

## Avalanche Biotechnologies, Inc.

### Fourth-Quarter Earnings a Nonevent; All Eyes on Midyear Data Readout, Maintain Outperform Rating

- Before the markets opened on Thursday, March 5, Avalanche Biotechnologies released fourth quarter 2014 earnings. We view earnings as a relative nonevent ahead of an important clinical calendar in 2015, particularly related to the company's lead product, AVA-101, which is expected to complete its Phase IIa study in 32 patients (2:1 active versus placebo) by mid-2015 and begin a Phase IIb study in the second half of the year. AVA-101 is delivered as a single subretinal injection that has the potential to serve as a "functional cure" for wet AMD, a disease that affects as many as 1.7 million individuals in the United States.
- AVA-101 is a gene therapy comprising an adeno-associated virus (AAV2) vector, which contains a gene-encoding sFLT-1, a naturally occurring VEGF inhibitor. We continue to view the gene therapy delivery of anti-VEGF by Avalanche as a best-in-class approach with the potential to be disruptive to the roughly \$6 billion worldwide wet AMD market. In the AVA-101 Phase I study, best-corrected visual acuity (the primary endpoint in all approved wet AMD therapy efficacy studies) increased in six of the seven patients tested, with a mean of +8.7 letters in the low-dose group and +6.3 in the high-dose group. On average, AVA-101-treated subjects needed only 0.33 Lucentis injections out of 12 opportunities over one year.
- All eyes remain focused on the upcoming Phase IIa data readout, which is still expected midyear. While shares have been punished during the first quarter, selling off 30% year-to-date, our enthusiasm for AVA-101 remains high ahead of the midyear data readout based on the strength of the company's Phase I data; we believe all patients treated with AVA-101 showed a benefit depending on the definition of response. In addition, we believe shares may begin to recover as soon as March 25, when the company will host an analyst event in the New York. And while the Phase IIa results will be a continued focus for investors, we believe the breadth of science and programs under way at Avalanche may broaden investor focus to the company's pipeline, which also includes AVA-201 for intermediate AMD, or wet AMD prevention; the rare disease X-linked retinoschisis (XLRS); and potentially several yet unnamed programs.

Recall that during the IPO, collaborator Regeneron Pharmaceuticals (REGN \$413.04) received a small equity stake in Avalanche as part of a larger partnership that included up to \$640 million in development milestones covering up to eight distinct therapeutic targets plus royalties and reimbursement of research costs. As part of the deal, Avalanche has the option to share up to 35% of profits and development costs for two of the targets, while Regeneron also has a right to see the Phase IIa data for AVA-101 and make an offer for the asset.

*Avalanche Biotechnologies is a biotechnology company located in Menlo Park, California, focused on developing gene-based therapies for the treatment of ocular diseases.*

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March 05, 2015

Stock Rating: **Outperform**  
Company Profile: **Aggressive Growth**  
Price Target: \$53.00

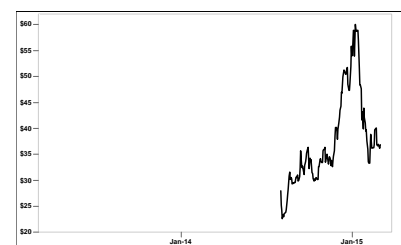
Symbol: AAVL (NASDAQ)  
Price: \$37.51 (52-Wk.: \$22-\$62)  
Market Value (mil.): \$896  
Fiscal Year End: December  
Long-Term EPS Growth Rate: NA  
Dividend/Yield: None

	2013A	2014A	2015E
<b>Estimates</b>			
EPS Q1	NA	A\$-0.45	NA
Q2	NA	A\$-2.27	NA
Q3	NA	A\$-0.50	NA
Q4	NA	\$-0.46	NA
FY	\$-1.44	\$-2.01	\$-2.45
CY		\$-2.01	\$-2.45
<b>Valuation</b>			
FY P/E	NM	NM	NM
CY P/E		NM	NM

<b>Trading Data (FactSet)</b>	
Shares Outstanding (mil.)	16
Float (mil.)	15
Average Daily Volume	257,661

<b>Financial Data (FactSet)</b>	
Long-Term Debt/Total Capital (MRQ)	0.0
Book Value Per Share (MRQ)	6.9
Return on Equity (TTM)	0.0

#### Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

However, Avalanche is not obligated to accept any terms given by Regeneron and may still partner AVA-101 away from the company if Avalanche believes other parties would make a more attractive offer following Phase IIb data. We believe there has been growing negative sentiment surrounding the use of gene therapy for a disease that has several efficacious treatment options already available, so it will be interesting to see how the Phase IIa data readout and the subsequent partnering discussions progress.

