FLASH NOTE

Company Update

USA | Healthcare | Biotechnology

June 15, 2015

Jefferies

Avalanche Biotechnologies (AAVL)

AVA-101 PIIa Data Supports Moving Forward But Not Without Controversy

BUY
Price target $51.00
Price $38.88

EQUITY RESEARCH AMERICAS

Key Takeaway

AAVL reported topline PIIa data for AVA-101 in wAMD w/ meaningful improvements in VA of +11.5 letters v. control at yr 1. While there is controversy on the VA decline in the control grp in light of imp'ts in retina thickness, we highlight a study in 58 tx-experienced pts switched to Eylea q4/q8 week (Arcinue et al) reporting a -4.5 letter decline from mos 6 to 12 despite imp'ts in retina thickness and suggests reductions in VA even w/ FDA approved therapies.

AAVL Reports Positive Topline PIIa Data for AVA-101 w/ Meaningful Improvements in VA: AAVL reported positive topline data from the PIIa trial for AVA-101 for wet AMD. The study enrolled 32 pts w/ wAMD and randomized them to AVA-101 (n=21) or control (n=11). Subjects in both grps received 2 Lucentis injections at day 0 and wk 4, and Lucentis rescue therapy was allowed according to pre-specified criteria beginning at wk 8. Three of the 32 pts were tx-naive and the remaining 29 were tx-exp w/ a median of 10 prior inj. Importantly, the BCVA mean change from baseline showed a significant difference of +11.5 letters b/w AVA-101 (+2.2 letters) v. control (-9.3 letters). Recall, we considered an optimal scenario for AVA-101 to show VA gains v. control of ~4-5 letter improvement w/ >60% reduction in inj frequency, thus we consider the +11.5 letter gain to be meaningful. Controversy lies in the 9-letter decline in VA in the control grp which appears poorer than might be expected, but we remind that pts were tx-exp w/ a median of 10 prior anti-VEGF injections which were given PRN, thus we do not view such a decline to be unusual. In fact, Arcinue et al (Am J Ophthal, Mar 2015) reported data in 58 tx experienced pts who switched to Eylea and had received a median of 13 prior inj and a baseline retinal thickness of 358 um. Mean VA gains were 2 letters at mos 6 and a 2.5 letter decline vs baseline at mos 12 w/ a 4.5 letter decline from mos 6 to 12 suggesting even regular q4/q8 wk administration w/ one of std of care in anti-VEGF was unable to improve/stabilize vision in a difficult to treat pt population, and therefore a 9 letter decline over 12 mos in the control group may not be an anomaly.

AVA-101 Also Showed Fewer No. of Rescue Injections v. Control: AAVL further reported that the median number of rescue injections was 2 (95% CI: 1-6 inj) in AVA-101 v. 4 (95% CI: 3-5 inj) in the control. More subjects req'd fewer re-txs in the tx group compared w/ control (w/ 52.4% receiving ≤2 inj w/ AVA-101 and 90.1% receiving >3 inj w/ control). Though inj frequency of less than what we hoped for, it clearly made up w/ differences in VA. Retinal thickness mean change from baseline was +25 mm for AVA-101 v. -56 mm in the control grp. The increase in thickness is odd to us, directionally, but mgmt disclosed that there was an imbalance in retinal thickness b/w the two arms with a general greater thickness in the control (baseline b/w each arm not disclosed; 328 microns for all). We note the reductions in retinal thickness was similar b/w the grps from wk 8 to wk 52 and this stabilization could be a direct result of re-treatment criteria.

AAVL Discloses PI 36-mo Safety Data: AAVL also announced long-term safety data from the PI study. Six of the 8 pts were available for evaluation at 36 mo w/ 4 from tx group and 2 in control. Two subjects withdrew from the study but were related to old age/comorbidities, and not to study drug. Among the 4 pts, the mean change from baseline to mo 36 was +0.5 letters and subjects received an avg of 0.71 rescue inj/yr.

PIIb to Begin by YE'15; AAVL to Disclose More Detail in Q3: AAVL intends to move forward w/ its PIIb trial in multiple centers across the U.S., w/ the first pt-in expected by YE'15. AAVL has not yet disclosed whether it intends to evaluate tx-naive or -exp pts, nor have they disclosed details on design. Mgmt plans to disclose more details in Q3.

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Company Description

Avalanche Biotechnologies, Inc., a clinical-stage biotechnology company, focuses on discovering and developing novel gene therapies for the treatment of ophthalmic diseases based on its Ocular BioFactory platform. Its lead product candidate includes AVA-101, which is in a Phase I/IIa trial for the treatment of wet age-related macular degeneration (AMD). The company is also developing AVA-201, an anti-vascular endothelial growth factor gene therapy product candidate for the prevention of wet AMD; and AVA-311 that is in preclinical studies for the treatment of juvenile X-linked retinoschisis, a rare genetic disease of the retina with no approved therapy. Avalanche Biotechnologies, Inc. has a collaboration agreement with Regeneron Pharmaceuticals, Inc. research, develop, and commercialize gene therapy products. The company was founded in 2006 and is headquartered in Menlo Park, California.

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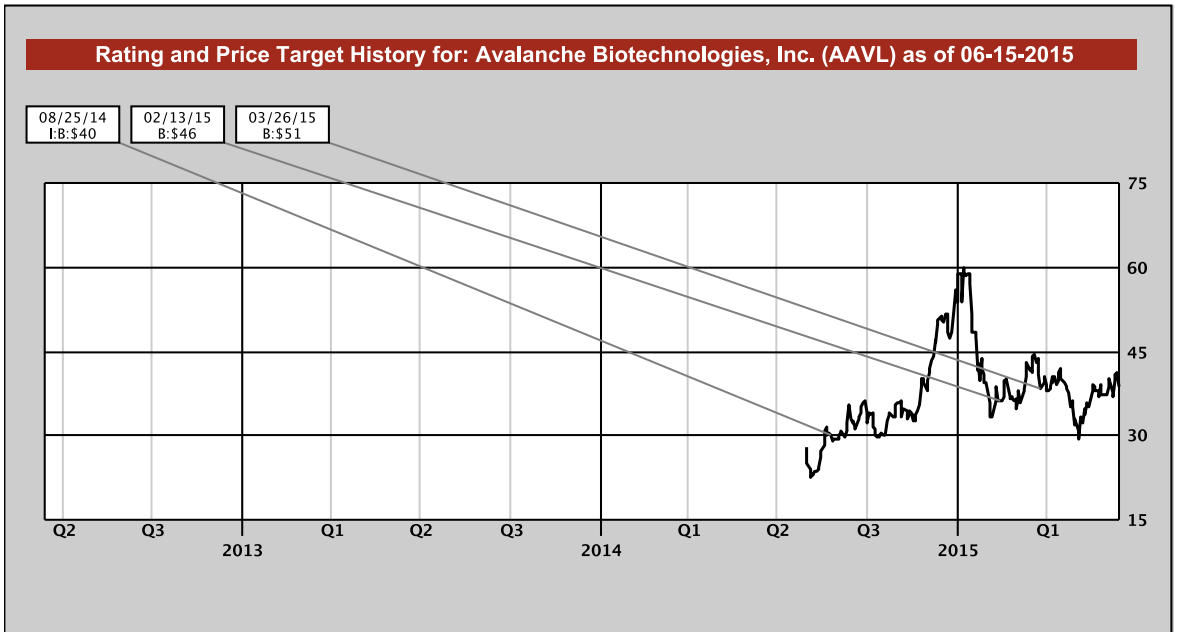

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