William Blair

Avalanche Biotechnologies, Inc.

November 12, 2014

Third-Quarter Earnings Suggest Company is Well-Positioned to Build Disruptive Ophthalmology Franchise

- Before the markets opened Wednesday, November 12, Avalanche released its third-quarter earnings. The company completed its IPO in August and raised net proceeds of about $106.5 million. With the IPO, collaborator Regeneron Pharmaceuticals (REGN $398.36) received a $10 million private placement of common stock, which is part of a larger partnership that included an $8 million up-front payment and up to $640 million in development milestones that covers up to eight distinct therapeutic targets plus royalties and reimbursement of research costs. In addition, Avalanche has the option to share up to 35% of profits and development costs for two of the targets and Regeneron has a right to see the Phase IIb wet-AMD data for AVA-101 and make an offer for the asset; however, Avalanche is not obligated to accept any terms given by Regeneron and may still partner AVA-101 away from the company if Avalanche chooses to partner the therapy post-Phase IIb data.

- AVA-101 is being developed as a single subretinal injection that is intended to be a "functional cure" for the disease, which affects as many as 15 million individuals in the United States. AVA-101 is a gene therapy composed of an adeno-associated virus (AAV2) vector platform which contains a gene encoding sFLT-1, an anti-VEGF protein. After attending the 2014 American Academy of Ophthalmology Annual meeting, 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 market in wet-AMD. The intravitreal injection approach used by competitor Sanofi's (SNY $46.12) AAV2-sFLT01 compound in the Phase I study results presented at the meeting showed only 4 out of 11 patients in the "expected responder" group with increased efficacy. In addition, improvements in response were defined in the trial as a 20% improvement in retinal fluid and no additional therapy. 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 out of the seven patients tested with a mean of +8.7 letters in the low dose and +6.3 in the high dose with no need for any rescue injections. The company expects the results of their Phase IIa study in 32 patients (2:1 active versus placebo) to report top-line data in mid-2015.

- The strong intellectual property portfolio of AVA-101 and the company's pipeline products includes 12 issued patents and 27 pending applications, including composition of matter, method of clinical use, and method of manufacture with coverage from issued patents through 2020 and coverage of pending applications through 2033 (upon issuance). The company's two disclosed pipeline products include AVA-201, a one-time intravitreal injection for the prevention of wet-AMD under pre-clinical development, and AVA-311, one of the partner compounds with Regeneron, a one-time intravitreal injection of the RS1 gene for the treatment of juvenile X-linked retinoschisis (XLRS).

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

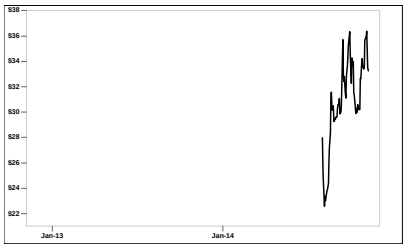
Stock Rating:	**Outperform**
Company Profile:	**Aggressive Growth**
Price Target:	$53.00
Symbol:	AAVL (NASDAQ)
Price:	$33.65 (52-Wk.: $22-$37)
Market Value (mil.):	$737
Fiscal Year End:	December
Long-Term EPS Growth Rate:	
Dividend/Yield:	None

		2013A	2014E	2015E
Estimates				
EPS	Q1	NA	A$-0.45	NA
	Q2	NA	A$-2.27	NA
	Q3	NA	A$-0.50	NA
	Q4	NA	$-0.47	NA
	FY	$-1.44	$-2.06	$-2.57
	CY		$-2.06	$-2.57
Valuation				
FY P/E		NM	NM	NM
CY P/E			NM	NM

Trading Data (FactSet)	
Shares Outstanding (mil.)	4
Float (mil.)	12
Average Daily Volume	209,104

Financial Data (FactSet)	
Long-Term Debt/Total Capital (MRQ)	0.0
Book Value Per Share (MRQ)	-4.5
Return on Equity (TTM)	0.0

Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

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- For third-quarter financials, the company reported total revenue of $204,000 due to licensing revenues, above our estimate of $30,000, but below consensus of $500,000. R&D expenses were $5.75 million, above our estimate of $3.2 million and consensus of $3.5 million; SG&A expenses were $2.4 million, above our estimate of $1.6 million and consensus of $1.4 million. Net income was reported as a loss of $8.3 million or $0.50 per share, below our estimates of a loss of $4.2 million or $0.16 per share and consensus of a loss of $4.1 million or $0.20 per share. We have included reported earnings, our estimates, and consensus estimates in exhibit 1. The company is well capitalized with $165.3 million in pro forma cash as of the end of the third quarter. In addition, in September, the company added Roman Rubio, M.D. as senior vice president and head of translational medicine, further strengthening the company's management team. We believe the team being assembled at Avalanche is impressive as Dr. Rubio's past experience includes several years at Genentech in various positions including Global Head of Ophthalmology where he was responsible for overseeing medical affairs and clinical development of Lucentis. Dr. Rubio follows another impressive recent hire. In July, Dr. Samuel Barone joined Avalanche as the company's chief medical officer. His past work includes time at the FDA where he worked as medical officer in the Office of Cellular, Tissue, and Gene Therapies and is also an active vitreoretinal surgeon. While we had previously viewed AVA-101 and the company's ocular gene therapy platform as a disruptive force in ophthalmology, the addition of top talent with significant eye care clinical development experience reinforces our enthusiasm for the opportunity being addressed by the company.

