COMPANY NOTE

Estimate Change

USA | Healthcare | Biotechnology

May 13, 2015

Jefferies

Avalanche Biotechnologies (AAVL)

Q1: Awaiting AVA-101 Phase IIa Wet AMD Data In Mid-2015

BUY

Price target $51.00

Price $33.88

EQUITY RESEARCH AMERICAS

Key Takeaway

The company issued a press release earlier today on its Q1 financials. We expect the company will announce topline data from its ongoing placebo-controlled Phase IIa study evaluating AVA-101 in 32 patients w/ treatment experienced wet AMD. We think the PIIa data will be a near-term catalyst and w/ shares driven by the magnitude of the treatment differences btwn the two arms.

AVA-101 Phase II Data Is Imminent: We expect AVA-101 PIIa data should be disclosed in the June/July timeframe. Recall, the company has enrolled 32 wet age-related macular degeneration (AMD) patients who are treatment experienced and have received a median of 10.5 or a mean of 11 inj prior to enrolling in the trial. The patients in the trial also are considered healthier than the prior Phase Ib trial w/ better baseline vision and a thinner retina. The company is expected to report the 12-month data from the Phase IIa trial, and we expect the outcome of the data could drive shares.

What Do We Expect From PIIa Data? The PIIa trial will report injection frequency of Lucentis rescue injections in both groups, and based on our conversations w/ key experts, we would expect a win would be > 60% inj reduction in the AVA-101 arm vs control. On retina thickness as measured by OCT, recall, the PIb trial reported reductions in retina thickness of 200 microns. Recall, also, that the Genzyme trial reported a 155 micron reduction over baseline. We would expect >155 micron reduction over baseline to support moving to a PIIb trial. Lastly, on visual acuity, based on expert feedback we know that there's going to be some variability in this endpt given the heterogenous pt population therefore we would expect a several letter gain.

Q1 Financials: AAVL reported Q1'15 rev of $0.2M and GAAP EPS of ($0.38) v. JEF est: ($0.41) and cons. of ($0.40). Cash and equivs were $290.1M end of Q1.

Valuation/Risks

Our $51 PT is DCF-based. Risks include clinical, manufacturing, competitive, regulatory, and commercial.

Financial Summary	
Net Debt (MM):	($290.1)
Long-Term Debt (MM):	$0.0
Cash & ST Invest. (MM):	$290.1
Cash/Share:	$11.63
Cash (MM):	$290.1

Market Data	
52 Week Range:	$62.48 - $22.00
Total Entprs. Value (MM):	$265.5
Market Cap. (MM):	$555.6
Shares Out. (MM):	16.4
Float (MM):	19.0
Avg. Daily Vol.:	321,923

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USD	Prev.	2013A	Prev.	2014A	Prev.	2015E	Prev.	2016E
Rev. (MM)	--	0.5	--	0.6	--	0.8	--	0.0
EV/Rev		NM		NM		NM		
EPS								
Mar	--	--	--	(0.11)	*(0.41)*	(0.38)A	--	--
Jun	--	--	--	(2.27)	*(0.41)*	(0.40)	--	--
Sep	--	--	--	(0.50)	*(0.41)*	(0.43)	--	--
Dec	--	--	--	(0.46)	*(0.43)*	(0.46)	--	--
FY Dec	--	(1.45)	--	(2.46)	*(1.69)*	(1.68)	*(1.87)*	(1.89)
FY P/E		NM		NM		NM		NM

Price Performance

Valuation

We arrive at our $51 PT (v. $46 previously) based on a DCF valuation model, which assumes a WACC of 14%, terminal growth rate of 0% and outstanding shares of 27.2 million, driven by sales of AVA-101. We assume market entry for AVA-101 for wet AMD in 2020 assuming positive data from a Phase III program. We estimate peak sales of $4.4 billion in the U.S. by 2026 for ophthalmic diseases including wet AMD, DME, and CRVO on an unadjusted-basis. If we apply a 65% risk discount to reflect the clinical risk of the AVA-101 program, we estimate $1.5 billion in U.S. sales by 2026. Additionally, we expect $118 million in royalty revenue for the same indications in 2026 using a 65% risk-discount.

