

Avalanche Biotechnologies (AAVL)

Overweight

Analyst Day: Needle Threaded. A Bright and Colorful Outlook; PT to \$52

CONCLUSION

This morning, AAVL hosted an analyst event which included leading retinal specialists. The event was stellar in our view; AAVL reasonably framed expectations for AVA-101 ahead of the mid-year data release and also focusing on the pipeline, positioning the company as a unique and differentiated ophthalmology platform. AAVL unveiled a new pipeline program at the event, AVA 322/323 for red/green colorblindness, which has demonstrated preclinical proof-of-concept in nonhuman primates and which we find very compelling. Reiterate OW rating and increasing PT to \$52 to reflect the potential value of this new and exciting program.

- **Specialists set expectations for AVA-101:** 3 KOLs are hoping to see OCT imaging benefit comparable to Lucentis in at least a subset of patients--meaning they aren't looking for 'cures' in everyone and are less focused on visual acuity. Retina specialists are eager to shift back to invasive procedures such as subretinal injection, clearly bored with high volumes of basic intravitreal therapies.
- **Healthy sentiment heading into the P2a:** Investors are engaged in a much more constructive dialogue for what to expect, which we believe now positions shares for meaningful upside (even if the P1a results aren't fully replicated in P2a). P2a data will inform the larger, multi-center Phase 2b trial set to begin in 2H15. While there is some debate over whether the vitrectomy component of the AVA-101 subretinal injection may be contributing to some of its benefit, we see this as a mute point since the control arm in P2b and P3 will not involve vitrectomy. Importantly, we believe the AVA-101 clinical program will be large enough to identify predictors of benefit which should help position the product for regulatory and commercial success.
- **New pipeline program hits our sweet spot:** AAVL introduced AVA-322/323 for color blindness, licensed from U Washington. This program inserts the missing opsin gene into cone photoreceptors via intravitreal injection and has shown impressive results in the preclinical setting, including monkeys (Cutest. Video. Ever.). The program should enter the clinic next year. We like this one because: 1) there is no current therapy or real competition; 2) the market is enormous even if it's mostly out-of-pocket; 3) animal models are robust; 4) it can validate AAVL's novel gene construct for intravitreal injection; 5) color vision endpoints are accepted by the FDA; and 6) the unmet need is surprisingly relevant. We see considerable value in this program and are increasing our terminal growth rate from 6% to 6.5%, driving our PT from \$47 to \$52.

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\$41.04

Note: priced as of the close 3/25/15.

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\$47.00	US\$52.00
FY15E Rev (mil)	—	US\$12.0
FY16E Rev (mil)	—	US\$12.0
FY15E EPS	—	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\$927.5	
Avg Daily Vol (ooo)	363	
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	1,545.8x	(0.40)	(0.54)	(0.50)	(0.46)	(2.46)	NM
2015E	3.0	3.0	3.0	3.0	12.0	77.3x	(0.31)	(0.35)	(0.42)	(0.46)	(1.54)	NM
2016E	—	—	—	—	12.0	77.3x	—	—	—	—	(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	\$1,407
Cash/equiv	\$163
Price Target	\$52
Target valuation	\$1,570
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%	\$107	\$68	\$48	\$35
	8.5%	\$180	\$97	\$63	\$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%	27%	(6%)	(29%)
	7.5%	160%	67%	17%	(14%)
	8.5%	339%	137%	52%	7%

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 25, 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>	25%	25%	25%	25%	25%	25%	25%	25%	25%	25%	25%	25%
<i>High frequency 101/201 penetr.</i>	0%	0%	0%	0%	0%	0%	0%	0%	8%	20%	35%	45%
<i>Mid frequency injection eyes</i>	55%	55%	55%	55%	55%	55%	55%	55%	55%	55%	55%	55%
<i>Mid frequency 101/201 penetr.</i>	0%	0%	0%	0%	0%	0%	0%	1%	2%	4%	8%	12%
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>	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
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>	0%	0%	0%	0%	0%	0%	0%	0%	1%	3%	4%	6%
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>	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	10%	11%
AVA-311 Royalty	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$0	\$3	\$9	\$12	\$20

Sources: Company Reports and Piper Jaffray

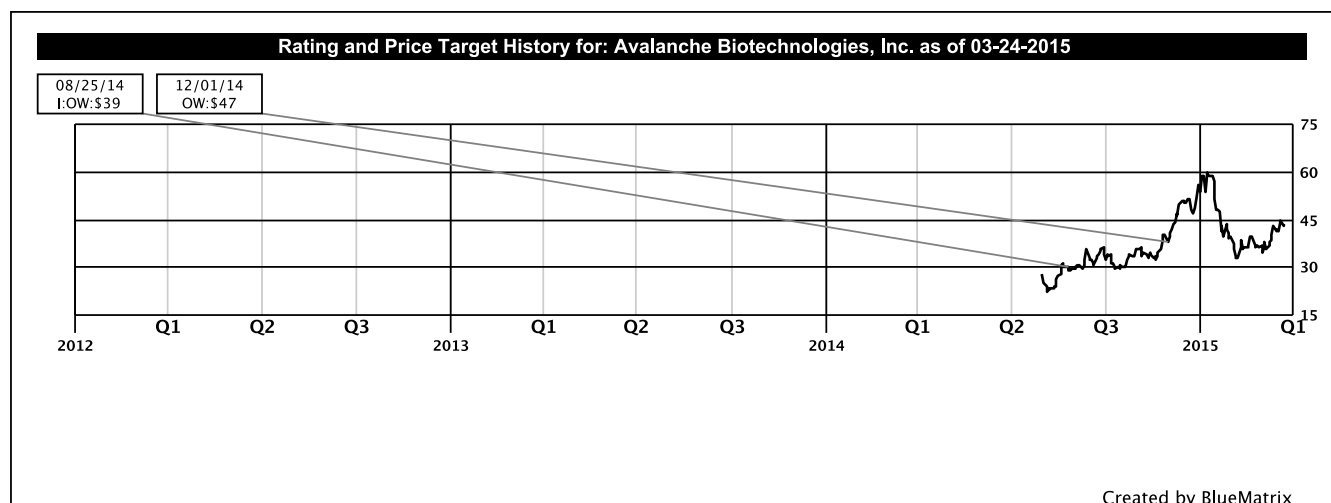
AAVL ANNUAL P&L												
	2013A	2014A	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	0.6	12	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$1	\$12	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$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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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]	370	59.68	102	27.57
HOLD [N]	235	37.90	18	7.66
SELL [UW]	15	2.42	0	0.00

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

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

— Jerry Yang, Ph.D., Research Analyst

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