Avalanche Biotechnologies (AAVL) — Overweight

Clarification on P2a AMD Data Expectation And Our Discussions With Management

CONCLUSION

Unfortunately, as a couple of investors have observed, our original industry note this morning erroneously wrote that AAVL "management notes they do know or see the data" and we corrected it to the point that "management notes they do NOT know or see the data" for the 1H15 P2a AVA-101 wet AMD data. Fortunately, investors we spoke with had all realized that was a typo. We've also had a chance to follow-up with management who highlighted that they did not intend for there to be any perceived change in sentiment or expectations. Perhaps it was our own selective hearing or the fatigue of a long week of conference, but we caution investors around over-interpreting any of our comments from this morning.

- **Management notes they don't know the data**: The company is insistent that there is nothing they know about the trial which would change their views or expectations for the study.
- **P2a is a 'safety' study**: Management highlighted to us that the P2a study is first and foremost a safety study, and that this has been their message consistently (which is accurate). Of course, given the signal in the P1a study, the efficacy profile in the P2a study will be intensely scrutinized by everyone.
- **All ranges of outcomes on the table**: The company notes they've consistently also said that all possible outcomes could be seen in the P2a study, ranging from no effect to a dramatic effect, which is also correct. And this is quite a reasonable point to make because of course, anything could be seen in P2a. Perhaps in our meeting with management this week, the fatigue of the week led to what we perceived to be a shift in communication since they started by highlighting the 'middle ground' scenario as one of the options first, but in our discussion today they presented a much more balanced overview of potential outcomes.
- **Maybe this is healthy**: Given the rapid appreciation of AAVL shares and high expectations from investors, having an opportunity to directly and clearly manage the expectations heading into a binary dataset is constructive for investors.
- **So, where SHOULD the bar be?** It actually is quite beneficial to start thinking about what level of efficacy would still support a meaningful commercial offering. Certainly, it doesn't need to be as solid as the P1a data, but we believe there needs to be a compelling benefit for a subretinal delivery product in terms of either a very meaningful (50% or more) average reduction in injection frequency or a very dramatic (>1 line vision) average improvement in visual acuity without a reduction in frequency.
- **Also consider:** AAVL has an intravitreous option in preclinical development which eliminates the subretinal delivery concerns. If 101 data isn't quite up to investors' broader expectations, it may still be a very attractive profile for an intravitreous route option (AVA-201).

COMPANY DESCRIPTION

AAVL is a pioneer in gene therapy, targeting ophthalmic indications.

PRICE: US$51.56
TARGET: US$47.00
DCF thru 2024, 11% discount rate, 6% terminal growth rate

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RISKS TO ACHIEVEMENT OF PRICE TARGET

AAVL gene therapy candidates may fail to achieve target development steps.

Price Performance - 1 Year

Source: Bloomberg

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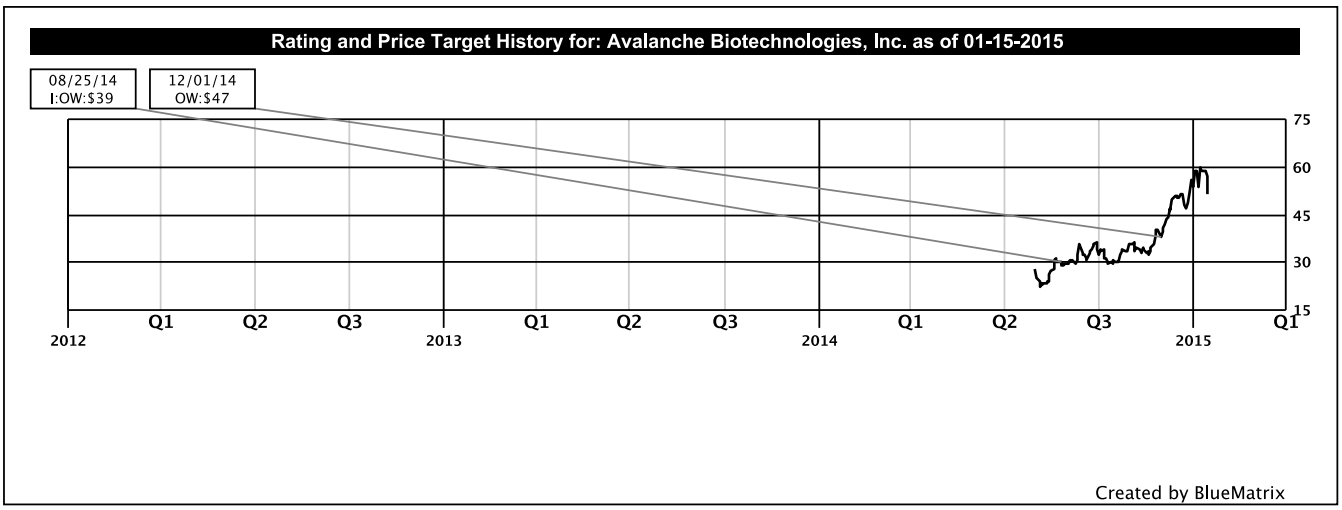

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N: Neutral
UW: Underweight
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