- We continue to rate Avalanche Biotechnologies as Outperform. We believe that the company continues to lead in the disruptive 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" of almost all patients treated with wet-AMD after prior heavy Lucentis use. In addition to the disruptive potential of AVA-101, we also believe that its 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.
Third Quarter Results and Estimates

	AAVL Q3 14A	WB Q3 14E	Consensus Q3 14E	Q/Q Growth	Variance (%)
($ in thousands except EPS)					
Total Revenue	**$ 204.0**	**$ 30.0**	**$ 500.0**	**NM**	**NM**
R&D	$ 5,746.0	$ 3,200.0	$ 3,500.0	86%	79.6%
G&A	$ 2,398.0	$ 1,600.0	$ 1,400.0	61%	49.9%
Operating Income (loss)	$ (7,940.0)	$ (4,770.0)	$ (4,400.0)	NM	NM
Net Income	$ (8,256.0)	$ (4,245.0)	$ (4,100.0)	NM	NM
EPS	**$ (0.50)**	**$ (0.16)**	**$ (0.20)**	**NM**	**NM**

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

Valuation

We rate shares of Avalanche as Outperform with a price target of $53, which is up from $52, based on a rolling of our valuation forward half a year and our 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 be 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 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 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.

Our model is included on the following page.

William Blair

Avalanche Biotech, Inc.
Earnings Model
11/12/14
($ in thousands except EPS data)

Rating: Outperform
Company Profile: Aggressive Growth
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	2012(A)	2013(A)	Q1(A)	Q2(A)	Q3(A)	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	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
License revenue	30	480	30	135	204	200	569	120	-	-	-	-	-	-	-
Grant revenue	-	-	-	-	-	-	-								
Total Revenue	30	480	30	135	204	200	569	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	204	200	569	120	120	-	-	84,802	642,373	1,129,434	1,270,035
R&D	1,310	2,151	910	3,094	5,746	6,500	16,250	47,125	54,194	37,936	66,387	79,665	55,672	61,382	62,121
Growth			-	-	-	-	655%	190%	15%	-30%	75%	20%	15%	15%	15%
SG&A	536	1,783	726	1,494	2,398	3,000	7,618	12,570	13,827	15,209	16,730	66,921.08	99,925	147,318	179,461
Growth							30%	65%	10%	10%	10%	300%	49%	47%	22%
Total Operating Expenses	1,846	3,934	1,636	4,588	8,144	9,500	23,868	59,695	68,020	53,145	83,118	146,586	155,597	208,700	241,583
growth			NA	NA	NA	NA	507%	150%	14%	-22%	56%	76%	6%	34%	16%
Operating Income	(1,816)	(3,454)	(1,606)	(4,453)	(7,940)	(9,300)	(23,299)	(59,575)	(67,900)	(53,145)	(83,118)	(61,784)	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)	(316.0)	(200.0)	(1,200)	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)	(8,256)	(9,500)	(27,743)	(57,575)	(66,400)	(51,645)	(75,118)	(53,784)	494,776	928,735	1,036,452
Income Tax Provision	-	-	-	-	-	-	-	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)	(8,256)	(9,500)	(27,743)	(58,575)	(67,400)	(51,645)	(75,118)	(53,784)	326,552	612,965	684,059
Net income to common per share	$ (0.50)	$ (1.44)	$ (0.45)	(2.27)	(0.50)	(0.47)	(2.06)	(2.57)	(2.60)	(1.96)	(2.84)	(1.96)	11.88	22.21	24.70
Basic avg. number of shares used in computing net income	3,643	3,673	3,673	2,250	16,394	20,300	12,981	22,800	23,950	24,350	26,450	26,550	26,650	26,750	26,850
Diluted avg. number of shares used in computing net income	3,643	3,673	3,673	3,673	16,394	20,300	13,456	22,800	25,950	26,350	26,450	27,394	27,494	27,594	27,694
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	56.4%	7.8%	5.0%	4.5%
SG&A (% Total Rev.)	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	47.3%	14.0%	12.0%	13.0%
Operating Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-43.7%	68.2%	75.0%	74.5%
Net Income Margin	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	NM	-38.1%	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	327%	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	655%	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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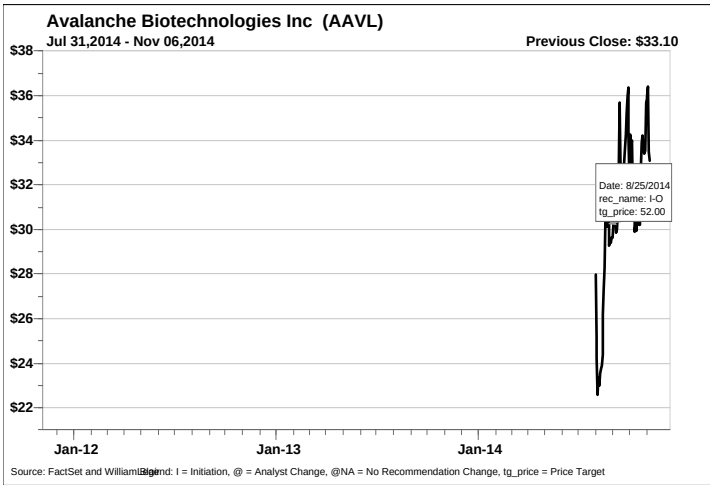
DOW JONES: 17,614.90
S&P 500: 2,039.68
NASDAQ: 4,660.56



Current Rating Distribution (as of 10/31/14)

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	65	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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