COWEN
AND COMPANY

Biotechnology

Avalanche Biotechnologies

Equity Research

Quick Take: Company Update

March 5, 2015

Price: $36.61 (03/4/2015)

Price Target: $45.00

OUTPERFORM (1)

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Key Data

Symbol	NASDAQ: AAVL
Market Cap (MM)	$896.2

Highlights From Lunch With Management

The Cowen Insight

Yesterday we hosted a lunch for senior Avalanche management. AVA-101's Phase IIa remains on track to complete in mid-2015. The consultants on the Gene Therapy and Ophthalmology panels at our 35th Annual Health Care Conference this week expect AVA-101 to be an effective treatment for wet AMD. We view AAVL as one of the leaders in AAV gene therapy for the eye, and continue to recommend shares.

Results From AVA-101's Phase IIa On Track For Mid-2015.

At Cowen and Company's 35th Annual Health Care Conference yesterday we hosted an investor lunch with Avalanche's Founder and CEO Thomas Chalberg and CFO Linda Bain. A key focus of investors is the upcoming data from AVA-101's Phase IIa trial in wet AMD. Management reiterated yesterday that data are on track for mid-2015. The Phase IIa trial enrolled 32 subjects with confirmed wet AMD. Compared to AVA-101's initial Phase I/II trial, patients in the Phase IIa could have better visual acuity at baseline. While patients in the prior Phase I/II were required to have visual acuity of 20/80 or worse, those in the Phase IIa could have visual acuity of 20/30 or worse with evidence of active disease. 21 patients were randomized to "high" dose AVA-101, while 11 were randomized to the control arm. Enrollment in the trial completed during H1:2014, and the last patient will exit the study (following their 12 month assessment) in June of 2015. Therefore results remain on track for mid-2015.

The trial included an interim safety analysis which was conducted in June of 2014, several months after dosing in most patients. Management noted that this safety analysis was successfully passed, with no serious or worrisome adverse events detected. As the study is ongoing, management said that it does not have knowledge of any adverse event or efficacy data other that the safety data from the June 2014 safety analysis. Nonetheless, management did say that the trial has a pharmacovigilance committee which monitors adverse events. Avalanche would be informed of any serious complications made known to the pharmacovigilance committee. Thus far the committee has not been notified of any serious adverse events in the trial. With nearly all patients at least nine months past their AVA-101 injection, we think this bodes well for AVA-101's safety profile in the Ph. IIa.

Results From Phase IIa To Inform Design Of Subsequent Studies

The primary endpoint of the Phase IIa is safety, while secondary endpoints include the change in retinal thickness, visual acuity, and the need for rescue therapy. Management noted that the results from the Phase IIa will inform the design of AVA-101's subsequent studies. In particular, Avalanche hopes to better understand the characteristics of patients who are likely to respond, and therefore the target patient population for Phase IIb and III trials. Avalanche expects to begin enrolling a U.S. Phase IIb trial during H2:15. Though finalization of the design of the Phase IIb must await results from the Phase IIa, Avalanche currently anticipates enrolling 100-200 patients at 12-24 U.S. centers, and randomizing them to 2 doses of AVA-101

or placebo. Following Phase IIb, Avalanche thinks that the path to approval has been well defined by Lucentis and Eylea. Despite the fact that AVA-101 is gene therapy, Avalanche expects the clinical requirements in its Phase III program will be similar to those of the proteins. Avalanche anticipates that it will need to demonstrate noninferiority or superiority in visual acuity improvement to Eylea or Lucentis at 12 months.

