

Avalanche Biotechnologies (AAVL)

Overweight

On The Road With Management; Increasing PT

CONCLUSION

We hosted AAVL management in Boston today for meetings with investors. The level of interest was high as the company continues to execute and prepare for initiation of the Phase 2b multi-center trial for AVA-101, the lead gene therapy candidate for wet-AMD, ahead of the Phase 2a data expected in mid-2015. Investors are beginning to look beyond the P2a data and increasingly toward the platform/pipeline which is a theme we expect to continue with gene therapy more broadly. We reiterate our OW rating and are increasing our PT from \$39 to \$47 as we increase our growth rate to reflect the broad LT opportunity from the pipeline. AAVL is presenting at our PJC Healthcare Conference this week.

- **Phase 2a Data Next Year:** AAVL remains on track to report topline data in the middle of 2015, following a prior interim safety look that revealed no safety concerns. The study is evaluating 32 patients, randomized 2:1 to drug vs placebo. Additional data is also expected on the initial cohort of 8, although we note this follow up data will be primarily safety related. Overall, these results are expected to inform the design of the Phase 2b, which will be a multi-center trial.
- **Focus On The Pipeline:** Regeneron (REGN) and AAVL announced their collaboration earlier this year utilizing AAVL's proprietary Ocular BioFactory platform. This partnership agreement covers 8 development candidates, one of which has been disclosed (AVA-311 for the genetic disorder X-Linked Juvenile Retinoschisis (XLRS)), and we expect details on additional targets to be revealed sometime next year.
- **Pricing: All Gene Therapy Is Not The Same:** AAVL's business model does not rely on ultra-premium pricing, and many investors have expressed comfort with the commercial model for wet-AMD. Initial pricing considerations will be informed by the anticipated 2 years of pivotal data required for approval (in line with prior anti-VEGF drugs), and benchmarked off the current two year treatment cost of approximately \$30,000 – \$50,000 for more refractory patients.
- **Valuation update:** A common theme to gene therapy platforms is their modularity. Success with one program should beget success with others. As investors are beginning to recognize this value, we are increasing our PT to better reflect the long-term ophtho opportunity for AAVL, in the form of terminal growth which is now 6% vs prior 5%.

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\$37.94

Note: priced as of the close 12/1/14.

TARGET: US\$47.00

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

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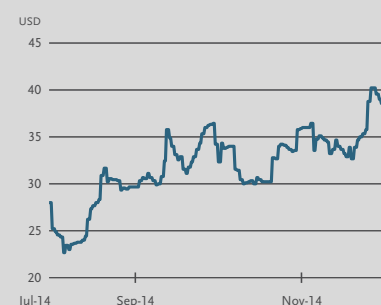
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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	US\$39.00	US\$47.00
FY14E Rev (mil)	—	US\$2.0
FY15E Rev (mil)	—	US\$12.0
FY14E EPS	—	US\$(1.45)
FY15E EPS	—	US\$(0.97)
52-Week High / Low	US\$40.99 / US\$22.00	
Shares Out (mil)	22.0	
Market Cap. (mil)	US\$834.7	
Avg Daily Vol (ooo)	118	
Book Value/Share	US\$7.08	
Net Cash Per Share	US\$7.51	
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
2013A	—	—	—	—	0.5	1,669.4x	—	—	—	—	(1.39)	NM
2014E	0.0A	0.1A	0.2A	1.5	2.0	417.3x	(0.40)A	(0.54)A	(0.50)A	(0.18)	(1.45)	NM
2015E	3.0	3.0	3.0	3.0	12.0	69.6x	(0.18)	(0.22)	(0.26)	(0.30)	(0.97)	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

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	\$1,246
Cash/equiv	\$163
Price Target	\$47
Target valuation	\$1,409
Shares Outstanding 2017E (million)	30.0

Source: PJC estimates

AAVL Valuation Sensitivity Analysis					
Terminal Growth	Discount Rate				
		10%	11%	12%	13%
	4.0%	\$43	\$33	\$26	\$21
	5.0%	\$52	\$39	\$30	\$24
	6.0%	\$65	\$47	\$35	\$27
	7.0%	\$88	\$59	\$43	\$32
	8.0%	\$133	\$79	\$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%	13%	(13%)	(31%)	(45%)
	5.0%	36%	2%	(21%)	(37%)
	6.0%	72%	24%	(7%)	(28%)
	7.0%	131%	56%	13%	(15%)
	8.0%	249%	109%	42%	2%

Source: Company Reports and Piper Jaffray.

