate of \$(0.41) and Street consensus of \$(0.40), primarily due to higher-than-expected R&D expense associated with the lomitapide P3 study and regulatory preparation. AEGR remains on-track for U.S./E.U. lomitapide filings by YE11, following a pre-NDA meeting (June) with a pre-MAA meeting scheduled for September. In addition, we await 78-week data in 4Q11. We forecast U.S. and E.U. approvals in 4Q12 (on a potential 10-month review) and YE12, respectively, and estimate lomitapide WW sales of \$233M+ in 2018 (vs. \$164M prior), with higher HoFH prevalence and pricing, a 6-month review, approval in pediatric HoFH (75%-100% of adult population, according to AEGR management) and FC representing upside. We are upwardly revising our lomitapide pricing estimates (\$250K (vs. \$175K prior) in the U.S. and \$225K (vs. \$160K) in E.U) to be in-line with guidance of \$200K-\$400K. As a result, we are increasing our PT to \$27 from \$23.

#### ■ Update on FC and pediatric HoFH indications:

AEGR received KOL feedback and expects to initiate both trials in 4Q11/1Q12, which should be similar in size (N=29) to the adult HoFH P3 study. The EMEA has validated the PIP plan for pediatric HoFH.

#### ■ ATU-based access in France should provide insight into launch:

Upon receipt of an ATU by YE11, AEGR will be able to commercialize lomitapide in France. In preparation, it has identified 14 treating KOLs and 75 HoFH patients. AEGR expects pricing in France to be in-line with the most recent guidance (at least \$200K/year).

#### 2Q11 results and updated FY11 cash burn guidance:

AEGR reported 2Q11 GAAP R&D expense of \$5.1M, above our estimate of \$3.5M while GAAP SG&A expense was \$3.2M, below our estimate of \$3.6M. AEGR exited 2Q11 with \$87.5M in cash and raised its FY11 cash burn guidance to \$29.5M-\$32.5M (from \$22.5M-\$27.5M), in anticipation of its regulatory filings, salesforce build-out and ramp-up in infrastructure ahead of an ATU in France in FY11 and U.S/E.U. launch in FY12.

Disclaimers regarding the content of this report as well as full disclosure of Collins Stewart LLC's ratings and information on the firm's position(s) in securities mentioned herein appear on page 5 of this report.

July 27, 2011

| Recommendation | BUY     |
|----------------|---------|
| Price          | \$15.62 |
| Target         | \$27.00 |

#### **Key Data**

| 52-Week Range           | \$25.92-\$9.00 |
|-------------------------|----------------|
| Shares Outstanding (MM) | 17.7           |
| Market Cap (MM)         | \$276          |
| Avg. Daily Volume (000) | 113            |
| Float (MM)              | 10.6           |
| % Held Institutionally  | 70.3%          |
| Total Debt (MM)         | \$9.4          |
| Cash Per Share          | \$4.94         |
| Short Interest          | 2.70%          |
|                         |                |

#### **Stock Performance**



|             | 1m   | 3m     | 12m |
|-------------|------|--------|-----|
| Performance | 1.4% | -16.9% |     |

Source: Bloomberg

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 3 for price target valuation method and risks to achieving that target.

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Figure 1: 2Q11 Variance Table

(\$thousands, except per share data)

