

## Avalanche Biotechnologies (AAVL)

Overweight

### AAVL Q1 Earnings And Model Update

#### CONCLUSION

AAVL released 4Q14 earnings this week disclosing a cash and cash equivalents balance of \$290M as of March 31 following a ~\$130M raise in January. Ahead of the Phase 2a trial readout in mid-2015, we continue to remain enthusiastic for the potential of AVA-101 to become a meaningful product, although we also see it as the first of what may be a series of iterations and optimizations as the company moves along the path of becoming an ophthalmology gene therapy platform leader. Behind AVA-101, the company continues to optimize vectors and advance its diverse pipeline. Reiterate OW and PT of \$52.

- **Framing The AVA-101 Data:** As we think about the upcoming Phase 2a dataset, we don't think the bar is set at the level seen in the Phase 1, although that would certainly be welcome. We note that specialists will be looking particularly for OCT imaging benefits similar to Lucentis. Additionally, visual acuity will be important as well as average reduction in injection frequency relative to the control arm, with meaningful improvements seen in at least subsets of patients for a commercially viable product. We note the baseline patient population is healthier than the prior patients, with thinner retinas, higher visual acuity and fewer anti-VEGF injections at baseline, which appears to generally represent the broader AMD population.
- **But Don't Forget The Broader Ophthalmology Platform:** We note that AAVL is developing several unique pipeline products: an intravitreal product, AVA-201, and more recently, the color blindness gene therapy program in-licensed from University of Washington as well as the partnered programs with Regeneron. In addition to taking pressure off the p2a data release, these programs highlight the continued evolution of vector designs and the potential broad applicability of the platform to target various tissues through both subretinal and intravitreal injection.

#### RISKS TO ACHIEVEMENT OF PRICE TARGET

AAVL gene therapy candidates may fail to achieve target development steps.

#### COMPANY DESCRIPTION

AAVL is a pioneer in gene therapy, targeting ophthalmic indications.

PRICE: US\$35.95

TARGET: US\$52.00

DCF thru 2024, 11% discount rate, 6.5% terminal growth rate

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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	—	US\$52.00
FY15E Rev (mil)	US\$12.0	US\$9.2
FY16E Rev (mil)	—	US\$12.0
FY15E EPS	US\$(1.54)	US\$(1.61)
FY16E EPS	—	US\$(1.35)

52-Week High / Low	US\$62.48 / US\$22.00
Shares Out (mil)	22.6
Market Cap. (mil)	US\$812.5
Avg Daily Vol (000)	321
Book Value/Share	US\$11.20
Net Cash Per Share	US\$8.10
Debt to Total Capital	0.0%
Div (ann)	US\$0.00
Fiscal Year End	Dec

#### Price Performance - 1 Year



Source: Bloomberg

YEAR	REVENUE (US\$ m)						EARNINGS PER SHARE (US\$)					
	Mar	Jun	Sep	Dec	FY	FY RM	Mar	Jun	Sep	Dec	FY	FY P/E
2014A	0.0	0.1	0.2	0.2	0.6	1,354.1x	(0.40)	(0.54)	(0.50)	(0.46)	(2.46)	NM
2015E	0.2A	3.0	3.0	3.0	9.2	88.3x	(0.38)A	(0.35)	(0.42)	(0.46)	(1.61)	NM
2016E	—	—	—	—	12.0	67.7x	—	—	—	—	(1.35)	NM

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AAVL Upcoming Catalysts				
Program	Indication	Type	Event	Expected Timing
AVA-101	wet AMD	Clinical	P2a data, AVA-101 subretinal	Mid-2015
		Commercial	REGN 45d exclusivity to discuss licensing	Mid-2015
		Regulatory	File IND	Mid-2015
		Clinical	Start P2b study in U.S.	2H15
AVA-201	wet AMD	Clinical	File IND	2016
AVA-311	XLRS	Clinical	File IND	2016
AVA-322, 323	Color blindness	Clinical	File IND	2016

Source: PJC and Company reports

AAVL DCF Analysis (2014-2024 CF Estimates)	
Discounted Cash Flow (DCF) Analysis	
Assumed Discount Rate (%)	11.0%
Terminal Growth Rate (%)	6.5%
Implied Terminal Year FCF Multiple	23.7x
NPV of FCF	\$1,406
Cash/equiv	\$163
<b>Price Target</b>	<b>\$52</b>
Target valuation	\$1,569
Shares Outstanding 2017E (million)	30.0

Source: PJC estimates

AAVL Valuation Sensitivity Analysis					
Terminal Growth	Discount Rate				
		10%	11%	12%	13%
	4.5%	\$46	\$35	\$27	\$22
	5.5%	\$57	\$42	\$32	\$25
	6.5%	\$75	<b>\$52</b>	\$38	\$29
	7.5%	\$106	\$68	\$48	\$35
	8.5%	\$180	\$97	\$62	\$44

Source: PJC estimates

AAVL Potential Upside From Current Levels					
Terminal Growth	Discount Rate				
		10.0%	11.0%	12.0%	13.0%
	4.5%	12%	(15%)	(34%)	(47%)
	5.5%	40%	3%	(22%)	(39%)
	6.5%	82%	<b>27%</b>	(6%)	(29%)
	7.5%	159%	67%	16%	(14%)
	8.5%	339%	137%	52%	7%

Source: Company Reports and Piper Jaffray.