We see the investment and interest by a company with the leading drug in the space (Eylea) as strong backing of the scientific strength of Avalanche's platform and approach. The additions of Roman Rubio, M.D., previously global head of ophthalmology at Roche, and Samuel Barone, M.D., a former medical officer in the Office of Cellular, Tissue, and Gene Therapies at the U.S. Food and Drug Administration, also reinforce our belief that Avalanche holds a significant opportunity in AMD and its additional pipeline programs.

- The recent IPO of Spark Therapeutics (ONCE \$54.53) provides additional backing of the scientific basis of treating ocular diseases with subretinal administration of gene therapy candidates due to the immuno-privileged nature of the eye. Spark is developing SPK-RPE65 for the treatment of inherited retinal dystrophy (IRD), a rare disease with about 3,500 patients that is caused by a RPE65 gene defect. The product is expected to report results from its second Phase III trial in the second half of 2015; if approved, the product could come to the market as early as 2016 as a result of SPK-RPE65's breakthrough therapy designation.
- Avalanche completed its IPO in August 2014, raising net proceeds of about \$106.5 million, and had a subsequent follow-on earlier this year raising net proceeds of roughly \$141.6 million, which is not included in the company's cash position of \$159.4 million reported today (reflecting the end of the fourth quarter). Following the recent raise, we believe Avalanche is in a strong cash position to continue the development of its ocular gene therapy programs; we estimate the company holds pro forma cash of about \$285 million.

In the fourth quarter, the company reported total revenue of \$203,000 as a result of licensing revenues, in line with our estimate of \$200,000 but below the consensus of \$1.5 million. R&D and SG&A expenses were \$7.23 million and \$3.4 million, respectively, above our estimates of \$6.5 million and \$3.0 million as well as consensus of \$5.1 million and \$2.2 million. The net loss was \$10.4 million, or \$0.46 per share, compared with our estimate of a loss of \$9.5 million, or \$0.47 per share, and the consensus of a loss of \$6.8 million, or \$0.29 per share. We have included reported earnings, our estimates, and consensus estimates in exhibit 1.

- We continue to assign an Outperform rating to Avalanche, and we maintain our \$53 price target. We believe that Avalanche continues to lead in the ocular gene therapy field, and while the company has treated only a handful of patients to date with AVA-101, the proof-of-concept results are impressive with a "functional cure" in patients with wet AMD after prior heavy Lucentis use. In addition to the disruptive potential of AVA-101, we believe that pipeline compound AVA-201 holds significant promise for the potential prophylactic treatment of patients at risk for developing wet AMD, which would be another major step for the field.

**Exhibit 1**  
**Avalanche Biotechnologies, Inc.**  
**Fourth Quarter Results and Estimates**

	AAVL Q4 14A	WB Q4 14E	Consensus Q4 14E	Q/Q Growth	Variance (%)
(\$ in thousands except EPS)					
<b>Total Revenue</b>	<b>\$ 203.0</b>	<b>\$ 200.0</b>	<b>\$ 1,500.0</b>	<b>NM</b>	<b>NM</b>
R&D	\$ 7,226.0	\$ 6,500.0	\$ 5,100.0	26%	11.2%
G&A	\$ 3,380.0	\$ 3,000.0	\$ 2,200.0	41%	12.7%
Operating Income (loss)	\$ (10,403.0)	\$ (9,300.0)	\$ (7,700.0)	NM	NM
Net Income	\$ (10,391.0)	\$ (9,500.0)	\$ (6,800.0)	NM	NM
<b>EPS</b>	<b>\$ (0.46)</b>	<b>\$ (0.47)</b>	<b>\$ (0.29)</b>	<b>NM</b>	<b>NM</b>

Source: Company reports, William Blair & Company L.L.C. estimates  
Consensus estimates reported by FactSet

## Valuation

We rate shares of Avalanche Outperform with a price target of \$53. In this calculation, we assume a launch of AVA-101 in 2019 following approval early that same year. We believe peak-year sales will approximate \$1.1 billion domestically. Our estimates assume penetration of 45% into the AMD incidence and a cost of \$30,000 (below the \$44,000 to \$46,000 cost of two years of Lucentis/Eylea); we also include minimal revenue from DME and RVO, and no revenue from outside the United States, which is likely conservative.

