William Blair

Avalanche Biotechnologies, Inc.

September 11, 2014

Second-Quarter Earnings Highlight Continued Development of AVA-101 and Growing Pipeline, Maintain Outperform Rating

- Before the open Thursday, Avalanche Biotechnologies reported second-quarter results—its first earnings filing since becoming a public company on July 31. Avalanche raised net proceeds of approximately $106.3 million, with use of proceeds focused on advancing the development of its lead product, AVA-101, as well as several earlier-stage products coming out of the company's ocular gene therapy platform. In general, we view the company's quarter-to-quarter financial results as a non-event outside of the development of AVA-101 and the development partnership between the company and Regeneron (REGN $348.88), which we believe are the current focus of investors. The two companies have a larger partnership that included an up-front payment and up to $640 million in development milestones.

- AVA-101 is under development in a Phase IIa clinical trial for wet age-related macular degeneration (wet AMD), a disease with an approximately $3 billion domestic and larger international market. AVA-101 is being developed as a single subretinal injection that is intended to provide a safe and effective treatment for wet AMD without the need for recurring injections that classify the current market leaders in therapy, thereby making it a "functional cure." AVA-101 is a gene therapy comprised of an adeno-associated virus (AAV2) vector, which contains a gene encoding sFLT-1, a naturally occurring VEGF inhibitor. Recall that in the company's Phase I trial, treated subjects on average required only 0.33 Lucentis injections over 52 weeks compared to 3 Lucentis injections in the control group, while 4/6 patients treated required no Lucentis injections at all, which we believe suggests a significant treatment effect. While we would characterize the clinical experience of AVA-101 as early, given the mechanism of action (VEGF inhibition), we believe the Phase IIa (N=32) results, which are due in the first half of 2015, could be a significant catalyst for shares.

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

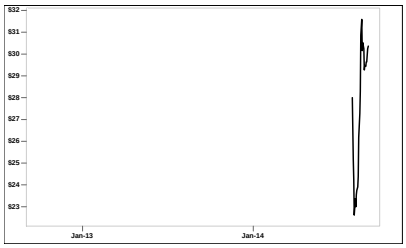
Stock Rating:	**Outperform**
Company Profile:	**Aggressive Growth**
Price Target:	$52.00
Symbol:	AAVL (NASDAQ)
Price:	$31.80 (52-Wk.: $22-$32)
Market Value (mil.):	$657
Fiscal Year End:	December
Long-Term EPS Growth Rate:	
Dividend/Yield:	None

		2013A	2014E	2015E
Estimates				
EPS	Q1	NA	A$-0.45	NA
	Q2	NA	A$-2.27	NA
	Q3	NA	$-0.16	NA
	Q4	NA	$-0.18	NA
	FY	$-1.44	$-1.00	$-1.35
	CY		$-1.00	$-1.35
Valuation				
FY P/E		NM	NM	NM
CY P/E			NM	NM

Trading Data (FactSet)	
Shares Outstanding (mil.)	15
Float (mil.)	11
Average Daily Volume	327,312

Financial Data (FactSet)	
Long-Term Debt/Total Capital (MRQ)	0.0
Book Value Per Share (MRQ)	7.1
Return on Equity (TTM)	0.0

Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

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- In the near term, we expect preliminary results presented from a Sanofi (SNY $55.56)/Applied Genetic Technologies Corporation (AGTC $17.96) competitor to AVA-101, AAV2-sFLT01, at the 2014 Annual Meeting of the Retina Society in Philadelphia on September 12. According to clinicaltrials.gov, AAV2-sFLT01 is involved in an ongoing Phase I study in 34 patients. The study involves four arms: 2×10^8 vector genomes AAV2-sFLT01, 2×10^9 vector genomes AAV2-sFLT01, 6×10^9 vector genomes AAV2-sFLT01, and 2×10^{10} vector genomes AAV2-sFLT01. The primary outcome measures are the maximum tolerated dose (MTD) of a single intravitreal injection at week 52 and the number of treatment emergent adverse events at week 52 and up to four years in an extended follow-up period. We expect limited efficacy and potential inflammation issues coming from this program given the preclinical data to date, with the most-glaring difference between the two programs being the intravitreal injection of Sanofi's AAV2-sFLT01 versus the subretinal injection of Avalanche's AVA-101, which preclinically is potentially 100-1,000 times more effective given the thickness of the inner limiting membrane of the retina. In Avalanche's Phase Ia study, efficacy was viewed at 10^{10} (low-dose) and 10^{11} (high-dose) vector genomes of AVA-101, toward the high range of the MTD study with AAV-sFLT01. We also expect additional presentations from several wet-AMD companies at the upcoming 2014 Annual Meeting of the American Academy of Ophthalmology in Chicago from October 18 through 21. However, as we view AVA-101 as the leading therapy that may be a "curative" option in wet-AMD, we believe more advanced programs are likely prone to a similar disruption in the market, which would be the outcome of a successful development of AVA-101.