AAVL QUARTERLY P&L											
	2013A	1Q14A	2Q14A	3Q14A	4Q14E	2014A	1Q15A	2Q15E	3Q15E	4Q15A	2015E
Product Revenue/Royalty	\$0	\$0	\$0	\$0	\$0	0.0	\$0	\$0	\$0	\$0	0
Funding/milestones	0.5	0.0	0.1	0.2	0.2	0.6	0.2	3	3	3	9.2
<b>Total Revenue</b>	<b>\$0.5</b>	<b>\$0.03</b>	<b>\$0.14</b>	<b>\$0.20</b>	<b>\$0.20</b>	<b>\$1</b>	<b>\$0</b>	<b>\$3</b>	<b>\$3</b>	<b>\$3</b>	<b>\$9</b>
COGS	0	0	0	0	0	0	0	0	0	0	0
<i>% product sales</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>
R&D	2	1	3	6	7	17	6	9	10	11	36
<i>% revenue</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>
SG&A	2	1	1	2	3	8	4	3	4	4	15
<i>% revenue</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>
<b>Total operating expenses</b>	<b>4</b>	<b>2</b>	<b>5</b>	<b>8</b>	<b>11</b>	<b>25</b>	<b>10</b>	<b>12</b>	<b>14</b>	<b>15</b>	<b>51</b>
Interest/other	-2	0	-1	0	0	-1	0	0	0	0	1
Pretax income	(\$5)	(\$2)	(\$5)	(\$8)	(\$10)	(\$25)	(\$10)	(\$9)	(\$11)	(\$12)	(\$41)
Taxes	0	0	0	0	0	0	0	0	0	0	0
<i>Tax rate</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0</i>
<b>Net income</b>	<b>-5</b>	<b>-2</b>	<b>-5</b>	<b>-8</b>	<b>-10</b>	<b>-29</b>	<b>-10</b>	<b>-9</b>	<b>-11</b>	<b>-12</b>	<b>-40.759</b>
<b>EPS</b>	<b>(\$1.39)</b>	<b>(\$0.40)</b>	<b>(\$0.54)</b>	<b>(\$0.50)</b>	<b>(\$0.46)</b>	<b>(\$2.46)</b>	<b>(\$0.38)</b>	<b>(\$0.35)</b>	<b>(\$0.42)</b>	<b>(\$0.46)</b>	<b>(\$1.61)</b>
Shares	4	4	9	16	23	12	25	25	25	26	25

Sources: Company Reports and Piper Jaffray

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Current disclosure information for this company can be found at <http://www.piperjaffray.com/researchdisclosures>.

Proprietary to Piper Jaffray & Co. May 14, 2015

AAVL ANNUAL PRODUCT MODEL												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
<b>AVA-101 Status</b>	P1	P2a	P2b	P2b	P3	P3	Filing	Launch	Market	Market	Market	Market
<b>AVA-201 Status</b>	N/A	Preclin	Preclin	P1	P2	P2	P3	P3	Filing	Launch	Market	Market
<b>AVA-311 Status</b>	N/A	Preclin	Preclin	P1	P2/3	P2/3	P2/3	Filing	Launch	Market	Market	Market
<b>Wet AMD eyes/yr, U.S. (000s)</b>	<b>100</b>	<b>100</b>	<b>101</b>	<b>102</b>	<b>103</b>	<b>104</b>	<b>105</b>	<b>106</b>	<b>107</b>	<b>108</b>	<b>109</b>	<b>110</b>
<i>High frequency injection eyes</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>	<i>25%</i>
<i>High frequency 101/201 penetr.</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>8%</i>	<i>20%</i>	<i>35%</i>	<i>45%</i>
<i>Mid frequency injection eyes</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>	<i>55%</i>
<i>Mid frequency 101/201 penetr.</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>1%</i>	<i>2%</i>	<i>4%</i>	<i>8%</i>	<i>12%</i>
AVA-101/201 injections (000s)	0	0	0	0	0	0	0	0	3	8	14	20
AVA-101/201 price/Rx (000s)	\$25	\$25	\$25	\$25	\$25	\$25	\$25	\$25	\$25	\$25	\$25	\$25
<b>AVA-101/201 Sales (mm)</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$7</b>	<b>\$83</b>	<b>\$195</b>	<b>\$360</b>	<b>\$493</b>
OUS AVA-101/201 Sales (mm)	0	0	0	0	0	0	0	0	0	50	100	200
<i>% Avalanche Royalty on OUS</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>	<i>15%</i>
<b>AVA-101/201 Royalty, OUS (mm)</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$8</b>	<b>\$15</b>	<b>\$30</b>
<b>XLRS patients, W/W</b>	20	20	20	20	20	20	20	20	20	20	20	20
<i>AVA-311 penetration</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>1%</i>	<i>3%</i>	<i>4%</i>	<i>6%</i>
AVA-311 price/Rx (000s)	\$150	\$150	\$150	\$150	\$150	\$150	\$150	\$150	\$150	\$150	\$150	\$150
<b>AVA-311 sales</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$30</b>	<b>\$90</b>	<b>\$120</b>	<b>\$180</b>
<i>% Avalanche Royalty on AVA-311</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>10%</i>	<i>11%</i>
<b>AVA-311 Royalty</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$0</b>	<b>\$3</b>	<b>\$9</b>	<b>\$12</b>	<b>\$20</b>