|   | Jun         |        | Jun     | Variance | Variance | Y/Y | Q/Q  |
|---|-------------|--------|---------|----------|----------|-----|------|
|   | 2Q11A       |        | 2Q11E   | A-E      | %        | %   | %    |
| Revenue                                   |             |        |         |          |          |     |      |
| Lomitapide Total Revenue                  | -           |        | -       | -        | n/a      | n/a | n/a  |
| Lomitapide HoFH - U.S.                    | -           | .      | -       | -        | n/a      | n/a | n/a  |
| Lomitapide HoFH - E.U.                    | -           |        | -       | -        | n/a      | n/a | n/a  |
| Milestone, Contract Revenues              | -           |        | -       | -        | n/a      | n/a | n/a  |
| Total Revenue                             | \$ -        |        | -       | -        | n/a      | n/a | n/a  |
| cogs                                      | -           |        | -       | -        | n/a      | n/a | n/a  |
| Gross profit                              | -           |        | -       | -        | n/a      | n/a | n/a  |
| Operating expense                         | -           |        |         |          |          |     |      |
| R&D                                       | 5,1         | 38     | 3,525   | 1,613    | -69%     | n/a | 56%  |
| SG&A                                      | 3,2         | 05     | 3,574   | (369)    | -112%    | n/a | -8%  |
| Total operating expense                   | 8,3         | 43     | 7,099   | 1,244    | -85%     | n/a | 23%  |
| Operating income (loss)                   | (8,3        | 43)    | (7,099) | (1,244)  | -85%     | n/a | 23%  |
| Interest Expense                          | (3          | 32)    | (260)   | (72)     | -78%     | n/a | 195% |
| Interest Income                           |             | 70     | 65      | 5        | -93%     | n/a | 4%   |
| Change in fair value of warrant liability | -           |        | -       | -        | n/a      | n/a | n/a  |
| Other income, net                         | -           |        | -       | -        | n/a      | n/a | n/a  |
| Total Other Income                        | (2          | 62)    | (195)   | (67)     | -75%     | n/a | 481% |
| Income Before Income Taxes                | (8,6        | 05)    | (7,294) | (1,311)  | -85%     | n/a | 26%  |
| Income Tax Provision                      | -           | .      | -       | -        | n/a      | n/a | n/a  |
| Accretion of preferred stock dividends    | _           |        | -       | -        | n/a      | n/a | n/a  |
| Net income (GAAP)                         | \$ (8,6     | 05) \$ | (7,294) | (1,311)  | -85%     | n/a | 26%  |
| CAAR EDG (dillected)                      | <b>^</b> (0 | 40) (  | (0.44)  | (0.07)   | 050/     | /-  | 050/ |
| GAAP EPS (diluted)                        | \$ (0.      | 49) \$ | (0.41)  | (0.07)   | -85%     | n/a | 25%  |
| Weighted shares outstanding               |             |        |         |          |          |     |      |
| diluted - GAAP                            | 17,7        | 29     | 17,730  | (1)      | -100%    | n/a | 0%   |

Source: Collins Stewart LLC Research and Company Reports

**Figure 2: AEGR Upcoming Milestones** 

| Product    | Indication     | Timing   | Milestone                        |
|------------|----------------|----------|----------------------------------|
|            | HoFH           | 4Q11     | Complete pre-clinical studies    |
|            | HoFH           | 4Q11     | Submit U.S. regulatory filing    |
|            | HoFH           | 4Q11     | Submit E.U. regulatory filing    |
|            | HoFH           | YE11     | Full Phase 3 78-week data        |
| Lomitapide | HoFH           | YE11     | Potential ATU approval in France |
|            | Pediatric HoFH | YE11     | Initiation of a pivotal study    |
|            | FC             | YE11     | Initiation of a pivotal study    |
|            | HoFH           | Mid-2012 | Potential FDA approval           |
|            | HoFH           | YE12     | Potential EMEA approval          |

