



November 11, 2014

## **Aerie Pharmaceuticals Reports Third Quarter 2014 Financial Results and Provides Business and Product Development Update**

**Focused on Building a Major Ophthalmic Pharmaceutical Company**

**Rhopressa™ and Roclatan™ Development Programs Advancing**

Conference Call and Webcast Today, November 11, at 5:00 p.m. ET

BEDMINSTER, N.J. & RESEARCH TRIANGLE PARK, N.C. & NEWPORT BEACH, Calif.--(BUSINESS WIRE)-- Aerie Pharmaceuticals, Inc. (NASDAQ:AERI), a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with glaucoma and other diseases of the eye, today reported financial results for third quarter 2014 and provided an update on the Company's business highlights.

### **Aerie Highlights**

- | Rhopressa™ Phase 3 registration trials are in progress, with three-month efficacy data expected by mid-2015.
- | Roclatan™ Phase 3 enabling activities also are ongoing, and Phase 3 registration trials are expected to commence in mid-2015.
- | Aerie ended the third quarter of 2014 with over \$171 million in cash, cash equivalents and investments. This cash and investment balance is expected to be sufficient to fund development activities and commercialization of Rhopressa™ and Roclatan™ and provides flexibility to pursue potential strategic growth opportunities.

"Aerie continues to make significant progress in advancing its novel product candidates, which were shown to be highly efficacious in Phase 2 clinical trials for the treatment of patients with glaucoma and ocular hypertension. Our Phase 3 clinical trials for Rhopressa™ are moving forward at a healthy pace, and Phase 3 preparations for Roclatan™ continue to point to a mid-2015 commencement of Phase 3 clinical trials. The strategies we laid out at our September 10<sup>th</sup> Investor Day remain intact, and we are pleased with our trajectory and progress," said Vicente Anido, Jr., Ph.D., Chairman and Chief Executive Officer.

Dr. Anido continued, "As previously announced, we recently completed a \$125 million senior secured convertible debt financing with Deerfield Management Company L.P. Combined with the proceeds from our IPO, we have raised nearly \$200 million since October 2013. We now have substantial financial resources to advance our clinical programs forward to product commercialization and the flexibility to execute our strategy of building a major ophthalmic pharmaceutical company."

### **Triple-Action Rhopressa™**

Aerie's first-in-class product candidates are all single drop, once-daily medications that are well-tolerated and have shown no systemic drug-related adverse events.

Rhopressa™ is a novel triple-action eye drop that we believe, if approved, would become the only once-daily product available that specifically targets the trabecular meshwork (TM), the eye's primary fluid drain and the diseased tissue responsible for elevated intraocular pressure (IOP) in glaucoma. Preclinical results have demonstrated that Rhopressa™ also lowers episcleral venous pressure (EVP), which contributes approximately half of IOP in healthy subjects. Further, Rhopressa™ provides an additional mechanism that reduces fluid production in the eye and therefore lowers IOP. Biochemically, Rhopressa™ is known to inhibit both Rho Kinase (ROCK) and norepinephrine transporter (NET).

In the Company's Phase 2b clinical trial, which was successfully completed in May 2013, Rhopressa™ demonstrated a strong IOP-lowering effect, with mean IOP reductions of 5.7 and 6.2 mmHg on days 28 and 14, respectively. In addition, Rhopressa™ demonstrated a consistent mean IOP-lowering effect irrespective of the baseline IOPs of the patients entered into the trial. This differentiates Rhopressa™ from currently marketed IOP-lowering agents such as market-leading prostaglandins (PGAs) and beta blockers, which have their highest effect at higher baseline IOPs, while losing efficacy as the baseline diminishes, as shown in published studies. This is significant given that the majority of glaucoma patients have low to moderately elevated IOPs of 26 mmHg or below at the time of diagnosis. In the Roclatan™ Phase 2b trial recently completed in June 2014, Rhopressa™ performed with similar results as in its Phase 2b trial completed in May 2013 and, in addition, demonstrated additive efficacy when used in combination with latanoprost, the most commonly prescribed PGA.