Sources: Company Reports and Piper Jaffray

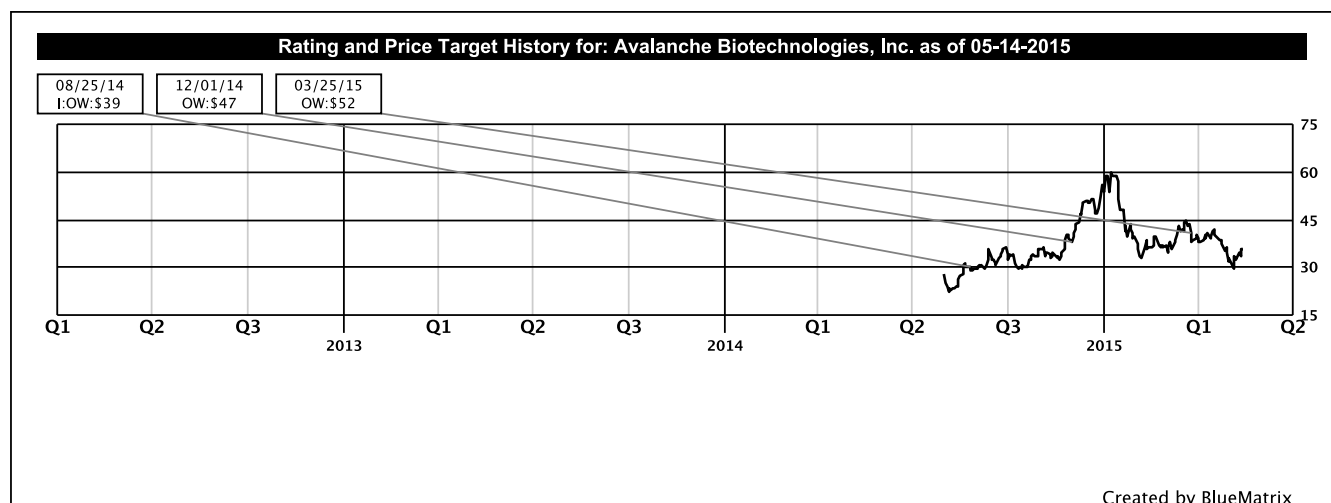
AAVL ANNUAL P&L												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	0.6	9.2	12	12	12	12	12	12	12	12	12
<b>Total Revenue</b>	<b>\$0.5</b>	<b>\$1</b>	<b>\$9</b>	<b>\$12</b>	<b>\$12</b>	<b>\$12</b>	<b>\$12</b>	<b>\$19</b>	<b>\$98</b>	<b>\$223</b>	<b>\$399</b>	<b>\$555</b>
COGS	0	0	0	0	0	0	0	1	8	19	36	49
% product sales	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%
R&D	2	17	36	40	60	80	80	65	75	80	85	90
% revenue	N/M	N/M	N/M	333%	500%	667%	667%	337%	76%	36%	21%	16%
SG&A	2	8	15	8	12	15	25	95	115	140	150	165
% revenue	N/M	N/M	N/M	67%	100%	125%	208%	492%	117%	63%	38%	30%
<b>Total operating expenses</b>	<b>4</b>	<b>25</b>	<b>51</b>	<b>48</b>	<b>72</b>	<b>95</b>	<b>105</b>	<b>161</b>	<b>198</b>	<b>239</b>	<b>271</b>	<b>304</b>
Interest/other	-2	-1	1	1	1	1	1	1	1	2	4	6
Pretax income	(\$5)	(\$25)	(\$41)	(\$35)	(\$59)	(\$82)	(\$92)	(\$140)	(\$99)	(\$14)	\$132	\$256
Taxes	0	0	0	0	0	0	0	0	0	-4	33	64
Tax rate	0%	0%	0%	0%	0%	0%	0%	0%	0%	25%	25%	25%
<b>Net income</b>	<b>-5</b>	<b>-29</b>	<b>-41</b>	<b>-35</b>	<b>-59</b>	<b>-82</b>	<b>-92</b>	<b>-140</b>	<b>-99</b>	<b>-11</b>	<b>99</b>	<b>192</b>
<b>EPS</b>	<b>(\$1.39)</b>	<b>(\$2.46)</b>	<b>(\$1.61)</b>	<b>(\$1.35)</b>	<b>(\$1.97)</b>	<b>(\$2.56)</b>	<b>(\$2.42)</b>	<b>(\$3.51)</b>	<b>(\$2.36)</b>	<b>(\$0.23)</b>	<b>\$2.06</b>	<b>\$3.85</b>
Shares	4	12	25	26	30	32	38	40	42	46	48	50