AVA-101's Potential Discussed During Ophthalmology and Gene Therapy Panels

The potential of AVA-101 was discussed during two forums at this week's conference, an ophthalmology panel held on Monday and a gene therapy panel yesterday. The experts on both panels are convinced that AVA-101 will produce sufficient sFLT-1 protein expression to result in efficacy that is at least non-inferior to Eylea and Lucentis. Moreover the experts on both panels are convinced that AVA-101 will not result in inflammation or side effects due to expression of sFLT-1. The physician on the ophthalmology panel does worry that the subretinal injection procedure could result in detached retinas and other adverse events in some patients. During yesterday's lunch management noted that the rate of serious or severe AE's associated with subretinal injections using modern equipment and techniques is vanishingly small (management suggested 1:1,000). The physician on the ophthalmology panel thinks that, should AVA-101 be approved and be shown to effectively manage wet AMD with injection frequency of less than once per year, it will capture a meaningful share of the anti-VEGF market. The scientist on our gene therapy panel was similarly convinced that AVA-101 will result in sufficient protein expression to be an effective treatment for wet AMD. He also was not worried about gene therapy-induced side effects. While he agreed that the biggest risk to AVA-101's development and regulatory approval is side effects associated with the subretinal injection, he nonetheless thinks that such side effects will most likely be rare and are therefore unlikely to derail 101's development or approval. While the scientist was less certain of the role that AVA-101 could have in the management of wet AMD, he is a PhD who does not see patients, not an ophthalmologist.

Avalanche's Early Pipeline Progressing, Update At R&D Day on March 25.

In addition to AVA-101, Avalanche has a number of candidates in preclinical testing. AVA-201 is also sFLT gene therapy for ophthalmological indications. Avalanche is developing new AAV vectors which it hopes will permit the intravitreal injection of AVA-201. Avalanche has several AVA-201 candidates in preclinical testing. AVA-311 is an optimized AAV vector which delivers the RS1 gene to maintain the integrity of the retina in boys with juvenile X-linked retinoschisis. AVA-311 has produced high levels of RS1 protein expression in mouse models of XLRS which have restored normal retina appearance and lead to improvement in vision after just one month. AVA-311 is also in preclinical testing, and is the one of the candidates partnered with Regeneron as part of the May 2014 collaboration agreement.

Avalanche expects to provide an update on AVA-101, AVA-201 and its early pipeline at its R&D day, scheduled for March 25 in NYC.

Valuation Methodology And Risks

Valuation Methodology

Biotechnology:

In calculating our 12-month target price, we employ one or more valuation methodologies, which include a discounted earnings analysis, discounted cash flow analysis, net present value analysis and/or a comparable company analysis. These analyses may or may not require the use of objective measures such as price-to-earnings or price-to-sales multiples as well as subjective measures such as discount rates.

We make investment recommendations on early stage (pre-commercial) biotechnology companies based upon an assessment of their technology, the probability of pipeline success, and the potential market opportunity in the event of success. However, because these companies lack traditional financial metrics, we do not believe there are any good methodologies for assigning a specific target price to such stocks.

Investment Risks

Biotechnology:

There are multiple risks that are inherent with an investment in the biotechnology sector. Beyond systemic risk, there is also clinical, regulatory, and commercial risk. Additionally, biotechnology companies require significant amounts of capital in order to develop their clinical programs. The capital-raising environment is always changing and there is risk that necessary capital to complete development may not be readily available.

Risks To The Price Target

The majority of Avalanche's market capitalization is dependent upon the success of lead candidate AVA-101. AVA-101's value could be adversely impacted should its clinical trials fail, should the regulatory agencies deny approval, or should its commercial opportunity not materialize as we project. In fact, all of Avalanche's drug candidates face clinical and regulatory risk. With the future development path depending on the evolution of clinical data, revenue forecasts are uncertain. The commercial outlook for Avalanche's candidates could additionally be altered by safety/efficacy findings, emerging competition, alterations in the medical treatment paradigm, or changes in the pricing environment. Some of Avalanche's projected market exclusivity depends on patents, which are subject to challenge by potential competitors.

