

Avalanche Biotechnologies (AAVL)

Overweight

AAVL Q2 2014 Model Update

CONCLUSION

AAVL reported its Q2 2014 earnings this morning. We are updating our model accordingly. Our FY15E and beyond revenue/EPS projections are unchanged. The FY14E loss per share estimate is a bit higher than projected as we sort through the co-funding of R&D under the new relationship with REGN, but this is a small factor and not impactful to our assessment. Our OW rating and \$39PT are unchanged; the rest of the year will see some data presentations by the SNY/AGTC gene therapy program for wet AMD, and potentially an AAVL event at the AAO conference in Chicago (Oct 18-21). We believe other advances in the broader gene therapy field may also help lift shares. P2a data for AVA-101 in mid-2015 for wet AMD will likely then mark another significant inflection point for the company.

- **Thoughts on SNY/AGTC data:** We had a number of questions from investors regarding the impact of this data on AAVL. SNY/AGTC are using an intravitreal approach with an otherwise similar vector and transgene, but are dosing intravitreally (unlike AAVL which is pursuing a subretinal delivery approach). Because this is a non-optimized approach (AAV-2 may not be ideal for intravitreal delivery as opposed to subretinal delivery), we believe the results will also be sub-optimal. But there may be some hints that using a better AAV construct, intravitreal delivery may some day be feasible, which would be a positive for all the players in this field, including AAVL. For the most part, any safety concerns or limited efficacy concerns would likely be attributable to intravitreal delivery with a sub-optimal vector, so should not have negative readthroughs.
- **Next generations:** Both AAVL and AGTC are developing optimized vectors to enable intravitreal dosing of a Flt-1 transgene which, in our view, would meaningfully improve ease of administration and expand the commercial potential for this approach.

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\$32.43

Note: Price as of the close September 11, 2014.

TARGET: US\$39.00

DCF thru 2024, 11% discount rate, 5% growth rate

Joshua E. Schimmer, MD

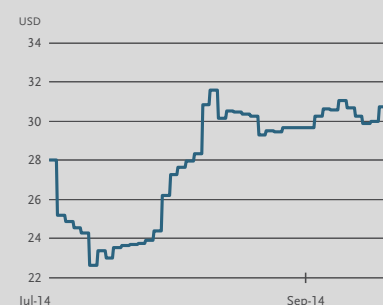
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Changes	Previous	Current
Rating	—	Overweight
Price Tgt	—	US\$39.00
FY14E Rev (mil)	US\$6.0	US\$3.2
FY15E Rev (mil)	—	US\$12.0
FY14E EPS	US\$(0.61)	US\$(0.77)
FY15E EPS	—	US\$(0.97)
52-Week High / Low	US\$32.38 / US\$22.00	
Shares Out (mil)	22.0	
Market Cap. (mil)	US\$713.5	
Avg Daily Vol (ooo)		
Net Cash Per Share	US\$7.55	
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	1,426.9x	—	—	—	—	(0.74)	NM
2014E	0.0A	0.1A	1.5	1.5	3.2	223.0x	(0.11)A	(0.28)A	(0.18)	(0.18)	(0.77)	NM
2015E	3.0	3.0	3.0	3.0	12.0	59.5x	(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	P1 data from AGTC/Genzyme intravitreal gene therapy study	Oct 2014 (AAO)
		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 (%)	5.0%
Implied Terminal Year FCF Multiple	17.5x
NPV of FCF	\$1,004
Cash/equiv	\$163
Price Target	\$39
Target valuation	\$1,167
Shares Outstanding 2017E (million)	30.0

Source: PJC estimates

AAVL Valuation Sensitivity Analysis					
Terminal Growth	Discount Rate				
		10%	11%	12%	13%
	3.0%	\$36	\$29	\$23	\$19
	4.0%	\$43	\$33	\$26	\$21
	5.0%	\$52	\$39	\$30	\$24
	6.0%	\$65	\$47	\$35	\$27
	7.0%	\$88	\$59	\$43	\$32

Source: PJC estimates

AAVL Potential Upside From Current Levels					
Terminal Growth	Discount Rate				
		10.0%	11.0%	12.0%	13.0%
	3.0%	21%	(5%)	(23%)	(37%)
	4.0%	42%	10%	(13%)	(30%)
	5.0%	72%	29%	(0%)	(21%)
	6.0%	116%	56%	17%	(9%)
	7.0%	191%	96%	42%	7%

Source: Company Reports and Piper Jaffray.