### Exhibit 2 Avalanche Biotechnologies, Inc. Sum of the Parts Valuation

	Peak Sales	Discount Rate	Probability of Success	Peak Sales	Value Per Share
AVA101	\$1,136	11%	50%	2024	\$ 46.46
Cash Per Share					\$ 6.26
NPV Value					\$1,226,019
NPV Value Per Share					\$ 52.72

Source: William Blair & Company L.L.C. estimates

## Risks

Risks to shares of Avalanche are similar to those of other development-stage therapeutics companies. The company faces clinical, manufacturing, and regulatory risks related to its product candidates. There are additional clinical risks in developing a new cutting-edge technology. Any clinical or regulatory setbacks for the AVA-101 program or other gene therapy products in development would weigh heavily on shares.

Our earnings model is shown on the following page.



**Avalanche Biotech, Inc.**  
**Earnings Model**  
 3/5/15  
 (\$ in thousands except EPS data)

**Rating: Outperform**  
**Company Profile: Aggressive Growth**  
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	2012(A)	2013(A)	2014(A)	Q1(E)	Q2(E)	Q3(E)	Q4(E)	2015(E)	2016(E)	2017(E)	2018(E)	2019(E)	2020(E)	2021(E)	2022(E)
AVA-101	-	-	-	-	-	-	-	-	-	-	-	141,336	713,747	1,227,646	1,380,473
AVA-201	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
AVA-311	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
License revenue	30	480	572	30	30	30	30	120	-	-	-	-	-	-	-
Grant revenue	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
<b>Total Revenue</b>	30	480	572	30	30	30	30	120	120	-	-	141,336	713,747	1,227,646	1,380,473
yr/yr growth	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	72.0%	12.4%
q/q growth				NA	NA	NA	NA								
incremental rev q/q															
<b>Cost of Goods Sold</b>	-	-	-	-	-	-	-	-	-	-	-	56,534	71,375	98,212	110,438
<b>Gross Profit</b>	30	480	572	30	30	30	30	120	120	-	-	84,802	642,373	1,129,434	1,270,035
<b>R&amp;D</b>	1,310	2,151	16,976	9,846	9,846	14,769	14,769	49,230	56,615	39,630	69,353	83,224	55,672	61,382	62,121
Growth			689%	982%	218%	157%	104%	190%	15%	-30%	75%	20%	15%	15%	15%
<b>SG&amp;A</b>	536	1,783	7,998	2,639	2,639	3,959	3,959	13,197	14,516	15,968	17,565	70,259.23	99,925	147,318	179,461
Growth			30%					65%	10%	10%	10%	300%	42%	47%	22%
<b>Total Operating Expenses</b>	1,846	3,934	24,974	12,485	12,485	18,728	18,728	62,427	71,131	55,598	86,918	153,483	155,597	208,700	241,583
growth			535%	663%	172%	130%	77%	150%	14%	-22%	56%	77%	1%	34%	16%
Operating Income	(1,816)	(3,454)	(24,402)	(12,455)	(12,455)	(18,698)	(18,698)	(62,307)	(71,011)	(55,598)	(86,918)	(68,682)	486,776	920,735	1,028,452
EBIT Margin			NM					NM	NM	NM	NM	NM	68%	75%	75%
growth y/y (%)			NM	676%	180%	135%	80%	NM	NM	NM	NM	NM	NM	NM	NM
Depreciation and Amortization	-	-	1,000	250	250	250	250	1,000	1,000	1,000	1,000	1,000	1,000	1,000	1,000
Interest income	(8)	(73)													
Other income (expense)	7	(96)	(988)	500	500.0	500.0	500.0	2,000	1,500	1,500	8,000	8,000	8,000	8,000	8,000
Change in fair value of embedded derivative	6	18													
Loss on extinguishment of conv. Notes	-	(1,671)													
Total Other (expense) income, net	5.0	(1,822)													
Deemed dividend															
Income Before Taxes	(1,811)	(5,276)	(28,634)	(11,955)	(11,955)	(18,198)	(18,198)	(60,307)	(69,511)	(54,098)	(78,918)	(60,682)	494,776	928,735	1,036,452
Income Tax Provision	-	-	-	250	250	250	250	1,000	1,000	-	-	-	168,224	315,770	352,394
Effective Tax Rate	0.0%	0.0%	NM	-2.1%	-2.1%	-1.4%	-1.4%	NA	NA	0%	0%	0%	34%	34%	34%
Foreign currency adjustment	8.0	19.0													
<b>Net Income (loss) Attributable to Common</b>	(1,803)	(5,257)	(28,634)	(12,205)	(12,205)	(18,448)	(18,448)	(61,307)	(70,511)	(54,098)	(78,918)	(60,681)	326,552	612,965	684,059
Net income to common per share	\$ (0.50)	(1.44)	(2.01)	(0.49)	(0.49)	(0.73)	(0.73)	(2.45)	(2.57)	(1.94)	(2.82)	(2.10)	11.26	21.07	23.43
Basic avg. number of shares used in computing net income	3,643	3,673	13,749	24,903	25,003	25,103	25,203	25,053	25,453	25,853	27,953	28,053	28,153	28,253	28,353
Diluted avg. number of shares used in computing net income	3,643	3,673	14,223	24,903	25,003	25,103	25,203	25,053	27,453	27,853	28,953	28,897	28,997	29,097	29,197
Share Growth				2300.0	100.0	100.0	100.0			100.0	100.0	100.0	100.0	100.0	100.0
Basic vs. Diluted															
												24280.12			
<b>Key Ratios (GAAP unless noted)</b>															
Gross Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	90.0%	50.0%	60.0%	90.0%	92.0%	92.0%
R&D (% Total Rev.)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	58.9%	7.8%	5.0%	4.5%
SG&A (% Total Rev.)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	49.7%	14.0%	12.0%	13.0%
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-48.6%	68.2%	75.0%	74.5%
Net Income Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-42.9%	45.8%	49.9%	49.6%
<b>Revenue Growth</b>															
Growth Yr/Yr	NM	1500%	NM	-78%	-85%	-85%	-85%	NM	NM	NM	NM	NM	405.0%	72.0%	12.4%
Growth Q/Q	NM			-85%	0%	0%	0%								
<b>SG&amp;A Growth</b>															
Growth Yr/Yr	NM	233%	349%	264%	77%	65%	17%	65%	10%	10%	10%	300%			
Growth Q/Q	NM			-22%	0%	50%	0%								
<b>R&amp;D Growth</b>															
Growth Yr/Yr	NM	64%	689%	982%	218%	157%	104%	190%	15%	-30%	75%	20%			
Growth Q/Q	NM			36%	0%	50%	0%								

