FLASH NOTE

Company Update

USA | Healthcare | Biotechnology

February 9, 2015

Jefferies

Avalanche Biotechnologies (AAVL)

Bascom Palmer Conference Debates Long-Term Implications of Anti-VEGF Therapy

BUY
Price target $40.00
Price $33.27

EQUITY RESEARCH AMERICAS

Key Takeaway

There were a number of presentations at the annual Bascom Palmer Angiogenesis meeting; however, more importantly we had time to sit down w/ AAVL management after a tumultuous last few weeks. Mgmt reiterated it expects to report PIIa data in mid-2015. AAVL continues to collect long-term safety data from the PI trial and plans to disclose in 2H '15. Mgmt disclosed baseline prior inj in the PII is an avg of 13 prior anti-VEGF inj compared to 18 prior inj in PI.

Self Manufactured Debate Rages Unnecessarily On Anti-VEGF Link To Geographic Atrophy: We thought the debate was addressed at AAO w/ the HARBOR data reporting there was no increased risk of geographic atrophy (GA) w/ long-term use of anti-VEGF therapies. The HARBOR data reported Lucentis 2.0 mg was not associated w/ increased risk of GA. Furthermore, vision was not impacted significantly in pts w/ GA. Also, there appeared to be no relationship btwn the frequency of injections in HARBOR and incidence of GA. The Angiogenesis meeting dedicated an entire session to this topic and it appears most physicians continue to remain unconvinced regarding the correlation of anti-VEGF therapy to GA. Interestingly, one physician noted that if anti-VEGF therapy caused GA then we would also have heard of reported cases in DME pts who may receive anti-VEGF therapy for a longer time period than wet AMD patients.

Anti-VEGF Competition Could Heat Up With Up To Three New Programs In Phase 3 This Year: A little known Chinese firm, Chengdu Kanghong, managed to receive approval in China for conbercept, an anti-VEGF w/ similar properties to Regeneron's (REGN, Hold, $406.86) Eylea. Chengdu is preparing to enter PIII trials in the U.S. w/ a pot'l q12 week regimen. The company presented data from the LAMP trial at Bascom Palmer in 30 wet AMD pts w/ less than 19 letters at baseline and w/ a mean of 8.9 letters. Pts were treated monthly for 3 mos followed by prn therapy for an add'l 3 mos and reported vision acuity (VA) gains of 24 letters at 6 mos. However, we would caution investors from extrapolating these data as these pts exhibit significant disease progression and yet also were not exposed to prior anti-VEGF injections. Actavis (ACT, $276.55) is also planning to move abicipar (formerly known as DARPin) to PIII w/ a q8 and/or a q12 week regimen. Abicipar is currently in PII trials evaluating a more refined mfr process in a wet AMD trial in 25 pts (CYPRESS trial) and in 140 DME pts (PALM trial) w/ data by mid- 2015. The previous PII REACH trial (cohort 3) reported a near 2-fold greater proportion of pts w/ 3 lines gained w/ abicipar 2.0 mg vs Lucentis at week 20 (26% vs 13%). Mean VA gains were 9.0 letters w/ abicipar 2.0 mg vs 4.7 letters w/ Lucentis at week 20. Earlier this year, Novartis (NOVN VX, Hold, CHF 91.85) reported it has initiated a PIII trial w/ RTH-258 vs Eylea in wet AMD w/ PIIb data results expected to be released later this month at the Macula meeting.

VEGF-PDGF Wars Heat Up: REGN released PI data from 12 pts w/ its single inj combo Eylea/PDGF in wet AMD pts. While the PI trial was primarily designed as a safety trial w/ no obvious safety events observed, the company also presented early efficacy data from this study. REGN evaluated 4 dose cohorts of PDGF (0.2 mg to 3 mg) in pts w/ a mean baseline VA of 63 letters and 50% receiving prior anti-VEGF inj. The 2 and 3 mg cohorts of PDGF will move forward into a randomized PII trial in 500 pts this qtr. The 1 mg and 3 mg cohorts reported VA gains of 4.3 letters and 3.3 letters, respectively at week 8 (after 2 mos inj) and OCT reduction of 77 um and 39 um, respectively. The 3 mg cohort appeared to be impacted by a more refractory pt group. Ophthotech (OPHT, $49.57, NC) also reported data from an investigator sponsored study evaluating pre-tx w/ Fovista in 27 tx resistant wet AMD pts w/ baseline VA of 55 letters and a mean of 25 prior anti-VEGF inj. Pts receiving pre-tx w/ Fovista (n = 10) observed a mean VA gain of 11 letters vs 5 letters gained in pts w/out pre-tx (n = 17). It appears the inj burden could be cumbersome as pre-tx would require a separate Fovista inj in addition to the current combo Fovista/anti-VEGF combo and therefore may be suitable for only hard to tx wet AMD pts.