- Regarding second-quarter financials, the company reported revenue of $0.1 million for the quarter with R&D and SG&A expenses of $3.1 million and $1.5 million, respectively, above our estimates of $1.3 million and $1 million. The company reported a net loss of $8.3 million for the second quarter, above our estimate of a loss of $1.6 million. As of June 30, 2014, the company reported cash of $52.4 million. However this cash number does not include the $106 million in proceeds from the company's IPO.

- Lastly in the quarter, the company made two significant hires to its management team: Samuel B. Barone, M.D. as chief medical officer (June 2014) and Linda C. Bain as chief financial officer (April 2014). Dr. Barone has a wide range of experience in the field of ophthalmology and has most recently served as a medical officer in the office of cellular, tissue, and gene therapies for the FDA. Ms. Bain previously served in various roles at the gene therapy company bluebird bio (BLUE $36.91). We view both hires as strong additions to an already impressive management team.

- Based on our belief that AVA-101 represents a potentially game-changing therapy for a growing ocular disease market and our confidence in the management team's ability to execute on its goals, we continue to rate shares of Avalanche Biotechnologies Outperform.

Exhibit 1
Avalanche Biotechnologies, Inc.
Timeline and Events

Date	Product	Event	Description/Comments
2014			
Sept 11 to 14	AAV2-sFLT01	Conference	Retina Society Meeting in Philadelphia, PA (Sanofi/AGTC data)
Oct 18 to 21	Wet AMD candidates	Conference	American Academy of Opthalmology Annual Meeting in Chicago, IL
2015			
H1 2015	AVA-101	Clinical	Phase IIa readout (N=32)
H2 2015	AVA-101	Clinical	Initiate Phase IIb study (N=120)
H2 2015	AVA-101	Regulatory	IND filing
2015	AVA-201	Clinical	Preclinical studies
2015	AAV2-sFLT01	Clinical	Phase I readout of Sanofi/AGTC product
2016			
2016	Multiple	Regulatory	IND filings of other product candidates
2016/2017	AVA-101	Clinical	Potential initiation of pivotal program

Sources: Company reports and William Blair & Company, L.L.C. estimates

Valuation

We rate shares of Avalanche Outperform with a price target of $52, based on a net present value of the company's lead development program, AVA-101. 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 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 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	$ 45.86
Cash Per Share					$ 5.77
NPV Value					$1,210,322
NPV Value Per Share					$ 51.64

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 around their 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.

William Blair

Avalanche Biotech, Inc.
Earnings Model
9/11/14
($ in thousands except EPS data)