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William Blair intends to seek investment banking compensation in the next three months from Avalanche Biotechnologies, Inc.

Within the past 12 months William Blair has provided or is providing investment banking services to or has an investment services relationship with Avalanche Biotechnologies, Inc.

Additional information is available upon request.

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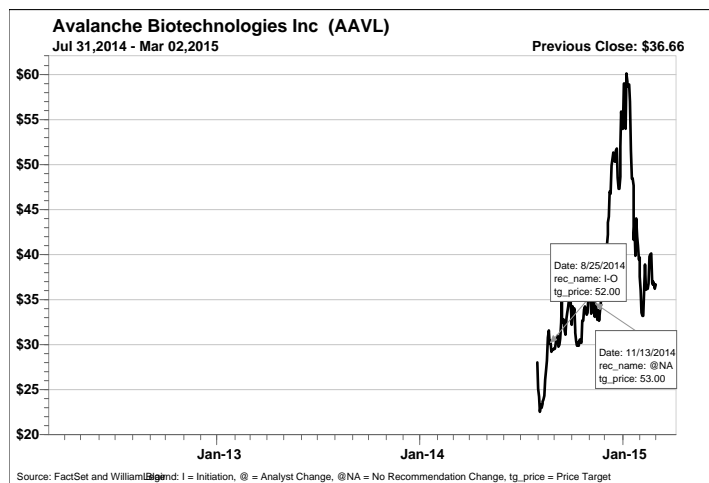
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DOW JONES: 18,096.90

S&P 500: 2,098.53

NASDAQ: 4,967.14



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Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	65	Outperform (Buy)	16
Market Perform (Hold)	32	Market Perform (Hold)	2
Underperform (Sell)	2	Underperform (Sell)	0

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