William Blair

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Avalanche Biotechnologies, Inc.

First-Quarter Earnings in Line; All Eyes on AVA-101 Phase IIa and 36-Month Phase I Follow-Up Data by Midyear

- Before the markets open Wednesday, May 13, Avalanche Biotechnologies reported first-quarter earnings. We view the company's financials as a non-event ahead of 12-month top-line data from the company's Phase IIa trial as well as 36-month follow-up from the Phase I trial with AVA-101, the company's lead candidate to treat wet age-related macular degeneration. Phase IIa data will also trigger a period of time where Regeneron (REGN $473.78) will be able to look at the data set and determine if it would like to make a partnership offer to Avalanche, an offer that Avalanche management is not obligated to accept. We believe this sets up an interesting decision point for the company as management may feel that a better offer will be gained following a Phase IIb study. We note that in 2014, Ophthotech (OPHT $52.52) received a deal totaling over $1 billion in development and commercial milestones as well as royalties from Novartis (NVS $102.83) in an outside-the-U.S. deal for Fovista, which included a $200 million upfront payment.

- The Phase IIa enrolled 32 patients and should provide a more significant proof of concept over the relatively small (albeit placebo controlled) Phase I study. We continue to believe a successful Phase IIa result will include meeting the primary endpoint of safety and tolerability over 52 weeks with an average of 1 Lucentis rescue injection or less in the AVA-101 group (versus 3-6 in the placebo group) along with stability or minimal letter improvement in visual acuity, although the company did see an increase of 6.3 letters in the high dose in the Phase I trial. Differences in visual acuity improvement between the Phase I and Phase IIa will make cross-trial comparison difficult with better visual acuity and in general a healthier patient population in the Phase IIa study, in addition to the inclusion of long-term Lucentis users who have already received a benefit in visual acuity from Lucentis, an improvement that usually plateaus over longer-term use. In addition, we would expect continued safety and tolerability as well as a reduction in Lucentis rescue injections in the Phase I 36-month follow-up; however, the study was not designed for long-term follow-up efficacy measurements and, again, could provide data that is difficult to compare to other long-term studies in the indication.

- At the recent Association for Research in Vision and Ophthalmology (ARVO) conference, Dr. Elizabeth Rakoczy from the University of Western Australia, a principal investigator for Avalanche's clinical trials, presented additional data from the AVA-101 Phase I study. The presentation included new analyses of systemic VEGF levels and immune response in the six treated patients (three high dose and three low dose). Levels of serum VEGF in the small data set look in line with if not improved to Lucentis and Eylea, suggesting a lack of systemic exposure and a likely continued strong systemic side-effect profile. While one patient developed an antibody response, it was deemed transient and not associated with a safety or efficacy signal. Results suggest AVA-101 should continue to look safe; however, given the limited number of patients treated to date, the Phase IIa results will be more informative than the subgroup analysis at ARVO.

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

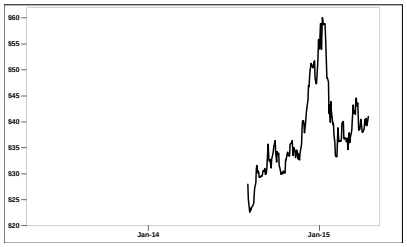
Stock Rating:	**Outperform**
Company Profile:	**Aggressive Growth**
Price Target:	$53.00
Symbol:	AAVL (NASDAQ)
Price:	$34.61 (52-Wk.: $22-$62)
Market Value (mil.):	$865
Fiscal Year End:	December
Long-Term EPS Growth Rate:	
Dividend/Yield:	None

	2014A	2015E	2016E
Estimates			
EPS Q1	$-0.45	A$-0.38	NA
Q2	$-2.27	$-0.40	NA
Q3	$-0.50	$-0.57	NA
Q4	$-0.46	$-0.56	NA
FY	$-2.01	$-1.92	$-2.53
CY		$-1.92	$-2.53
Sales (mil.)	1	1	0
Valuation			
FY P/E	NM	NM	NM
CY P/E		NM	NM

Trading Data (FactSet)	
Shares Outstanding (mil.)	23
Float (mil.)	15
Average Daily Volume	358,990

Financial Data (FactSet)	
Book Value Per Share (MRQ)	6.6
Return on Equity (TTM)	-38.4

Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

- In addition, in the quarter, Avalanche continued to broaden its development platform by announcing a partnership with the University of Washington to develop products for the treatment of red-green color blindness using the company's Ocular BioFactory platform, now called AVA-322 and AVA-323. Avalanche's approach will build on the research conducted by Dr. Jay Neitz and Dr. Maureen Neitz of the University of Washington's Department of Ophthalmology. In the University of Washington published study, two adult male squirrel monkeys showed that, after therapy with a gene encoding the M3 pigment, they could tell blue-green from gray. The authors monitored the time course of function, as well, and found that the M3-cone pigment appeared about 20 weeks after injection, and it was associated with the change in visual function (Mancuso et al. *Nature* 2009). At the company's analyst event also conducted in the first quarter, Dr. Neitz stated that the company expects the pipeline products to enter the clinic within two years, and we believe that single-gene mutations that result in ocular dysfunctions represent diseases where AAV gene therapies could be particularly effective; the doctors stated that they are seeing efficacy using Avalanche's proprietary vectors within one month.

- For first-quarter financials, Avalanche reported a net loss of $9.5 million, or $0.38 per share, above both consensus of a loss of $10 million or $0.40 per share and our estimate of a loss of $12 million or $0.49 per share. Research and development costs were $5.6 million, lower than both consensus of $7.7 million and our estimate of $9.85 million. SG&A costs were $4.1 million, higher than both consensus of $3 million and our estimate of $2.6 million. As of the end of the first quarter, the company had $290.1 million in cash and cash equivalents including the net proceeds of roughly $130.6 million from a secondary offering conducted in January. The company's cash position places it in a strong position to achieve significant pipeline developments. We note that the company is planning to initiate a Phase IIb trial for AVA-101 with roughly 120 patients in the second half of 2015.

- We continue to rate Avalanche shares Outperform and maintain our $53 price target, which is derived from conservative assumptions on AVA-101 alone. We believe that the company continues to lead in the ocular gene therapy field with an impressive management team. While Avalanche has treated only a handful of patients to date with AVA-101, the proof-of-concept results are impressive in previously heavy Lucentis use. In addition to the disruptive potential of AVA-101, the company's pipeline includes multiple other compounds that may be transformational for other ocular diseases.

Exhibit 1
Phase IIa Baseline Characteristics Compared to Phase I

	Phase I	Phase IIa
Age (years)	79 (71-86)	79.5 (62-95)
Baseline BCVA (ETDRS letters)	36.5 (28-56)	63 (35-78)
Baseline center point thickness (μm)	549 (193-1094)	332.5 (179-816)
Number treatment naïve (n/N)	0/8	4/32
Previous anti-VEGF injections (for non-naïve)	11.5 (1-29)	10.5 (1-25)
Time since diagnosis (months)	49.2 (2-65)	16.2 (0-85)

all values median (range)

Source: Company reports

Exhibit 2
Avalanche Biotechnologies, Inc.
First Quarter Results and Estimates

	AAVL Q1 15A	WB Q1 15E	Consensus Q1 15E	Q/Q Growth	Variance (%)
($ in thousands except EPS)					
Total Revenue	**$ 203.0**	**$ 30.0**	**$ 200.0**	**NM**	**NM**
R&D	$ 5,621.0	$ 9,846.0	$ 7,700.0	-22%	-42.9%
G&A	$ 4,143.0	$ 2,639.0	$ 3,000.0	23%	57.0%
Operating Income (loss)	$ (9,561.0)	$(12,455.0)	$ (10,600.0)	NM	NM
Net Income	$ (9,509.0)	$(12,205.0)	$ (10,000.0)	NM	NM
EPS	**$ (0.38)**	**$ (0.49)**	**$ (0.40)**	**NM**	**NM**

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

Valuation
We rate Avalanche shares 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 reach about $1.1 billion domestically. Our estimates assume a 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 diabetic macular edema (DME) and retinal vein occlusion (RVO) and no revenue from outside the United States, which is likely conservative.

Risks
Risks to shares of Avalanche are similar to those of other development-stage therapeutics companies. The company faces clinical, manufacturing, and regulatory risks on 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 model is included on the following page.

William Blair

Avalanche Biotech, Inc.
Earnings Model
5/13/15
($ in thousands except EPS data)

