

Avalanche Biotechnologies (AAVL)

Overweight

AAVL Earnings And Model Update

CONCLUSION

AAVL released 4Q14 earnings this morning disclosing a cash balance of \$159.4M as of December 31, but then raised an additional ~\$130M in January. This year is an important year for the company as we will see Phase 2a clinical data in wet-AMD mid-year, but before that event we expect to learn more about the development programs at the company's March 25th analyst event. We remain enthusiastic for the potential of AVA-101 to become a meaningful product, although also see it as the first of what may be a series of iterations and optimizations as the company moves down the path of becoming an ophthalmology gene therapy platform leader, beyond wet-AMD and into other indications, particularly orphan diseases. Reiterate OW and PT of \$47.

- **Framing The AVA-101 Data:** As we think about the potential profile of AVA-101, we don't think the bar is set at the level seen in the Phase 1, although that would certainly be welcome. However, there does need to be a compelling clinical benefit for a subretinal delivery product in terms of either a very meaningful (50% or more) average reduction in injection frequency or a relevant (>1 line vision) average improvement in visual acuity. These results are expected to frame and inform the larger, multi-center Phase 2b trial set to begin in 2H2015.
- **More Than AVA-101, AAVL Is A Broader Ophthalmology Platform:** In the wings, we note that AAVL is developing an intravitreal product, AVA-201, with the same transgene as '101 but with a new vector optimized for this more challenging delivery method-- representing a more appealing commercial profile and one for which the efficacy bar may be set even lower. In collaboration with REGN, we note that AAVL is also developing additional candidates, with some designed for orphan indications (e.g., AVA-311 for XLRS), an appealing but increasingly crowded space.
- **Model Updates:** Following minor model updates and rolling the DCF forward one year, our price target remains \$47.

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\$36.61

TARGET: US\$47.00

DCF thru 2024, 11% discount rate, 6% terminal growth rate

Joshua E. Schimmer, MD

Sr Research Analyst, Piper Jaffray & Co.
212 284-9322, joshua.e.schimmer@pjc.com

Jerry Yang, Ph.D.

Research Analyst, Piper Jaffray & Co.
212 284-9331, jerry.n.yang@pjc.com

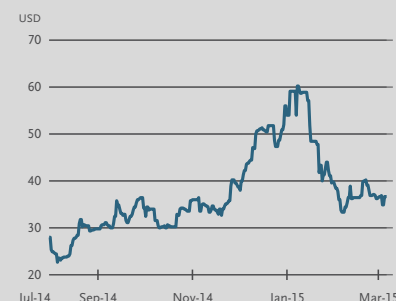
Steven P. Breazzano, Ph.D.

Research Analyst, Piper Jaffray & Co.
212 284-6114, steven.p.breazzano@pjc.com

Changes	Previous	Current
Rating	—	Overweight
Price Tgt	—	US\$47.00
FY15E Rev (mil)	—	US\$12.0
FY16E Rev (mil)	—	US\$12.0
FY15E EPS	US\$(0.97)	US\$(1.54)
FY16E EPS	—	US\$(1.35)

52-Week High / Low	US\$62.48 / US\$22.00
Shares Out (mil)	22.6
Market Cap. (mil)	US\$827.4
Avg Daily Vol (000)	337
Book Value/Share	US\$6.61
Net Cash Per Share	US\$7.05
Debt to Total Capital	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	1,379.0x	(0.40)	(0.54)	(0.50)	(0.46)	(2.46)	NM
2015E	3.0	3.0	3.0	3.0	12.0	68.9x	(0.31)	(0.35)	(0.42)	(0.46)	(1.54)	NM
2016E	—	—	—	—	12.0	68.9x	—	—	—	—	(1.35)	NM

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AAVL Upcoming Catalysts				
Program	Indication	Type	Event	Expected Timing
AVA-101	wet AMD	Development	Analyst Day	March 2015
		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

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 (%)	6.0%
Implied Terminal Year FCF Multiple	21.2x
NPV of FCF	\$1,238
Cash/equiv	\$163
Price Target	\$47
Target valuation	\$1,401
Shares Outstanding 2017E (million)	30.0

Source: PJC estimates

AAVL Valuation Sensitivity Analysis					
Terminal Growth	Discount Rate				
		10%	11%	12%	13%
	4.0%	\$42	\$32	\$25	\$20
	5.0%	\$51	\$38	\$29	\$23
	6.0%	\$65	\$47	\$35	\$27
	7.0%	\$88	\$59	\$43	\$32
	8.0%	\$134	\$80	\$54	\$39

Source: PJC estimates

AAVL Potential Upside From Current Levels					
Terminal Growth	Discount Rate				
		10.0%	11.0%	12.0%	13.0%
	4.0%	11%	(15%)	(33%)	(47%)
	5.0%	35%	1%	(22%)	(39%)
	6.0%	71%	23%	(8%)	(29%)
	7.0%	132%	56%	13%	(16%)
	8.0%	254%	112%	43%	3%

Source: Company Reports and Piper Jaffray.