Sources: Company Reports and Piper Jaffray

AAVL STATEMENT OF CASH FLOWS												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Net Income	-5	-29	-41	-35	-59	-82	-92	-140	-99	-11	99	192
Depreciation	0	0	0	0	0	0	0	0	0	0	0	0
Stock based comp	1	6	7	10	12	14	16	18	20	22	24	25
Other	3	3	0	0	0	0	0	0	0	0	0	0
Change in NWC	0	4	-2	-4	-5	-10	-15	-15	-15	-15	-15	0
<b>Cash from operations</b>	<b>-2</b>	<b>-15</b>	<b>-36</b>	<b>-29</b>	<b>-52</b>	<b>-78</b>	<b>-91</b>	<b>-137</b>	<b>-94</b>	<b>-4</b>	<b>108</b>	<b>217</b>
PP&E	0	0	0	-2	-2	-2	-2	-2	-2	-2	-2	-2
<b>Free cash flow</b>	<b>-2</b>	<b>-15</b>	<b>-36</b>	<b>-31</b>	<b>-54</b>	<b>-80</b>	<b>-93</b>	<b>-139</b>	<b>-96</b>	<b>-6</b>	<b>106</b>	<b>215</b>
<b>Financing Cash</b>	<b>2</b>	<b>174</b>	<b>139</b>	<b>0</b>	<b>125</b>	<b>0</b>	<b>175</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>
Cash start	0	1	159	262	231	302	222	304	165	69	63	169
<b>Cash end</b>	<b>1</b>	<b>159</b>	<b>262</b>	<b>231</b>	<b>302</b>	<b>222</b>	<b>304</b>	<b>165</b>	<b>69</b>	<b>63</b>	<b>169</b>	<b>384</b>

Sources: Company Reports and Piper Jaffray

## IMPORTANT RESEARCH DISCLOSURES



Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written during the past three years.

## Legend:

I: Initiating Coverage  
 R: Resuming Coverage  
 T: Transferring Coverage  
 D: Discontinuing Coverage  
 S: Suspending Coverage  
 OW: Overweight  
 N: Neutral  
 UW: Underweight  
 NA: Not Available  
 UR: Under Review

Distribution of Ratings/IB Services Piper Jaffray				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
<b>BUY [OW]</b>	380	61.59	97	25.53
<b>HOLD [N]</b>	222	35.98	14	6.31
<b>SELL [UW]</b>	15	2.43	0	0.00

Note: Distribution of Ratings/IB Services shows the number of companies currently in each rating category from which Piper Jaffray and its affiliates received compensation for investment banking services within the past 12 months. FINRA rules require disclosure of which ratings most closely correspond with "buy," "hold," and "sell" recommendations. Piper Jaffray ratings are not the equivalent of buy, hold or sell, but instead represent recommended relative weightings. Nevertheless, Overweight corresponds most closely with buy, Neutral with hold and Underweight with sell. See Stock Rating definitions below.

**Analyst Certification — Joshua E. Schimmer, MD, Sr Research Analyst**

— Steven P. Breazzano, Ph.D., Research Analyst

— Jerry Yang, Ph.D., Research Analyst

The views expressed in this report accurately reflect my personal views about the subject company and the subject security. In addition, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendations or views contained in this report.

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Piper Jaffray research analysts receive compensation that is based, in part, on overall firm revenues, which include investment banking revenues.

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- **Overweight (OW):** Anticipated to outperform relative to the median of the group of stocks covered by the analyst.
- **Neutral (N):** Anticipated to perform in line relative to the median of the group of stocks covered by the analyst.
- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

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