Source: Collins Stewart LLC Research and Company Reports



Figure 3: Changes to Model

| (\$thousands, except per share data)      |             |             |             |             |             |             |           |           |            |           |            |       |
|---|-------------|-------------|-------------|-------------|-------------|-------------|-----------|-----------|------------|-----------|------------|-------|
|   | FY          | 11E         | FY          | 12E         | FY          | 13E         | FY        | 14E       | FY         | 15E       | FY1        | 6E    |
|   | New         | Prior       | New         | Prior       | New         | Prior       | New       | Prior     | New        | Prior     | New        | Prior |
| Revenue                                   |             |             |             |             |             |             |           |           |            |           |            |       |
| Lomitapide Total Revenue                  | -           | -           | \$ 8,236    | \$ 5,779    | \$ 40,074   | \$ 28,232   | \$ 76,740 | \$ 54,081 | \$ 122,860 | \$ 86,598 | \$ 166,075 |       |
| Lomitapide HoFH - U.S.                    | -           | -           | 6,940       | 4,858       | 23,882      | 16,717      | 44,087    | 30,861    | 69,247     | 48,473    | 91,817     |       |
| Lomitapide HoFH - E.U.                    | -           | -           | 1,296       | 921         | 16,192      | 11,514      | 32,653    | 23,220    | 53,613     | 38,125    | 74,258     |       |
| Milestone, Contract Revenues              | -           | -           | -           | -           | -           | -           | -         | -         | -          | -         | -          |       |
| Total Revenue                             | \$ -        | \$ -        | \$ 8,236    | \$ 5,779    | \$ 40,074   | \$ 28,232   | \$ 76,740 | \$ 54,081 | \$ 122,860 | \$ 86,598 | \$ 166,075 |       |
| cogs                                      | -           |             | 659         | 462         | 5,210       | 3,670       | 9,976     | 7,031     | 14,743     | 10,392    | 18,268     |       |
| Gross profit                              | -           | -           | 7,577       | 5,317       | 34,865      | 24,562      | 66,764    | 47,050    | 108,117    | 76,206    | 147,807    |       |
| Operating expense                         |             |             |             |             |             |             |           |           |            |           |            |       |
| R&D                                       | 21,988      | 14,586      | 25,523      | 13,001      | 27,002      | 13,003      | 28,412    | 14,523    | 30,211     | 16,491    | 31,783     |       |
| SG&A                                      | 15,846      | 14,865      | 20,321      | 17,226      | 25,433      | 20,047      | 28,001    | 23,547    | 30,523     | 27,046    | 32,683     |       |
| Total operating expense                   | 37,834      | 29,450      | 45,844      | 30,227      | 52,435      | 33,050      | 56,413    | 38,070    | 60,734     | 43,537    | 64,466     |       |
| Operating income (loss)                   | (37,834)    | (29,450)    | (38,267)    | (24,910)    | (17,570)    | (8,488)     | 10,351    | 8,980     | 47,383     | 32,669    | 83,341     |       |
| Interest Expense                          | (1,108)     | (893)       | (2,887)     | (2,453)     | (2,887)     | (2,453)     | (2,887)   | (2,453)   | (1,925)    | (1,635)   | -          |       |
| Interest Income                           | 404         | 229         | 105         | 40          | 126         | 26          | 127       | 20        | 253        | 84        | 523        |       |
| Change in fair value of warrant liability | -           | -           |             |             |             |             | -         | -         | -          | -         | -          |       |
| Other income, net                         | -           | -           | -           | -           | -           | -           | -         | -         | -          | -         | -          |       |
| Total Other Income                        | (704)       | (664)       | (2,782)     | (2,412)     | (2,762)     | (2,427)     | (2,760)   | (2,433)   | (1,672)    | (1,551)   | 523        |       |
| Income Before Income Taxes                | (38,537)    | (30,114)    | (41,049)    | (27,322)    | (20,332)    | (10,915)    | 7,591     | 6,548     | 45,710     | 31,118    | 83,864     |       |
| Income Tax Provision                      | -           | -           | -           | -           | -           | -           | -         | -         | -          | -         | (4,193)    |       |
| Accretion of preferred stock dividends    | _           | _           | _           | -           | _           | -           | _         | -         | _          | -         | _          |       |
| Net income (GAAP)                         | \$ (38,537) | \$ (30,114) | \$ (41,049) | \$ (27,322) | \$ (20,332) | \$ (10,915) | \$ 7,591  | \$ 6,548  | \$ 45,710  | \$ 31,118 | \$ 79,671  |       |
| GAAP EPS (diluted)                        | \$ (1.96)   | \$ (1.69)   | \$ (1.90)   | \$ (1.51)   | \$ (0.93)   | \$ (0.60)   | \$ 0.32   | \$ 0.32   | \$ 1.90    | \$ 1.52   | \$ 3.28    |       |
| Weighted shares outstanding               | , , , ,     | ,,          | , , ,       | , , ,       | ,,          | ,,          |           |           |            |           |            |       |
| diluted - GAAP                            | 19,502      | 17,774      | 21,625      | 18,087      | 21,841      | 18,267      | 23,814    | 20,204    | 24,052     | 20,406    | 24,293     |       |

Source: Collins Stewart LLC Research and Company Reports

### Valuation and Risks

Our 12-month PT of \$27 (prev. \$23) is based on 35x our fully taxed, fully diluted FY15 GAAP EPS estimate of \$1.90 (prev. \$1.52) discounted back to YE12 (prev. mid-2012) at 30%. 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.