In July 2014, the Company commenced its Phase 3 registration trials for Rhopressa™, which will measure efficacy over three months and safety over 12 months. Two trials are being conducted in the United States, named "Rocket 1" and "Rocket 2," where the primary efficacy endpoint will be to demonstrate non-inferiority of IOP lowering for Rhopressa™ compared to timolol. Timolol is the most widely used comparator in registration trials for glaucoma. A third safety-only registration trial is being conducted in Canada. Three-month efficacy results are expected by mid-2015. If the trials are successful, the Company expects to submit an NDA filing by mid-2016.

### **Quadruple-Action Roclatan™**

Roclatan™ is a once-daily eye drop that combines our triple-action Rhopressa™ with latanoprost, a PGA that is the most widely prescribed glaucoma drug. If approved, we believe that Roclatan™ would be the first glaucoma product to lower IOP through all known mechanisms: (i) increasing fluid outflow through the TM, the eye's primary drain, (ii) increasing fluid outflow through the uveoscleral pathway, the eye's secondary drain, (iii) reducing fluid production in the eye, and (iv) reducing EVP.

A successful 28-day Phase 2b clinical trial for Roclatan™ was completed in June 2014. Roclatan™ achieved its primary efficacy endpoint on day 29 and demonstrated statistical superiority over the product's individual components at all time points. We believe that Roclatan™, if approved, would be the only glaucoma product that covers the full spectrum of known IOP-lowering mechanisms, giving it the potential to provide a greater IOP-lowering effect than any currently approved glaucoma product. Therefore, we believe Roclatan™, if approved, could compete in both the PGA and non-PGA markets and become the product of choice for patients requiring maximal IOP lowering, including those with IOPs in excess of 26 mmHg and those who present with significant disease progression despite currently available therapies. We expect Phase 3 registration trials to commence in mid-2015. Preparatory steps for such trials are in progress.

### **Third Quarter 2014 Financial Results**

As of September 30, 2014, the Company had cash, cash equivalents and investments of \$171.1 million. For the third quarter ended September 30, 2014, the Company reported a net loss attributable to common stockholders, as measured in accordance with U.S. generally accepted accounting principles ("GAAP"), of \$13.1 million, or \$0.54 per share, compared to \$10.9 million and \$10.81 per share for the third quarter 2013, when the Company was privately held. The weighted average number of shares of common stock outstanding utilized in the calculation of net loss per common share was 24,325,166 and 1,006,893 for the third quarter 2014 and 2013, respectively. The increase in the weighted average number of shares of common stock outstanding is attributable to changes to the Company's capital structure in connection with the IPO in October 2013.

The \$13.1 million net loss attributable to common stockholders for the third quarter 2014 includes \$13.2 million in operating expenses, reflecting \$8.2 million in research and development expenses and \$4.9 million in general and administrative expenses. Included in the \$13.2 million net loss is \$2.4 million of non-cash stock-based compensation expense included in operating expenses. Excluding non-cash stock-based compensation expense, adjusted operating expenses of \$10.8 million include adjusted research and development expenses of \$8.0 million and adjusted general and administrative expenses of \$2.8 million. Total adjusted net loss was \$10.8 million and adjusted net loss per share was \$0.44.

The \$10.9 million net loss attributable to common stockholders for the third quarter 2013 includes \$5.7 million in operating expenses, reflecting research and development expenses of \$2.4 million and general and administrative expenses of \$3.3 million, non-cash interest expense and certain valuation-related losses of \$5.1 million included in other income (expense), net and convertible preferred stock-related charges of \$0.1 million. Excluding non-cash stock-based compensation expense of \$1.1 million, adjusted operating expenses for third quarter 2013 were approximately \$4.6 million, reflecting adjusted research and development expenses of \$2.3 million and adjusted general and administrative expenses of \$2.2 million. Total adjusted net loss was \$4.6 million.