Addendum

Stocks Mentioned In Important Disclosures

Ticker	Company Name
AAVL	Avalanche Biotechnologies

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Outperform (1): The stock is expected to achieve a total positive return of at least 15% over the next 12 months

Market Perform (2): The stock is expected to have a total return that falls between the parameters of an Outperform and Underperform over the next 12 months

Underperform (3): Stock is expected to achieve a total negative return of at least 10% over the next 12 months

Assumption: The expected total return calculation includes anticipated dividend yield

Cowen and Company Rating System until May 25, 2013

Outperform (1): Stock expected to outperform the S&P 500

Neutral (2): Stock expected to perform in line with the S&P 500

Underperform (3): Stock expected to underperform the S&P 500

Assumptions: Time horizon is 12 months; S&P 500 is flat over forecast period

Cowen Securities, formerly known as Dahlman Rose & Company, Rating System until May 25, 2013

Buy – The fundamentals/valuations of the subject company are improving and the investment return is expected to be 5 to 15 percentage points higher than the general market return

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Hold – The fundamentals/valuations of the subject company are neither improving nor deteriorating and the investment return is expected to be in line with the general market return

Cowen And Company Rating Definitions

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/14

Rating	Count	Ratings Distribution	Count	IB Services/Past 12 Months
Buy (a)	461	60.50%	109	23.64%
Hold (b)	288	37.80%	14	4.86%
Sell (c)	13	1.71%	0	0.00%

(a) Corresponds to "Outperform" rated stocks as defined in Cowen and Company, LLC's rating definitions. (b) Corresponds to "Market Perform" as defined in Cowen and Company, LLC's ratings definitions. (c) Corresponds to "Underperform" as defined in Cowen and Company, LLC's ratings definitions.

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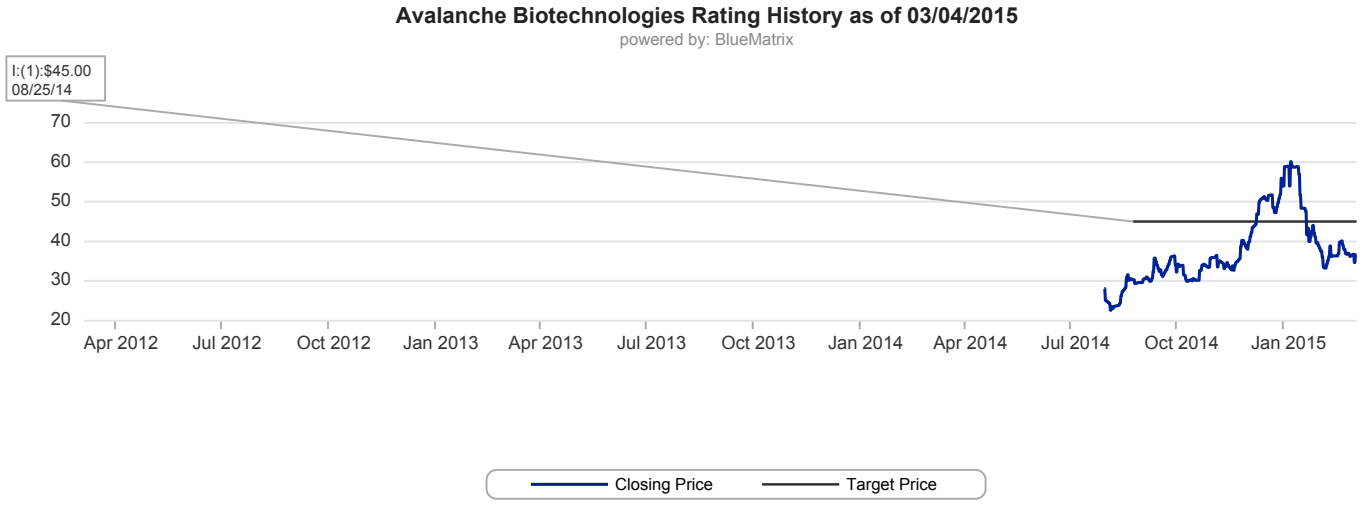

Legend for Price Chart:

I = Initiation | 1 = Outperform | 2 = Market Perform | 3 = Underperform | UR = Price Target Under Review | T = Terminated Coverage | $xx = Price Target | NA = Not Available | S=Suspended

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