# **Aegerion Pharmaceuticals**

(NASDAQ: AEGR)

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Consolidated Income Statement

(\$thousands, except per share data)

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|   | FY          | Mar        | Jun        | Sep         | Dec         | FY          | FY          | FY          | FY        | FY         | FY         |
|---|-------------|------------|------------|-------------|-------------|-------------|-------------|-------------|-----------|------------|------------|
|   | 2010A       | 1Q11A      | 2Q11A      | 3Q11E       | 4Q11E       | 2011E       | 2012E       | 2013E       | 2014E     | 2015E      | 2016E      |
| Revenue                                   |             |            |            |             |             |             |             |             |           |            |            |
| Lomitapide Total Revenue                  | -           | -          | -          | -           | -           | -           | \$ 8,236    | \$ 40,074   | \$ 76,740 | \$ 122,860 | \$ 166,075 |
| Lomitapide HoFH - U.S.                    | -           | -          | -          | -           | -           | -           | 6,940       | 23,882      | 44,087    | 69,247     | 91,817     |
| Lomitapide HoFH - E.U.                    | -           | -          | -          | -           | -           | -           | 1,296       | 16,192      | 32,653    | 53,613     | 74,258     |
| Total Revenue                             | \$ -        | \$ -       | \$ -       | \$ -        | \$ -        | \$ -        | \$ 8,236    | \$ 40,074   | \$ 76,740 | \$ 122,860 | \$ 166,075 |
| cogs                                      |             |            | _          |             |             | _           | 659         | 5,210       | 9,976     | 14,743     | 18,268     |
| Gross profit                              | -           | -          |            | -           | -           |             | 7,577       | 34,865      | 66,764    | 108,117    | 147,807    |
| Gross profit                              | -           | -          | -          | -           | -           | -           | 7,577       | 34,005      | 00,704    | 100,117    | 147,007    |
| Operating expense                         |             |            |            |             |             |             |             |             |           |            |            |
| R&D (GAAP)                                | 7,629       | 3,297      | 5,138      | 6,032       | 7,521       | 21,988      | 25,523      | 27,002      | 28,412    | 30,211     | 31,783     |
| SG&A (GAAP)                               | 5,922       | 3,491      | 3,205      | 3,898       | 5,252       | 15,846      | 20,321      | 25,433      | 28,001    | 30,523     | 32,683     |
| Total operating expense                   | 13,551      | 6,787      | 8,343      | 9,930       | 12,773      | 37,834      | 45,844      | 52,435      | 56,413    | 60,734     | 64,466     |
|   |             |            |            | -           |             |             |             |             |           |            |            |
| Operating income (loss)                   | (13,551)    | (6,787)    | (8,343)    | (9,930)     | (12,773)    | , , ,       | (38,267)    | (17,570)    | 10,351    | 47,383     | 83,341     |
| Interest Expense                          | (2,404)     | (113)      | (332)      | (332)       | (332)       | (1,108)     | (2,887)     | (2,887)     | (2,887)   | (1,925)    | -          |
| Interest Income                           | 109         | 68         | 70         | 140         | 126         | 404         | 105         | 126         | 127       | 253        | 523        |
| Change in fair value of warrant liability | (416)       | -          | -          |             |             | -           |             |             | -         | -          | -          |
| Other income, net                         | 215         | Ī          | -          |             |             |             |             |             |           |            |            |
| Total Other Income                        | (2,496)     | (45)       | (262)      | (191)       | (206)       | (704)       | (2,782)     | (2,762)     | (2,760)   | (1,672)    | 523        |
| Income Before Income Taxes                | (16,047)    | (6,832)    | (8,605)    | (10,121)    | (12,979)    | (38,537)    | (41,049)    | (20,332)    | 7,591     | 45,710     | 83,864     |
| Income Tax Provision                      | 1,793       | -          | -          | -           | -           | -           | -           | -           | -         | -          | (4,193)    |
| Accretion of preferred stock dividends    | (8,751)     | _          | _          | _           | _           | _           | _           | _           | _         | _          | _          |
| Net income (GAAP)                         | \$ (23,005) | \$ (6,832) | \$ (8,605) | \$ (10,121) | \$ (12,979) | \$ (38,537) | \$ (41,049) | \$ (20,332) | \$ 7,591  | \$ 45,710  | \$ 79,671  |
| GAAP EPS (diluted)                        | \$ (5.07)   | \$ (0.39)  | \$ (0.49)  | \$ (0.48)   | \$ (0.61)   | \$ (1.96)   | \$ (1.90)   | \$ (0.93)   | \$ 0.32   | \$ 1.90    | \$ 3.28    |
| Weighted shares outstanding               |             |            |            |             |             |             |             |             |           |            |            |
