

Avalanche Biotechnologies (AAVL)

Overweight

AAVL 3Q14 Earnings: Model Update

CONCLUSION

AAVL released 3Q14 earnings this morning disclosing a cash balance of \$165M following their successful IPO in August. The company continues to advance its pipeline with lead gene therapy candidate AVA-101 currently being evaluated in a Phase 2a clinical trial for the treatment of patients with wet age-related macular degeneration. We expect to see data mid 2015. Meanwhile, AAVL continues to advance preclinical candidates in collaboration with partner Regeneron. We remain optimistic on AAVL and the ophthalmology focused gene therapy platform; reiterate OW and PT of \$39.

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\$33.12

TARGET: US\$39.00

DCF thru 2024, 11% discount rate, 5% 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\$39.00
FY14E Rev (mil)	US\$3.2	US\$2.0
FY15E Rev (mil)	—	US\$12.0
FY14E EPS	US\$(0.77)	US\$(1.45)
FY15E EPS	—	US\$(0.97)

52-Week High / Low	US\$37.38 / US\$22.00
Shares Out (mil)	22.0
Market Cap. (mil)	US\$728.6
Avg Daily Vol (ooo)	121
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	1,457.3X	—	—	—	—	(1.39)	NM
2014E	0.0A	0.1A	0.2A	1.5	2.0	364.3X	(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	60.7X	(0.18)	(0.22)	(0.26)	(0.30)	(0.97)	NM

Piper Jaffray does and seeks to do business with companies covered in its research reports. As a result, investors should be aware that the firm may have a conflict of interest that could affect the objectivity of this report. Investors should consider this report as only a single factor in making their investment decisions. This report should be read in conjunction with important disclosure information, including an attestation under Regulation Analyst certification, found on pages 7 - 8 of this report or at the following site: <http://www.piperjaffray.com/researchdisclosures>.

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 (%)	5.0%
Implied Terminal Year FCF Multiple	17.5x
NPV of FCF	\$1,003
Cash/equiv	\$163
Price Target	\$39
Target valuation	\$1,166
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%)	(24%)	(38%)
	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	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

Joshua Schimmer: 212-284-9322

Proprietary to Piper Jaffray & Co. November 12, 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

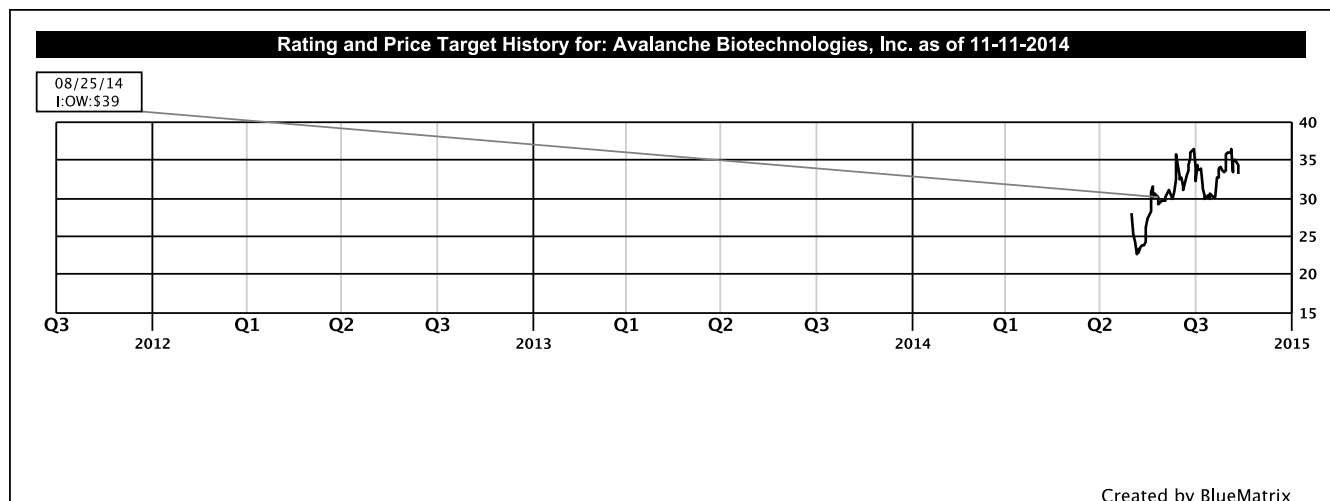
AAVL ANNUAL P&L												
	2013A	2014E	2015E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E
Product Revenue/Royalty	\$0	0.0	0	\$0	\$0	\$0	\$0	\$7	\$86	\$211	\$387	\$543
Funding/milestones	0.5	1.9	12	12	12	12	12	12	12	12	12	12
Total Revenue	\$0.5	\$2	\$12	\$12	\$12	\$12	\$12	\$19	\$98	\$223	\$399	\$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

IMPORTANT RESEARCH DISCLOSURES



Notes: The boxes on the Rating and Price Target History chart above indicate the date of the Research Note, the rating, and the price target. Each box represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first Note written during the past three years.

