

Avalanche Biotechnologies (AAVL)

Overweight

Framing The AVA-101 P2a Data - Setting The Record Straight; Reiterate OW

CONCLUSION

We've had a high volume of incoming calls from investors trying to frame the upcoming P2a data of AAVL's gene therapy product (AVA-101) for treatment of wet AMD. Many investors are, in our opinion, focusing on the wrong metrics. We think the most important datapoint is the percent of patients with a meaningful reduction in Lucentis injections vs. baseline, since that seems to be the key driver of adoption. Many investors are looking for reductions in visual acuity and/or OCT retinal thickness, but since most patients are already controlled at baseline, this is a less realistic objective.

- **Who are these patients?** 32 patients were randomized 2:1 to AVA-101 vs control. 90.6% of patients were on anti-VEGF injections at baseline (mean of 10 injections pre-enrollment).
- **What's the design?** All patients received Lucentis at baseline and 4 weeks. Over the year of follow up, patients in both arms receive additional Lucentis injections on a PRN basis following standard re-injection criteria.
- **What's a good outcome?** Most of the patients were stable on anti-VEGF therapy heading into the trial, so the goal of AVA-101 is to preserve the effects and wean patients off injections. As such, visual acuity and retinal thickness should have little room for added improvement. A "responder" is one with meaningfully reduced Lucentis requirement (ideally to 0-1/yr, but potentially higher if achieved safely). A ~40% response rate would be a good starting point for expectations.
- **What about VA and OCT benefits?** Only 3 treatment naive patients (i.e. uncontrolled, active disease) are enrolled; too few to draw conclusions regarding VA or OCT impact. There may be other patients with active baseline disease despite prior anti-VEGF therapy, so there might be a signal of benefit in them as well, but also small numbers.
- **What will the stock do?** Investor expectations are diverse, and there are too many variables to consider all scenarios (definition of "responder", % responder, and safety). At the extremes, if there is no benefit or a safety signal (highly unlikely), AAVL shares would probably decline substantially whereas if the P1a data are reproduced, upside would probably be considerable. We expect a healthy debate over the rest of the potential outcomes, but keep in mind retina specialists enjoy performing invasive procedures and, if safe, will accept more modest gains than many investors might have thought. Overall we see a favorable risk reward ahead of the data, in part because we see a strong emerging AAVL pipeline in ophtho gene therapy and a vector evolution strategy that investors are overlooking.

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.

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

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Avalanche Biotechnologies, Inc.

PRICE: US\$37.52

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\$9.2
FY16E Rev (mil)	—	US\$12.0
FY15E EPS	—	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\$848.0
Avg Daily Vol (000)	332
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

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
		Clinical	Report long-term follow up of P1a data	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
Price Target	\$52
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	\$52	\$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%	25%	(5%)	(26%)	(42%)
	5.5%	55%	14%	(13%)	(33%)
	6.5%	102%	41%	4%	(21%)
	7.5%	188%	85%	29%	(5%)
	8.5%	387%	163%	69%	18%

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
Total Revenue	\$0.5	\$0.03	\$0.14	\$0.20	\$0.20	\$1	\$0	\$3	\$3	\$3	\$9
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>
Total operating expenses	4	2	5	8	11	25	10	12	14	15	51
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>
Net income	-5	-2	-5	-8	-10	-29	-10	-9	-11	-12	-40.759
EPS	(\$1.39)	(\$0.40)	(\$0.54)	(\$0.50)	(\$0.46)	(\$2.46)	(\$0.38)	(\$0.35)	(\$0.42)	(\$0.46)	(\$1.61)
Shares	4	4	9	16	23	12	25	25	25	26	25

Sources: Company Reports and Piper Jaffray

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Proprietary to Piper Jaffray & Co. June 2, 2015