Rating: Outperform
Company Profile: Aggressive Growth
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	2012(A)	2013(A)	2014(A)	Q1(A)	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	203	203	203	203	812	-	-	-	-	-	-	-
Grant revenue	-	-	-												
Total Revenue	30	480	572	203	203	203	203	812	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															
Cost of Goods Sold	-	-	-	-	-	-	-	-	-	-	-	56,534	71,375	98,212	110,438
Gross Profit	30	480	572	203	203	203	203	812	120	-	-	84,802	642,373	1,129,434	1,270,035
R&D	1,310	2,151	16,976	5,621	6,400	9,846	9,846	49,230	56,615	39,630	69,353	83,224	55,672	61,382	62,121
Growth			*689%*	*518%*	*107%*	*71%*	*36%*	*190%*	*15%*	*-30%*	*75%*	*20%*	*15%*	*15%*	*15%*
SG&A	536	1,783	7,998	4,143	3,959	4,619	4,619	13,197	14,516	15,968	17,565	70,259.23	99,925	147,318	179,461
Growth			*30%*	*471%*	*165%*	*93%*	*37%*	*65%*	*10%*	*10%*	*10%*	*300%*	*42%*	*47%*	*22%*
Total Operating Expenses	1,846	3,934	24,974	9,764	10,359	14,465	14,465	49,053	71,131	55,598	86,918	153,483	155,597	208,700	241,583
growth			535%	497%	126%	78%	36%	96%	45%	-22%	56%	77%	1%	34%	16%
Operating Income	(1,816)	(3,454)	(24,402)	(9,561)	(10,156)	(14,262)	(14,262)	(48,241)	(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*	*495%*	*128%*	*80%*	*37%*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*	*NM*
Interest income	(8)	(73)													
Other income (expense)	7	(96)	(988)	52	52.0	52.0	52.0	208	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)	(9,509)	(10,104)	(14,210)	(14,210)	(48,033)	(69,511)	(54,098)	(78,918)	(60,682)	494,776	928,735	1,036,452
Income Tax Provision	-	-	-					-	-	-	-	-	168,224	315,770	352,394
Effective Tax Rate	0.0%	0.0%	NM	0.0%	0.0%	0.0%	0.0%	NA	NA	0%	0%	0%	34%	34%	34%
Foreign currency adjustment	8.0	19.0													
Net Income (loss) Attributable to Common	(1,803)	(5,257)	(28,634)	(9,509)	(10,104)	(14,210)	(14,210)	(48,033)	(69,511)	(54,098)	(78,918)	(60,681)	326,552	612,965	684,059
Net income to common per share	$ (0.50)	(1.44)	(2.01)	(0.38)	(0.40)	(0.57)	(0.56)	(1.92)	(2.53)	(1.94)	(2.82)	(2.10)	11.27	21.08	23.44
Basic avg. number of shares used in computing net income	3,643	3,673	13,749	24,887	24,987	25,087	25,187	25,037	25,437	25,837	27,937	28,037	28,137	28,237	28,337
Diluted avg. number of shares used in computing net income	3,643	3,673	14,223	24,887	24,987	25,087	25,187	25,037	27,437	27,837	27,937	28,881	28,981	29,081	29,181

IMPORTANT DISCLOSURES

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William Blair or an affiliate expects to receive or intends to seek compensation for investment banking services from Avalanche Biotechnologies, Inc. within the next three months.

William Blair or an affiliate received compensation for investment banking services from Avalanche Biotechnologies, Inc. within the last 12 months. Avalanche Biotechnologies, Inc. is or was, within the last 12 months, an investment banking client of William Blair & Company and/or one or more of its affiliates.

Additional information is available upon request.

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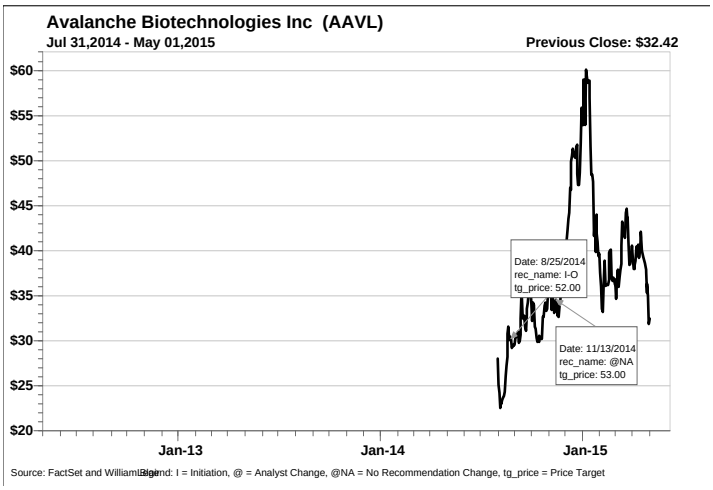
DOW JONES: 18,068.23
S&P 500: 2,099.12
NASDAQ: 4,976.19



Current Rating Distribution (as of 04/30/15)

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	65	Outperform (Buy)	14
Market Perform (Hold)	32	Market Perform (Hold)	2
Underperform (Sell)	2	Underperform (Sell)	0

*Percentage of companies in each rating category that are investment banking clients, defined as companies for which William Blair has received compensation for investment banking services within the past 12 months.

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