AAVL QUARTERLY P&L											
	2013A	1Q14A	2Q14A	3Q14A	4Q14E	2014E	1Q15E	2Q15E	3Q15E	4Q15E	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	1.5	1.9	3	3	3	3	12
Total Revenue	\$0.5	\$0.03	\$0.14	\$0.20	\$1.50	\$2	\$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	4	14	6	7	8	9	30
<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	2	6	2	2	2	2	6
<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	6	20	8	9	10	11	36
Interest/other	-2	0	-1	0	0	-1	0	0	0	0	1
Pretax income	(\$5)	(\$2)	(\$5)	(\$8)	(\$4)	(\$19)	(\$4)	(\$5)	(\$6)	(\$7)	(\$23)
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	-4	-19	-4	-5	-6	-7	-23
EPS	(\$1.39)	(\$0.40)	(\$0.54)	(\$0.50)	(\$0.18)	(\$1.45)	(\$0.18)	(\$0.22)	(\$0.26)	(\$0.30)	(\$0.97)
Shares	4	4	9	16	22	13	23	24	24	25	24

Sources: Company Reports and Piper Jaffray

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Proprietary to Piper Jaffray & Co. December 1, 2014

AAVL ANNUAL PRODUCT MODEL												
	2013A	2014E	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

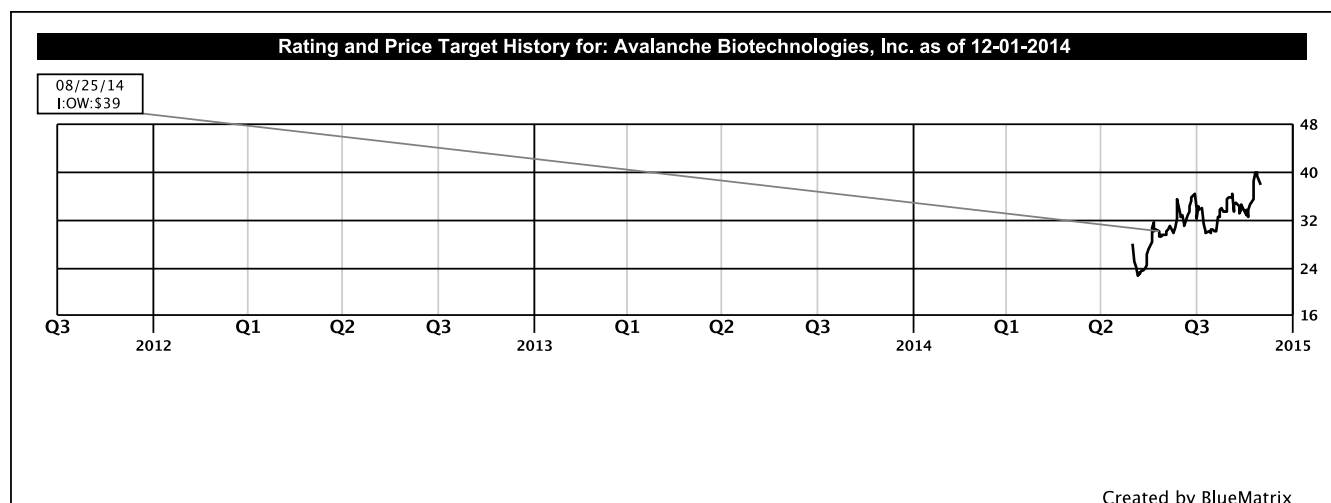
AAVL ANNUAL P&L												
	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	1.9	12	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$2	\$12	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$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	14	30	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	6	6	8	10	10	12	80	100	125	135	145
% revenue	N/M	N/M	N/M	67%	83%	83%	100%	415%	102%	56%	34%	26%
Total operating expenses	4	20	36	48	70	90	92	146	183	224	256	284
Interest/other	-2	-1	1	1	1	1	1	1	1	2	4	6
Pretax income	(\$5)	(\$19)	(\$23)	(\$35)	(\$57)	(\$77)	(\$79)	(\$125)	(\$84)	\$1	\$147	\$276
Taxes	0	0	0	0	0	0	0	0	0	0	37	69
Tax rate	0%	0%	0%	0%	0%	0%	0%	0%	0%	25%	25%	25%
Net income	-5	-19	-23	-35	-57	-77	-79	-125	-84	1	110	207
EPS	(\$1.39)	(\$1.45)	(\$0.97)	(\$1.35)	(\$1.90)	(\$2.41)	(\$2.08)	(\$3.14)	(\$2.01)	\$0.02	\$2.29	\$4.15
Shares	4	13	24	26	30	32	38	40	42	46	48	50

Sources: Company Reports and Piper Jaffray

AAVL STATEMENT OF CASH FLOWS												
	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Net Income	-5	-19	-23	-35	-57	-77	-79	-125.43	-84	1	110	207
Depreciation	0	0	0	0	0	0	0	0	0	0	0	0
Stock based comp	1	5	8	10	12	14	16	18	20	22	24	25
Other	3	0	0	0	0	0	0	0	0	0	0	0
Change in NWC	0	1	-4	-4	-5	-10	-15	-15	-15	-15	-15	0
Cash from operations	-2	-13	-19	-29	-50	-73	-78	-122.43	-79	8	119	232
PP&E	0	0	0	-2	-2	-2	-2	-2	-2	-2	-2	-2
Free cash flow	-2	-12	-19	-31	-52	-75	-80	-124.43	-81	6	117	230
Financing Cash	2.48	173.5	0	0	125	0	175	0	0	0	0	0
Cash start	0	1	162	143	112	185	110	205	80	-1	5	122
Cash end	1	162	143	112	185	110	205	80	-1	5	122	352

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

Distribution of Ratings/IB Services Piper Jaffray				
Rating	Count	Percent	IB Serv./Past 12 Mos.	
			Count	Percent
BUY [OW]	378	61.97	101	26.72
HOLD [N]	220	36.07	22	10.00
SELL [UW]	12	1.97	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

— 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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