AAVL ANNUAL PRODUCT MODEL												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
AVA-101 Status	P1	P2a	P2b	P2b	P3	P3	Filing	Launch	Market	Market	Market	Market
AVA-201 Status	N/A	Preclin	Preclin	P1	P2	P2	P3	P3	Filing	Launch	Market	Market
AVA-311 Status	N/A	Preclin	Preclin	P1	P2/3	P2/3	P2/3	Filing	Launch	Market	Market	Market
Wet AMD eyes/yr, U.S. (000s)	100	100	101	102	103	104	105	106	107	108	109	110
<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
AVA-101/201 Sales (mm)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$7	\$83	\$195	\$360	\$493
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>
AVA-101/201 Royalty, OUS (mm)	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$8	\$15	\$30
XLRS patients, W/W	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
AVA-311 sales	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$30	\$90	\$120	\$180
<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>
AVA-311 Royalty	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$3	\$9	\$12	\$20

Sources: Company Reports and Piper Jaffray

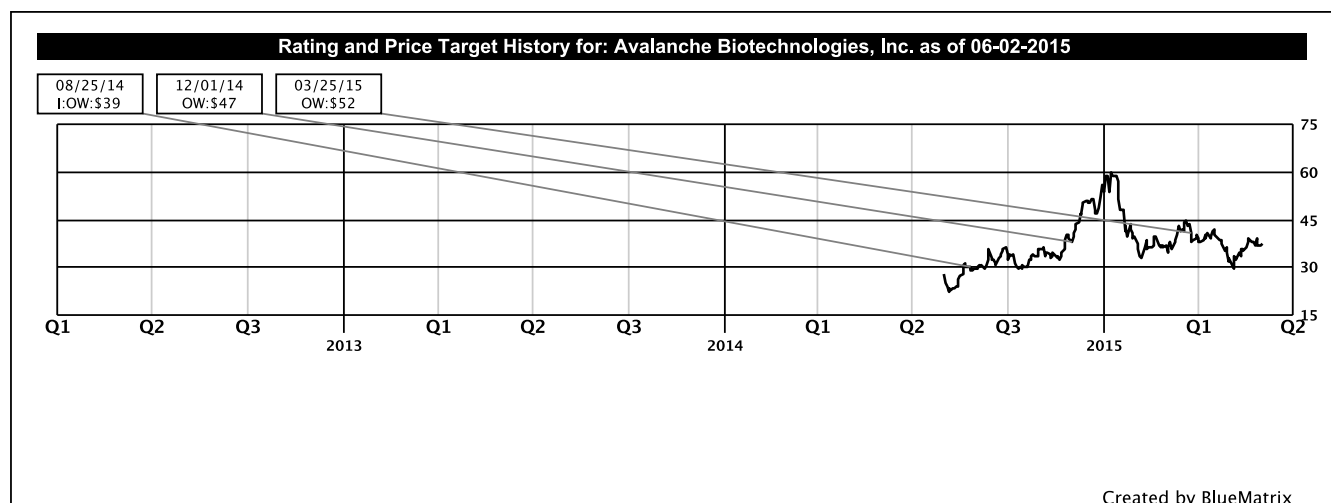
AAVL ANNUAL P&L												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	0.6	9.2	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$1	\$9	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$555
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%
Total operating expenses	4	25	51	48	72	95	105	161	198	239	271	304
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%
Net income	-5	-29	-41	-35	-59	-82	-92	-140	-99	-11	99	192
EPS	(\$1.39)	(\$2.46)	(\$1.61)	(\$1.35)	(\$1.97)	(\$2.56)	(\$2.42)	(\$3.51)	(\$2.36)	(\$0.23)	\$2.06	\$3.85
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
Cash from operations	-2	-15	-36	-29	-52	-78	-91	-137	-94	-4	108	217
PP&E	0	0	0	-2	-2	-2	-2	-2	-2	-2	-2	-2
Free cash flow	-2	-15	-36	-31	-54	-80	-93	-139	-96	-6	106	215
Financing Cash	2	174	139	0	125	0	175	0	0	0	0	0
Cash start	0	1	159	262	231	302	222	304	165	69	63	169
Cash end	1	159	262	231	302	222	304	165	69	63	169	384

Sources: Company Reports and Piper Jaffray

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

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Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OW]	392	60.87	101	25.77
HOLD [N]	237	36.80	15	6.33
SELL [UW]	15	2.33	0	0.00

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

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