AAVL QUARTERLY P&L											
	2013A	1Q14A	2Q14A	3Q14A	4Q14E	2014A	1Q15E	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	3	3	3	3	12
Total Revenue	\$0.5	\$0.03	\$0.14	\$0.20	\$0.20	\$1	\$3	\$3	\$3	\$3	\$12
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	8	9	10	11	38
<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	3	3	4	4	14
<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	11	12	14	15	52
Interest/other	-2	0	-1	0	0	-1	0	0	0	0	1
Pretax income	(\$5)	(\$2)	(\$5)	(\$8)	(\$10)	(\$25)	(\$8)	(\$9)	(\$11)	(\$12)	(\$39)
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	-8	-9	-11	-12	-39
EPS	(\$1.39)	(\$0.40)	(\$0.54)	(\$0.50)	(\$0.46)	(\$2.46)	(\$0.31)	(\$0.35)	(\$0.42)	(\$0.46)	(\$1.54)
Shares	4	4	9	16	23	12	25	25	26	26	25

Sources: Company Reports and Piper Jaffray

Joshua Schimmer: 212-284-9322

Proprietary to Piper Jaffray & Co. March 5, 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

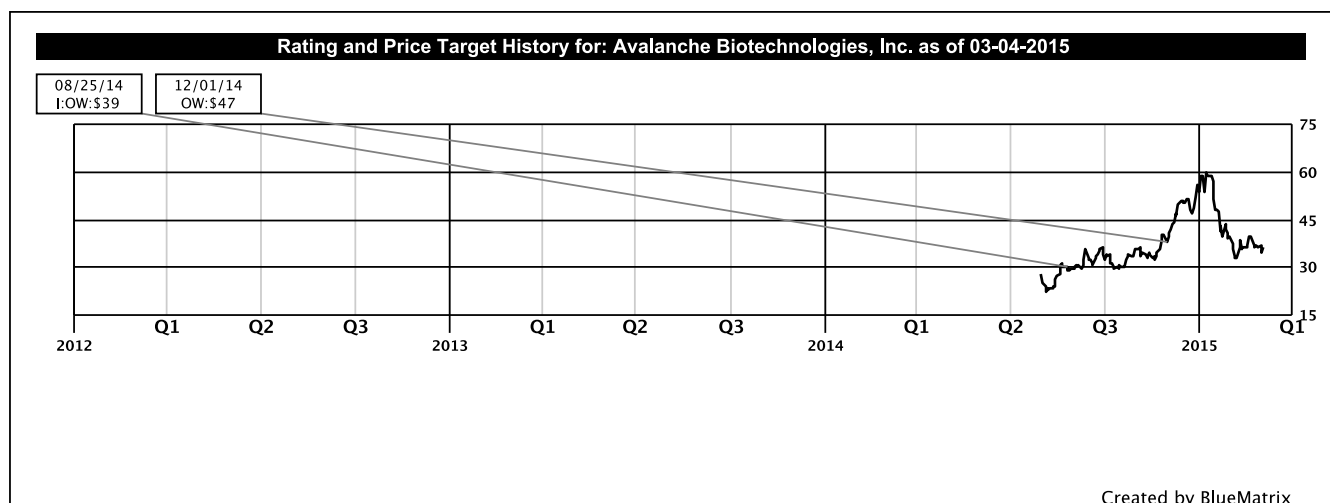
AAVL ANNUAL P&L												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	0.6	12	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$1	\$12	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$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	38	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	14	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	52	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)	(\$39)	(\$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	-39	-35	-59	-82	-92	-140	-99	-11	99	192
EPS	(\$1.39)	(\$2.46)	(\$1.54)	(\$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	-39	-35	-59	-82	-92	-140.43	-99	-11	99	192
Depreciation	0	0	0	0	0	0	0	0	0	0	0	0
Stock based comp	1	6	8	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	-4	-4	-5	-10	-15	-15	-15	-15	-15	0
Cash from operations	-2	-15	-35	-29	-52	-78	-91	-137.43	-94	-4	108	217
PP&E	0	0	0	-2	-2	-2	-2	-2	-2	-2	-2	-2
Free cash flow	-2	-15	-35	-31	-54	-80	-93	-139.43	-96	-6	106	215
Financing Cash	2.48	173.5	130.5	0	125	0	175	0	0	0	0	0
Cash start	0	1	159	254	223	294	214	296	157	61	55	161
Cash end	1	159	254	223	294	214	296	157	61	55	161	376

Sources: Company Reports and Piper Jaffray

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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
BUY [OW]	374	59.65	101	27.01
HOLD [N]	238	37.96	19	7.98
SELL [UW]	15	2.39	1	6.67

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

— Jerry Yang, Ph.D., Research Analyst

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

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- **Neutral (N):** Anticipated to perform in line relative to the median of the group of stocks covered by the analyst.
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