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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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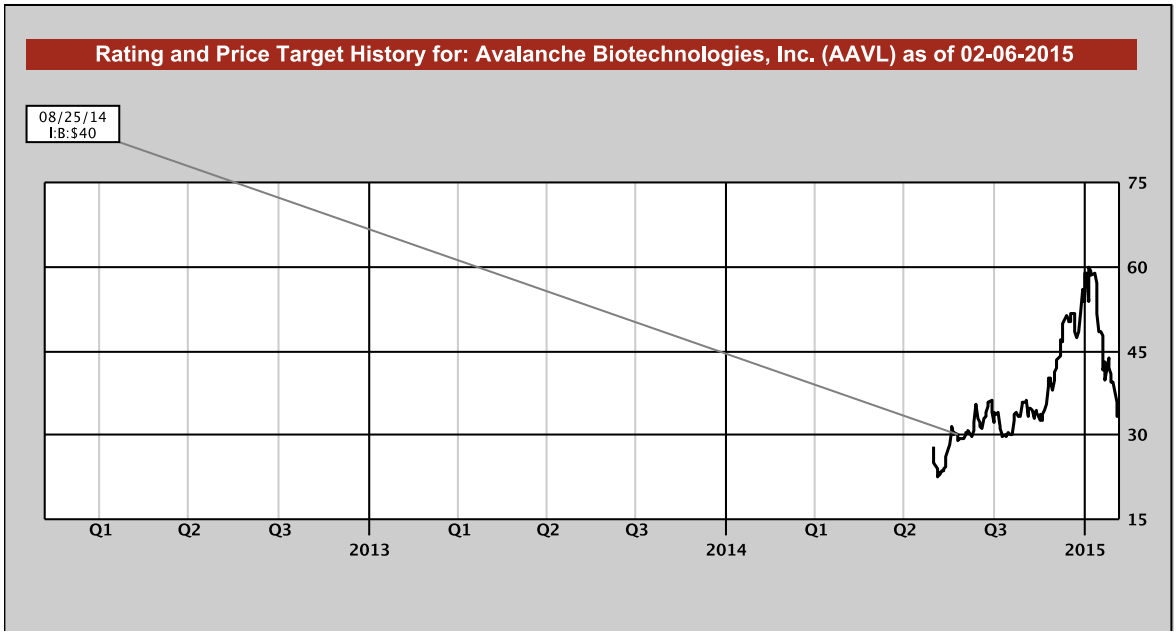
- Actavis (ACT: $276.55, Suspended)
- Novartis AG (NOVN VX: CHF91.85, BUY)
- Regeneron Pharmaceuticals, Inc. (REGN: $406.86, HOLD)



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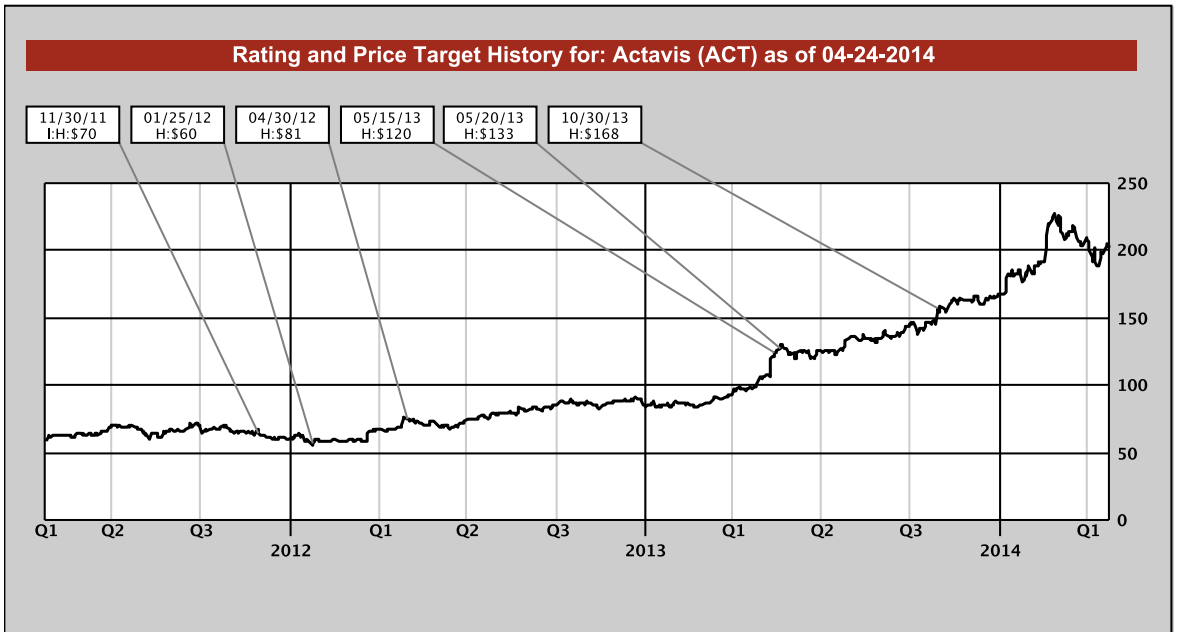