At this time, we do not model AVA-201 for wAMD or AVA-311 for juvenile X-linked retinoschisis (XLRS), and these products represent upside. We have included $135 million risk-adjusted peak sales for AVA-322/-323 for color blindness. We expect R&D expense to reach $26 million by YE 2015, increasing to $61 million by 2026 as AAVL ramps up clinical development of AVA-101 into DME and CRVO, AVA-201 and AVA-311. We expect SG&A expense to be $18 million by YE 2015, increasing to $58 million by 2026. We include $25 million in launch expenses for AVA-101 in 2021, risk-adjusted by 70%.

Exhibit 1: DCF sensitivity analysis

Discount rate	Equity Value	Price/Share
10.0%	$1,930.6	$71.77
12.0%	$1,618.5	$60.17
14.0%	**$1,367.1**	**$50.83**
16.0%	$1,163.6	$43.26
18.0%	$998.1	$37.11

Source: Jefferies estimates

Risks

Clinical Failure: As with all companies in biotechnology and pharmaceuticals developing treatments of the future, a clinical failure can lead to delays in approval or possibly discontinuation of programs.

Regulatory Failure: The FDA could determine the Biologic Licensing Application is inadequate for AVA-101 for wet AMD and could delay approval. Furthermore, to date the FDA has not approved any gene therapy products for any indication. There is therefore no historical precedence for approval of such products, and the FDA may deem AAVL's clinical package for AVA-101 as insufficient for approval. Any delays in approval timelines could impact our earnings estimates, price target, and/or rating.

Commercial Failure: We currently estimate peak sales of $4.4 billion in the U.S. by 2026 for ophthalmic diseases including wet AMD, DME, and CRVO on an unadjusted-basis. If we apply a 65% risk discount to reflect the clinical risk of the AVA-101 program, we estimate $1.5 billion in U.S. sales by 2026. Additionally, we expect $117 million in royalty revenue for the same indications in 2026 using an 70% risk-discount. Our estimates may rely on the success of the company/partners to receive drug reimbursement from private/public payors.

Manufacturing Risks: AAVL relies on its proprietary baculovirus expression system (BVES) to produce its gene therapy products, including AVA-101. AAVL believes its BVES is efficient and scalable, with production yields up to 100x greater than those obtained by

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conventional AAV production system, allowing it to manufacture commercial grade production for large markets as wet AMD. If AVA-101 is approved, AAVL will need a consistent and reliable process, while limiting contamination risks, for manufacturing these candidates on large-scale for the approved patient population. Any supply or manufacturing disruption could negatively impact AVA-101 supply and sales.

Competitive Risks: Other companies are rapidly developing gene therapy product candidates in various stages of clinical development for ophthalmic diseases including wet AMD that may compete with AVA-101. If any of these product candidates have an improved therapeutic profile over AVA-101 and is approved, AVA-101's growth trajectory in the marketplace, even if approved, could be adversely impacted.

Financing Risks: We expect AAVL to have adequate cash through the majority of AVA-101's clinical development, and we model an $80 million equity raise on 2 million shares in 2019. AAVL may need additional dilutive financing to fund the potential U.S. launch of AVA-101 and its R&D programs in additional indications.

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Exhibit 2: AAVL Income Statement

Avalanche Biotechnologies, Inc.

Quarterly Income Statement

(All values in $MM except EPS and average shares)