| diluted - GAAP                            | 4,537       | 17,642     | 17,729     | 21,226      | 21,411      | 19,502      | 21,625      | 21,841      | 23,814    | 24,052     | 24,293     |
| dildied - SAAF                            | 4,557       | 17,042     | 17,729     | 21,220      | 21,411      | 19,502      | 21,023      | 21,041      | 23,014    | 24,032     | 24,293     |
| Margin Analysis:                          |             |            |            |             |             |             |             |             |           |            |            |
| Cost of product sales                     | nm          | nm         | nm         | nm          | nm          | nm          | 8%          | 13%         | 13%       | 12%        | 11%        |
| Product gross margin                      | nm          | nm         | nm         | nm          | nm          | nm          | 92%         | 87%         | 87%       | 88%        | 89%        |
| R&D (non-GAAP)                            | nm          | nm         | nm         | nm          | nm          | nm          | 310%        | 67%         | 37%       | 25%        | 19%        |
| SG&A (non-GAAP)                           | nm          | nm         | nm         | nm          | nm          | nm          | 247%        | 63%         | 36%       | 25%        | 20%        |
| Stock-based compensation expense          | nm          | nm         | nm         | nm          | nm          | nm          | 79%         | 19%         | 11%       | 8%         | 6%         |
| Total operating expense                   | nm          | nm         | nm         | nm          | nm          | nm          | 557%        | 131%        | 74%       | 49%        | 39%        |
| Operating margin                          | nm          | nm         | nm         | nm          | nm          | nm          | -465%       | -44%        | 13%       | 39%        | 50%        |
| Income tax provision                      | nm          | nm         | nm         | nm          | nm          | nm          | 0%          | 0%          | 0%        | 0%         | 5%         |
| Net margin (GAAP)                         | nm          | nm         | nm         | nm          | nm          | nm          | nm          | nm          | 10%       | 37%        | 48%        |
| Y/Y change:                               |             |            |            |             |             |             |             |             |           |            |            |
| Total revenue                             | nm          | nm         | nm         | nm          | nm          | nm          | nm          | 387%        | 91%       | 60%        | 35%        |
| R&D                                       | 8%          | nm         | nm         | nm          | nm          | 188%        | 16%         | 6%          | 5%        | 6%         | 5%         |
| SG&A                                      | 93%         | nm         | nm         | nm          | nm          | 168%        | 28%         | 25%         | 10%       | 9%         | 7%         |
| Stock-based compensation expense          | -24%        | nm         | nm         | nm          | nm          | 668%        | 19%         | 14%         | 13%       | 12%        | 11%        |
| Total operating expense                   | 34%         | nm         | nm         | nm          | nm          | 179%        | 21%         | 14%         | 8%        | 8%         | 6%         |
| Operating income                          | 34%         | nm         | nm         | nm          | nm          | 179%        | 1%          | -54%        | -159%     | 358%       | 76%        |
| Net income (GAAP)                         | 49%         | nm         | nm         | nm          | nm          | 68%         | 7%          | -50%        | -137%     | 502%       | 74%        |
| GAAP EPS (diluted)                        | -46%        | nm         | nm         | nm          | nm          | -61%        | -3%         | -51%        | -134%     | 496%       | 73%        |
| Shares outstanding - GAAP                 | 174%        | nm         | nm         | nm          | l nm        | 330%        | 11%         | 1%          | 9%        | 1%         | 1%         |

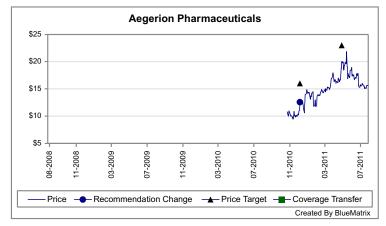
Source: Collins Stewart LLC Research and Company Reports



# Important Disclosure / Disclaimer Information

Other Public Companies Mentioned in this Report

CompanyTickerPriceRecommendationAegerion PharmaceuticalsAEGR\$15.62BUY



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

Collins Stewart LLC Ratings

#### Valuation and Risks

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

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

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

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

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|             | % of CSTI Universe with<br>this rating | % of rating tier for which CSTI provided IB services |
|-------------|--|--|
| Buy<br>Hold | 55%                                    | 0%   |
| Hold        | 42%                                    | 0%   |
| Sell        | 3%                                     | 0%   |

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