The higher operating expenses in third quarter 2014 as compared to third quarter 2013 primarily reflect increased clinical and non-clinical activities for Rhopressa™, increased non-clinical activities for Roclatan™ and increased activity related to supporting the growth in our operations.

For the nine months ended September 30, 2014, the Company reported a net loss attributable to common stockholders, as measured in accordance with GAAP, of \$31.6 million, or \$1.32 per share, compared to \$21.3 million and \$21.61 per share for the nine months ended September 30, 2013. The weighted average number of shares of common stock outstanding utilized in the calculation of net loss per common share was 23,980,963 and 984,727 for the nine months ended September 30, 2014 and 2013, respectively.

The \$31.6 million net loss attributable to common stockholders for the nine months ended September 30, 2014 includes \$34.0 million in operating expenses, reflecting \$20.3 million in research and development expenses and \$13.7 million in general and administrative expenses, partially offset by \$2.4 million in proceeds from the sale of a New Jersey state tax benefit, which is recorded as income in other income (expense), net. Included in the \$31.6 million net loss is \$6.7 million of non-cash stock-based compensation expense included in operating expenses. Excluding non-cash stock-based compensation expense, adjusted operating expenses of \$27.3 million include adjusted research and development expenses of \$19.2 million and adjusted general and administrative expenses of \$8.1 million. Total adjusted net loss was \$24.9 million and adjusted net loss per share was \$1.04.

The \$21.3 million net loss attributable to common stockholders for the nine months ended September 30, 2013 includes \$15.4 million in operating expenses, reflecting research and development expenses of \$8.7 million and general and administrative expenses of \$6.7 million. Additionally, it includes non-cash interest and amortization expense and certain valuation-related losses of \$6.7 million, partially offset by \$1.3 million in proceeds from the sale of a New Jersey state tax benefit included in other income (expense), net and convertible preferred stock-related charges of \$0.4 million. Excluding non-cash stock-based compensation expense of \$1.5 million, adjusted operating expenses for the nine months ended September 30, 2013 were approximately \$13.9 million, reflecting adjusted research and development expenses of \$8.6 million and adjusted general and administrative expenses of \$5.3 million. Total adjusted net loss was \$12.6 million.

The higher operating expenses for the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013 primarily reflect increased clinical and non-clinical activities for Rhopressa™, increased clinical and non-clinical activities for Roclatan™ and increased activity related to supporting the growth in our operations.

### **Conference Call / Web Cast Information**

Aerie management will host a live conference call and webcast at 5:00 p.m. Eastern Time today to discuss the Company's financial results and provide a general business update.

The live webcast and a replay may be accessed by visiting Aerie's website at <http://investors.aeriepharma.com>. Please connect to the Company's website at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call (888) 734-0328 (U.S.) or (678) 894-3054 (international) to listen to the live conference call. The conference ID number for the live call is 20105726. Please dial in approximately 10 minutes prior to the call. Telephone replay will be available approximately two hours after the call. To access the replay, please call (855)-859-2056 (U.S.) or (404) 537-3406 (international). The conference ID number for the replay is 20105726. The telephone replay will be available until November 18, 2014.

### **About Aerie Pharmaceuticals, Inc.**

Aerie is a clinical-stage pharmaceutical company focused on the discovery, development and commercialization of first-in-class therapies for the treatment of patients with glaucoma and other diseases of the eye. The Company is conducting two Phase 3 registration trials in the United States, named "Rocket 1" and "Rocket 2," where the primary efficacy endpoint will be to demonstrate non-inferiority of IOP lowering for Rhopressa™ compared to timolol, along with a third Phase 3 registration safety-only trial, named "Rocket 3," in Canada. The Company also completed a Phase 2b clinical trial where Roclatan™ met the primary efficacy endpoint, demonstrating the statistical superiority of Roclatan™ to each of its components. Phase 3 registration trials for Roclatan™ are expected to commence in mid-2015. Preparatory steps for these trials are in progress.