	2012A	2013E	2014A	2015E					2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
	FY	FY	FY	1QA	2QE	3QE	4QE	FY	FY	FY	FY	FY	FY	FY	FY	FY
Revenue:																
AVA101- U.S.	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	43.5	112.8	420.6	845.7
AVA101- ROW royalty	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	10.2	49.3	94.2
AVA322/323	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	50.0	75.0
License and collaboration revenues	0.0	0.5	0.6	0.2	0.2	0.2	0.2	0.8	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Total revenue, net	**0.0**	**0.5**	**0.6**	**0.2**	**0.2**	**0.2**	**0.2**	**0.8**	**0.0**	**0.0**	**0.0**	**0.0**	**43.5**	**123.0**	**519.9**	**1,014.9**
Costs and expenses:																
Cost of goods sold	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	10.9	30.8	130.0	253.7
Research & development	13	2.2	17.0	5.6	6.2	6.8	7.5	26.1	31.3	36.0	39.6	43.6	45.7	48.0	50.4	52.9
Selling, general & administrative	0.5	1.8	8.0	4.1	4.4	4.6	4.8	17.9	19.6	21.6	23.8	25.0	26.2	45.0	47.3	49.6
Total operating expenses	**1.8**	**3.9**	**25.0**	**9.8**	**10.5**	**11.4**	**12.3**	**43.9**	**50.9**	**57.6**	**63.4**	**68.5**	**82.8**	**123.8**	**227.7**	**356.3**
Income (loss) from operations	**(1.8)**	**(3.5)**	**(24.4)**	**(9.6)**	**(10.3)**	**(11.2)**	**(12.1)**	**(43.1)**	**(50.9)**	**(57.6)**	**(63.4)**	**(68.5)**	**(39.3)**	**(0.8)**	**292.2**	**658.6**
Other income (expense):																
Miscellaneous (expense) income	(0.0)	(1.9)	(4.2)	0.1	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Interest income	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Interest expense	0.0	0.0	(0.0)	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net profit (loss) before income taxes	**(1.8)**	**(5.3)**	**(28.6)**	**(9.5)**	**(10.3)**	**(11.2)**	**(12.1)**	**(43.1)**	**(50.9)**	**(57.6)**	**(63.4)**	**(68.5)**	**(39.3)**	**(0.8)**	**292.2**	**658.6**
Income tax expense (benefit)										0.0	0.0	0.0	0.0	0.0	29.2	230.5
Income tax (%)										*0.0%*	*0.0%*	*0.0%*	*0.0%*	*0.0%*	*10.0%*	*35.0%*
Net Income (GAAP)	**(1.8)**	**(5.3)**	**(28.6)**	**(9.5)**	**(10.3)**	**(11.2)**	**(12.1)**	**(43.1)**	**(50.9)**	**(57.6)**	**(63.4)**	**(68.5)**	**(39.3)**	**(0.8)**	**15.0**	**428.1**
Adjusted Items (Non-GAAP)																
Stock options	0.0	0.0	0.0	0.6	0.7	0.8	0.9	3.0	4.0	5.0	6.0	7.0	14.0	16.0	18.0	20.0
Depreciation and amortization expense	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
Net Income (Non-GAAP)	**(1.8)**	**(5.3)**	**(28.6)**	**(8.9)**	**(9.6)**	**(10.4)**	**(11.2)**	**(40.1)**	**(46.9)**	**(52.6)**	**(57.4)**	**(61.5)**	**(25.3)**	**15.2**	**33.0**	**448.1**
EPS, GAAP																
Basic	(0.50)	(1.45)	(2.46)	(0.38)	(0.40)	(0.43)	(0.46)	(1.68)	(1.89)	(2.12)	(2.31)	(2.31)	(1.31)	(0.03)	0.49	13.84
Diluted	**$ (0.50)**	**$ (1.45)**	**$ (2.46)**	**$ (0.38)**	**$ (0.40)**	**$ (0.43)**	**$ (0.46)**	**$ (1.68)**	**$ (1.89)**	**$ (2.12)**	**$ (2.31)**	**$ (2.31)**	**$ (1.31)**	**$ (0.03)**	**$ 0.49**	**$ 13.84**
Weighted average share- Basic	3.6	3.7	11.7	24.9	25.6	25.9	26.1	25.6	26.9	27.2	27.4	29.7	30.0	30.3	30.6	30.9
Weighted average share- Diluted	3.6	3.7	11.7	24.9	25.6	25.9	26.1	25.6	26.9	27.2	27.4	29.7	30.0	30.3	30.6	30.9

Source: Jefferies, company data

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Exhibit 3: AAVL DCF analysis