Legend:

I: Initiating Coverage
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]	375	61.68	95	25.33
HOLD [N]	221	36.35	23	10.41
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

The views expressed in this report accurately reflect my personal views about the subject company and the subject security. In addition, no part of my compensation was, is, or will be directly or indirectly related to the specific recommendations or views contained in this report.

Research Disclosures

Piper Jaffray was making a market in the securities of Avalanche Biotechnologies, Inc. at the time this research report was published. Piper Jaffray will buy and sell Avalanche Biotechnologies, Inc. securities on a principal basis.

Piper Jaffray expects to receive or intends to seek compensation for investment banking services from Avalanche Biotechnologies, Inc. in the next 3 months.

Piper Jaffray has received compensation for investment banking services from or has had a client relationship with Avalanche Biotechnologies, Inc. within the past 12 months.

Within the past 12 months Piper Jaffray was a managing underwriter of a public offering of, or dealer manager of a tender offer for, the securities of Avalanche Biotechnologies, Inc. or the securities of an affiliate.

Within the past 3 years Piper Jaffray participated in a public offering of, or acted as a dealer manager for, Avalanche Biotechnologies, Inc. securities.

Piper Jaffray research analysts receive compensation that is based, in part, on overall firm revenues, which include investment banking revenues.

Rating Definitions

Stock Ratings: Piper Jaffray ratings are indicators of expected total return (price appreciation plus dividend) within the next 12 months. At times analysts may specify a different investment horizon or may include additional investment time horizons for specific stocks. Stock performance is measured relative to the group of stocks covered by each analyst. Lists of the stocks covered by each are available at www.piperjaffray.com/researchdisclosures. Stock ratings and/or stock coverage may be suspended from time to time in the event that there is no active analyst opinion or analyst coverage, but the opinion or coverage is expected to resume. Research reports and ratings should not be relied upon as individual investment advice. As always, an investor's decision to buy or sell a security must depend on individual circumstances, including existing holdings, time horizons and risk tolerance. Piper Jaffray sales and trading personnel may provide written or oral commentary, trade ideas, or other information about a particular stock to clients or internal trading desks reflecting different opinions than those expressed by the research analyst. In addition, Piper Jaffray technical research products are based on different methodologies and may contradict the opinions contained in fundamental research reports.

- **Overweight (OW):** Anticipated to outperform relative to the median of the group of stocks covered by the analyst.
- **Neutral (N):** Anticipated to perform in line relative to the median of the group of stocks covered by the analyst.
- **Underweight (UW):** Anticipated to underperform relative to the median of the group of stocks covered by the analyst.

Other Important Information

The material regarding the subject company is based on data obtained from sources we deem to be reliable; it is not guaranteed as to accuracy and does not purport to be complete. This report is solely for informational purposes and is not intended to be used as the primary basis of investment decisions. Piper Jaffray has not assessed the suitability of the subject company for any person. Because of individual client requirements, it is not, and it should not be construed as, advice designed to meet the particular investment needs of any investor. This report is not an offer or the solicitation of an offer to sell or buy any security. Unless otherwise noted, the price of a security mentioned in this report is the market closing price as of the end of the prior business day. Piper Jaffray does not maintain a predetermined schedule for publication of research and will not necessarily update this report. Piper Jaffray policy generally prohibits research analysts from sending draft research reports to subject companies; however, it should be presumed that the analyst(s) who authored this report has had discussions with the subject company to ensure factual accuracy prior to publication, and has had assistance from the company in conducting diligence, including visits to company sites and meetings with company management and other representatives.

Notice to customers: This material is not directed to, or intended for distribution to or use by, any person or entity if Piper Jaffray is prohibited or restricted by any legislation or regulation in any jurisdiction from making it available to such person or entity. Customers in any of the jurisdictions where Piper Jaffray and its affiliates do business who wish to effect a transaction in the securities discussed in this report should contact their local Piper Jaffray representative. **Europe:** This material is for the use of intended recipients only and only for distribution to professional and institutional investors, i.e. persons who are authorised persons or exempted persons within the meaning of the Financial Services and Markets Act 2000 of the United Kingdom, or persons who have been categorised by Piper Jaffray Ltd. as professional clients under the rules of the Financial Conduct Authority. **United States:** This report is distributed in the United States by Piper Jaffray & Co., member SIPC, FINRA and NYSE, Inc., which accepts responsibility for its contents. The securities described in this report may not have been registered under the U.S. Securities Act of 1933 and, in such case, may not be offered or sold in the United States or to U.S. persons unless they have been so registered, or an exemption from the registration requirements is available.

This report is produced for the use of Piper Jaffray customers and may not be reproduced, re-distributed or passed to any other person or published in whole or in part for any purpose without the prior consent of Piper Jaffray & Co. Additional information is available upon request.

Copyright 2014 Piper Jaffray. All rights reserved.