AAVL QUARTERLY P&L											
	2013A	1Q14A	2Q14E	3Q14E	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	1.5	1.5	3.2	3	3	3	3	12
Total Revenue	\$0.5	\$0.03	\$0.14	\$1.50	\$1.50	\$3	\$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	4	4	12	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	5	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	6	6	17	8	9	10	11	36
Interest/other	-2	0	-1	0	0	-1	0	0	0	0	1
Pretax income	(\$5)	(\$2)	(\$5)	(\$4)	(\$4)	(\$15)	(\$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	-4	-4	-15	-4	-5	-6	-7	-23
EPS	(\$0.74)	(\$0.11)	(\$0.28)	(\$0.18)	(\$0.18)	(\$0.77)	(\$0.18)	(\$0.22)	(\$0.26)	(\$0.30)	(\$0.97)
Shares	7	15	18	22	22	19	23	24	24	25	24

Sources: Company Reports and Piper Jaffray

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Proprietary to Piper Jaffray & Co. September 11, 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

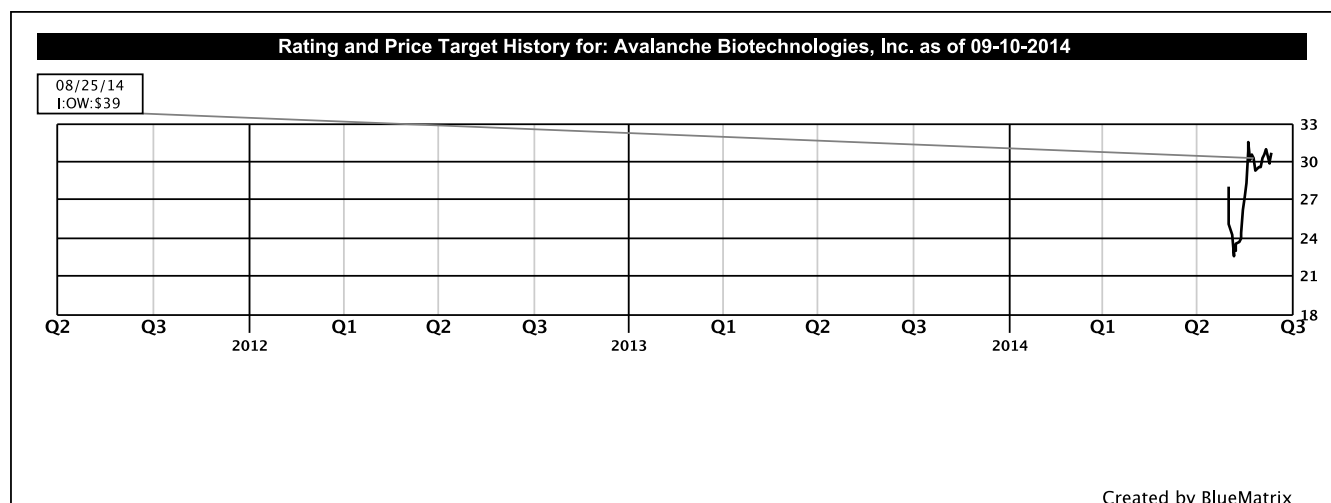
AAVL ANNUAL P&L												
	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	3.2	12	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$3	\$12	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$555
COGS	0	0	0	0	0	0	0	1	8	19	36	49
<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>	<i>10%</i>
R&D	2	12	30	40	60	80	80	65	75	80	85	90
<i>% revenue</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>333%</i>	<i>500%</i>	<i>667%</i>	<i>667%</i>	<i>337%</i>	<i>76%</i>	<i>36%</i>	<i>21%</i>	<i>16%</i>
SG&A	2	5	6	8	10	10	12	80	100	125	135	145
<i>% revenue</i>	<i>N/M</i>	<i>N/M</i>	<i>N/M</i>	<i>67%</i>	<i>83%</i>	<i>83%</i>	<i>100%</i>	<i>415%</i>	<i>102%</i>	<i>56%</i>	<i>34%</i>	<i>26%</i>
Total operating expenses	4	17	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)	(\$15)	(\$23)	(\$35)	(\$57)	(\$77)	(\$79)	(\$125)	(\$84)	\$1	\$147	\$276
Taxes	0	0	0	0	0	0	0	0	0	0	37	69
<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>25%</i>	<i>25%</i>	<i>25%</i>
Net income	-5	-15	-23	-35	-57	-77	-79	-125	-84	1	110	207
EPS	(\$0.74)	(\$0.77)	(\$0.97)	(\$1.35)	(\$1.90)	(\$2.41)	(\$2.08)	(\$3.14)	(\$2.01)	\$0.02	\$2.29	\$4.15
Shares	7	19	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	-15	-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	-2	-4	-4	-5	-10	-15	-15	-15	-15	-15	0
Cash from operations	-2	-11	-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	-11	-19	-31	-52	-75	-80	-124.43	-81	6	117	230
Financing Cash	2.48	173.3	0	0	125	0	175	0	0	0	0	0
Cash start	0	1	162	143	112	185	110	205	81	0	6	122
Cash end	1	162	143	112	185	110	205	81	0	6	122	353

Sources: Company Reports and Piper Jaffray

IMPORTANT RESEARCH DISCLOSURES



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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]	360	61.02	100	27.78
HOLD [N]	219	37.12	23	10.50
SELL [UW]	11	1.86	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

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