Avalanche Biotechnologies

Discounted Cash Flow Analysis

(All values in $MM)	*2012A*	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Sales	*0.0*	0.5	0.6	0.8	0.0	0.0	0.0	0.0	43.5	123.0	519.9	1,014.9
Operating Expenses	*1.8*	3.9	25.0	43.9	50.9	57.6	63.4	68.5	82.8	123.8	227.7	356.3
EBIT	*(1.8)*	(3.5)	(24.4)	(43.1)	(50.9)	(57.6)	(63.4)	(68.5)	(39.3)	(0.8)	292.2	658.6
(-): Taxes	*0.0*	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	29.2	230.5
EBIAT	*(1.8)*	(3.5)	(24.4)	(43.1)	(50.9)	(57.6)	(63.4)	(68.5)	(39.3)	(0.8)	263.0	428.1
(+):Depreciation	*0.0*	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0	0.0
(+):FAS-123 Options	*0.0*	0.0	0.0	3.0	4.0	5.0	6.0	7.0	14.0	16.0	18.0	20.0
(-): Capital expenditures	*0.0*	0.1	0.1	0.1	0.1	0.1	0.1	0.1	0.1	0.1	0.1	0.1
Unlevered free cash flow	***(1.8)***	***(3.5)***	***(24.5)***	***(40.2)***	***(47.0)***	***(52.7)***	***(57.4)***	***(61.6)***	***(25.4)***	***15.2***	***280.9***	***448.0***

Source: Jefferies estimates, company data

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Company Description

Avalanche Biotechnologies, Inc., a clinical-stage biotechnology company, focuses on discovering and developing novel gene therapies for the treatment of ophthalmic diseases based on its Ocular BioFactory platform. Its lead product candidate includes AVA-101, which is in a Phase I/IIa trial for the treatment of wet age-related macular degeneration (AMD). The company is also developing AVA-201, an anti-vascular endothelial growth factor gene therapy product candidate for the prevention of wet AMD; and AVA-311 that is in preclinical studies for the treatment of juvenile X-linked retinoschisis, a rare genetic disease of the retina with no approved therapy. Avalanche Biotechnologies, Inc. has a collaboration agreement with Regeneron Pharmaceuticals, Inc. research, develop, and commercialize gene therapy products. The company was founded in 2006 and is headquartered in Menlo Park, California.

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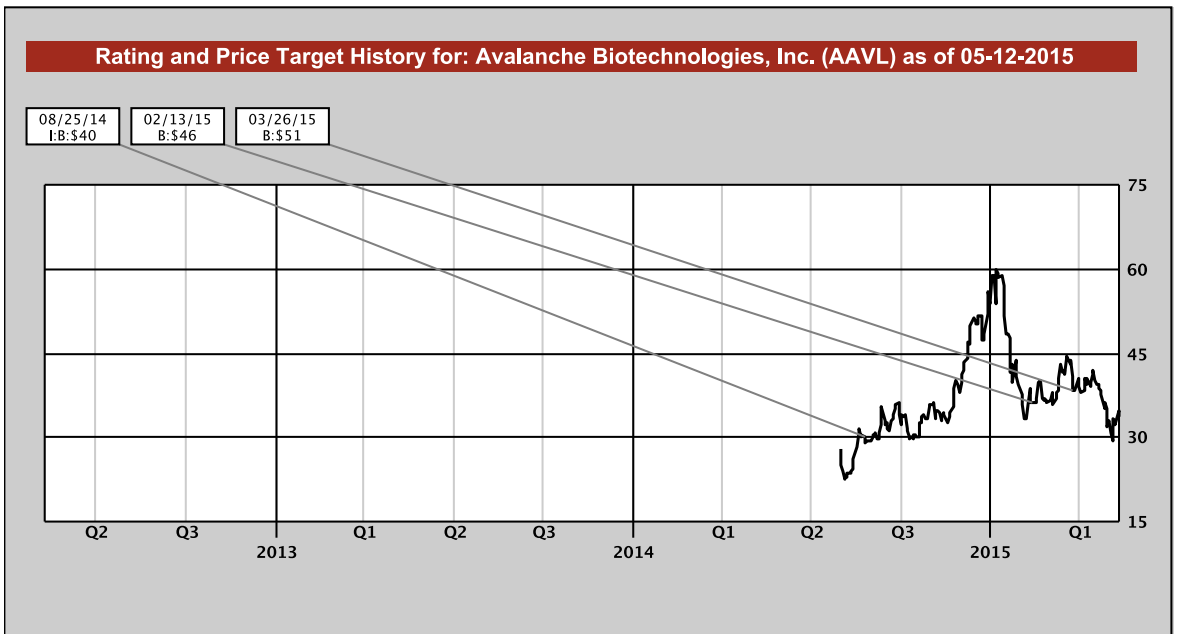
Other Companies Mentioned in This Report

- Avalanche Biotechnologies, Inc. (AAVL: $33.88, BUY)



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