### **Forward-Looking Statements**

This press release contains forward-looking statements for purposes of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "exploring,"

"pursuing" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Forward-looking statements include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things: the success, timing and cost of our ongoing and anticipated preclinical studies and clinical trials for our current product candidates, including statements regarding the timing of initiation and completion of the studies and trials; our expectations regarding the clinical effectiveness of our product candidates and results of our clinical trials; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates; our expectations regarding the commercialization of our product candidates; our expectations related to the use of proceeds from our initial public offering and the issuance and sale of our senior secured convertible notes; our estimates regarding anticipated capital requirements and our needs for additional financing; the potential advantages of our product candidates; our plans to pursue development of our product candidates for additional indications and other therapeutic opportunities; our plans to explore possible uses of our existing proprietary compounds beyond glaucoma; and our ability to protect our proprietary technology and enforce our intellectual property rights. By their nature, forward-looking statements involve risks and uncertainties because they relate to events, competitive dynamics and industry change, and depend on regulatory approvals and economic circumstances that may or may not occur in the future or may occur on longer or shorter timelines than anticipated. We discuss many of these risks in greater detail under the heading "Risk Factors" in the quarterly and annual reports that we file with the Securities and Exchange Commission (SEC). Forward-looking statements are not guarantees of future performance and our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this press release. Any forward-looking statements that we make in this press release speak only as of the date of this press release. We assume no obligation to update our forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

### Non-GAAP Financial Measures

To supplement our financial statements, which are prepared and presented in accordance with GAAP, we use the following non-GAAP financial measures, some of which are discussed above: adjusted net loss, adjusted operating expenses, adjusted research and development expenses, adjusted general and administrative expenses, adjusted other income (expense) and adjusted net loss per share. For a description of the adjusted calculations and reconciliation to the nearest GAAP measure, please see the "Reconciliation of GAAP Net Loss to Adjusted Net Loss" and "Reconciliation of GAAP Net Loss per Share to Adjusted Net Loss per Share" tables in this press release.

We believe these non-GAAP financial measures provide investors with useful supplemental information about the financial performance of our business, enable comparison of financial results between periods where certain items may vary independent of business performance, and allow for greater transparency with respect to key metrics used by management in operating our business.

The presentation of these financial measures is not intended to be considered in isolation from, or as a substitute for, financial information prepared and presented in accordance with GAAP. Investors are cautioned that there are material limitations associated with the use of non-GAAP financial measures as an analytical tool. In particular, the adjustments to our GAAP financial measures reflect the exclusion of non-cash stock-based compensation expense, which is recurring and will be reflected in our financial results for the foreseeable future. In addition, these measures may be different from non-GAAP financial measures used by other companies, limiting their usefulness for comparison purposes. We compensate for these limitations by providing specific information regarding the GAAP amounts excluded from these non-GAAP financial measures.

**AERIE PHARMACEUTICALS, INC.**  
**(A Development Stage Company)**  
**Balance Sheets**  
**(Unaudited)**

(in thousands, except share and per share data)

	<b>SEPTEMBER 30, 2014</b>	<b>DECEMBER 31, 2013</b>
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 148,884	\$ 69,649
Short-term investments	22,177	—
Prepaid expenses and other current assets	656	618

Total current assets	171,717	70,267
Furniture, fixtures and equipment, net	228	132
Other assets, net	1,338	59
Total assets	<u>\$ 173,283</u>	<u>\$ 70,458</u>

### Liabilities and Stockholders' Equity

Current liabilities		
Accounts payable and other current liabilities	\$ 6,999	\$ 3,482
Total current liabilities	<u>6,999</u>	<u>3,482</u>
Convertible notes, net of discounts	124,125	—
Total liabilities	<u>131,124</u>	<u>3,482</u>
Commitments and contingencies		
Stockholders' Equity		
Preferred stock, \$0.001 par value; 15,000,000 shares authorized as of September 30, 2014 and December 31, 2013; None issued and outstanding	—	—
Common stock, \$0.001 par value; 150,000,000 shares authorized as of September 30, 2014 and December 31, 2013; 23,967,696 and 23,285,549 shares issued and outstanding as of September 30, 2014 and December 31, 2013, respectively	24	23
Additional paid-in capital	168,844	162,021
Accumulated other comprehensive loss	(9)	—
Deficit accumulated during the development stage	<u>(126,700)</u>	<u>(95,068)</u>
Total stockholders' equity	<u>42,159</u>	<u>66,976</u>
Total liabilities and stockholders' equity	<u>\$ 173,283</u>	<u>\$ 70,458</u>

