William Blair

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Avalanche Biotechnologies, Inc.

Management Changes as Analysis of Phase IIa Continues, Some Clarity Possibly in Fall, Maintain Market Perform

- After the close Thursday, July 23, Avalanche Biotech reported the resignation of company co-founder, board member, and Chief Executive Officer Dr. Thomas Chalberg from the company effective immediately. Hans P. Hull, the second employee at the company, was appointed interim CEO and president as the board begins a search for new leadership as well as an additional board member. The resignation of Dr. Chalberg follows disappointing top-line results from the company's Phase IIa trial and the 36-month follow-up from its Phase I study with lead candidate AVA-101 for the treatment of wet age-related macular degeneration (or wet-AMD) reported in June. The Phase IIa trial, which was powered for safety and tolerability, disappointed investors on the secondary efficacy endpoints related to visual acuity, number of rescue injections, and a signal for increased retinal thickness. While the Phase IIa results showed an improvement in the treatment arm over placebo, this improvement was driven by a 9.3 letter loss in the Lucentis arm, which did not compare well with historical results, including the CATT study where between year 1 and 2, patients showed only a 1-2 letter decrease.

- Ultimately, we continue to believe the performance of Lucentis in the control arm raised more questions than answers, and given the innovative gene therapy approach and the high bar for safety and efficacy in the VEGF class of therapies, we remain on the sidelines. However, we note that the company continues to develop additional compounds aside from AVA-101. After speaking with management, we believe it is continuing analysis of the Phase IIa data. While we are uncertain of the possibility of Regeneron (REGN $556.61) and Avalanche partnering for future development of AVA-101 given the top-line data released, it seems likely beneficial for both parties to wait until Phase IIb results, when it should be easier to assess the value of AVA-101.

- Until we hear further details about the Regeneron partnership, we assume the next catalyst for shares will likely be a further examination of the Phase IIa data, which may occur either at the Retina Society Annual meeting on October 7 in Paris or at the American Academy of Ophthalmology in November. Within that data, we anticipate more details on patients in the Lucentis arm given their visual acuity performance, the potential for hyper-/hypo-responsive groups, more detail on the increased retina thickness in the AVA-101 arm, and if results were skewed by the mix of VEGF treatment naïve and experienced patients.

- Dr. Chalberg's resignation still leaves us with questions that we believe may only be answered by Phase IIb results, which are likely some time away given the guided initiation in the second half of 2015 and potential trial design (noting previously approved wet-AMD products that have run Phase IIb clinical trials). However, we would welcome continued development of the company's deep pipeline outside AVA-101, and ultimately, these programs—specifically AVA-201, AVA-311, or the color blindness programs—may be the next catalysts to once again pique investor interest in the name. Until we see further clarity and stronger signs of efficacy from the company's lead programs, we are staying on the sidelines and are withdrawing our previous $24 price target while continuing to rate shares of Avalanche Biotechnologies as Market Perform. The company holds $290 million, or about $12/share, in cash.

Avalanche Biotechnologies is a biotechnology company located in Menlo Park, California, focused on developing gene-based therapies for the treatment of ocular diseases.

Stock Rating:	**Market Perform**
Company Profile:	**Aggressive Growth**

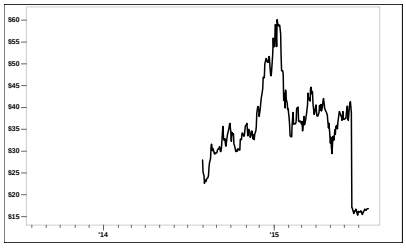
Symbol:	AAVL (NASDAQ)
Price:	$16.16 (52-Wk.: $15-$62)
Market Value (mil.):	$413
Fiscal Year End:	December
Long-Term EPS Growth Rate:	
Dividend/Yield:	None

	2014A	2015E	2016E
Estimates			
EPS FY	$-2.01	$-1.92	$-2.53
CY		$-1.92	$-2.53
Sales (mil.)	1	1	0
Valuation			
FY P/E	NM	NM	NM
CY P/E		NM	NM

Trading Data (FactSet)	
Shares Outstanding (mil.)	25
Float (mil.)	17
Average Daily Volume	644,371

Financial Data (FactSet)	
Book Value Per Share (MRQ)	11.0
Return on Equity (TTM)	-38.4

Two-Year Price Performance Chart



Sources: FactSet, William Blair & Company estimates

Exhibit 1
Phase IIa Baseline Characteristics Compared to Phase I

	Phase I	Phase IIa	Lucentis PRN regimen in CATT 2 Year Study*
Age (years)	79 (71-86)	79.5 (62-95)	78.4 (70.6-86.2)
Baseline BCVA (ETDRS letters)	36.5 (28-56)	63 (35-78)	61.5 (48.3-74.7)
Baseline center point thickness (μm)	549 (193-1094)	332.5 (179-816)	458 (265-651)
Number treatment naïve (n/N)	0/8	3/32	all
Previous anti-VEGF injections (for non-naïve)	11.5 (1-29)	10.5 (1-25)	6.9 (3.9-9.9)
Time since diagnosis (months)	49.2 (2-65)	16.2 (0-85)	12

all AAVL study values median (range)

*comparison is from year 1 to year 2 of therapy

Source: Company reports, Martin et al. *Ophthalmology* 2012

Valuation

We rate shares of Avalanche Market Perform based on the lack of near-term catalysts and uncertainty generated by the company's Phase IIa data. We continue to believe that the company has assembled a strong management team that has significant experience in the field of retinal diseases and note an intriguing (albeit very early stage) pipeline that could yield new product candidates.

Risks

Risks to shares of Avalanche are similar to those of other development-stage therapeutics companies. The company faces clinical, manufacturing, and regulatory risks on its product candidates. There are additional clinical risks in developing a new cutting-edge technology and we believe Phase IIa data to date has not confirmed a clear efficacy signal that appeared in the smaller Phase Ib results.

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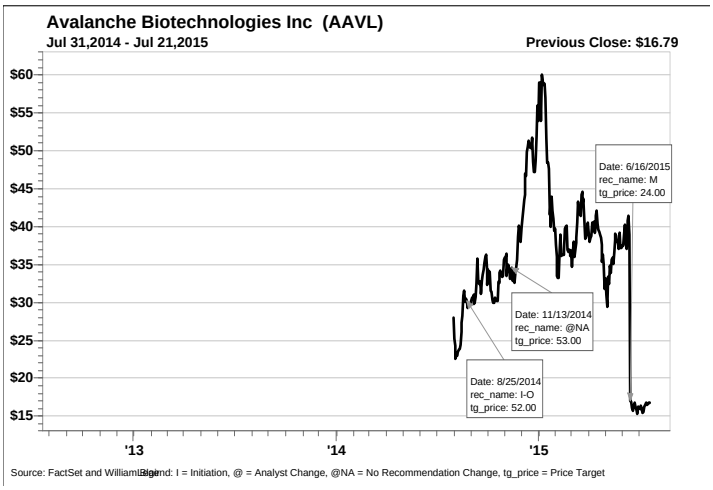
DOW JONES: 17,731.92
S&P 500: 2,102.15
NASDAQ: 5,146.41



Current Rating Distribution (as of 06/30/15)

Coverage Universe	Percent	Inv. Banking Relationships*	Percent
Outperform (Buy)	66	Outperform (Buy)	15
Market Perform (Hold)	32	Market Perform (Hold)	2
Underperform (Sell)	2	Underperform (Sell)	0

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