Rating: Outperform
Company Profile: Aggressive Growth
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	2012(A)	2013(A)	Q1(A)	Q2(A)	Q3(E)	Q4(E)	2014(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	-	-	-	135	-	-	135	-	-	-	-	-	-	-	-
License revenue	30	480	30	-	30	30	90	120	-	-	-	-	-	-	-
Grant revenue	-	-	-	-	-	-	-								
Total Revenue	30	480	30	135	30	30	225	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															
Cost of Goods Sold	-	-	-	-	-	-	-	-	-	-	-	56,534	71,375	98,212	110,438
Gross Profit	30	480	30	135	30	30	225	120	120	-	-	84,802	642,373	1,129,434	1,270,035
R&D	1,310	2,151	910	3,094	3,200	3,500	10,704	31,042	35,698	24,988	43,730	52,476	55,672	61,382	62,121
Growth			-	-	-	-	398%	190%	15%	-30%	75%	20%	15%	15%	15%
SG&A	536	1,783	726	1,494	1,600	1,700	5,520	9,108	10,019	11,021	12,123	48,490.99	99,925	147,318	179,461
Growth							30%	65%	10%	10%	10%	300%	106%	47%	22%
Total Operating Expenses	1,846	3,934	1,636	4,588	4,800	5,200	16,224	40,150	45,717	36,009	55,853	100,967	155,597	208,700	241,583
growth			NA	NA	NA	NA	312%	147%	14%	-21%	55%	81%	54%	34%	16%
Operating Income	(1,816)	(3,454)	(1,606)	(4,453)	(4,770)	(5,170)	(15,999)	(40,030)	(45,597)	(36,009)	(55,853)	(16,165)	486,776	920,735	1,028,452
EBIT Margin							NM	NM	NM	NM	NM	NM	68%	75%	75%
growth y/y (%)			NA	NA	NA	NA	NM	NM	NM	NM	NM	NM	NM	NM	NM
Depreciation and Amortization	-	-	-	250	250	250	1,000	1,000	1,000	1,000	1,000	1,000	1,000	1,000	1,000
Interest income	(8)	(73)	(14)												
Other income (expense)	7	(96)	(43)	(641.0)	750.0	750.0	3,000	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)	(57)												
Deemed dividend				(3230.0)											
Income Before Taxes	(1,811)	(5,276)	(1,663)	(8,324)	(4,020)	(4,420)	(18,427)	(38,030)	(44,097)	(34,509)	(47,853)	(8,165)	494,776	928,735	1,036,452
Income Tax Provision	-	-	-	-	225	225	450	1,000	1,000	-	-	-	168,224	315,770	352,394
Effective Tax Rate	0.0%	0.0%	NA	NA	NA	NA	NM	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)	(1,663)	(8,324)	(4,245)	(4,645)	(18,877)	(39,030)	(45,097)	(34,509)	(47,853)	(8,165)	326,552	612,965	684,059
Net income to common per share	$ (0.50)	$ (1.44)	$ (0.45)	(2.27)	(0.16)	(0.18)	(1.00)	(1.35)	(1.40)	(1.06)	(1.47)	(0.24)	9.69	18.14	20.19
Basic avg. number of shares used in computing net income	3,643	3,673	3,673	2,250	22,257	22,357	15,621	24,857	26,007	26,407	32,641	32,741	32,841	32,941	33,041
Diluted avg. number of shares used in computing net income	3,643	3,673	3,673	3,673	26,391	26,491	18,852	28,991	32,141	32,541	32,641	33,585	33,685	33,785	33,885
Key Ratios (GAAP unless noted)															
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	37.1%	7.8%	5.0%	4.5%
SG&A (% Total Rev.)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	34.3%	14.0%	12.0%	13.0%
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-11.4%	68.2%	75.0%	74.5%
Net Income Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-5.8%	45.8%	49.9%	49.6%
Revenue Growth															
Growth Yr/Yr	NM	1500%	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	405.0%	72.0%	12.4%
Growth Q/Q	NM		NM	NM	NM	NM									
SG&A Growth															
Growth Yr/Yr	NM	233%	NM	NM	NM	NM	210%	65%	10%	10%	10%	300%			
Growth Q/Q	NM		NM	NM	NM	NM									
R&D Growth															
Growth Yr/Yr	NM	64%	NM	NM	NM	NM	398%	190%	15%	-30%	75%	20%			
Growth Q/Q	NM		NM	NM	NM	NM									

IMPORTANT DISCLOSURES

William Blair was a manager or co-manager of a public offering of equity securities for Avalanche Biotechnologies, Inc. within the prior 12 months.

William Blair is a market maker in the security of Avalanche Biotechnologies, Inc. and may have a long or short position.

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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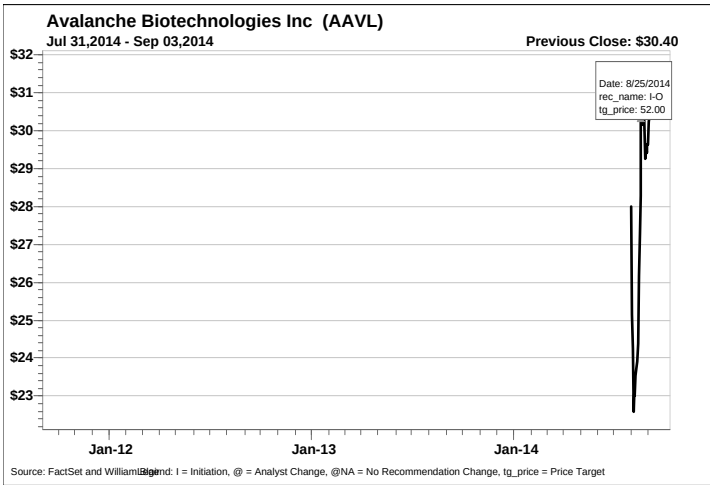
DOW JONES: 17,068.71
S&P 500: 1,995.69
NASDAQ: 4,586.52



Current Rating Distribution (as of 08/31/14)

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	66	Outperform (Buy)	16
Market Perform (Hold)	31	Market Perform (Hold)	3
Underperform (Sell)	1	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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