**AERIE PHARMACEUTICALS, INC.**  
**(A Development Stage Company)**  
**Statements of Operations and Comprehensive Loss**  
**Unaudited**  
(in thousands, except share and per share data)

	<b>THREE MONTHS ENDED</b>		<b>NINE MONTHS ENDED</b>	
	<b>SEPTEMBER 30,</b>		<b>SEPTEMBER 30,</b>	
	<b>2014</b>	<b>2013</b>	<b>2014</b>	<b>2013</b>
<b>Operating expenses</b>				
General and administrative	\$ (4,944)	\$ (3,287)	\$ (13,723)	\$ (6,693)
Research and development	<u>(8,230)</u>	<u>(2,399)</u>	<u>(20,276)</u>	<u>(8,727)</u>
Loss from operations	(13,174)	(5,686)	(33,999)	(15,420)
Other income (expense), net	27	(5,062)	2,367	(5,446)
Net loss	<u>\$ (13,147)</u>	<u>\$ (10,748)</u>	<u>\$ (31,632)</u>	<u>\$ (20,866)</u>
Net loss attributable to common stockholders—basic and diluted	<u>\$ (13,147)</u>	<u>\$ (10,887)</u>	<u>\$ (31,632)</u>	<u>\$ (21,279)</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (0.54)</u>	<u>\$ (10.81)</u>	<u>\$ (1.32)</u>	<u>\$ (21.61)</u>
Weighted average number of common shares outstanding—basic and diluted	<u>24,325,166</u>	<u>1,006,893</u>	<u>23,980,963</u>	<u>984,727</u>
Net loss	\$ (13,147)	\$ (10,748)	\$ (31,632)	\$ (20,866)
Unrealized gain (loss) on available-for-sale investments	4	—	(9)	—
Comprehensive loss	<u>\$ (13,143)</u>	<u>\$ (10,748)</u>	<u>\$ (31,641)</u>	<u>\$ (20,866)</u>

**Aerie Pharmaceuticals, Inc.**  
**(A Development Stage Company)**  
**Reconciliation of GAAP Net Loss to Adjusted Net Loss**  
**and GAAP Net Loss per Share to Adjusted Net Loss per Share**