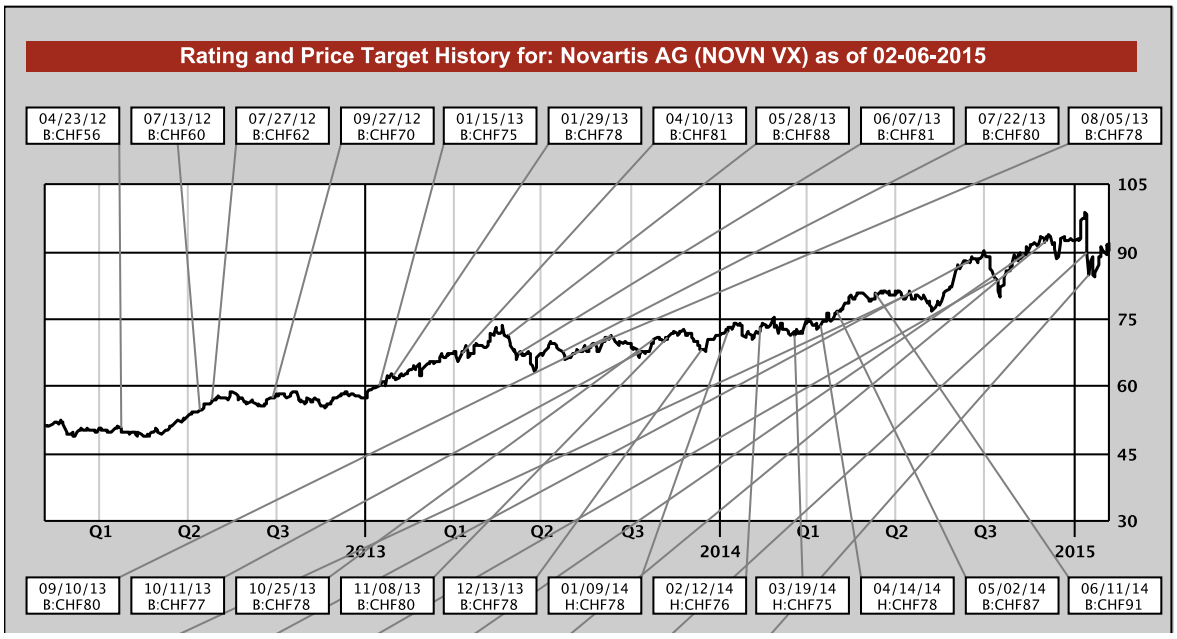

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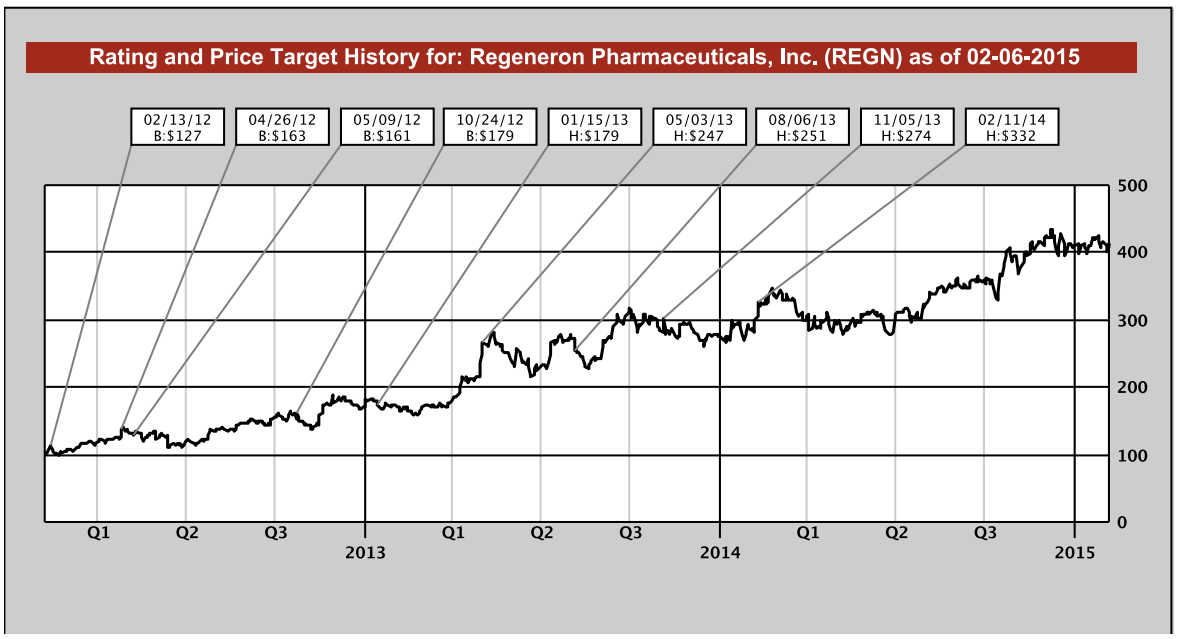

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