**Reconciliation of GAAP Net Loss to Adjusted Net Loss**  
**(Unaudited)**  
(in thousands)

	<b>THREE MONTHS ENDED SEPTEMBER 30,</b>		<b>NINE MONTHS ENDED SEPTEMBER 30,</b>	
	<b>2014</b>	<b>2013</b>	<b>2014</b>	<b>2013</b>
<b>Net loss attributable to common stockholders - basic and diluted:</b>				
Net loss attributable to common stockholders - basic and diluted (GAAP)	\$ (13,147)	\$ (10,887)	\$ (31,632)	\$ (21,279)
<b>Adjustments:</b>				
Stock-based compensation (a)	2,392	1,130	6,696	1,531
Change in fair value measurements of warrant liabilities (b)	—	3,585	—	3,850
Interest and amortization expense related to notes subsequently converted to common equity (c)	—	1,477	—	2,865
Accretion related to convertible preferred stock (d)	—	139	—	413
<b>Adjusted Net loss</b>	<b><u>\$ (10,755)</u></b>	<b><u>\$ (4,556)</u></b>	<b><u>\$ (24,936)</u></b>	<b><u>\$ (12,620)</u></b>
<b>Operating expenses:</b>				
<b>General and administrative expense:</b>				
General and administrative expense (GAAP)	\$ (4,944)	\$ (3,287)	\$ (13,723)	\$ (6,693)
<b>Adjustments:</b>				
Stock-based compensation (a)	2,172	1,068	5,624	1,426
<b>Adjusted general and administrative expense</b>	<b><u>\$ (2,772)</u></b>	<b><u>\$ (2,219)</u></b>	<b><u>\$ (8,099)</u></b>	<b><u>\$ (5,267)</u></b>
<b>Research and development expense:</b>				
Research and development expense (GAAP)	\$ (8,230)	\$ (2,399)	\$ (20,276)	\$ (8,727)
<b>Adjustments:</b>				
Stock-based compensation (a)	220	62	1,072	105
<b>Adjusted research and development expense</b>	<b><u>\$ (8,010)</u></b>	<b><u>\$ (2,337)</u></b>	<b><u>\$ (19,204)</u></b>	<b><u>\$ (8,622)</u></b>
Operating expenses (GAAP)	\$ (13,174)	\$ (5,686)	\$ (33,999)	\$ (15,420)
<b>Adjustments:</b>				
Stock-based compensation (a)	2,392	1,130	6,696	1,531
<b>Adjusted operating expenses</b>	<b><u>\$ (10,782)</u></b>	<b><u>\$ (4,556)</u></b>	<b><u>\$ (27,303)</u></b>	<b><u>\$ (13,889)</u></b>
<b>Other income (expense):</b>				
Other income (expense) (GAAP)	\$ 27	\$ (5,062)	\$ 2,367	\$ (5,446)
<b>Adjustments:</b>				
Change in fair value measurements of warrant liabilities (b)	—	3,585	—	3,850
Interest and amortization expense related to notes subsequently converted to common equity (c)	—	1,477	—	2,865
<b>Adjusted other income (expense)</b>	<b><u>\$ 27</u></b>	<b><u>\$ —</u></b>	<b><u>\$ 2,367</u></b>	<b><u>\$ 1,269</u></b>

**Reconciliation of GAAP Net Loss Per Share to Adjusted Net Loss Per Share**  
**(Unaudited)**

	THREE MONTHS ENDED		NINE MONTHS ENDED	
	SEPTEMBER 30,		SEPTEMBER 30,	
	2014	2013	2014	2013
<b>Net loss per share attributable to common stockholders - basic and diluted:</b>				
Net loss per share attributable to common stockholders - basic and diluted (GAAP)	\$ (0.54)	\$ (10.81)	\$ (1.32)	\$ (21.61)
<b>Adjustments:</b>				
Stock-based compensation (a)	0.10	1.12	0.28	1.55
Change in fair value measurements of warrant liabilities (b)	—	3.56	—	3.91
Interest and amortization expense related to notes subsequently converted to common equity (c)	—	1.47	—	2.91
Accretion related to convertible preferred stock (d)	—	0.14	—	0.42
<b>Adjusted Net loss per share</b>	<b>\$ (0.44)</b>	<b>\$ (4.52)</b>	<b>\$ (1.04)</b>	<b>\$ (12.82)</b>
<b>Weighted average number of common shares outstanding - basic and diluted</b>	<b>24,325,166</b>	<b>1,006,893</b>	<b>23,980,963</b>	<b>984,727</b>

Aerie is providing adjusted information that excludes certain items because of the nature of these items and the impact they have on the analysis of underlying business performance and trends. Management believes that providing this information enhances investors' understanding of the Company's performance. This information should be considered in addition to, but not in lieu of, information prepared in accordance with GAAP.

Explanation of adjustments:

**(a) Stock-based compensation:** Exclude the non-cash stock-based compensation.

**(b) Change in fair value measurements of warrant liabilities:** Exclude the non-cash change in fair value.

**(c) Interest and amortization expense related to notes subsequently converted to common equity:** Exclude the non-cash interest and amortization expense.

**(d) Accretion related to convertible preferred stock:** Exclude the accretion related to convertible preferred stock.

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